# UNITED STATES SECURITIES AND EXCHANGE COMMISSION Washington, D.C. 20549

FORM S-1 REGISTRATION STATEMENT UNDER THE SECURITIES ACT OF 1933

## 4D Molecular Therapeutics, Inc.

(Exact name of Registrant as specified in its charter)

**Delaware** (State or other jurisdiction of incorporation or organization)

2836 (Primary Standard Industrial Classification Code Number) 47-3506994 (I.R.S. Employer Identification Number)

5858 Horton Street #455 Emeryville, California 94608 (510) 505-2680

(Address, including zip code, and telephone number, including area code, of Registrant's principal executive offices)

David Kirn, M.D.
Chairman and Chief Executive Officer
5858 Horton Street #455
Emeryville, California 94608
(510) 505-2680

Emeryville, California 94608 (510) 505-2680 (Name, address, including zip code, and telephone number, including area code, of agent for service)

Copies to:

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Emerging growth company

August J. Moretti Chief Financial Officer 4D Molecular Therapeutics, Inc. 5858 Horton Street #455 Emeryville, California 94608 Telephone: (510) 505-2680 David Peinsipp Charles S. Kim Kristin VanderPas Will H. Cai Cooley LLP 101 California Street, 5th Floor San Francisco, California 94111 Telephone: (415) 693-2000

Appro	eximate date of cor	nmencement of proposed sa	lle to the public: As soon a	as practicable after the effec	ctive date of this Registra	ation Statement.	
If any the following b		eing registered on this Form	are to be offered on a del	ayed or continuous basis p	ursuant to Rule 415 und	ler the Securities Act of 1933, cl	heck
		gister additional securities for ent number of the earlier eff				he following box and list the	
		ective amendment filed pursi effective registration stateme			ne following box and list	the Securities Act registration	
		ective amendment filed pursi effective registration stateme			ne following box and list	the Securities Act registration	
		whether the registrant is a la ler," "accelerated filer," "sma				reporting company. See the exchange Act.	
Large accelera	ated filer					Accelerated filer	
Non-accelerat	ed filer	$\boxtimes$				Smaller reporting company	

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided to Section 7(a)(2)(B) of the Securities Act.  $\Box$ 

## **CALCULATION OF REGISTRATION FEE**

Title of each class of securities to be registered	Proposed maximum aggregate offering price <sup>(1)</sup>	Amount of registration fee <sup>(2)</sup>
Common Stock, \$0.0001 par value per share	\$100,000,000	\$12,120.00

- (1) Estimated solely for the purpose of calculating the amount of the registration fee in accordance with Rule 457(o) under the Securities Act of 1933, as amended. Includes shares that the underwriters have the option to purchase solely to cover over-allotments, if any.
- (2) Calculated pursuant to Rule 457(o) based on an estimate of the proposed maximum offering price.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine

The information contained in this preliminary prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell these securities and is not soliciting an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

Subject to Completion, Dated September 30, 2019

## **Shares**



## Common Stock

This is the initial public offering of shares of common stock of 4D Molecular Therapeutics, Inc. We are offering shares of our common stock.

Prior to this offering, there has been no public market for our common stock. It is currently estimated that the initial public offering price will be between \$ and \$ per share.

We have applied to list our common stock on The Nasdag Global Market under the symbol "DDDD."

We are an "emerging growth company" as defined under the federal securities laws and, as such, have elected to comply with certain reduced public company reporting requirements in this prospectus and may elect to do so in future filings.

See the section titled "<u>Risk Factors</u>" beginning on page 13 to read about factors you should consider before deciding to invest in shares of our common stock.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

	Per Share	Total
Initial public offering price	\$	\$
Underwriting discounts and commissions(1)	\$	\$
Proceeds, before expenses, to 4D Molecular Therapeutics, Inc.	\$	\$

<sup>1)</sup> See the section titled "Underwriting" for a description of the compensation payable to the underwriters.

To the extent that the underwriters sell more than shares of common stock, the underwriters have an over-allotment option to purchase up to an additional shares from us at the initial public offering price, less the underwriting discounts and commissions.

The underwriters expect to deliver the shares against payment in New York, New York on , 2019.

Goldman Sachs & Co. LLC Evercore ISI William Blair
Chardan

Prospectus dated , 2019.

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We and the underwriters have not authorized anyone to provide you any information other than that contained in this prospectus or in any free writing prospectus prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We and the underwriters are not making an offer to sell these securities in any jurisdiction where the offer or sale is not permitted. You should assume that the information appearing in this prospectus is accurate only as of the date on the front cover of this prospectus. Our business, financial condition, results of operations and prospects may have changed since that date.

For investors outside of the United States: we have not and the underwriters have not done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than the United States. Persons outside of the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside of the United States.

4D Molecular Therapeutics™, Therapeutic Vector Evolution™, and our logo are some of our trademarks and tradenames used in this prospectus. This prospectus also includes trademarks, tradenames and service marks that are the property of other organizations. Solely for convenience, our trademarks and tradenames referred to in this prospectus may appear without the ® and ™ symbol, but those references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights, or the right of the applicable licensor to these trademarks and tradenames.

## **PROSPECTUS SUMMARY**

This summary highlights information contained elsewhere in this prospectus and does not contain all of the information that you should consider in making your investment decision. Before deciding to invest in our common stock, you should read this entire prospectus carefully, including the sections of this prospectus titled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes included elsewhere in this prospectus. Unless the context otherwise requires or as otherwise noted, references in this prospectus to the "company," "4D Molecular Therapeutics," "4DMT," "we," "us" and "our" refer to 4D Molecular Therapeutics, Inc.

## 4D Molecular Therapeutics, Inc.

## Overview

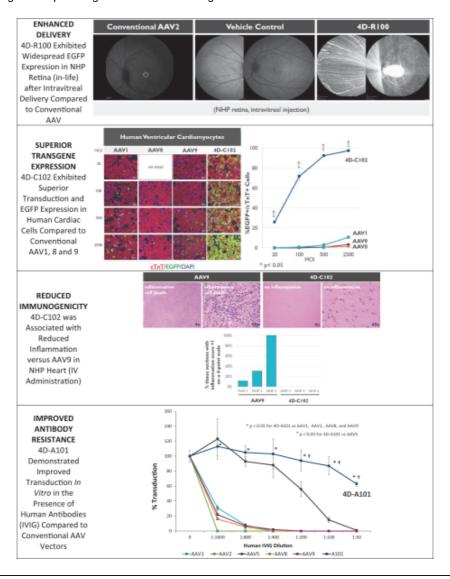
We are a development stage precision gene therapy company dedicated to pioneering the development of targeted therapies based on our next-generation AAV vectors. Our proprietary Therapeutic Vector Evolution platform enables our "disease first" approach to product discovery and development, thereby allowing us to customize our AAV vectors to target specific tissue types associated with the underlying disease. Our proprietary AAV vectors are designed to provide targeted delivery by routine clinical routes, efficient transduction, reduced immunogenicity and resistance to pre-existing antibodies, which we believe will enable us to develop gene therapies that could overcome known limitations of conventional AAVs. As a result, we believe these key attributes will enable us to develop gene therapies with improved therapeutic profiles, pursue previously untreatable diseases and address a broad range of rare and large market diseases.

Our product candidates that are in or have completed Investigational New Drug (IND)-enabling studies include a wholly-owned asset in development for the treatment of Fabry disease, as well as two assets in ophthalmology. In ophthalmology, our product candidate for the treatment of X-linked Retinitis Pigmentosa (XLRP) is wholly-owned subject to an exclusive option for Roche to develop and commercialize the asset, and our product candidate for the treatment of Choroideremia is licensed to Roche. In addition, our wholly-owned product candidate for the treatment of Cystic Fibrosis has completed lead optimization and we expect to initiate IND-enabling studies in the first half of 2020. We expect to initiate our first clinical trials for these three product candidates in 2020. We also have a pipeline of product candidates in the lead optimization stage, including for the treatment of Duchenne Muscular Dystrophy (DMD) and Wet Age-Related Macular Degeneration (Wet AMD).

## **Our Therapeutic Vector Evolution Platform**

While gene therapy holds tremendous promise as a transformative therapeutic class, the majority of gene therapeutic approaches utilize naturally-occurring or conventional AAV vectors that have encountered limitations such as high dose requirements, limited efficacy, inflammation and toxicity, and neutralization by pre-existing antibodies. As a result of these shortcomings, current efforts with conventional AAV vectors have focused on diseases in which a surgical technique is used for direct cell access such as subretinal injection for the retina, or a low level of gene expression may be sufficient for patient benefit, such as Hemophilia A or B treatment. Rather than using conventional AAV vectors for our products, we instead take a "disease first" approach by creating proprietary customized AAV vectors for each disease through our Therapeutic Vector Evolution platform. Our Therapeutic Vector Evolution platform is based on directed evolution, a high-throughput biological engineering and screening approach harnessing the power of natural selection to identify novel AAVs with desirable characteristics.

As of September 1, 2019, we have a total of 37 distinct libraries with an estimated over one billion AAV vector sequences. We select customized AAVs based on the Target Vector Profile (TVP) for the disease, and we subsequently characterize each lead vector through extensive studies in non-human primates (NHPs) and proprietary human cell and organotypic tissue assays. Based on our preclinical data to date from NHPs and human cell models, including preclinical head-to-head comparisons with conventional AAVs, we observed that our precision gene therapy AAVs were well-tolerated and achieved enhanced delivery, increased transgene expression, reduced immunogenicity and/or improved antibody resistance when compared to conventional AAVs. Our preclinical data comparing vectors carrying and expressing a marker *GFP* transgene is summarized in the table below.



## **Our Pipeline**

Our initial focus is on developing a broad pipeline of transformative gene therapy product candidates designed to treat patients suffering from lysosomal storage diseases, lung diseases, muscular dystrophies and ophthalmological diseases. We are developing a diverse pipeline of wholly-owned and partnered programs for both rare and large market diseases. We have not conducted any clinical trials but we expect to initiate our first clinical trials for these three product candidates in 2020. We believe our AAV products are modular, in that a single vector can be armed with different transgene payloads in order to treat multiple diseases within the same tissue types or therapeutic areas. Our pipeline is represented in the diagram below.



- \* Expected timing of milestone. FIH refers to first-in-human, Phase 1 or Phase 1/2, clinical studies.
- \*\* We are responsible for development of this product candidate and Roche has an exclusive option to assume development and commercialization at its sole cost and expense. Such option may be exercised prior to pivotal trial initiation.
- We are independently optimizing lead assets in this field. If we elect to declare product candidates in the field specified in our collaboration and license agreement with Roche, Roche could exercise the option to license, further develop and commercialize this product candidate at any time prior to pivotal trial initiation. See the section titled "Business—Strategic Collaborations—Collaboration and License Agreement with F. Hoffman Ltd. and Hoffman-La Roche Inc." for further information.
- ‡ Proof of concept studies for 4D-710 were conducted in a pig model of Cystic Fibrosis and utilized a species-specific (i.e., pig-specific) vector rather than the primate-specific 4D-A101 vector, which is the vector for Cystic Fibrosis product candidate 4D-710.

## Lysosomal Storage Diseases

Our lead product candidate in lysosomal storage diseases, 4D-310, is wholly-owned and under development for the treatment of Fabry disease, a progressive and fatal condition with cardiac complications cited as the leading cause of death. Classic Fabry disease is known to affect at least 5,000 males in the United States. However, we estimate the incidence rate may be higher as recent newborn screening suggests the total number of individuals with Fabry-related genetic mutations in the United States, as well as France, Germany, Italy, Spain and the United Kingdom (the EU-5) could fall between 50,000 and 70,000. Moreover, a recent multicenter study estimated that 57% of patients seen

in the clinic are of the classic phenotype. Thus, we estimate the total addressable male Fabry patient population in the United States to be approximately 10,000 individuals, and including the EU-5, we estimate the total addressable male population to be approximately 18,800 individuals. 4D-310 uses a proprietary vector designed for efficient, low dose intravenous delivery to cardiac and other relevant tissues and to have minimal toxicities, and potential resistance to pre-existing antibodies in the patient population. The approved enzyme replacement therapies (ERT) reportedly do not adequately address Fabry cardiomyopathy, a leading cause of death in this disease. In 2019, we initiated IND-enabling pharmacology and Good Laboratory Practices (GLP) toxicology and biodistribution studies with 4D-310, and in April 2019, we held a pre-IND meeting with the U.S. Food and Drug Administration (FDA). 4D-310 Phase 1/2 clinical trial initiation is planned for the second half of 2020. We expect initial human safety and biomarker data by

## Lung Diseases

Our lead product candidate in the lung therapeutic area, 4D-710, is wholly-owned and under development for the aerosol treatment of Cystic Fibrosis lung disease. Cystic Fibrosis is the most fatal inherited disease, affecting more than 70,000 patients worldwide. Cystic Fibrosis causes impaired lung function, inflammation and bronchiectasis and is commonly associated with repeat and persistent lung infections due to the inability to clear thickened mucus from the lung, often resulting in frequent exacerbations, hospitalizations and eventual end stage respiratory failure. We produced 4D-710 using a proprietary vector designed for efficient aerosol delivery to the lung airway, with enhanced penetration through mucus, efficient airway cell transduction and transgene expression, and resistance to pre-existing antibodies in the patient population. While several products have been approved for Cystic Fibrosis, they fall short of restoring normal lung function in most patients, and these chronic therapies require daily dosing for the patient's lifetime. Additionally, approximately 10% of patients remain without a corrective treatment option. We expect to initiate IND-enabling study activities with 4D-710 in the first half of 2020.

## Muscular Dystrophies

We are currently in the lead optimization phase for our wholly-owned research candidates for the treatment of certain muscular dystrophies, including DMD. DMD, a progressive and fatal muscular-wasting disease, is one of the most prevalent rare genetic diseases, with an estimated prevalence between 10,000 to 15,000 cases in the United States. Our product candidates are engineered from proprietary vectors designed for efficient, low dose intravenous delivery to cardiac and skeletal muscle tissues with minimal toxicities, and potential resistance to pre-existing antibodies in the human patient population. Several companies are developing gene therapies for DMD using conventional AAVs. While early clinical data in some programs have been encouraging, AAV gene therapies using conventional AAV vectors have been subject to inflammation-related toxicity issues and manufacturing challenges due to the relatively high doses required. In addition to requiring high doses, patients have been excluded from clinical trials due to pre-existing antibodies to the conventional vector used. We expect to complete lead optimization in 2020.

## Ophthalmological Diseases

We have two ophthalmology product candidates that utilize our proprietary intravitreal vector for rare monogenic blinding diseases: 4D-125 in development for the treatment of XLRP and 4D-110 in development for the treatment of Choroideremia. Our proprietary vector for these ophthalmological diseases is designed for intravitreal injection, potentially leading to transgene expression across the surface area of the retina, and major cell layers of the retina. We have a broad partnership in

ophthalmology with Roche, excluding DNA-directed RNA interference (ddRNAi) delivery. The partnership includes an option for Roche to license 4D-125 in XLRP, as well as other ophthalmology product candidates we may declare, prior to pivotal trial initiation. 4D-125 is expected to enter clinical trials in mid-2020. In addition, Roche currently holds a license to 4D-110 in Choroideremia. We expect to initiate 4D-110 clinical trials in the second half of 2020. We expect initial human safety and biomarker data for 4D-125 by

We are currently in lead optimization for multiple product candidates that utilize our proprietary intravitreal vector, the same as for 4D-125 and 4D-110 described above, for the treatment of Wet AMD, each of which is engineered to express a different protein to neutralize vascular endothelial growth factor (VEGF). We expect to complete lead optimization in the first half of 2020.

## Manufacturing

Our team has extensive experience with the manufacturing and analytical testing of numerous unique AAV capsids. To date, our team has internally manufactured approximately 50 unique AAV vector capsids, including both proprietary evolved 4DMT capsid variants and naturally-occurring capsids. Our team has manufactured over 100 total lots of AAV vectors. This total includes 9 lots of material for GLP toxicology and biodistribution studies. We initiated our first in-house manufacturing run for clinical trial material for use in patients, according to current Good Manufacturing Practices (cGMP), in 2019.

## **Our Team**

We have an experienced team of biotherapeutics developers, innovative gene therapy scientists and clinicians to execute our product design and development strategy. Led by our Chief Executive Officer and co-founder, David Kirn, M.D., our team has more than 100 years of combined experience in the field of viral vector gene therapy, including leadership of over 30 clinical trials from Phase 1 through Phase 3. Our Chief Technical Officer and Head of Regulatory and Quality, Fred Kamal, Ph.D., has over 20 years of industry experience in product manufacturing and quality, including most recently with AveXis where he was a key contributor to the development and writing of the quality and manufacturing sections of the biologics license application (BLA) for the AAV product Zolgensma. Our Chief Scientific Advisor and co-founder, David Schaffer, Ph.D., pioneered the application of directed evolution to the capsid of AAV vectors over 15 years ago.

## **Our Investors**

We have raised \$108.6 million from the sale and issuance of our preferred stock to leading investors including Viking Global Investors, Janus Henderson Investors, The Biotechnology Value Fund, Arrowmark Partners, Pfizer Ventures, Mirae Asset Financial Group, Perceptive Advisors, Ridgeback Capital Investments, Pappas Capital and Chiesi Ventures.

## **Our Strategy**

To achieve our goal of unlocking the full potential of gene therapy for many patient populations, we strive to achieve the following:

- Develop transformative precision gene therapies using the power of directed evolution and our "disease first" approach.
- Design, develop and commercialize a diverse portfolio of product candidates in a broad range of therapeutic areas for both rare and large market diseases.

- Continue to expand our cGMP manufacturing capabilities.
- Protect, defend and expand our intellectual property portfolio of proprietary AAV vectors discovered through directed evolution or Therapeutic Vector Evolution.
- Selectively partner with leading pharmaceutical companies to harness the full potential of our platform while retaining
  product rights in key markets, and work closely with patient advocacy groups to inform our programs.

#### **Risks Associated with Our Business**

Our ability to implement our business strategy is subject to numerous risks that you should be aware of before making an investment decision. These risks are described more fully in the section titled "Risk Factors," immediately following this prospectus summary. These risks include the following, among others:

- We are in the early stages of drug development, and we have a very limited operating history and no products approved for commercial sale, which may make it difficult to evaluate our current business and predict our future success and viability.
- We have incurred significant net losses since inception and we expect to continue to incur significant net losses for the foreseeable future.
- Even if this offering is successful, we will require substantial additional capital to finance our operations. If we fail or are unable to raise such capital when needed, or on acceptable terms, we may be forced to delay, reduce and/or eliminate one or more of our research and drug development programs, future commercialization efforts or other operations.
- All of our product candidates are based on a novel AAV gene therapy technology with which there is limited regulatory and
  no clinical experience to date, which makes it difficult to predict the time and cost of product candidate development and
  subsequently obtaining regulatory approval.
- Gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in
  delays in our development or commercialization programs, limit the supply of our products or otherwise seriously harm our
  business.
- Adverse public perception or regulatory scrutiny of gene therapy technology may negatively impact the developmental progress or commercial success of products that we develop alone or with collaborators.
- Our clinical trials may fail to demonstrate substantial evidence of the safety and efficacy of our product candidates, which
  would prevent, delay or limit the scope of regulatory approval and commercialization.
- The regulatory approval processes of the FDA, EMA and comparable foreign regulatory authorities are lengthy, expensive, time consuming, and inherently unpredictable. If we are ultimately unable to obtain regulatory approval for our product candidates, we will be unable to generate product revenue and our business will be substantially harmed.
- Our employees, independent contractors, consultants, research or commercial partners or collaborators and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.
- Our success depends on our ability to protect our intellectual property and our proprietary technologies.

• Our rights to develop and commercialize our product candidates are subject in part to the terms and conditions of licenses granted to us by others, and the patent protection, prosecution and enforcement for some of our product candidates may be dependent on our licensors.

## **Corporate Information**

We were formed on September 12, 2013 as a Delaware limited liability corporation under the name 4D Molecular Therapeutics, LLC. On March 11, 2015, 4D Molecular Therapeutics, Inc. was incorporated as a Delaware corporation. On March 20, 2015, 4D Molecular Therapeutics, LLC merged with 4D Molecular Therapeutics, Inc. with 4D Molecular Therapeutics, Inc. being the surviving entity. Our principal executive offices are located at 5858 Horton Street #455, Emeryville, California 94608, and our telephone number is (510) 505-2680. Our website address is www.4dmoleculartherapeutics.com. The information on, or that can be accessed through, our website is not part of this prospectus. We have included our website address as an inactive textual reference only.

## Implications of Being an Emerging Growth Company

We are an emerging growth company as defined in the Jumpstart Our Business Startups Act of 2012 (the JOBS Act). We will remain an emerging growth company until the earlier of (i) the last day of the year following the fifth anniversary of the completion of this offering, (ii) the last day of the year in which we have total annual gross revenue of at least \$1.07 billion, (iii) the last day of the year in which we are deemed to be a "large accelerated filer" as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended (the Exchange Act), which would occur if the market value of our common stock held by non-affiliates exceeded \$700.0 million as of the last business day of the second fiscal quarter of such year, or (iv) the date on which we have issued more than \$1.0 billion in non-convertible debt securities during the prior three-year period. An emerging growth company may take advantage of specified reduced reporting requirements and is relieved of certain other significant requirements that are otherwise generally applicable to public companies. As an emerging growth company:

- we will present only two years of audited financial statements, plus unaudited financial statements for any interim period, and related management's discussion and analysis of financial condition and results of operations;
- we will avail ourselves of the exemption from the requirement to obtain an attestation and report from our auditors on the
  assessment of our internal control over financial reporting pursuant to the Sarbanes-Oxley Act of 2002 (the Sarbanes-Oxley
  Act);
- we will provide less extensive disclosure about our executive compensation arrangements;
- we will not require stockholder non-binding advisory votes on executive compensation or golden parachute arrangements;
   and
- we will take advantage of extended transition periods to comply with new or revised accounting standards, delaying the
  adoption of these accounting standards until they would apply to private companies.

As a result, the information in this prospectus and that we provide to our investors in the future may be different than what you might receive from other public reporting companies.

## THE OFFERING

Common stock offered by us

Underwriters' over-allotment option

Common stock to be immediately outstanding after the offering

Use of proceeds

Risk factors

Proposed Nasdaq Global Market trading symbol

shares.

We have granted the underwriters a 30-day over-allotment option to purchase up to additional shares of our common stock.

shares (or shares if the underwriters exercise their over-allotment option in full).

We estimate that the net proceeds from this offering will be approximately \$\\$million, or approximately \$\\$million if the underwriters exercise their over-allotment option in full, assuming an initial public offering price of \$\\$per share, the midpoint of the estimated price range set forth on the cover of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

We currently expect to use the net proceeds from this offering to fund the ongoing and planned preclinical and clinical development of our product candidates, further the development and expansion of our pipeline, the continued expansion of our manufacturing and research and development capabilities and the remainder for working capital and general corporate purposes. See the section titled "Use of Proceeds" for a more complete description of the intended use of proceeds from this offering.

See the section titled "Risk Factors" and other information included in this prospectus for a discussion of factors that you should consider carefully before deciding to invest in our common stock.

The number of shares of our common stock to be outstanding after this offering is based on 12,516,975 shares of our common stock (including our redeemable convertible preferred stock on an as-converted basis) outstanding as of June 30, 2019, and

• 2,192,230 shares of our common stock issuable upon the exercise of stock options to purchase common stock that were outstanding as of June 30, 2019, with a weighted-average exercise price of \$6.10 per share;

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• 68,669 shares of our common stock issuable upon the exercise of outstanding warrants with weighted-average exercise price of \$1.85 per share;

excludes:

- 399,886 shares of our common stock reserved for issuance pursuant to future awards under our 2015 Equity Incentive Plan (the Plan) and associated amendments as of June 30, 2019;
- shares of our common stock reserved for issuance pursuant to future awards under our 2019 Equity Incentive Award
  Plan (the 2019 Plan), as well as any automatic increases in the number of shares of our common stock reserved for future
  issuance under this plan, which will become effective immediately prior to the completion of this offering; and
- shares of our common stock reserved for issuance pursuant to future awards under our 2019 Employee Stock Purchase Plan (the ESPP), as well as any automatic increases in the number of shares of our common stock reserved for future issuance under this plan, which will become effective immediately prior to the completion of this offering.

In addition, unless we specifically state otherwise, all information in this prospectus reflects and assumes the following:

- a -for- forward stock split of our capital stock to be effected prior to the effectiveness of the registration statement of which this prospectus is a part;
- the conversion of 7,375,631 shares of our outstanding redeemable convertible preferred stock as of June 30, 2019 into an equivalent number of shares of our common stock immediately prior to the completion of this offering;
- the filing and effectiveness of our amended and restated certificate of incorporation in Delaware and the adoption of our amended and restated bylaws, each of which will occur immediately prior to the completion of this offering;
- · no exercise of outstanding stock options or warrants described above; and
- · no exercise of the underwriters' over-allotment option.

Unless otherwise specified and unless the context otherwise requires, we refer to our Series A, Series A-1 and Series B redeemable convertible preferred stock collectively as, redeemable convertible preferred stock in this prospectus, as well as for financial reporting purposes and in the financial tables included elsewhere in this prospectus, as more fully explained in Note 10 to our financial statements included elsewhere in this prospectus.

## **Summary Financial Data**

The following tables summarize our financial data for the periods and as of the dates indicated. We derived the summary statements of operations and comprehensive loss data for the years ended December 31, 2017 and 2018 and the summary balance sheet data as of December 31, 2018 from our audited financial statements included elsewhere in this prospectus. We derived the summary statements of operations and comprehensive loss data for the six months ended June 30, 2018 and 2019 and the summary balance sheet data as of June 30, 2019 from our unaudited interim financial statements that are included elsewhere in this prospectus. Our unaudited interim financial statements are prepared in accordance with U.S. generally accepted accounting principles (GAAP) on the same basis as our audited financial statements and include, in the opinion of management, all adjustments, consisting of normal recurring adjustments, that are necessary for the fair statement of the financial information set forth in those financial statements. Our historical results are not necessarily indicative of the results that may be expected in the future and our historical results for the six months ended June 30, 2019 are not necessarily indicative of the results that may be expected for the remainder of 2019.

You should read this data together with our financial statements and related notes included elsewhere in this prospectus and the information under the sections titled "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations."

	Vear Ended	Year Ended December 31.		Six Months Ended June 30.		
	2017 2018		2018	2019		
	(in thousands, except share and per share data)					
Revenue:						
Collaboration and research revenue, related parties	\$ 3,176	\$ 5,143	\$ 143	\$ 26		
Collaboration and research revenue	2,614	8,987	4,926	4,390		
Total revenue	5,790	14,130	5,069	4,416		
Operating expenses:						
Research and development	13,573	18,362	8,208	16,985		
General and administrative	3,489	6,167	2,198	4,884		
Total operating expenses	17,062	24,529	10,406	21,869		
Loss from operations	(11,272)	(10,399)	(5,337)	(17,453)		
Other income (expense):	, ,	,	, ,	, ,		
Interest income	89	850	147	945		
Other income (expense), net	(40)	(2)	4	(8)		
Total other income (expense)	49	848	151	937		
Net loss and comprehensive loss	\$ (11,223)	\$ (9,551)	\$ (5,186)	\$ (16,516)		
Net loss per share attributable to common stockholders, basic and diluted $^{(1)}$	(2.27)	(1.89)	(1.03)	(3.22)		
Weighted-average shares outstanding used in computing net loss per share attributable to common stockholders, basic and diluted(1)	4,953,419	5,049,203	5,021,003	5,132,714		
Pro forma net loss per share, basic and diluted(1)						
Weighted-average shares outstanding used in computing pro forma net loss per share, basic and diluted <sup>(1)</sup>						

<sup>(1)</sup> See Notes 2 and 14 to our financial statements included elsewhere in this prospectus for an explanation of the calculations of our basic and diluted net loss per share attributable to common stockholders, pro forma net loss per share, and the weighted-average number of shares of our common stock used in the computation of the per share amounts.

	_	As of June 30, 2019			
		Actual		Pro Forma <sup>(1)</sup> (in thousands)	Pro Forma As Adjusted <sup>(2)(3)</sup>
Balance Sheet Data:				(iii tiiousaiius)	
Cash and cash equivalents	\$	72	2,869	\$	\$
Working capital <sup>(4)</sup>		66	,842		
Total assets		80	,500		
Redeemable convertible preferred stock		102	2,980		
Accumulated deficit		(46	5,235)		
Total stockholders' (deficit) equity		(42	2,304)		

- (1) The pro forma column in the balance sheet data table above gives effect to (i) the conversion of 7,375,631 shares of our outstanding redeemable convertible preferred stock as of June 30, 2019 into an equivalent number of shares of our common stock immediately prior to the completion of this offering; and (ii) the filing and effectiveness of our amended and restated certificate of incorporation, which will occur, in each case, immediately prior to the completion of this offering.
- (2) The pro forma as adjusted column in the balance sheet data table above gives effect to (i) the pro forma adjustments set forth in footnote (i) above and (ii) the sale and issuance of shares of our common stock in this offering, assuming an initial public offering price of \$ per share, the midpoint of the estimated price range set forth on the cover of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.
- payable by us.

  (3) Each \$1.00 increase or decrease in the assumed initial public offering price of \$ per share, the midpoint of the estimated price range set forth on the cover of this prospectus, would increase or decrease, as applicable, each of the amount of our cash and cash equivalents, working capital, total assets and total stockholders' (deficit) equity by \$ million, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting the estimated underwriting discount and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of shares we are offering. Each increase or decrease of 1.0 million in the number of shares we are offering would increase or decrease, as applicable, each of the amount of our cash and cash equivalents, working capital, total assets and total stockholders' (deficit) equity by approximately \$ million, assuming the assumed initial public offering price per share, as set forth on the cover page of this prospectus, remains the same. The pro forma as adjusted information is illustrative only and we will adjust this information based on the actual initial public offering price and other terms of this offering determined at pricing.
- (4) We define working capital as current assets less current liabilities. See our financial statements and related notes included elsewhere in this prospectus for further details regarding our current assets and current liabilities.

#### **RISK FACTORS**

Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this prospectus, including our financial statements and the related notes and the section of this prospectus titled "Management's Discussion and Analysis of Financial Condition and Results of Operations," before deciding whether to invest in our common stock. If any of the following risks actually occurs, our business, reputation, financial condition, results of operations, revenue and future prospects could be seriously harmed. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties that we are unaware of, or that we currently believe are not material, may also become important factors that adversely affect our business. Unless otherwise indicated, references to our business being seriously harmed in these risk factors and elsewhere will include harm to our business, reputation, financial condition, results of operations, future prospects and stock price. In that event, the market price of our common stock could decline, and you could lose part or all of your investment.

## Risks Related to Our Limited Operating History, Financial Condition and Capital Requirements

We are in the early stages of drug development and have a very limited operating history and no products approved for commercial sale, which may make it difficult to evaluate our current business and predict our future success and viability.

We are a development-stage biopharmaceutical company specializing in the development of novel, precision AAV vectors, for gene therapies that are generated through our proprietary directed evolution platform. We commenced operations in September 2013, have no products approved for commercial sale and have not generated any product revenue. Drug development is a highly uncertain undertaking and involves a substantial degree of risk. We expect to begin our first clinical trial in the middle of 2020. If our product candidates are not successfully developed and approved, we may never generate any revenue. To date, we have not initiated or completed any clinical trials (including any pivotal clinical trial), obtained marketing approval for any product candidates, manufactured commercial scale quantities of any of our product candidates or arranged for a third party to do so on our behalf, or conducted sales and marketing activities necessary for successful product commercialization. Our limited operating history as a company and early stage of drug development make any assessment of our future success and viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by early-stage biopharmaceutical companies in rapidly evolving fields, and we have not yet demonstrated an ability to successfully overcome such risks and difficulties. If we do not address these risks and difficulties successfully, our business will be seriously harmed.

We have incurred significant net losses since inception and we expect to continue to incur significant net losses for the foreseeable future.

We have incurred net losses in each reporting period since our inception, including net losses of \$11.2 million and \$9.6 million for the years ended December 31, 2017 and 2018, respectively. As of June 30, 2019, we had an accumulated deficit of \$46.2 million.

We have devoted substantially all of our financial resources and efforts in research and development activities, including for our product candidates and our Therapeutic Vector Evolution platform. We do not expect to generate revenue from product sales for several years, if at all. We continue to incur significant research and development and other expenses related to our ongoing operations. The amount of our future net losses will depend, in part, on the level of our future expenditures and our ability to generate revenue. Moreover, our net losses may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance.

We expect to continue to incur significant and increasingly higher expenses and operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- progress our current and any future product candidates through preclinical and clinical development;
- expand our manufacturing facilities and work with our contract manufacturers to scale up the manufacturing processes for our product candidates;
- · continue our research and discovery activities;
- continue the development of our Therapeutic Vector Evolution platform;
- initiate and conduct additional preclinical, clinical or other studies for our product candidates;
- · change or add additional contract manufacturers or suppliers;
- seek regulatory approvals and marketing authorizations for our product candidates;
- establish sales, marketing and distribution infrastructure to commercialize any products for which we obtain approval;
- · acquire or in-license product candidates, intellectual property and technologies;
- make milestone, royalty or other payments due under any current or future collaboration or license agreements;
- obtain, maintain, expand, protect and enforce our intellectual property portfolio;
- · attract, hire and retain qualified personnel;
- · experience any delays or encounter other issues related to our operations;
- · meet the requirements and demands of being a public company; and
- · defend against any product liability claims or other lawsuits related to our products.

Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' deficit and working capital. In any particular quarter or quarters, our operating results could be below the expectations of securities analysts or investors, which could cause our stock price to decline.

Even if this offering is successful, we will require substantial additional capital to finance our operations. If we fail or are unable to raise such capital when needed, or on acceptable terms, we may be forced to delay, reduce and/or eliminate one or more of our research and drug development programs, future commercialization efforts or other operations.

Developing biopharmaceutical products, including conducting preclinical studies and clinical trials, is a very time consuming, expensive and uncertain process that takes years to complete. Our operations have required substantial amounts of cash since inception. To date, we have financed our operations primarily through the sale of equity securities, and to a lesser extent, payments pursuant to our collaboration agreements. We expect to initiate clinical trials in the middle of 2020 and have several other product candidates in preclinical development that may enter clinical development shortly thereafter. Developing our product candidates is expensive, and we expect to continue to spend substantial amounts as we fund our early-stage research projects, continue preclinical development of our product candidates and, in particular, advance our product candidates through clinical trials. Even if we are successful in developing our product candidates, obtaining regulatory approvals and launching and commercializing any product candidate will require substantial additional funding beyond the net proceeds of this offering.

As of June 30, 2019, we had \$72.9 million in cash and cash equivalents. Based on our current operating plan, we estimate that our existing cash and cash equivalents, together with the anticipated net proceeds from this offering, will be sufficient to fund our operating expenses and capital expenditure requirements through at least the next months. Our estimate as to how long we expect our existing cash and cash equivalents to be available to fund our operations is based on assumptions that may prove inaccurate, and we could use our available capital resources sooner than we currently expect. In addition, changing circumstances may cause us to increase our spending significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. We may need to raise additional funds sooner than we anticipate if we choose to expand more rapidly than we presently anticipate.

Additional funds may not be available when we need them, on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis, we may be required to:

- delay, limit, reduce or terminate preclinical studies, clinical trials or other research and development activities or eliminate one or more of our development programs altogether; or
- delay, limit, reduce or terminate our efforts to establish manufacturing and sales and marketing capabilities or other activities that
  may be necessary to commercialize our product candidates, or reduce our flexibility in developing or maintaining our sales and
  marketing strategy.

We also could be required to seek funds through arrangements with collaborators or others that may require us to relinquish rights to, or jointly own some aspects of, our product candidates or technologies that we would otherwise pursue on our own. We do not expect to realize revenue from sales of products or royalties from licensed products in the foreseeable future, if at all, and unless and until a product candidate is clinically tested, approved for commercialization and successfully marketed.

We will be required to seek additional funding in the future and currently intend to do so through collaborations, public or private equity offerings or debt financings, credit or loan facilities or a combination of one or more of these funding sources. Our ability to raise additional funds will depend on financial, economic and other factors, many of which are beyond our control. Additional funds may not be available to us on acceptable terms or at all. If we raise additional funds by issuing equity securities, our stockholders will suffer dilution and the terms of any financing may adversely affect the rights of our stockholders. In addition, as a condition to providing additional funds to us, future investors may demand, and may be granted, rights superior to those of existing stockholders. Debt financing, if available, is likely to involve restrictive covenants limiting our flexibility in conducting future business activities, and, in the event of insolvency, debt holders would be repaid before holders of our equity securities received any distribution of our corporate assets.

If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our product candidates or one or more of our other research and development initiatives. Any of the above events could significantly harm our business and cause the price of our common stock to decline.

Due to the significant resources required for the development of our product candidates, and depending on our ability to access capital, we must prioritize development of certain product candidates. Moreover, we may expend our limited resources on product candidates that do not yield a successful product and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Due to the significant resources required for the development of our product candidates, in particular our product candidates in IND-enabling studies or those in clinical trials, we must decide

which product candidates and indications to pursue and advance and the amount of resources to allocate to each. Our decisions concerning the allocation of research, development, collaboration, management and financial resources toward particular product candidates or therapeutic areas may not lead to the development of any viable commercial product and may divert resources away from better opportunities. Similarly, our potential decisions to delay, terminate or collaborate with third parties in respect of certain product candidates may subsequently also prove to be less than optimal and could cause us to miss valuable opportunities. If we make incorrect determinations regarding the viability or market potential of any of our product candidates or misread trends in the biopharmaceutical industry, in particular for lysosomal storage diseases, lung diseases, muscular dystrophies and ophthalmological diseases, our business could be seriously harmed. As a result, we may fail to capitalize on viable commercial products or profitable market opportunities, be required to forego or delay pursuit of opportunities with other product candidates or other diseases that may later prove to have greater commercial potential than those we choose to pursue, or relinquish valuable rights to such product candidates through collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to invest additional resources to retain sole development and commercialization rights.

The amount of our future losses is uncertain and our quarterly operating results may fluctuate significantly or may all below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline.

Our quarterly and annual operating results may fluctuate significantly, which makes it difficult for us to predict our future operating results. These fluctuations may occur due to a variety of factors, many of which are outside of our control and may be difficult to predict, including:

- the timing and success or failure of preclinical studies and clinical trials for our product candidates or competing product candidates, or any other change in the competitive landscape of our industry, including consolidation among our competitors or collaboration partners;
- the timing and cost of, and level of investment in research, development and commercialization activities, which may change from time to time;
- the timing of receipt of approvals from regulatory authorities in the United States and internationally;
- the timing and status of enrollment and safety and efficacy readouts for our clinical trials;
- the cost of manufacturing, as well as building out our supply chain, which may vary depending on the quantity of production, the
  cost of continuing to establish and scale up our internal manufacturing capabilities, and the terms of any agreements we enter
  into with third-party suppliers;
- timing and amount of any option, milestone, royalty or other payments due under any current or future collaboration or license agreement;
- coverage and reimbursement policies with respect to our gene therapy product candidates and potential future drugs that compete with our products, if approved;
- expenditures that we may incur to acquire, develop or commercialize additional products and technologies;
- the level of demand for our gene therapy products, if approved, which may vary significantly over time; and
- future accounting pronouncements or changes in our accounting policies.

The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-

period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance.

This variability and unpredictability could also result in our failing to meet the expectations of financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue or earnings guidance we may provide.

Risks Related to the Research, Discovery, Development and Commercialization of Our Product Candidates

All of our product candidates are based on a novel AAV gene therapy technology with which there is limited regulatory and no clinical experience to date, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval.

All of our product candidates are based on gene therapy technology and our future success depends on the successful development of this novel therapeutic approach. We cannot assure you that any development problems we or other gene therapy companies experience in the future related to gene therapy technology will not cause significant delays or unanticipated costs in the development of our product candidates, or that such development problems can be solved. In addition, the clinical study requirements of the U.S. Food and Drug Administration (FDA) and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied therapeutic modalities. Further, as we are developing novel treatments for diseases in which there is limited clinical experience with new endpoints and methodologies, there is heightened risk that the FDA, European Medicines Agency (EMA) or comparable foreign regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. To date, few gene therapy products have been approved by the FDA or comparable foreign regulatory authorities, which makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the European Union or other jurisdictions. Further, approvals by one regulatory agency may not be indicative of what other regulatory agencies may require for approval.

Regulatory requirements governing gene therapy products have evolved and may continue to change in the future. For example, the FDA established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research (CBER) to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. These and other regulatory review agencies, committees and advisory groups and the requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval limitations or restrictions.

Under the National Institutes of Health (NIH) Guidelines for Research Involving Recombinant DNA Molecules (NIH Guidelines), supervision of human gene transfer trials, including evaluation and assessment by an Institutional Biosafety Committee (IBC) a local institutional committee that reviews

and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution, is required. The IBC assesses the safety of the research and identifies any potential risk to the public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

We are subject to significant regulatory oversight by the FDA, and in addition to the government regulators, the applicable IBC and Institutional Review Board (IRB), of each institution at which we or our collaborators conduct clinical trials of our product candidates, or a central IRB if appropriate, would need to review and approve the proposed clinical trial.

Similarly, the EMA governs the development of gene therapies in the European Union and may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines.

Changes in applicable regulatory guidelines may lengthen the regulatory review process, require us to perform additional studies or trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions.

As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of such product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

Adverse public perception or regulatory scrutiny of gene therapy technology may negatively impact the developmental progress or commercial success of products that we develop alone or with collaborators.

The developmental and commercial success of our current product candidates, or any that we develop alone or with collaborators in the future, will depend in part on public acceptance of the use of gene therapy technology, including the use of AAVs, for the prevention or treatment of human diseases. Adverse public perception of gene therapies may negatively impact our ability to raise capital or enter into strategic agreements for the development of product candidates.

Gene therapy remains a novel technology. The commercial success of our gene therapy products, if successfully developed and approved, may be adversely affected by claims that gene therapy is unsafe, unethical or immoral. This may lead to unfavorable public perception and the inability of any of our product candidates to gain the acceptance of the public or the medical community. Unfavorable public perceptions may also adversely impact our or our collaborators' ability to enroll clinical trials for our product candidates. Moreover, success in commercializing any product candidates that receive regulatory approval will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of such product candidates in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available.

Publicity of any adverse events in, or unfavorable results of, preclinical studies or clinical trials for any current or future product candidates, or with respect to the studies or trials of our competitors or of

academic researchers utilizing similar technologies, even if not ultimately attributable to our technology or product candidates, could negatively influence public opinion. Negative public perception about the use of AAV technology in human therapeutics, whether related to our technology or a competitor's technology, could result in increased governmental regulation, delays in the development and commercialization of product candidates or decreased demand for the resulting products, any of which may have a negative impact on our business and financial condition.

Our product candidates may cause undesirable side effects or have other properties that could halt their clinical development, prevent their regulatory approval, limit their commercial potential or result in significant negative consequences.

Adverse events or other undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign regulatory authorities.

During the conduct of clinical trials, patients report changes in their health, including illnesses, injuries, and discomforts, to their study doctor. Often, it is not possible to determine whether or not the product candidate being studied caused these conditions. It is possible that as we test our product candidates in larger, longer and more extensive clinical trials, or as use of these product candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in previous trials, as well as conditions that did not occur or went undetected in previous trials, will be reported by patients. Many times, side effects are only detectable after investigational products are tested in large-scale, Phase 3 clinical trials or, in some cases, after they are made available to patients on a commercial scale after approval.

If any serious adverse events occur, clinical trials or commercial distribution of any product candidates or products we develop alone or with collaborators could be suspended or terminated, and our business could be seriously harmed. Treatment-related side effects could also affect patient recruitment and the ability of enrolled patients to complete the trial or result in potential liability claims. Regulatory authorities could order us or our collaborators to cease further development of, deny approval of, or require us to cease selling any product candidates or products for any or all targeted indications. If we or our collaborators elect, or are required, to delay, suspend or terminate any clinical trial or commercialization efforts, the commercial prospects of such product candidates or products may be harmed, and our ability to generate product revenues from them or other product candidates that we develop may be delayed or eliminated. Additionally, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects or adverse events caused by such products, a number of potentially significant negative consequences could result, including but not limited to:

- regulatory authorities may suspend, limit or withdraw approvals of such product, or seek an injunction against its manufacture or distribution;
- regulatory authorities may require additional warnings on the label including "boxed" warnings, or issue safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- · we may be required to change the way the product is administered or conduct additional clinical trials or post-approval studies;
- we may be required to create a Risk Evaluation and Mitigation Strategy (REMS), which could include a medication guide
  outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers and/or other
  elements to assure safe use;
- · the product may become less competitive;

- we may be subject to fines, injunctions or the imposition of criminal penalties;
- · we could be sued and held liable for harm caused to patients; and
- · our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could seriously harm our business.

Drug development is a highly uncertain undertaking and involves a substantial degree of risk. We have no products approved for commercial sale, and we have never generated any revenue from product sales, and we may never generate revenue or be profitable.

We have no products approved for commercial sale and have not generated any revenue from product sales. We do not anticipate generating any revenue from product sales until after we have successfully completed clinical development and received regulatory approval for the commercial sale of a product candidate, which will not occur for several years if ever.

Our ability to generate revenue and achieve profitability depends significantly on many factors, including:

- · successfully completing research and preclinical and clinical development of our product candidates;
- obtaining regulatory approvals and marketing authorizations for product candidates for which we successfully complete clinical development and clinical trials;
- developing a sustainable and scalable manufacturing process for our product candidates, as well as establishing and
  maintaining commercially viable supply relationships with third parties that can provide adequate products and services to
  support clinical activities and any commercial demand for our product candidates;
- identifying, assessing, acquiring and/or developing new product candidates;
- · negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;
- the prevalence, duration and severity of potential side effects or other safety issues experienced with our product candidates or future approved products, if any;
- launching and successfully commercializing product candidates for which we obtain marketing approval, either by collaborating with a partner or, if launched independently, by establishing a sales, marketing and distribution infrastructure;
- obtaining and maintaining an adequate price for our product candidates, both in the United States and in foreign countries where our products are commercialized;
- obtaining adequate reimbursement for our product candidates or procedures using our product candidates from payors;
- the convenience and durability of our treatment or dosing regimen;
- acceptance by physicians, payors and patients of the benefits, safety and efficacy of our product candidate or any future product candidates, if approved, including relative to alternative and competing treatments;
- patient demand for any of our product candidates that may be approved;
- · addressing any competing technological and market developments;

- maintaining, protecting, expanding and enforcing our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- · attracting, hiring and retaining qualified personnel.

Because of the numerous risks and uncertainties associated with drug development, we are unable to predict the timing or amount of our expenses, or when we will be able to generate any meaningful revenue or achieve or maintain profitability, if ever. In addition, our expenses could increase beyond our current expectations if we are required by the FDA or foreign regulatory agencies, to perform studies in addition to those that we currently anticipate, or if there are any delays in any of our or our future collaborators' clinical trials or the development of any of our product candidates. Even if one or more of our product candidates is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate and ongoing compliance efforts.

Even if we are able to generate revenue from the sale of any approved products, we may not become profitable, and we will need to obtain additional funding through one or more equity or debt financings in order to continue operations. Revenue from the sale of any product candidate for which regulatory approval is obtained will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to get reimbursement at any price and whether we own the commercial rights for that territory. If the number of addressable patients is not as significant as we anticipate, the indication approved by regulatory authorities is narrower than we expect, the reasonably accepted population for treatment is narrowed by competition, physician choice or treatment guidelines or the price and available third party reimbursement are lower than anticipated, we may not generate significant revenue from sales of such products, even if approved. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our pipeline of product candidates or continue our operations and cause a decline in the value of our common stock, all or any of which may seriously harm our business.

We may encounter substantial delays in our clinical trials, or may not be able to conduct or complete our clinical trials on the timelines we expect, if at all.

Clinical testing is expensive, time consuming, and subject to uncertainty. To date, neither we nor our collaborators have initiated any clinical trials for any product candidates. We cannot guarantee that any clinical trials will be initiated or conducted as planned or completed on schedule, if at all. For example, recently a manufacturing batch of our product candidate 4D-110 produced at a CMO failed to meet the specifications required for use in our planned clinical trial due to an issue identified with one of the plasmids used in the manufacturing process. As a result, we delayed the initiation of our planned first-in-human trial of 4D-110 until the second half of 2020, so that we could produce the clinical-grade material required for the trial using our in-house manufacturing facility. We also cannot be sure that submission of an IND or a clinical trial application (CTA) will result in the FDA or other regulatory authority, as applicable, allowing clinical trials to begin in a timely manner, if at all. Moreover, even if these trials begin, issues may arise that could suspend or terminate such clinical trials. A failure of one or more clinical trials can occur at any stage of testing, and our future clinical trials may not be successful. Events that may prevent successful or timely initiation or completion of clinical trials include:

• inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation or continuation of clinical trials;

- delays in reaching a consensus with regulatory agencies on study design or implementation of the clinical trials;
- delays or failure in obtaining regulatory authorization to commence a trial;
- delays in reaching agreement on acceptable terms with prospective contract research organizations (CROs) and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- · delays in identifying, recruiting and training suitable clinical investigators;
- · delays in obtaining required IRB approval at each clinical trial site;
- delays in manufacturing, testing, releasing, validating, or importing/exporting sufficient stable quantities of our product candidates for use in clinical trials or the inability to do any of the foregoing;
- insufficient or inadequate supply or quality of product candidates or other materials necessary for use in clinical trials, or delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for clinical trials;
- imposition of a temporary or permanent clinical hold by regulatory agencies for a number of reasons, including after review of an IND or amendment, CTA or amendment, or equivalent foreign application or amendment; as a result of a new safety finding that presents unreasonable risk to clinical trial participants; or a negative finding from an inspection of our clinical trial operations or study sites;
- developments on trials conducted by competitors for related technology that raises FDA or foreign regulatory authority concerns about risk to patients of the technology broadly; or if the FDA or a foreign regulatory authority finds that the investigational protocol or plan is clearly deficient to meet its stated objectives;
- delays caused by patients withdrawing from clinical trials or failing to return for post-treatment follow-up;
- difficulty collaborating with patient groups and investigators;
- failure by our CROs, other third parties, or us to adhere to clinical trial protocols;
- failure to perform in accordance with the FDA's or any other regulatory authority's good clinical practice requirements (GCPs) or applicable regulatory guidelines in other countries;
- occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- · changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- · changes in the standard of care on which a clinical development plan was based, which may require new or additional trials;
- · the cost of clinical trials of our product candidates being greater than we anticipate;
- clinical trials of our product candidates producing negative or inconclusive results, which may result in our deciding, or regulators
  requiring us, to conduct additional clinical trials or abandon development of such product candidates;
- transfer of manufacturing processes to larger-scale facilities operated by a contract manufacturing organization (CMO) or by us, and delays or failure by our CMOs or us to make any necessary changes to such manufacturing process; and
- · third parties being unwilling or unable to satisfy their contractual obligations to us.

Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the severity and difficulty of diagnosing the disease under investigation, size of the patient population and process for identifying subjects, eligibility and exclusion criteria for the trial in question, design of the trial protocol, availability and efficacy of approved therapies or other clinical trials for the disease or condition under investigation, perceived risks and benefits of the product candidate under trial or testing, availability of genetic testing for potential patients, efforts to facilitate timely enrollment in clinical trials, patient referral practices of physicians, ability to obtain and maintain subject consent, the risk that enrolled subjects will drop out before completion of the trial, the ability to monitor patients adequately during and after treatment, and the proximity and availability of clinical trial sites for prospective patients. Furthermore, we rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials, and while we have agreements governing their committed activities, we have limited influence over their actual performance.

Any inability to successfully initiate or complete clinical trials could result in additional costs to us or impair our ability to generate revenue. In addition, if we make manufacturing or formulation changes to our product candidates, we may be required to or we may elect to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which our products have patent protection and may allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may seriously harm our business.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the data safety monitoring board for such trial or by the FDA or any other regulatory authority, or if the IRBs of the institutions in which such trials are being conducted suspend or terminate the participation of their clinical investigators and sites subject to their review. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Delays in the completion of any clinical trial of our product candidates will increase our costs, slow down our product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

The limited number of patients who have the diseases for which our product candidates are being studied may make it more difficult for us to enroll or complete clinical studies, or may result in findings in our clinical studies that do not reach levels of statistical significance sufficient for marketing approval.

Each of the conditions for which we plan to evaluate our current product candidates is a rare genetic disease. Accordingly, there are limited patient pools from which to draw for clinical studies. In addition to the rarity of these diseases, the eligibility criteria of our clinical studies will further limit the pool of available study participants as we will require that patients have specific characteristics that we can measure or to assure their disease is either severe enough or not too advanced to include them in a study. We or our collaborators may not be able to initiate or continue clinical trials on a timely basis or at all for any of our product candidates if we or our collaborators are unable to locate and enroll a sufficient number of eligible patients to participate in the trials as required by applicable regulations or as needed to provide appropriate statistical power for a given trial. Similarly, because most of the

conditions we intend to treat are rare in nature, we plan to design and conduct clinical trials utilizing a small number of patients in order to evaluate the safety and therapeutic activity of our product candidates. Conducting trials in smaller subject populations increases the risk that any safety or efficacy issues observed in only a few patients could prevent such studies from reaching statistical significance or otherwise meeting their specified endpoints, which could require us to conduct additional clinical studies, or delay or prevent our product candidates from receiving regulatory approval, which would seriously harm our business.

Research and development of biopharmaceutical products is inherently risky. We cannot give any assurance that any of our product candidates will receive regulatory approval, which is necessary before they can be commercialized.

We are at an early stage of development of our product candidates. Our future success is dependent on our ability to successfully develop, obtain regulatory approval for, and then successfully commercialize our product candidates, and we may fail to do so for many reasons, including the following:

- · our product candidates may not successfully complete preclinical studies or clinical trials;
- a product candidate may on further study be shown to have harmful side effects or other characteristics that indicate it does not
  meet applicable regulatory criteria;
- our competitors may develop therapeutics that render our product candidates obsolete or less attractive;
- our competitors may develop platform technologies that render our Therapeutic Vector Evolution platform technology obsolete or less attractive;
- the product candidates and Therapeutic Vector Evolution platform technology that we develop may not be sufficiently covered by
  intellectual property for which we hold exclusive rights or may be covered by third party patents or other intellectual property or
  exclusive rights;
- the market for a product candidate may change so that the continued development of that product candidate is no longer reasonable or commercially attractive;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all;
- if a product candidate obtains regulatory approval, we may be unable to establish sales and marketing capabilities, or successfully market such approved product candidate; and
- a product candidate may not be accepted as safe and effective by patients, the medical community or third-party payors.

If any of these events occur, we or our collaborators may be forced to abandon our development efforts for a product candidate or candidates, which would seriously harm our business. Failure of a product candidate may occur at any stage of preclinical or clinical development, and, because our product candidates and our Therapeutic Vector Evolution platform technology are in an early stage of development, there is a relatively higher risk of failure and we may never succeed in developing marketable products or generating product revenue.

We may not be successful in our efforts to further develop our Therapeutic Vector Evolution platform technology and current product candidates. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates. Each of our product candidates is in the early stages of development and will require

significant additional clinical development, management of preclinical, clinical, and manufacturing activities, regulatory approval, adequate manufacturing supply, a commercial organization, and significant marketing efforts before we generate any revenue from product sales, if at all. Any clinical studies that we may conduct may not demonstrate the efficacy and safety necessary to obtain regulatory approval to market our product candidates. If the results of our ongoing or future clinical studies are inconclusive with respect to the efficacy of our product candidates or if we do not meet the clinical endpoints with statistical significance or if there are safety concerns or adverse events associated with our product candidates, we may be prevented or delayed in obtaining marketing approval for our product candidates.

If any of our product candidates successfully completes clinical trials, we generally plan to seek regulatory approval to market our product candidates in the United States, the European Union, and in additional foreign countries where we believe there is a viable commercial opportunity. We have never commenced, compiled or submitted an application seeking regulatory approval to market any product candidates. We may never receive regulatory approval to market any product candidates even if such product candidates successfully complete clinical trials, which would adversely affect our viability. To obtain regulatory approval in countries outside the United States, we must comply with numerous and varying regulatory requirements of such other countries regarding safety, efficacy, chemistry, manufacturing and controls, clinical trials, commercial sales, pricing and distribution of our product candidates. We may also rely on our collaborators or collaboration partners to conduct the required activities to support an application for regulatory approval, and to seek approval, for one or more of our product candidates. We cannot be sure that our collaborators or collaboration partners will conduct these activities successfully or do so within the timeframe we desire. Even if we (or our collaborators or collaboration partners) are successful in obtaining approval in one jurisdiction, we cannot ensure that we will obtain approval in any other jurisdictions. Failure to obtain approval for our product candidates in multiple jurisdictions, will seriously harm our business.

Even if we receive regulatory approval to market any of our product candidates, we cannot assure you that any such product candidate will be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available alternatives. Any approval we may obtain could be for indications or patient populations that are not as broad as intended or desired or may require labeling that includes significant use or distribution restrictions or safety warnings. We may also be required to perform additional or unanticipated clinical studies to obtain approval or be subject to additional post-marketing testing requirements to maintain approval. In addition, regulatory authorities may withdraw their approval of a product or impose restrictions on its distribution, such as in the form of a REMS. The failure to obtain timely regulatory approval of product candidates, any product marketing limitations or a product withdrawal would seriously harm our business.

Investment in biopharmaceutical product development involves significant risk that any product candidate will fail to demonstrate adequate efficacy or an acceptable safety profile, gain regulatory approval, and become commercially viable. We cannot provide any assurance that we will be able to successfully advance any of our product candidates through the development process or, if approved, successfully commercialize any of our product candidates.

Our clinical trials may fail to demonstrate substantial evidence of the safety and efficacy of our product candidates, which would prevent, delay or limit the scope of regulatory approval and commercialization.

Before obtaining regulatory approvals for the commercial sale of any of our product candidates, we or our collaborators must demonstrate through lengthy, complex and expensive preclinical studies and clinical trials that our product candidates are both safe and effective for use in each target indication. Further, because our product candidates are subject to regulation as biological drug

products, we will need to demonstrate that they are safe, pure, and potent for use in their target indications. Each product candidate must demonstrate an adequate risk versus benefit profile in its intended patient population and for its intended use.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies of our product candidates may not be predictive of the results of early-stage or later-stage clinical trials, and results of early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. The results of clinical trials in one set of patients or disease indications may not be predictive of those obtained in another. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or unacceptable safety issues, notwithstanding promising results in earlier trials. Most product candidates that begin clinical trials are never approved by regulatory authorities for commercialization.

We have limited experience in designing clinical trials and may be unable to design and execute a clinical trial to support marketing approval. We cannot be certain that our planned clinical trials or any other future clinical trials will be successful. Additionally, any safety concerns observed in any one of our clinical trials in our targeted indications could limit the prospects for regulatory approval of our product candidates in those and other indications, which could seriously harm our business.

In addition, even if such clinical trials are successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. To the extent that the results of the trials are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates. Even if regulatory approval is secured for any of our product candidates, the terms of such approval may limit the scope and use of our product candidate, which may also limit its commercial potential.

Interim "top-line" and preliminary data from studies or trials that we announce or publish from time to time may change as more data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim "top-line" or preliminary data from preclinical studies or clinical trials. Interim data are subject to the risk that one or more of the outcomes may materially change as more data become available. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the top-line results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Preliminary or "top-line" data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Additionally, interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Adverse differences between preliminary or interim data and final data could seriously harm our business.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure. Any information we determine not to disclose may ultimately be deemed significant by you or others with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product candidate or our business. If the top-line data that we report differ from final results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, product candidates may be harmed, which could seriously harm our business.

We may not be successful in our efforts to continue to create a pipeline of product candidates or to develop commercially successful products. If we fail to successfully identify and develop additional product candidates, our commercial opportunity may be limited.

One of our strategies is to identify and pursue clinical development of additional product candidates through our Therapeutic Vector Evolution platform technology. Our Therapeutic Vector Evolution platform technology may not produce a pipeline of viable product candidates, or our competitors may develop platform technologies that render our Therapeutic Vector Evolution platform technology obsolete or less attractive. Our research methodology may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make them unmarketable or unlikely to receive marketing approval. Identifying, developing and obtaining regulatory approval and commercializing additional product candidates will require substantial additional funding beyond the net proceeds of this offering and is prone to the risks of failure inherent in drug development. If we are unable to successfully identify, acquire, develop and commercialize additional product candidates, our commercial opportunity may be limited.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than us.

The development and commercialization of new drug products is highly competitive. We may face competition with respect to any product candidates that we seek to develop or commercialize in the future from major pharmaceutical companies and biotechnology companies worldwide. Potential competitors also include academic institutions, government agencies, and other public and private research organizations that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing, and commercialization.

There are a number of large pharmaceutical and biotechnology companies that are currently pursuing the development of products for the treatment of the indications for which we have product candidates, including Fabry disease, Cystic Fibrosis, XLRP and Choroideremia. Certain of our competitors have commercially approved products for the treatment of the diseases that we are pursuing or may pursue in the future, including Roche, Sanofi, Takeda and Vertex. These drugs are well established therapies and are widely accepted by physicians, patients and third-party payors, which may make it difficult to convince these parties to switch to our product candidates. Companies that we are aware are developing therapeutics in the lysosomal storage diseases, lung diseases, muscular dystrophies and ophthalmological diseases areas include large companies with significant financial resources, such as Allergan, Novartis, Pfizer, Regeneron, Roche, Sanofi, Takeda and Vertex, and biopharmaceutical companies such as Avrobio, Freeline, Sangamo and Sarepta. In addition to competition from other companies targeting lysosomal storage, lung, muscular dystrophy and

ophthalmologic indications, any products we may develop may also face competition from other types of therapies, such as gene-editing therapies and drug delivery devices.

Many of our current or potential competitors, either alone or with their strategic partners, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our product candidates. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than any products that we may develop. Furthermore, currently approved products could be discovered to have application for treatment of lysosomal storage, lung, muscular dystrophy and ophthalmologic indications, which could give such products significant regulatory and market timing advantages over any of our product candidates. Our competitors also may obtain FDA, EMA or other regulatory approval for their products more rapidly than we may obtain approval for ours. Additionally, products or technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing any product candidates we may develop against competitors.

If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market any product candidates we may develop, we may not be successful in commercializing those product candidates if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product for which we retain sales and marketing responsibilities, we must either develop a sales and marketing organization or outsource these functions to third parties. In the future, we may choose to build a focused sales, marketing and commercial support infrastructure to sell, or participate in sales activities with our collaborators for, some of our product candidates if and when they are approved.

There are risks involved with both establishing our own commercial capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force or reimbursement specialists is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing and other commercialization capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our commercialization personnel.

Factors that may inhibit our efforts to commercialize any approved product on our own include:

- our inability to recruit and retain adequate numbers of effective sales, marketing, reimbursement, compliance, customer service, medical affairs and other support personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future approved products;

- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement, and other acceptance by payors;
- the inability to price our products at a sufficient price point to ensure an adequate and attractive level of profitability;
- restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- · unforeseen costs and expenses associated with creating an independent commercialization organization.

If we enter into arrangements with third parties to perform sales, marketing, commercial support and distribution services, our product revenue or the profitability of product revenue may be lower than if we were to market and sell any products we may develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to commercialize our product candidates or may be unable to do so on terms that are favorable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish commercialization capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates if approved and our business would be seriously harmed.

Even if any product candidates we develop receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors and others in the medical community necessary for commercial success.

The commercial success of any of our product candidates will depend upon its degree of market acceptance by physicians, patients, third-party payors and others in the medical community. Even if any product candidates we may develop receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors and others in the medical community. The degree of market acceptance of any product candidates we may develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in pivotal clinical trials and published in peer-reviewed journals;
- · the potential and perceived advantages compared to alternative treatments;
- the ability to offer our products for sale at competitive prices;
- the ability to offer appropriate patient access programs, such as co-pay assistance;
- sufficient third-party coverage or reimbursement;
- · the extent to which physicians recommend our products to their patients;
- convenience and ease of dosing and administration compared to alternative treatments;
- the clinical indications for which the product candidate is approved by FDA, EMA or other regulatory agencies;
- product labeling or product insert requirements of the FDA, EMA or other comparable foreign regulatory authorities, including
  any limitations, contraindications or warnings contained in a product's approved labeling;
- · restrictions on how the product is distributed;

- the timing of market introduction of competitive products;
- · publicity concerning our products or competing products and treatments;
- · the strength of marketing and distribution support; and
- · the prevalence and severity of any side effects.

If any product candidates we develop do not achieve an adequate level of acceptance, we may not generate significant product revenue, and we may not become profitable.

## If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates.

We face an inherent risk of product liability as a result of the clinical testing of our product candidates and will face an even greater risk when and if we commercialize any products. For example, we may be sued if our product candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to cease commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- · decreased or interrupted demand for our products;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- · injury to our reputation;
- withdrawal of clinical trial participants and inability to continue clinical trials;
- initiation of investigations by regulators;
- · costs to defend the related litigation;
- · a diversion of management's time and our resources;
- · loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any product candidate: and
- · a decline in our share price.

Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop, alone or with collaborators. Our insurance policies may have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

## **Risks Related to Manufacturing**

Gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products or otherwise seriously harm our business.

We currently have a development, manufacturing and testing agreement and cooperation agreement with Paragon and Wuxi, to manufacture supplies of our product candidates in the future. Our product candidates require processing steps that are more complex than those required for most chemical and protein pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we employ multiple steps to control our manufacturing process to assure that the process works and the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory, which could delay or prevent the initiation of clinical trials or receipt of regulatory approvals. We may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet FDA, or other comparable applicable foreign standards or specifications with consistent and acceptable production yields and costs. For example, recently a manufacturing batch of our product candidate 4D-110 produced at a CMO failed to meet the specifications required for use in our planned clinical trial due to an issue identified with one of the plasmids used in the manufacturing process. As a result, we delayed the initiation of our planned first-in-human trial of 4D-110 until the second half of 2020, so that we could produce the clinical-grade material required for the trial using our in-house manufacturing facility.

In addition, FDA and other comparable foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA or other comparable foreign regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay clinical trials or product launches which could be costly to us and otherwise seriously harm our business.

We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to operate our manufacturing process which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

Any problems in our manufacturing process or the facilities with which we contract could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies, which could limit our access to additional attractive development programs. Problems in third-party manufacturing process or facilities also could restrict our ability to meet market demand for our products. Additionally, should our agreements with Paragon or WuXi or agreements with other parties with whom we have manufacturing agreements be terminated for any reason, there are a limited number of manufacturers who would be suitable replacements, and it would take a significant amount of time to transition the manufacturing to a replacement.

Delays in obtaining regulatory approval of our manufacturing process or disruptions in our manufacturing process may delay or disrupt our commercialization efforts.

Before we can begin to commercially manufacture our product candidates in third-party or our own facilities, we must obtain regulatory approval from the FDA to market our product using the

manufacturing process and facility we proposed in our marketing application. In addition, we must pass a pre-approval inspection of our manufacturing facility by the FDA before any of our product candidates can obtain marketing approval, if ever. In order to obtain approval of a BLA for our product candidates, we will need to ensure that all of our manufacturing processes, methods and equipment are compliant with current cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. If any of our vendors, contract laboratories or suppliers is found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we work with these third parties to remedy the violation or while we work to identify suitable replacement vendors. The cGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with cGMP, we will be obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we would be subject to possible regulatory action and may not be permitted to sell any products that we may develop.

Delays in developing our manufacturing capabilities or failure to achieve operating efficiencies from it may require us to devote additional resources and management time to manufacturing operations and may delay our product development timelines.

We have a small operational manufacturing facility that we are using to manufacture clinical trial material. In addition, we have leased approximately 17,000 square feet of space for our second manufacturing facility in Emeryville, California, at which we plan to devote manufacturing activities for our clinical studies. We may face delays in the production of clinical supply at our manufacturing facility, and cannot guarantee when our facility will be able to produce sufficient quantities of product candidates needed to initiate our planned clinical studies. Any delays in developing our internal manufacturing capabilities may disrupt or delay the supply of our product candidates if we have not maintained a sufficient back-up supply of such product candidates through third-party manufacturers. Moreover, changing manufacturing facilities during the clinical development process may also require that we or our collaborators conduct additional studies, make notifications to regulatory authorities, make additional filings to regulatory authorities, and obtain regulatory authority approval for the new facilities, which may be delayed or which we may never receive. We will further need to comply with the FDA's and applicable foreign regulatory authorities' cGMP requirements for the production of product candidates for clinical trials and, if approved, commercial supply, and will be subject to FDA and comparable foreign regulatory authority inspection. These requirements include the qualification and validation of our manufacturing equipment and processes. We may not be able to develop or acquire the internal expertise and resources necessary for compliance with these requirements.

In order to develop internal manufacturing expertise, we may be forced to devote greater resources and management time than anticipated, particularly in areas relating to operations, quality, regulatory, facilities and information technology. We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to operate our manufacturing processes. If we experience unanticipated employee shortage or turnover in any of these areas, we may not be able to effectively manage our ongoing manufacturing operations and we may not achieve the operating efficiencies that we anticipate from developing these capabilities, which may negatively affect our product development timeline or result in difficulties in maintaining compliance with applicable regulatory requirements. Any such problems could result in the delay, prevention or impairment of clinical development and commercialization of our product candidates and would seriously harm our business.

We currently rely and expect to continue to rely on third parties to conduct product manufacturing for certain of our product candidates, and these third parties may not perform satisfactorily.

Although we are in process of expanding internal manufacturing capabilities, we currently rely, and expect to continue to rely, on third parties for the production of some of our preclinical study and planned clinical trial materials and, therefore, we can control only certain aspects of their activities. The facilities used by us and our contract manufacturers to manufacture certain of our product candidates must be reviewed by the FDA pursuant to inspections that will be conducted after we submit our BLA to the FDA. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with the cGMPs for manufacture of our products. If we or our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, we will not be able to obtain and/or maintain regulatory approval for our products as manufactured at their manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved.

In addition, we rely on additional third parties to manufacture plasmids used in the manufacture of our product candidates and to perform quality testing, and reliance on these third parties entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- reduced control for certain aspects of manufacturing activities;
- termination or nonrenewal of manufacturing and service agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers and service providers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or service provider.

Any of these events could lead to clinical trial delays or failure to obtain regulatory approval, or impact our ability to successfully commercialize future product candidates. Some of these events could be the basis for FDA or European Union Member State regulatory authority action, including injunction, recall, seizure or total or partial suspension of product manufacture.

Any contamination in our manufacturing process, shortages of raw materials or failure of any of our key suppliers to deliver necessary components could result in delays in our research studies, preclinical, clinical development or marketing schedules.

Given the nature of biologics manufacturing, there is a risk of contamination during manufacturing. Any contamination could materially harm our ability to produce product candidates on schedule and could harm our results of operations and cause reputational damage.

Some of the raw materials required in our manufacturing process, such as plasmids, are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could seriously harm our business.

We depend on third-party suppliers for key raw materials used in our manufacturing processes, and the loss of these third-party suppliers or their inability to supply us with adequate raw materials could harm our business.

We rely on third-party suppliers for the raw materials required for the production of our product candidates. Our dependence on these third-party suppliers and the challenges we may face in obtaining adequate supplies of raw materials involve several risks, including limited control over pricing, availability, quality and delivery schedules. As a small company, our negotiation leverage is limited and we are likely to get lower priority than our competitors who are larger than we are. We cannot be certain that our suppliers will continue to provide us with the quantities of these raw materials that we require or satisfy our anticipated specifications and quality requirements. Any interruption in supply of raw materials could materially harm our ability to manufacture our product candidates until a new source of supply, if any, could be identified and qualified. We may be unable to find a sufficient alternative supplier in a reasonable time or on commercially reasonable terms. Any performance failure on the part of our suppliers could delay the development and potential commercialization of our product candidates, including limiting supplies necessary for clinical trials and regulatory approvals, which would seriously harm our business.

### Risks Related to Regulatory Approval and Other Legal Compliance Matters

The regulatory approval processes of the FDA, EMA and comparable foreign regulatory authorities are lengthy, expensive, time consuming, and inherently unpredictable. If we are ultimately unable to obtain regulatory approval for our product candidates, we will be unable to generate product revenue and our business will be seriously harmed.

We and any collaborators are not permitted to commercialize, market, promote or sell any product candidate in the United States without obtaining marketing approval from the FDA. Foreign regulatory authorities impose similar requirements. The time required to obtain approval by the FDA and comparable foreign regulatory authorities is unpredictable, typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the type, complexity and novelty of the product candidates involved. In addition, approval policies, regulations or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions, which may cause delays in the approval or the decision not to approve an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. We have not submitted for or obtained regulatory approval for any product candidate. We and any collaborators must complete additional preclinical or nonclinical studies and clinical trials to demonstrate the safety and efficacy of our product candidates in humans to the satisfaction of the regulatory authorities before we will be able to obtain these approvals, and it is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval.

Applications for our product candidates could fail to receive regulatory approval for many reasons, including but not limited to the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design, implementation or results of our or our collaborators' clinical trials;
- the FDA or comparable foreign regulatory authorities may determine that our product candidates are not safe and effective, only moderately effective or have undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use of our products;
- the population studied in the clinical program may not be sufficiently broad or representative to assure efficacy and safety in the full population for which we seek approval;

- we or our collaborators may be unable to demonstrate to the FDA, or comparable foreign regulatory authorities that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our or our collaborators' interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of a BLA or other submission or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes, test procedures and specifications, or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our or our collaborators' clinical data insufficient for approval.

This lengthy approval process, as well as the unpredictability of the results of clinical trials, may result in our failing to obtain regulatory approval to market any of our product candidates, which would seriously harm our business.

In addition, even if we or our collaborators were to obtain approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, may impose significant limitations in the form of narrow indications, warnings, or a REMS. Regulatory authorities may not approve the price we or our collaborators intend to charge for products we may develop, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of the foregoing scenarios could seriously harm our business.

# Even if we or our collaborators obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny.

If one of our product candidates is approved, it will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies, and submission of safety, efficacy, and other post- market information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities.

Manufacturers and manufacturers' facilities are required to comply with extensive FDA and comparable foreign regulatory authority requirements, including ensuring that quality control and manufacturing procedures conform to cGMP regulations. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any approved marketing application. Accordingly, we and others with whom we work must continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, and quality control.

We will have to comply with requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drugs and biologics are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we may not promote our products "off-label" for indications or uses for which they do not have approval, though we may share truthful and not misleading information that is otherwise

consistent with our product's FDA approved labeling. The holder of an approved application must submit new or supplemental applications and obtain approval for certain changes to the approved product, product labeling, or manufacturing process. We could also be asked to conduct post-marketing clinical studies to verify the safety and efficacy of our products in general or in specific patient subsets. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval or label restrictions.

If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing or labeling of a product, such regulatory agency may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If we fail to comply with applicable regulatory requirements, a regulatory agency or enforcement authority may, among other things:

- · issue warning letters;
- · impose civil or criminal penalties;
- · suspend or withdraw regulatory approval;
- · suspend any of our clinical studies;
- refuse to approve pending applications or supplements to approved applications submitted by us;
- · impose restrictions on our operations, including closing our contract manufacturers' facilities; or
- seize or detain products, or require a product recall.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response, and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may adversely affect our ability to commercialize and generate revenue from our products. If regulatory sanctions are applied or if regulatory approval is withdrawn, our business will be seriously harmed.

Moreover, the policies of the FDA and of other regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

Changes in funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also increase the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would seriously harm our

business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could seriously harm our business.

We have received orphan drug designation for 4D-110 for the treatment of Choroideremia, and we may seek orphan drug designation for certain future product candidates, but we may be unable to obtain such designations or to maintain the benefits associated with orphan drug designation, including market exclusivity, which may cause our revenue, if any, to be reduced.

We have received orphan drug designation in the United States for 4D-110 for the treatment of choroideremia. Although we may seek orphan product designation for some or all of our other product candidates, we may never receive such designations. Under the Orphan Drug Act, the FDA may designate a drug or biologic product as an orphan drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. Orphan drug designation must be requested before submitting a BLA. In the European Union, the EMA's Committee for Orphan Medicinal Products (COMP), grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biological product or where there is no satisfactory method of diagnosis, prevention, or treatment, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and application fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA.

In addition, if a product receives the first FDA approval for the indication for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity for the orphan patient population. Exclusive marketing rights in the United States may also be unavailable if we or our collaborators seek approval for an indication broader than the orphan designated indication and may be lost if the FDA later determines that the request for designation was materially defective. In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity following drug or biological product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Even if we obtain orphan drug designation, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products. Further, even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product from competition because different drugs with different active

moieties can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is safer, more effective, or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug or biologic nor gives the drug or biologic any advantage in the regulatory review or approval process.

If the product candidates that we or our collaborators may develop receive regulatory approval in the United States or another jurisdiction, they may never receive approval in other jurisdictions, which would limit market opportunities for such product candidate and seriously harm our business.

Approval of a product candidate in the United States by the FDA or by the requisite regulatory agencies in any other jurisdiction does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions. The approval process varies among countries and may limit our or our collaborators' ability to develop, manufacture, promote and sell product candidates internationally. Failure to obtain marketing approval in international jurisdictions would prevent the product candidates from being marketed outside of the jurisdictions in which regulatory approvals have been received. In order to market and sell product candidates in the European Union and many other jurisdictions, we and our collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and may involve additional preclinical studies or clinical trials both before and after approval. In many countries, any product candidate for human use must be approved for reimbursement before it can be approved for sale in that country. In some cases, the intended price for such product is also subject to approval. Further, while regulatory approval of a product candidate in one country does not ensure approval in any other country, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in others. If we or our collaborators fail to comply with the regulatory requirements in international markets or to obtain all required marketing approvals, the target market for a particular potential product will be reduced, which would limit our ability to realize the full market potential for the product and seriously harm our business.

Enacted and future healthcare legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

In the United States, the European Union and other jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (collectively the ACA) was enacted, which substantially changed the way healthcare is financed by both governmental and private payors. Among the provisions of the ACA, those of greatest importance to the pharmaceutical and biotechnology industries include the following:

- an annual, non-deductible fee payable by any entity that manufactures or imports certain branded prescription drugs and biologic agents (other than those designated as orphan drugs), which is apportioned among these entities according to their market share in certain government healthcare programs;
- new requirements to report certain financial arrangements with physicians and teaching hospitals, including reporting "transfers
  of value" made or distributed to prescribers and other healthcare providers and reporting investment interests held by physicians
  and their immediate family members;

- an increase to the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and an extension the rebate program to individuals enrolled in Medicaid managed care organizations;
- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to
  certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer's
  Medicaid rebate liability;
- a licensure framework for follow-on biologic products;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical
  effectiveness research, along with funding for such research; and
- establishment of a Center for Medicare Innovation at the Centers for Medicare & Medicaid Services (CMS) to test innovative
  payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

Since its enactment, there have been judicial, U.S. Congressional and executive branch challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. For example, the Tax Cuts and Jobs Act of 2017 was enacted, which includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas (Texas District Court Judge) ruled that the individual mandate is a critical and inseverable feature of the ACA, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the ACA are invalid as well. While the Texas District Court Judge, as well as the Trump Administration and CMS, have stated that the ruling will have no immediate effect, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the ACA will impact the ACA and our business.

Since the enactment of the Tax Cuts and Jobs Act of 2017, there have been additional amendments to certain provisions of the ACA, and we expect the current Trump administration and Congress will likely continue to seek to modify, repeal, or otherwise invalidate all, or certain provisions of, the ACA. It is uncertain the extent to which any such changes may harm our business.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, led to aggregate reductions of Medicare payments to providers of 2% per fiscal year. These reductions went into effect in April 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2027 unless additional action is taken by Congress. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several types of providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws or any other similar laws introduced in the future may result in additional reductions in Medicare and other health care funding, which could negatively affect our customers and accordingly, our financial operations.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in

several U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under government payor programs, and review the relationship between pricing and manufacturer patient programs. The Trump administration's budget proposals for fiscal years 2019 and 2020 contains further drug price control measures that could be enacted during the budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Further, the Trump administration released a "Blueprint", or plan, to lower drug prices and reduce out of pocket costs of prescription drugs that contains additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers. The Department of Health and Human Services (HHS) has started soliciting feedback on some of these measures and, at the same time, is implementing others under its existing authority. While some measures will require additional authorization to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

Individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could seriously harm our business. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our product candidates or put pressure on our product pricing. Furthermore, there has been increased interest by third party payors and governmental authorities in reference pricing systems and publication of discounts and list prices. Prescription drugs and biological products that are in violation of these requirements will be included on a public list. These reforms could reduce the ultimate demand for our product candidates or put pressure on our product pricing.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most European Union member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with everincreasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved. In markets outside of the United States and European Union, reimbursement and healthcare payment

systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or judicial action in the United States, the European Union or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

Even if we are able to commercialize any product candidates, due to unfavorable pricing regulations and/or third-party coverage and reimbursement policies, we may not be able to offer such products at competitive prices which would seriously harm our business.

The regulations that govern marketing approvals, pricing and reimbursement for new drugs vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenue we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if any product candidates we may develop obtain marketing approval.

Our ability to successfully commercialize any products that we may develop also will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers, and other organizations. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Government authorities currently impose mandatory discounts for certain patient groups, such as Medicare, Medicaid and Veterans Affairs (VA), hospitals, and may seek to increase such discounts at any time. Future regulation may negatively impact the price of our products, if approved. Increasingly, other third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. For gene therapy and other products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. We cannot be sure that reimbursement will be available for any product candidate that we commercialize and, if reimbursement is available, that the level of reimbursement will be sufficient.

Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. In order to get reimbursement, physicians may need to show that patients have superior treatment outcomes with our products compared to standard of care drugs, including lower-priced generic versions of standard of care drugs. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval. In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors and coverage and reimbursement levels for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time consuming and costly process that may require us to provide scientific and

clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

There may be significant delays in obtaining reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the medicine is approved by the FDA, EMA or other comparable foreign regulatory authorities. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale, and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products we may develop could seriously harm our business.

Even if we obtain FDA approval of any of our product candidates, we may never obtain approval or commercialize such products outside of the United States, which would limit our ability to realize their full market potential.

In order to market any products outside of the United States, we must establish and comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not mean that regulatory approval will be obtained in any other country. Approval procedures vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approvals could result in significant delays, difficulties and costs for us and may require additional preclinical studies or clinical trials which would be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. Satisfying these and other regulatory requirements is costly, time consuming, uncertain and subject to unanticipated delays. In addition, our failure to obtain regulatory approval in any country may delay or have negative effects on the process for regulatory approval in other countries. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, our ability to realize the full market potential of our products will be harmed.

We currently have no sales organization. If we are unable to establish sales capabilities on our own or through third parties, we may not be able to market and sell any products effectively, if approved, or generate product revenue.

We currently do not have a marketing or sales organization. In order to commercialize any product, if approved, in the United States and foreign jurisdictions, we must build our marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services, and we may not be successful in doing so. In advance of any of our product candidates receiving regulatory approval, we expect to establish a sales organization with technical expertise and supporting distribution capabilities to commercialize each such product candidate, which will be expensive and time-consuming. We have no prior experience in the marketing, sale and distribution of pharmaceutical products, and there are significant risks involved in building and

managing a sales organization, including our ability to hire, retain, and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of these products. We may choose to collaborate with third parties that have direct sales forces and established distribution systems, either to augment our own sales force and distribution systems or in lieu of our own sales force and distribution systems. If we are unable to enter into such arrangements on acceptable terms or at all, we may not be able to successfully commercialize our product candidates. If we are not successful in commercializing products, either on our own or through arrangements with one or more third parties, we may not be able to generate any future product revenue and we would incur significant additional losses.

Our business operations and current and future relationships with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers will be subject to applicable healthcare regulatory laws, which could expose us to penalties.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers, may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our product candidates, if approved. Such laws include:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or providing any remuneration (including any kickback, bribe, or certain rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under any U.S. federal healthcare program, such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation:
- the U.S. federal civil and criminal false claims and civil monetary penalties laws, including the civil federal False Claims Act, which can be enforced through civil whistleblower or qui tam actions, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, to the U.S. federal government, claims for payment or approval that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. Pharmaceutical manufacturers can cause false claims to be presented to the U.S. federal government by engaging in impermissible marketing practices, such as the off-label promotion of a product for an indication for which it has not received FDA approval. In addition, the government may assert that a claim including items and services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act;
- The federal Health Insurance Portability and Accountability Act of 1996 (HIPAA), which imposes criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the healthcare fraud statute implemented under HIPAA or specific intent to violate it in order to have committed a violation;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 (HITECH), and its
  implementing regulations, which also imposes certain obligations, including mandatory contractual terms, with respect to
  safeguarding the privacy and security of individually identifiable health information of covered entities subject to the rule, such as
  health plans, healthcare clearinghouses and certain healthcare providers as well as their business associates, independent
  contractors of a covered entity that perform certain services involving the use or disclosure of individually identifiable health
  information on their behalf;
- the FDCA, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;
- the U.S. Public Health Service Act, which prohibits, among other things, the introduction into interstate commerce of a biological product unless a biologics license is in effect for that product;
- the U.S. Physician Payments Sunshine Act and its implementing regulations, which requires certain manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to CMS information related to certain payments and other transfers of value to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;
- analogous U.S. state laws and regulations, including: state anti-kickback and false claims laws, which may apply to our business practices, including but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by any third-party payor, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws and regulations that require drug manufacturers to file reports relating to pricing and marketing information, which requires tracking gifts and other remuneration and items of value provided to healthcare professionals and entities; state and local laws requiring the registration of pharmaceutical sales representatives; and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts; and
- similar healthcare laws and data protection laws in the European Union and other jurisdictions, including reporting requirements detailing interactions with and payments to healthcare providers and laws governing the privacy and security of certain protected information, such as the General Data Protection Regulation (GDPR), which imposes obligations and restrictions on the collection and use of personal data relating to individuals located in the European Economic Area (the EEA) and the United Kingdom (including health data).

We may also be subject to additional federal laws, such as the U.S. Foreign Corrupt Practices Act of 1977, as amended, which prohibits, among other things, U.S. companies and their employees and agents from authorizing, promising, offering, or providing, directly or indirectly, corrupt or improper payments or anything else of value to foreign government officials, employees of public international organizations and foreign government owned or affiliated entities, candidates for foreign political office, and foreign political parties or officials thereof, and federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that

governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, individual imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and imprisonment, which could affect our ability to operate our business. Further, defending against any such actions can be costly, time-consuming and may require significant personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

Our employees, independent contractors, consultants, research or commercial partners or collaborators and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk of fraud, misconduct or other illegal activity by our employees, independent contractors, consultants, research or commercial partners or other collaborators, including the foundations we work with, and vendors. Misconduct by these parties could include intentional, reckless and negligent conduct that fails to: comply with the laws of the FDA, EMA and other comparable foreign regulatory authorities; provide true, complete and accurate information to the FDA, EMA and other comparable foreign regulatory authorities; comply with manufacturing standards we have established; comply with healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws; or report financial information or data accurately or to disclose unauthorized activities to us. If we obtain FDA approval of any of our product candidates and begin commercializing those products in the United States, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. In particular, research, sales, marketing, education and other business arrangements in the healthcare industry are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, educating, marketing and promotion, sales and commission, certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws. In addition, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

If we or any contract manufacturers and suppliers we engage fail to comply with environmental, health, and safety laws and regulations, we could become subject to fines or penalties or incur costs that could seriously harm our business.

We and any contract manufacturers and suppliers we engage are subject to numerous federal, state and local environmental, health, and safety laws, regulations, and permitting requirements,

including those governing laboratory procedures; the generation, handling, use, storage, treatment and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. Our operations also produce hazardous waste. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Under certain environmental laws, we could be held responsible for costs relating to any contamination at our current or past facilities and at third-party facilities. We also could incur significant costs associated with civil or criminal fines and penalties.

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our research, product development and manufacturing efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty, and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended, which could seriously harm our business.

### We use and generate materials that may expose us to material liability.

Our research programs involve the use of hazardous materials and chemicals, which are currently only handled by third parties. We are subject to foreign, federal, state and local environmental and health and safety laws and regulations governing, among other matters, the use, manufacture, handling, storage and disposal of hazardous materials and waste products such as human tissue samples that may have the potential to transmit diseases. We may incur significant costs to comply with these current or future environmental and health and safety laws and regulations. In addition, we cannot completely eliminate the risk of contamination or injury from hazardous materials and may incur material liability as a result of such contamination or injury. In the event of an accident, an injured party may seek to hold us liable for any damages that result. Any liability could exceed the limits or fall outside the coverage of our workers' compensation, property and business interruption insurance and we may not be able to maintain insurance on acceptable terms, if at all. We currently carry no insurance specifically covering environmental claims.

Compliance with governmental regulations regarding the treatment of animals used in research could increase our operating costs, which would adversely affect the commercialization of our products.

The Animal Welfare Act (AWA) is the federal law that covers the treatment of certain animals used in research. Currently, the AWA imposes a wide variety of specific regulations that govern the humane handling, care, treatment and transportation of certain animals by producers and users of research animals, most notably relating to personnel, facilities, sanitation, cage size, and feeding, watering and shipping conditions. Third parties with whom we contract are subject to registration, inspections and reporting requirements under the AWA. Furthermore, some states have their own regulations, including general anti-cruelty legislation, which establish certain standards in handling animals. Comparable rules, regulations, and or obligations exist in many foreign jurisdictions. If we or

our contractors fail to comply with regulations concerning the treatment of animals used in research, we may be subject to fines and penalties and adverse publicity, and our operations could be adversely affected.

### Risks Related to Our Reliance on Third Parties

We expect to rely on third parties to conduct our clinical trials and some aspects of our research and preclinical testing, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research or testing.

We currently rely and expect to continue to rely on third parties, such as CROs, clinical data management organizations, clinical data assessments and analysis organizations, medical institutions and clinical investigators, to conduct some aspects of our research, preclinical testing and clinical trials. Any of these third parties may terminate their engagements with us or be unable to fulfill their contractual obligations. If we need to enter into alternative arrangements, it would delay our product development activities.

Our reliance on these third parties for research and development activities reduces our control over these activities, but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with GCPs for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible, reproducible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We are also required to register ongoing clinical trials and to post the results of completed clinical trials on a government-sponsored database within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for any product candidates we may develop and will not be able to, or may be delayed in our efforts to, successfully commercialize our medicines.

We also expect to rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of any product candidates we may develop or commercialization of our product candidates, producing additional losses and depriving us of potential product revenue.

Reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Reliance on third parties to conduct clinical trials, assist in research and development and to manufacture our product candidates, will at times require us to share trade secrets with them. We seek to protect our proprietary technology by in part entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements.

Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's independent discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may seriously harm our business.

We may depend on collaborations with third parties for the research, development and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to realize the market potential of those product candidates.

We have sought, and may in the future seek third-party collaborators for the research, development and commercialization of certain of the product candidates we may develop. Our likely collaborators for any other collaboration arrangements include large and mid-size pharmaceutical companies, regional, national and international pharmaceutical companies, biotechnology companies and academic institutions. If we enter into any such arrangements with any third parties, we will likely have shared or limited control over the amount and timing of resources that our collaborators dedicate to the development or potential commercialization of any product candidates we may seek to develop with them. Our ability to generate revenue from these arrangements with commercial entities will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of any collaboration that we enter into.

Collaborations involving our product candidates we may develop, pose the following risks to us:

- collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not properly obtain, maintain, enforce or defend intellectual property or proprietary rights relating to our
  product candidates or may use our proprietary information in such a way as to expose us to potential litigation or other
  intellectual property related proceedings, including proceedings challenging the scope, ownership, validity and enforceability of
  our intellectual property;
- collaborators may own or co-own intellectual property covering our product candidates that result from our collaboration with them, and in such cases, we may not have the exclusive right to commercialize such intellectual property or such product candidates:
- disputes may arise with respect to the ownership of intellectual property developed pursuant to collaborations;
- we may need the cooperation of our collaborators to enforce or defend any intellectual property we contribute to or that arises out of our collaborations, which may not be provided to us;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our product candidates or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborators may decide not to pursue development and commercialization of any product candidates we develop or may elect
  not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's
  strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing
  priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials, or require a new formulation of a product candidate for clinical testing;

- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- collaborators with marketing and distribution rights to one or more product candidates may not commit sufficient resources to the marketing and distribution of such product candidates;
- we may lose certain valuable rights under circumstances identified in our collaborations, including if we undergo a change of control;
- collaborators may undergo a change of control and the new owners may decide to take the collaboration in a direction which is not in our best interest;
- collaborators may become party to a business combination transaction and the continued pursuit and emphasis on our development or commercialization program by the resulting entity under our existing collaboration could be delayed, diminished or terminated:
- collaborators may become bankrupt, which may significantly delay our research or development programs, or may cause us to
  lose access to valuable technology, devices, materials, know-how or intellectual property of the collaborator relating to our
  products, product candidates;
- key personnel at our collaborators may leave, which could negatively impact our ability to productively work with our collaborators;
- collaborations may require us to incur short and long-term expenditures, issue securities that dilute our stockholders, or disrupt our management and business;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates or our Therapeutic Vector Evolution platform technology; and
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all.

We may face significant competition in seeking appropriate collaborations. Recent business combinations among biotechnology and pharmaceutical companies have resulted in a reduced number of potential collaborators. In addition, the negotiation process is time-consuming and complex, and we may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate or delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop product candidates or bring them to market and generate product revenue.

If we enter into collaborations to develop and potentially commercialize any product candidates, we may not be able to realize the benefit of such transactions if we or our collaborator elect not to exercise the rights granted under the agreement or if we or our collaborator are unable to successfully integrate a product candidate into existing operations and company culture. In addition, if our agreement with any of our collaborators terminates, our access to technology and intellectual property licensed to us by that collaborator may be restricted or terminate entirely, which may delay our continued development of our product candidates utilizing the collaborator's technology or intellectual property or require us to stop development of those product candidates completely. We may also find it

more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. Any collaborator may also be subject to many of the risks relating to product development, regulatory approval, and commercialization described in this "Risk Factors" section, and any negative impact on our collaborators may adversely affect us.

### **Risks Related to Our Intellectual Property**

### Our success depends on our ability to protect our intellectual property and our proprietary technologies.

Our commercial success depends in part on our ability to obtain and maintain patent protection and trade secret protection for our product candidates, proprietary technologies and their uses as well as our ability to operate without infringing upon the proprietary rights of others. We generally seek to protect our proprietary position by filing patent applications in the United States and abroad related to our product candidates, proprietary technologies and their uses that are important to our business. Our patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless, and until, patents issues from such applications, and then only to the extent the issued claims cover the technology. There can be no assurance that our patent applications or those of our licensors will result in additional patents being issued or that issued patents will afford sufficient protection against competitors with similar technology, nor can there be any assurance that the patents issued will not be infringed, designed around or invalidated by third parties. Even issued patents may later be found invalid or unenforceable or may be modified or revoked in proceedings instituted by third parties before various patent offices or in courts. The degree of future protection for our proprietary rights is uncertain. Only limited protection may be available and may not adequately protect our rights or permit us to gain or keep any competitive advantage. If we do not adequately protect our intellectual property and proprietary technology, competitors may be able to use our product candidates and proprietary technologies and erode or negate any competitive advantage we may have, which could seriously harm our business.

We and our licensors have applied, and we intend to continue applying, for patents covering aspects of our product candidates, proprietary technologies and their uses that we deem appropriate. However, we may not be able to apply for patents on certain aspects of our current or future product candidates, proprietary technologies and their uses in a timely fashion, at a reasonable cost, in all jurisdictions, or at all, and any potential patent coverage we obtain may not be sufficient to prevent substantial competition.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our actual or potential future collaborators or licensors will be successful in protecting our product candidates, proprietary technologies and their uses by obtaining and defending patents. These risks and uncertainties include the following:

- the U.S. Patent and Trademark Office (USPTO) and various foreign governmental patent agencies require compliance with a
  number of procedural, documentary, fee payment and other provisions during the patent process, the noncompliance with which
  can result in abandonment or lapse of a patent or patent application, and partial or complete loss of patent rights in the relevant
  jurisdiction;
- patent applications may not result in any patents being issued;
- patents that may be issued or in-licensed may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;

- our competitors, many of whom have substantially greater resources than we do and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use and sell our potential product candidates;
- other parties may have designed around our claims or developed technologies that may be related or competitive to our
  platform, may have filed or may file patent applications and may have received or may receive patents that overlap or conflict
  with our patent applications, either by claiming the same methods or devices or by claiming subject matter that could dominate
  our patent position;
- any successful opposition to any patents owned by or licensed to us could deprive us of rights necessary for the practice of our technologies or the successful commercialization of any products or product candidates that we may develop;
- because patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we or our licensors were the first to file any patent application related to our product candidates, proprietary technologies and their uses;
- an interference proceeding can be provoked by a third party or instituted by the USPTO to determine who was the first to invent
  any of the subject matter covered by the patent claims of our applications for any application with an effective filing date before
  March 16, 2013;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent
  protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy
  regarding worldwide health concerns; and
- countries other than the United States may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing product candidates.

The patent prosecution process is also expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants and other third parties, any of these parties may breach such agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection.

The patent position of biopharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has been the subject of much litigation in recent years. Moreover, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents, if issued, or the patent rights that we license from others, may be challenged in the courts or patent offices in the United States and abroad. Once granted, patents may remain open to opposition, interference, re-examination, post-grant review, *inter partes* review, nullification or derivation action in court or before patent offices or similar proceedings for a given period after allowance or grant, during which time third parties can raise objections against such initial grant. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical products or product candidates, or limit the duration of the patent protection of our product candidates. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such

candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Our ability to enforce our patent rights depends on our ability to detect infringement. It may be difficult to detect infringers who do not advertise the components or methods that are used in connection with their products and services. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor's or potential competitor's product or service. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded if we were to prevail may not be commercially meaningful.

In addition, proceedings to enforce or defend our patents could put our patents at risk of being invalidated, held unenforceable or interpreted narrowly. Such proceedings could also provoke third parties to assert claims against us, including that some or all of the claims in one or more of our patents are invalid or otherwise unenforceable. If any of our patents covering our product candidates are invalidated or found unenforceable, or if a court found that valid, enforceable patents held by third parties covered one or more of our product candidates, our competitive position could be harmed or we could be required to incur significant expenses to enforce or defend our rights. If we initiate lawsuits to protect or enforce our patents, or litigate against third party claims, such proceedings would be expensive and would divert the attention of our management and technical personnel.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

- any of our patents, or any of our pending patent applications, if issued, or those of our licensors, will include claims having a scope sufficient to protect our product candidates;
- any of our pending patent applications or those of our licensors may issue as patents;
- others will not or may not be able to make, use, offer to sell, or sell products that are the same as or similar to our own but that are not covered by the claims of the patents that we own or license;
- we will be able to successfully commercialize our product candidates on a substantial scale, if approved, before the relevant
  patents that we own or license expire;
- we were the first to make the inventions covered by each of the patents and pending patent applications that we own or license;
- we or our licensors were the first to file patent applications for these inventions;
- others will not develop similar or alternative technologies that do not infringe the patents we own or license;
- any of the patents we own or license will be found to ultimately be valid and enforceable;
- any patents issued to us or our licensors will provide a basis for an exclusive market for our commercially viable products or will
  provide us with any competitive advantages;
- a third party may not challenge the patents we own or license and, if challenged, a court would hold that such patents are valid, enforceable and infringed;
- we may develop or in-license additional proprietary technologies that are patentable;
- · the patents of others will not have an adverse effect on our business;
- our competitors do not conduct research and development activities in countries where we do not have enforceable patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets:

- · we will develop additional proprietary technologies or product candidates that are separately patentable; or
- our commercial activities or products will not infringe upon the patents of others.

Where we obtain licenses from or collaborate with third parties, in some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties, or such activities, if controlled by us, may require the input of such third parties. We may also require the cooperation of our licensors and collaborators to enforce any licensed patent rights, and such cooperation may not be provided. Therefore, these patents and patent applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. Moreover, if we do obtain necessary licenses, we will likely have obligations under those licenses, and any failure to satisfy those obligations could give our licensor the right to terminate the license. Termination of a necessary license, or expiration of licensed patents or patent applications, could seriously harm our business.

Furthermore, our owned and in-licensed intellectual property rights may be subject to a reservation of rights by one or more third parties. For example, the research resulting in certain of our in-licensed patent rights and technology was funded in part by the U.S. government. As a result, the government may have certain rights, or march-in rights, to such patent rights and technology. When new technologies are developed with government funding, the government generally obtains certain rights in any resulting patents, including a non-exclusive license authorizing the government to use the invention for non-commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. The government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government of such rights could harm our business.

### The lives of our patents may not be sufficient to effectively protect our product candidates and business.

Patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after its first effective non-provisional filing date. Although various extensions may be available, the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates, proprietary technologies and their uses are obtained, once the patent life has expired, we may be open to competition. In addition, although upon issuance in the United States a patent's life can be extended based on certain delays caused by the USPTO and clinical development, this extension can be reduced or eliminated based on certain delays caused by the patent applicant during patent prosecution. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. If we do not have sufficient patent life to protect our product candidates, proprietary technologies and their uses, our business and results of operations will be adversely affected.

## If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

We rely on the protection of our trade secrets, including unpatented know-how, technology and other proprietary information to maintain our competitive position. We have taken steps to protect our

trade secrets and unpatented know-how, including entering into confidentiality agreements with third parties, and confidential information and inventions agreements with employees and consultants. In addition to contractual measures, we try to protect the confidential nature of our proprietary information using commonly accepted physical and technological security measures. Despite these efforts, we cannot provide any assurances that all such agreements have been duly executed, and any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. In addition, such security measures may not provide adequate protection for our proprietary information, for example, in the case of misappropriation of a trade secret by an employee, consultant, customer or third party with authorized access. Our security measures may not prevent an employee, consultant, collaborator or customer from misappropriating our trade secrets and providing them to a competitor, and recourse we take against such misconduct may not provide an adequate remedy to protect our interests fully. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. Unauthorized parties may also attempt to copy or reverse engineer certain aspects of our product candidates that we consider proprietary. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret can be difficult, expensive and time-consuming, and the outcome is unpredictable. Even though we use commonly accepted security measures, the criteria for protection of trade secrets can vary among different jurisdictions.

In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. Moreover, third parties may still obtain this information or may come upon this or similar information independently, and we would have no right to prevent them from using that technology or information to compete with us. Trade secrets will over time be disseminated within the industry through independent development, the publication of journal articles and the movement of personnel skilled in the art from company to company or academic to industry scientific positions. Though our agreements with third parties typically restrict the ability of our employees, collaborators, licensors, third-party contractors and consultants to publish data potentially relating to our trade secrets, our agreements may contain certain limited publication rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position. Because from time to time we have relied on, and in future expect to rely on third parties in the development, manufacture, and distribution of our product candidates and provision of our services, we must, at times, share trade secrets with them. Despite employing the contractual and other security precautions described above, the need to share trade secrets increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. If any of these events occurs or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced and our competitive position would be harmed. If we do not apply for patent protection prior to such publication or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

Our rights to develop and commercialize our product candidates are subject in part to the terms and conditions of licenses granted to us by others, and the patent protection, prosecution and enforcement for some of our product candidates may be dependent on our licensors.

We currently are reliant upon licenses of certain patent rights and proprietary technology from third parties that is important or necessary to the development of our technology, including technology related to our product candidates. For example, we rely on our exclusive license agreements with The Regents of the University of California (the UC Regents) for all of our rights with respect to the

intellectual property covering certain compositions of matter and methods of use of certain AAV variants related to our research candidates in lead optimization stage. These and other licenses we may enter into in the future may not provide adequate rights to use such intellectual property and technology in all relevant fields of use or in all territories in which we may wish to develop or commercialize our technology and product candidates in the future. As a result, we may not be able to develop and commercialize our technology and product candidates in fields of use and territories for which we are not granted rights pursuant to such licenses.

Licenses to additional third party technology that may be required for our development programs may not be available in the future or may not be available on commercially reasonable terms, which could seriously harm our business.

In some circumstances, we may not have the right to control the preparation, filing, prosecution and enforcement of patent applications, or to maintain the patents, covering technology that we license from third parties. In addition, some of our agreements with our licensors require us to obtain consent from the licensor before we can enforce patent rights, and our licensor may withhold such consent or may not provide it on a timely basis. Therefore, we cannot be certain that our licensors or collaborators will prosecute, maintain, enforce and defend such intellectual property rights in a manner consistent with the best interests of our business, including by taking reasonable measures to protect the confidentiality of know-how and trade secrets, or by paying all applicable prosecution and maintenance fees related to intellectual property registrations for any of our product candidates. We also cannot be certain that our licensors have drafted or prosecuted the patents and patent applications licensed to us in compliance with applicable laws and regulations, which may affect the validity and enforceability of such patents or any patents that may issue from such applications. If they fail to do so, this could cause us to lose rights in any applicable intellectual property that we in-license, and as a result our ability to develop and commercialize products or product candidates may be adversely affected and we may be unable to prevent competitors from making, using and selling competing products.

Our current licenses, and our future licenses likely will, impose various royalty payments, milestones, and other obligations on us. If we fail to comply with any of these obligations, we may be required to pay damages and the licensor may have the right to terminate the license. Termination by the licensor would cause us to lose valuable rights, and could prevent us from developing and commercializing our product candidates and proprietary technologies. Our business would suffer if any current or future licenses terminate, if the licensors fail to abide by the terms of the license, if the licensors fail to enforce licensed patents against infringing third parties, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. Furthermore, if any current or future licenses terminate, or if the underlying patents fail to provide the intended exclusivity, competitors or other third parties may gain the freedom to seek regulatory approval of, and to market, products identical to ours. Moreover, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights. In addition, while we cannot currently determine the amount of royalty obligations we would be required to pay on the sales of future products, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in product candidates that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize any product candidates, we may be unable to achieve or maintain profitability.

We may fail to comply with any of our obligations under existing or future agreements pursuant to which we license or have otherwise acquired intellectual property rights or technology, which could result in the loss of rights or technology that are material to our business.

We are party to various agreements that we depend on to operate our business. Our rights to use currently licensed intellectual property, or intellectual property to be licensed in the future, are or will be

subject to the continuation of and our compliance with the terms of these agreements. These agreements are complex, and certain provisions in such agreements may be susceptible to multiple interpretations which could lead to disputes, including but not limited to those regarding:

- · the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our proprietary technology and product candidates infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- · the sublicensing of patent and other rights;
- · diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the creation or use of intellectual property by us or our counterparties, alone or jointly;
- · the scope and duration of our payment obligations;
- · rights upon termination of such agreement; and
- · the scope and duration of exclusivity obligations of each party to the agreement.

The resolution of any contractual interpretation dispute that may arise, if unfavorable to us, could seriously harm our business. Such resolution could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, increase what we believe to be our financial or other obligations under the relevant agreement, or decrease the third party's financial or other obligations under the relevant agreement.

If disputes over intellectual property rights that we have licensed or acquired from third parties prevent or impair our ability to maintain our current license agreements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. If we fail to comply with our obligations under current or future license agreements, these agreements may be terminated or the scope of our rights under them may be reduced and we might be unable to develop, manufacture or market any product that is licensed under these agreements.

# Litigation or other proceedings or third party claims of intellectual property infringement could require us to spend significant time and money and could prevent us from selling our products.

Our commercial success depends significantly on our ability to operate without infringing the patents and other proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Claims by third parties that we infringe their proprietary rights may result in liability for damages or prevent or delay our developmental and commercialization efforts. We cannot assure you that our operations do not, or will not in the future, infringe existing or future patents or other proprietary rights of third parties.

Third parties may have or obtain patents or other proprietary rights that could limit our ability to make, use, sell, offer for sale or import our product candidates and future approved products, if any, or impair our competitive position. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions, reexaminations, *inter partes* review proceedings and post-grant review proceedings before the USPTO and/or corresponding foreign patent offices. Numerous third party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing product candidates. There may be third party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates.

Furthermore, the scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history and can involve other factors such as expert opinion. Our interpretation of the relevance or the scope of claims in a patent or a pending application may be incorrect, which may negatively impact our ability to market our products. Further, we may incorrectly determine that our technologies or product candidates are not covered by a third party patent or may incorrectly predict whether a third party's pending patent application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our product candidates.

As the biotechnology industry expands and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties. Our competitors in both the United States and abroad, many of which have substantially greater resources and have made substantial investments in patent portfolios and competing technologies, may have applied for or obtained or may in the future apply for and obtain, patents that will prevent, limit or otherwise interfere with our ability to make, use and sell our product candidates. We do not always conduct independent reviews of pending patent applications of and patents issued to third parties. Patent applications in the United States and elsewhere are typically published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Certain U.S. applications that will not be filed outside the United States can remain confidential until patents issue. In addition, patent applications in the United States and elsewhere can be pending for many years before issuance, or unintentionally abandoned patents or applications can be revived. Furthermore, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, our product candidates or the use of our product candidates. As such, there may be applications of others now pending or recently revived patents of which we are unaware. These applications may later result in issued patents, or the revival of previously abandoned patents, that will prevent, limit or otherwise interfere with our ability to make, use or sell our product candidates. As a result, we may be unaware of third party patents that may be infringed by commercialization of our product candidates, and cannot be certain that we were the first to file a patent application related to a product candidate or technology. Moreover, because patent applications can take many years to issue, there may be currently-pending patent applications that may later result in issued patents that our product candidates may infringe. In addition, identification of third party patent rights that may be relevant to our technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. Any claims of patent infringement asserted by third parties would be time consuming and could:

- · result in costly litigation;
- · divert the time and attention of our technical personnel and management;
- · cause development delays;
- prevent us from commercializing our product candidates until the asserted patent expires or is held finally invalid or not infringed in a court of law;
- require us to develop non-infringing technology, which may not be possible on a cost-effective basis;
- require us to pay damages to the party whose intellectual property rights we may be found to be infringing, which may include treble damages if we are found to have been willfully infringing such intellectual property;
- require us to pay the attorney's fees and costs of litigation to the party whose intellectual property rights we may be found to be infringing; and/or

require us to enter into royalty or licensing agreements, which may not be available on commercially reasonable terms, or at all.

Although no third party has asserted a claim of patent infringement against us as of the date of this prospectus, others may hold proprietary rights that could prevent our product candidates or any future products from being marketed. Any patent-related legal action against us claiming damages and seeking to enjoin commercial activities relating to our product candidates or proprietary technologies could subject us to potential liability for damages, including treble damages if we were determined to willfully infringe, and require us to obtain a license to manufacture or market our product candidates or any future products. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be time-consuming and a substantial diversion of management and employee resources from our business. We cannot predict whether we would prevail in any such actions or that any license required under any of these patents would be made available on commercially acceptable terms, if at all. Even if such licenses are available, we could incur substantial costs related to royalty payments for licenses obtained from third parties, which could negatively affect our gross margins, and the rights may be non-exclusive, which could give our competitors access to the same technology or intellectual property rights licensed to us. In addition, we cannot be certain that we could redesign our product candidates or proprietary technologies to avoid infringement, if necessary, or on a cost-effective basis. Accordingly, an adverse determination in a judicial or administrative proceeding, or the failure to obtain necessary licenses, could prevent us from developing and commercializing our product candidates or any future products which could seriously harm our business. In addition, intellectual property litigation, regardless of its outcome, may cause negative publicity and could prohibit us from marketing or otherwise commercializing our product candidates and technology.

If we collaborate with third parties in the development of technology in the future, our collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to litigation or potential liability. Further, collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability. Also, we may be obligated under our agreements with our collaborators, licensors, suppliers and others to indemnify and hold them harmless for damages arising from intellectual property infringement by us.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time consuming, and unsuccessful. Further, our issued patents could be found invalid or unenforceable if challenged.

Competitors may infringe our intellectual property rights or those of our licensors. To prevent infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in a patent infringement proceeding, a court or administrative tribunal may decide that a patent we own or license is not valid, is unenforceable and/or is not infringed. If we or any of our potential future collaborators were to initiate legal proceedings against a third party to enforce a patent directed at one of our product candidates, the defendant could counterclaim that our patent is invalid and/or unenforceable in whole or in part. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge include an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could include an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution. Third parties may also raise similar claims before the USPTO, even outside the context of litigation. Similar mechanisms for challenging the validity and enforceability of a patent exist in foreign

patent offices and may result in the revocation, cancellation, or amendment of any foreign patents we hold in the future. The outcome following legal assertions of invalidity and unenforceability is unpredictable, and prior art could render our patents or those of our licensors invalid. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we could lose at least part, and perhaps all, of the patent protection on an affected product candidate. Such a loss of patent protection would seriously harm our business.

Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO, or equivalent actions brought in foreign jurisdictions, may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be seriously harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties or enter into development or manufacturing partnerships that would help us bring our product candidates to market.

Even if resolved in our favor, litigation or other legal proceedings relating to our intellectual property rights may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

# We may not be successful in obtaining or maintaining necessary rights to our product candidates through acquisitions and licenses.

We currently have rights to the intellectual property, through licenses from third parties and under patents that we own, to develop our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business will depend in part on our ability to acquire, license or use these proprietary rights. We may be unable to acquire or license any compositions, methods of use, processes or other third party intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing and acquisition of third party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to

their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We have collaborated with, and are currently collaborating with, U.S. academic institutions and may in the future collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. These institutions may provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of that program and our business could be seriously harmed.

We may be subject to claims that we have wrongfully hired an employee from a competitor or that we, our employees or consultants have wrongfully used or disclosed alleged confidential information or trade secrets of their former or concurrent employers or former or current clients.

As is common in the biotechnology and biopharmaceutical industries, in addition to our employees, we engage the services of consultants to assist us in the development of our product candidates. Many of these consultants, and many of our employees, were previously employed at, or may have previously provided or may be currently providing consulting services to, other biotechnology or biopharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees and consultants do not use the proprietary information or know-how of others in their work for us, we may become subject to claims that we, our employees or a consultant inadvertently or otherwise used or disclosed trade secrets or other information proprietary to their former employers or their former or current clients. We may also be subject to claims that patents and applications we have filed to protect inventions of our employees or consultants, even those related to one or more of our product candidates, are rightfully owned by their former or concurrent employer or former or current client. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, which could seriously harm our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management and other employees.

## We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

Our agreements with employees and consultants provide that any inventions conceived by an individual in the course of rendering services to us shall be our exclusive property. Although our policy is to have all such individuals complete these agreements, we may not obtain these agreements in all circumstances, and individuals with whom we have these agreements may not comply with their terms. The assignment of intellectual property may not be self-executing and despite such agreement, such inventions may become assigned to third parties. In the event of unauthorized use or disclosure of our trade secrets or proprietary information, these agreements, even if obtained, may not provide meaningful protection, particularly for our trade secrets or other confidential information. We may also be subject to claims that former employees, consultants, or other third parties have an ownership

interest in our patents or other intellectual property. In addition, we may face claims by third parties that our agreements with employees or consultants obligating them to assign intellectual property to us are ineffective or in conflict with prior or competing contractual obligations of assignment, which could result in ownership disputes regarding intellectual property we have developed or will develop and interfere with our ability to capture the commercial value of such intellectual property. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Such an outcome could seriously harm our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and distraction to our management and other employees.

### If we do not obtain patent term extension for our product candidates, our business may be seriously harmed.

Depending upon the timing, duration and specifics of FDA marketing approval of any of our product candidates, one or more of our U.S. patents may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, also known as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. The Hatch-Waxman Act allows a maximum of one patent to be extended per FDA approved product as compensation for the patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. Patent term extension may also be available in certain foreign countries upon regulatory approval of our product candidates. However, we may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration of the applicable product, and our business may be seriously harmed.

If our trademarks and trade names, whether registered in the future or unregistered now, are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Any trademarks we may register in the future or any current unregistered trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. We may license our trademarks and trade names, to the extent any are registered, to third parties, such as distributors. Though these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names. Our efforts to enforce or protect our proprietary rights related to trademarks, trade names, trade secrets, domain names, copyrights or other intellectual property may

be ineffective and could result in substantial costs and diversion of resources and could seriously harm our business.

Changes in patent law in the United States or in other countries could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

Our patent rights may be affected by developments or uncertainty in the U.S. or foreign patent statutes, patent case laws, USPTO rules and regulations or in the rules and regulations of foreign patent offices.

There are a number of recent changes to the U.S. patent laws that may have a significant impact on our ability to protect our technology and enforce our intellectual property rights. For example, on September 16, 2011, the Leahy-Smith America Invents Act (Leahy-Smith Act) was signed into law. The Leahy-Smith Act includes a number of significant changes to the U.S. patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. In particular, under the Leahy-Smith Act, the United States transitioned in March 2013 to a "first to file" system in which the first inventor to file a patent application will be entitled to the patent. Third parties are allowed to submit prior art before the issuance of a patent by the USPTO, and may become involved in post-grant proceedings including opposition, derivation, reexamination, inter partes review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope or enforceability of, or invalidate, our patent rights, which could adversely affect our competitive position. This could have a negative impact on some of our intellectual property and could increase uncertainties surrounding obtaining and enforcement or defense of our issued patents. In addition, Congress may pass patent reform legislation that is unfavorable to us. The Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents we might obtain in the future. Similarly, statutory or judicial changes to the patent laws of other countries may increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. Complying with these laws and regulations could limit our ability to obtain new patents in the future that may be important for our business.

### We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending all current and future patents in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but enforcement is not as strong as that in the United States. These products may compete with our product candidates, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

The legal systems of many foreign countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights. For example, some foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. In addition, some countries limit the enforceability of patents against third parties, including government agencies or government contractors. In these countries, patents may provide limited or no benefit. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our business may be seriously harmed.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the USPTO and various governmental patent agencies outside of the US in several stages over the lifetime of the patents and/or applications. We employ reputable professionals and rely on such third parties to help us comply with these requirements and effect payment of these fees with respect to the patents and patent applications that we own, and if we license intellectual property we may have to rely upon our licensors to comply with these requirements and effect payment of these fees with respect to any patents and patent applications that we license. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case.

## Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make gene therapy products that are similar to our product candidates or utilize similar gene therapy technology but that are not covered by the claims of the patents that we own or have exclusively licensed;
- we or our licensors or future collaborators might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or have exclusively licensed;
- we or our licensors or future collaborators might not have been the first to file patent applications covering certain of our inventions;

- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or have exclusively licensed may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- · we may not develop additional proprietary technologies that are patentable; and
- · the patents of others may have an adverse effect on our business.

Should any of these events occur, they could seriously harm our business.

# Any collaboration arrangements that we may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our products.

Any future collaborations that we enter into may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborations are subject to numerous risks, which may include that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or
  renew development or commercialization programs based on trial or test results, changes in their strategic focus due to the
  acquisition of competitive products, availability of funding or other external factors, such as a business combination that diverts
  resources or creates competing priorities;
- collaborators could independently develop, or develop with third parties, products and product candidates that compete directly or indirectly with our product candidates;
- a collaborator with marketing, manufacturing and distribution rights to one or more products and product candidates may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or
  proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual
  property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that causes the delay or termination of the research, development or commercialization of our current or future product candidates or that results in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated, and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable current or future product candidates;
- collaborators may own or co-own intellectual property covering our product candidates that results from our collaborating with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property; and

a collaborator's sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

### **Risks Related to Our Operations**

We are highly dependent on our key personnel, and if we are not successful in attracting, motivating and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract, motivate and retain highly qualified managerial, scientific and medical personnel. The loss of the services provided by any of our executive officers, other key employees, and other scientific and medical advisors, and our inability to find suitable replacements, could result in delays in the development of our product candidates and harm our business.

We conduct our operations at our facilities in Emeryville, California, in a region that is headquarters to many other biopharmaceutical companies and many academic and research institutions. Competition for skilled personnel is intense and the turnover rate can be high, which may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all. We expect that we may need to recruit talent from outside of our region, and doing so may be costly and difficult.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided restricted stock and stock option grants. The value to employees of these equity grants that vest over time may be significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies. In addition, our employees are employed at-will, which means that any of our employees could leave our employment at any time, with or without notice. If we are unable to attract, incentivize and retain quality personnel on acceptable terms, or at all, it may seriously harm our business.

### We will need to grow the size and capabilities of our organization, and we may experience difficulties in managing this growth.

As of June 30, 2019, we had 57 full-time employees. As our development plans and strategies develop, and as we transition into operating as a public company, we must add a significant number of additional managerial, operational, financial and other personnel. Future growth will impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, retaining and motivating additional employees;
- managing our internal development efforts effectively, including the clinical and FDA review process for our current and future product candidates, while complying with our contractual obligations to contractors and other third parties;
- expanding our operational, financial and management controls, reporting systems and procedures; and
- managing increasing operational and managerial complexity.

Our future financial performance and our ability to continue to develop and, if approved, commercialize our product candidates will depend, in part, on our ability to effectively manage any future growth. Our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to manage these growth activities.

We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services. There can be no

assurance that the services of these independent organizations, advisors and consultants will continue to be available to us on a timely basis when needed, or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed, or terminated, and we may not be able to obtain regulatory approval of our product candidates or otherwise advance our business. There can be no assurance that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, if at all.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop our product candidates and, accordingly, may not achieve our research, development and commercialization goals.

## Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. Portions of our future clinical trials may be conducted outside of the United States and unfavorable economic conditions resulting in the weakening of the U.S. dollar would make those clinical trials more costly to operate. Furthermore, the most recent global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn could result in a variety of risks to our business, including a reduced ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy or international trade disputes could also strain our suppliers, some of which are located outside of the United States, possibly resulting in supply disruption. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

The estimates of market opportunity and forecasts of market growth included in this prospectus may prove to be inaccurate, and even if the markets in which we compete achieve the forecasted growth, our business may not grow at similar rates, or at all.

Market opportunity estimates and growth forecasts included in this prospectus are subject to significant uncertainty and are based on assumptions and estimates which may not prove to be accurate. The estimates and forecasts included in this prospectus relating to size and expected growth of our target market may prove to be inaccurate. Even if the markets in which we compete meet the size estimates and growth forecasts included in this prospectus, our business may not grow at similar rates, or at all. Our growth is subject to many factors, including our success in implementing our business strategy, which is subject to many risks and uncertainties.

If we engage in acquisitions or strategic partnerships, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

We may engage in various acquisitions and strategic partnerships in the future, including licensing or acquiring complementary products, intellectual property rights, technologies or businesses. Any acquisition or strategic partnership may entail numerous risks, including:

- · increased operating expenses and cash requirements;
- · the assumption of indebtedness or contingent liabilities;
- · the issuance of our equity securities which would result in dilution to our stockholders;

- assimilation of operations, intellectual property, products and product candidates of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing product candidates and initiatives in pursuing such an acquisition or strategic partnership;
- retention of key employees, the loss of key personnel, and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their
  existing products or product candidates and regulatory approvals; and
- our inability to generate revenue from acquired intellectual property, technology and/or products sufficient to meet our objectives or even to offset the associated transaction and maintenance costs.

In addition, if we undertake such a transaction, we may incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense.

Our information technology systems, or those used by our third-party research institution collaborators, CROs or other contractors or consultants, may fail or suffer security breaches and other disruptions.

In September 2019, we experienced a phishing attack in which the email account of a single non-officer employee was compromised. Our security controls detected the compromise, and we were able to block the unauthorized access, but not before the attacker was able to use the account to send out additional phishing emails. We do not believe the phishing attack was a material incursion because, among other reasons, none of our data was accessed or compromised, and we have not incurred any related material remediation costs. Despite the implementation of security measures like those that detected the phishing attack, our internal information technology systems and those of our collaborators, future CROs and other contractors and consultants may be vulnerable to damage from computer viruses, natural disasters, terrorism, war, telecommunication and electrical failures, hacking, cyberattacks, phishing attacks and other social engineering schemes, denial or degradation of service attacks, unauthorized access or use, persons inside our organization, or persons with access to systems inside our organization. The risk of a security breach or disruption, particularly through cyberattacks or cyber intrusion, including by computer hackers, foreign governments and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. The costs to us to investigate and mitigate network security problems, bugs, viruses, worms, malicious software programs and security vulnerabilities could be significant, and while we have implemented security measures to protect our data security and information technology systems, our efforts to address these problems may not be successful, and these problems could result in unexpected interruptions, delays, cessation of service, negative publicity and other harm to our business and our competitive position. Although to our knowledge we have not experienced any such material system failure or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss, corruption or unauthorized disclosure of our trade secrets, personal data or other proprietary or sensitive information or other similar disruptions.

For example, the loss of clinical trial data from completed, ongoing or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. If a security breach or other incident were to result in the unauthorized access to or unauthorized use, disclosure, release or other processing of clinical trial data or personal data, it may necessary to notify individuals, governmental authorities, supervisory bodies, the media, and other parties pursuant to privacy and security laws. Likewise, we rely on our third-party research institution

collaborators for research and development of our product candidates and other third parties for the manufacture of our product candidates and to conduct clinical trials, and similar events relating to their information technology systems could also seriously harm our business. Any security compromise affecting us, our partners or our industry, whether real or perceived, could harm our reputation, erode confidence in the effectiveness of our security measures, and lead to regulatory scrutiny. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or systems, or inappropriate disclosure of confidential or proprietary or personal information, we could incur liability, our competitive position could be harmed, and the further development and commercialization of our product candidates could be delayed.

### Business disruptions could seriously harm our business.

Our operations, and those of our CROs, CMOs, suppliers, and other contractors and consultants, could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for which we are partly uninsured. In addition, we rely on our third-party research institution collaborators for conducting research and development of our product candidates, and they may be affected by government shutdowns or withdrawn funding. The occurrence of any of these business disruptions could seriously harm our business.

All of our operations including our corporate headquarters are located in multiple facilities in Emeryville, California. Damage or extended periods of interruption to our corporate, development or research facilities due to fire, earthquake and other natural disaster, power loss, communications failure, unauthorized entry or other events could cause us to cease or delay development of some or all of our product candidates. Although we maintain property damage and business interruption insurance coverage on these facilities, our insurance might not cover all losses under such circumstances and our business may be seriously harmed by such delays and interruption.

Actual or perceived failures to comply with applicable data protection, privacy and security laws, regulations, standards and other requirements could adversely affect our business, results of operations, and financial condition.

The global data protection landscape is rapidly evolving, and we are or may become subject to numerous state, federal and foreign laws, requirements and regulations governing the collection, use, disclosure, retention, and security of personal data, such as information that we may collect in connection with clinical trials in the United States and abroad. Implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future, and we cannot yet determine the impact future laws, regulations, standards, or perception of their requirements may have on our business. This evolution may create uncertainty in our business, affect our ability to operate in certain jurisdictions or to collect, store, transfer use and share personal information, necessitate the acceptance of more onerous obligations in our contracts, result in liability or impose additional costs on us. The cost of compliance with these laws, regulations and standards is high and is likely to increase in the future. Any failure or perceived failure by us to comply with federal, state or foreign laws or regulation, our internal policies and procedures or our contracts governing our processing of personal information could result in negative publicity, government investigations and enforcement actions, claims by third parties and damage to our reputation, any of which could have a material adverse effect on our operations, financial performance and business.

As our operations and business grow, we may become subject to or affected by new or additional data protection laws and regulations and face increased scrutiny or attention from regulatory authorities. In the United States, HIPAA imposes, among other things, certain standards relating to the privacy, security, transmission and breach reporting of individually identifiable health information.

Certain states have also adopted comparable privacy and security laws and regulations, some of which may be more stringent than HIPAA. Such laws and regulations will be subject to interpretation by various courts and other governmental authorities, thus creating potentially complex compliance issues for us and our future customers and strategic partners. In addition, California enacted the California Consumer Privacy Act (CCPA) on June 28, 2018, which takes effect on January 1, 2020. The CCPA creates individual privacy rights for California consumers and increases the privacy and security obligations of entities handling certain personal data. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. The CCPA may increase our compliance costs and potential liability, and many similar laws have been proposed at the federal level and in other states. In the event that we are subject to or affected by HIPAA, the CCPA or other domestic privacy and data protection laws, any liability from failure to comply with the requirements of these laws could adversely affect our financial condition. In Europe, the GDPR went into effect in May 2018 and introduces strict requirements for processing the personal data of EU data subjects. Companies that must comply with the GDPR face increased compliance obligations and risk, including more robust regulatory enforcement of data protection requirements and potential fines for noncompliance of up to €20 million or 4% of the annual global revenues of the noncompliant company, whichever is greater.

Although we work to comply with applicable laws, regulations and standards, our contractual obligations and other legal obligations, these requirements are evolving and may be modified, interpreted and applied in an inconsistent manner from one jurisdiction to another, and may conflict with one another or other legal obligations with which we must comply. Any failure or perceived failure by us or our employees, representatives, contractors, consultants, CROs, collaborators, or other third parties to comply with such requirements or adequately address privacy and security concerns, even if unfounded, could result in additional cost and liability to us, damage our reputation, and adversely affect our business and results of operations.

## Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

Under Sections 382 and 383 of the United States Internal Revenue Code of 1986, as amended (the Code), if a corporation undergoes an "ownership change" (generally defined as a greater than 50-percentage-point cumulative change (by value) in the equity ownership of certain stockholders over a rolling three-year period), the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change taxable income or taxes may be limited. We have experienced ownership changes in the past. We may also experience ownership changes as a result of this offering or as a result of subsequent shifts in our stock ownership, some of which are outside our control. As a result, our ability to use our pre-change net operating loss carryforwards and other pre-change tax attributes to offset post-change taxable income or taxes may be subject to limitation. We will be unable to use our NOLs if we do not attain profitability sufficient to offset our available NOLs prior to their expiration.

## Changes in tax laws or regulations that are applied adversely to us or our customers may seriously harm our business.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could affect the tax treatment of any of our future domestic and foreign earnings. Any new taxes could adversely affect our domestic and international business operations, and our business and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us.

## Risks Related to This Offering and Ownership of Our Common Stock

We do not know whether an active market will develop for our common stock or what the market price of our common stock will be, and, as a result, it may be difficult for you to sell your shares of our common stock.

Before this offering, there was no public trading market for our common stock. If an active market for our common stock does not develop or is not sustained, it may be difficult for you to sell your shares of our common stock at an attractive price, or at all. We cannot predict the prices at which our common stock will trade. It is possible that in one or more future periods our results of operations and progression of our product pipeline may not meet the expectations of public market analysts and investors, and, as a result of these and other factors including those identified in this "Risk Factors" section, the price of our common stock may fall.

# The market price of our common stock may be volatile, which could result in substantial losses for investors purchasing shares in this offering.

The initial public offering price for our common stock was determined through negotiations with the underwriters. This initial public offering price may differ from the market price of our common stock after the offering. As a result, you may not be able to sell your common stock at or above the initial public offering price. Some of the factors that may cause the market price of our common stock to fluctuate include:

- · the success of existing or new competitive products or technologies;
- the timing and results of clinical trials for our current product candidates and any future product candidates that we may develop;
- · commencement or termination of collaborations for our product candidates;
- · failure or discontinuation of any of our product candidates;
- failure to develop our Therapeutic Vector Evolution platform technology;
- results of preclinical studies, clinical trials or regulatory approvals of product candidates of our competitors, or announcements about new research programs or product candidates of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- · the recruitment or departure of key personnel;
- · the commencement of litigation;
- the level of expenses related to any of the research programs or product candidates that we may develop;
- the results of our efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- announcement or expectation of additional financing efforts;
- · sales of our common stock by us, our insiders, or other stockholders;
- · expiration of market standoff or lock-up agreements;

- variations in our financial results or those of companies that are perceived to be similar to us;
- · changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- changes in the structure of healthcare payment systems;
- · market conditions in the pharmaceutical and biotechnology sectors;
- · general economic, industry, and market conditions; and
- · the other factors described in this "Risk Factors" section.

In recent years, the stock market in general, and the market for pharmaceutical and biotechnology companies in particular, has experienced significant price and volume fluctuations that have often been unrelated or disproportionate to changes in the operating performance of the companies whose stock is experiencing those price and volume fluctuations. Broad market and industry factors may seriously affect the market price of our common stock, regardless of our actual operating performance. These fluctuations may be even more pronounced in the trading market for our stock shortly following this offering. Following periods of such volatility in the market price of a company's securities, securities class action litigation has often been brought against that company. Because of the potential volatility of our stock price, we may become the target of securities litigation in the future. Securities litigation could result in substantial costs and divert management's attention and resources from our business.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock will rely in part on the research and reports that industry or financial analysts publish about us or our business. We do not currently have and may never obtain research coverage by industry or financial analysts. If no or few analysts commence coverage of us, the trading price of our stock could decrease. Even if we do obtain analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

A significant portion of our total outstanding shares is restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, upon the expiration of the market standoff and lock-up agreements, the early release of these agreements or the perception in the market that the holders of a large number of shares of our common stock intend to sell shares, could reduce the market price of our common stock. After this offering and after giving effect to the conversion of 7.375.631 outstanding shares of our redeemable convertible preferred stock into an equivalent number of shares of our common stock immediately prior to the completion of this offering, we will have shares of our common stock outstanding based on shares of our common stock outstanding as of June 30, 2019. Of shares we are selling in this offering may be resold in the public market immediately, unless purchased by our these shares, the % of our outstanding shares after this offering, are currently prohibited or otherwise affiliates. The remaining shares, or restricted under securities laws, market standoff agreements entered into by our stockholders with us, or lock-up agreements entered into by our stockholders with the underwriters. However, subject to applicable securities law restrictions and excluding shares of restricted stock that will remain unvested, prohibitions and restrictions on the sale of these shares in the public market will be lifted beginning 180 days after the

date of this prospectus. Goldman Sachs & Co. LLC and Evercore Group L.L.C may, in their sole discretion, release all or some portion of the shares subject to lock-up agreements at any time and for any reason. Shares issued upon the exercise of stock options outstanding under our equity incentive plans, or pursuant to future awards granted under those plans, will become available for sale in the public market to the extent permitted by the provisions of applicable vesting schedules, any applicable market standoff and lock-up agreements, and Rule 144 and Rule 701 under the Securities Act of 1933, as amended (the Securities Act). See the section of this prospectus titled "Shares Eligible for Future Sale" for additional information.

Moreover, after this offering, holders of an aggregate of shares of our common stock will have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also plan to register all shares of our common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates and the lock-up agreements described in the section of this prospectus titled "Underwriters." If any of these additional shares are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

# Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We will seek additional capital through one or a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. We, and indirectly, our stockholders, will bear the cost of issuing and servicing such securities. Because our decision to issue debt or equity securities in any future offering will depend on market conditions and other factors beyond our control, we cannot predict or estimate the amount, timing or nature of any future offerings. To the extent that we raise additional capital through the sale of equity securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences that adversely affect your rights as a stockholder. The incurrence of indebtedness would result in increased fixed payment obligations and could involve restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Additionally, any future collaborations we enter into with third parties may provide capital in the near term but limit our potential cash flow and revenue in the future. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses on terms unfavorable to us.

# Insiders will continue to have substantial influence over us after this offering, which could limit your ability to affect the outcome of key transactions, including a change of control.

After this offering, our directors, executive officers, holders of more than 5% of our outstanding stock and their respective affiliates will beneficially own shares representing approximately % of our outstanding common stock. As a result, these stockholders, if they act together, will be able to influence our management and affairs and all matters requiring stockholder approval, including the election of directors and approval of significant corporate transactions. This concentration of ownership may have the effect of delaying or preventing a change in control of our company and might affect the market price of our common stock.

We are an "emerging growth company" and as a result of the reduced disclosure and governance requirements applicable to emerging growth companies, our common stock may be less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act, and we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. In addition, as an "emerging growth company" the JOBS Act allows us to delay adoption of new or revised accounting pronouncements applicable to public companies until such pronouncements are made applicable to private companies. We have elected to use this extended transition period under the JOBS Act. As a result, our financial statements may not be comparable to the financial statements of issuers who are required to comply with the effective dates for new or revised accounting standards that are applicable to public companies, which may make comparison of our financials to those of other public companies more difficult.

We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the earlier of (1) the last day of the year following the fifth anniversary of the consummation of this offering, (2) the last day of the year in which we have total annual gross revenue of at least \$1.07 billion, (3) the last day of the year in which we are deemed to be a "large accelerated filer" as defined in Rule 12b-2 under the Exchange Act, which would occur if the market value of our common stock held by non-affiliates exceeded \$700.0 million as of the last business day of the second fiscal quarter of such year or (4) the date on which we have issued more than \$1.0 billion in non-convertible debt securities during the prior three-year period.

We will incur significant costs as a result of operating as a public company, and our management will devote substantial time to new compliance initiatives. We may fail to comply with the rules that apply to public companies, including Section 404 of the Sarbanes-Oxley Act of 2002, which could result in sanctions or other penalties that would seriously harm our business.

We will incur significant legal, accounting and other expenses as a public company, including costs resulting from public company reporting obligations under the Exchange Act and regulations regarding corporate governance practices. The listing requirements of The Nasdaq Global Market and the rules of the Securities and Exchange Commission (SEC) require that we satisfy certain corporate governance requirements relating to director independence, filing annual and interim reports, stockholder meetings, approvals and voting, soliciting proxies, conflicts of interest and a code of conduct. Our management and other personnel will need to devote a substantial amount of time to ensure that we comply with all of these requirements. Moreover, the reporting requirements, rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. Any changes we make to comply with these obligations may not be sufficient to allow us to satisfy our obligations as a public company on a timely basis, or at all. These reporting requirements, rules and regulations, coupled with the increase in potential litigation exposure associated with being a public company, could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors or board committees or to serve as executive officers, or to obtain certain types of insurance, including directors' and officers' insurance, on acceptable terms.

After this offering, we will be subject to Section 404 of The Sarbanes-Oxley Act of 2002 (Section 404) and the related rules of the SEC, which generally require our management and independent registered public accounting firm to report on the effectiveness of our internal control over financial reporting. Beginning with the second annual report that we will be required to file with the SEC, Section 404 requires an annual management assessment of the effectiveness of our internal control over financial reporting. However, for so long as we remain an emerging growth company as defined in the JOBS Act, we intend to take advantage of certain exemptions from various reporting requirements that are applicable to public companies that are not emerging growth companies, including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404. Once we are no longer an emerging growth company or, if prior to such date, we opt to no longer take advantage of the applicable exemption, we will be required to include an opinion from our independent registered public accounting firm on the effectiveness of our internal controls over financial reporting.

To date, we have never conducted a review of our internal control for the purpose of providing the reports required by these rules. During the course of our review and testing, we may identify deficiencies and be unable to remediate them before we must provide the required reports. Furthermore, if we have a material weakness in our internal controls over financial reporting, we may not detect errors on a timely basis and our financial statements may be materially misstated. We or our independent registered public accounting firm may not be able to conclude on an ongoing basis that we have effective internal control over financial reporting, which could harm our operating results, cause investors to lose confidence in our reported financial information and cause the trading price of our stock to fall. In addition, as a public company we will be required to file accurate and timely quarterly and annual reports with the SEC under the Exchange Act. In order to report our results of operations and financial statements on an accurate and timely basis, we will depend in part on CROs to provide timely and accurate notice of their costs to us. Any failure to report our financial results on an accurate and timely basis could result in sanctions, lawsuits, delisting of our shares from The Nasdaq Global Market or other adverse consequences that would seriously harm our business.

# Purchasers in this offering will experience immediate and substantial dilution in the book value of their investment.

The initial public offering price of our common stock is substantially higher than the pro forma net tangible book value per share of our common stock before giving effect to this offering. Accordingly, if you purchase our common stock in this offering, you will incur immediate substantial dilution of approximately \$ per share, based on an assumed initial public offering price of \$ per share, the midpoint of the estimated price range set forth on the cover of this prospectus, and our pro forma net tangible book value as of June 30, 2019. In addition, following this offering, purchasers in this offering will have contributed approximately \$% of the total gross consideration paid by stockholders to us to purchase shares of our common stock, through June 30, 2019, but will own only approximately % of the shares of our common stock outstanding immediately after this offering. Furthermore, if the underwriters exercise their overallotment option, or outstanding options and warrants are exercised, you could experience further dilution. For a further description of the dilution that you will experience immediately after this offering, see the section titled "Dilution."

## We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

We cannot specify with certainty the uses of the majority of the net proceeds we will receive from this offering. Our management will have broad discretion in the application of the net proceeds, including for any of the purposes described in the section of this prospectus titled "Use of Proceeds." Our management may spend a portion or all of the net proceeds from this offering in ways that our

stockholders may not desire or that may not yield a favorable return. The failure by our management to apply these funds effectively could seriously harm our business. Pending their use, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

Delaware law and provisions in our certificate of incorporation and bylaws that will become effective upon the closing of this offering might discourage, delay, or prevent a change in control of our company or changes in our management and, therefore, depress the trading price of our common stock.

Provisions in our certificate of incorporation and bylaws that will become effective upon the closing of this offering may discourage, delay, or prevent a merger, acquisition, or other change in control that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares of our common stock. These provisions may also prevent or frustrate attempts by our stockholders to replace or remove our management. Therefore, these provisions could adversely affect the price of our common stock. Among other things, our charter documents will:

- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;
- · eliminate cumulative voting in the election of directors;
- authorize our board of directors to issue shares of preferred stock and determine the price and other terms of those shares, including preferences and voting rights, without stockholder approval;
- provide our board of directors with the exclusive right to elect a director to fill a vacancy or newly created directorship;
- · provide that our directors may be removed only for cause;
- permit stockholders to only take actions at a duly called annual or special meeting and not by written consent;
- prohibit stockholders from calling a special meeting of stockholders;
- provide for a staggered board, which will result in only a couple directors being up for re-election in each calendar year;
- require that stockholders give advance notice to nominate directors or submit proposals for consideration at stockholder meetings;
- · authorize our board of directors, by a majority vote, to amend the bylaws;
- require the affirmative vote of at least 66 2/3% or more of the outstanding shares of our common stock to amend many of the provisions described above; and
- · limit the liability of, and provide indemnification to, our directors and officers.

In addition, Section 203 of the General Corporation Law of the State of Delaware (DGCL) prohibits a publicly-held Delaware corporation from engaging in a business combination with an interested stockholder, generally a person which together with its affiliates owns, or within the last three years has owned, 15% of our voting stock, for a period of three years after the date of the transaction in which the person became an interested stockholder, unless the business combination is approved in a prescribed manner.

Any provision of our certificate of incorporation, bylaws, or Delaware law that has the effect of delaying or preventing a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our capital stock and could also affect the price that some investors are willing to pay for our common stock.

Claims for indemnification by our directors and officers may reduce our available funds to satisfy successful third-party claims against us and may reduce the amount of money available to us.

Our amended and restated certificate of incorporation and amended and restated bylaws will provide that we will indemnify our directors and officers, in each case to the fullest extent permitted by Delaware law.

In addition, as permitted by Section 145 of the Delaware General Corporation Law, our amended and restated bylaws to be effective immediately prior to the completion of this offering and our indemnification agreements that we have entered into with our directors and officers will provide that:

- We will indemnify our directors and officers for serving us in those capacities or for serving other business enterprises at our request, to the fullest extent permitted by Delaware law. Delaware law provides that a corporation may indemnify such person if such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the registrant and, with respect to any criminal proceeding, had no reasonable cause to believe such person's conduct was unlawful.
- We may, in our discretion, indemnify employees and agents in those circumstances where indemnification is permitted by applicable law.
- We are required to advance expenses, as incurred, to our directors and officers in connection with defending a proceeding, except that such directors or officers shall undertake to repay such advances if it is ultimately determined that such person is not entitled to indemnification.
- We will not be obligated pursuant to our amended and restated bylaws to indemnify a person with respect to proceedings
  initiated by that person against us or our other indemnitees, except with respect to proceedings authorized by our board of
  directors or brought to enforce a right to indemnification.
- The rights conferred in our amended and restated bylaws are not exclusive, and we are authorized to enter into indemnification
  agreements with our directors, officers, employees and agents and to obtain insurance to indemnify such persons.
- We may not retroactively amend our amended and restated bylaw provisions to reduce our indemnification obligations to directors, officers, employees and agents.

Our amended and restated certificate of incorporation and our amended and restated bylaws will provide that the Court of Chancery of the State of Delaware will be the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation and our amended and restated bylaws will provide that the Court of Chancery of the State of Delaware is the exclusive forum for any derivative action or proceeding brought on our behalf; any action asserting a breach of fiduciary duty; any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws; or any action asserting a claim against us that is governed by the internal affairs doctrine; provided that, the exclusive forum provision will not apply to suits brought to enforce any liability or duty created by the Securities Act or the Exchange Act or any other claim for which the federal courts have exclusive jurisdiction; and provided further that, if and only if the Court of Chancery of the State of Delaware dismisses any such action for lack of subject matter jurisdiction, such action may be brought in another state or federal court sitting in the State of Delaware. Nothing in our amended and restated bylaws precludes stockholders that assert claims under the Securities Act or the Exchange Act from bringing such claims in state or federal court, subject to applicable law.

We believe these provisions may benefit us by providing increased consistency in the application of Delaware law and federal securities laws by chancellors and judges, as applicable, particularly experienced in resolving corporate disputes, efficient administration of cases on a more expedited schedule relative to other forums and protection against the burdens of multi-forum litigation. This choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or any of our directors, officers, other employees or stockholders, which may discourage lawsuits with respect to such claims, although our stockholders will not be deemed to have waived our compliance with federal securities laws and the rules and regulations thereunder. If a court were to find the choice of forum provision that will be contained in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could seriously harm our business.

We do not currently intend to pay dividends on our common stock, and, consequently, your ability to achieve a return on your investment will depend on appreciation in the price of our common stock.

We do not currently intend to pay any cash dividends on our common stock for the foreseeable future. We currently intend to invest our future earnings, if any, to fund our growth. Therefore, you are not likely to receive any dividends on your common stock for the foreseeable future. Since we do not intend to pay dividends, your ability to receive a return on your investment will depend on any future appreciation in the market value of our common stock. There is no guarantee that our common stock will appreciate or even maintain the price at which our holders have purchased it.

## We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us, because biotechnology companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could seriously harm our business.

#### SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements concerning our business, operations and financial performance and condition, as well as our plans, objectives and expectations for our business operations and financial performance and condition. Any statements contained herein that are not statements of historical facts may be deemed to be forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as "aim," "anticipate," "assume," "believe," "contemplate," "continue," "could," "due," "estimate," "expect," "goal," "intend," "may," "objective," "plan," "predict," "potential," "positioned," "seek," "should," "target," "will," "would" and other similar expressions that are predictions of or indicate future events and future trends, or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- the success, cost and timing of our development activities, preclinical studies and clinical trials, including our planned clinical trials for 4D-310, 4D-125 and 4D-110;
- the timing of IND-enabling studies and results from such studies, including our IND-enabling studies in 4D-710;
- the timing and success of lead optimization for our product candidates in lead optimization, including our product candidates for the treatment of DMD;
- the translation of our preclinical results and data into future clinical trials in humans;
- the timing of any manufacturing runs for materials to be used in patient trials;
- the number, size and design of our planned clinical trials, and what regulatory authorities may require to obtain marketing approval
- · the timing or likelihood of regulatory filings and approvals;
- our ability to obtain and maintain regulatory approval of our product candidates, and any related restrictions, limitations and/or warnings in the label of any approved product candidate;
- our ability to obtain funding for our operations, including funding necessary to develop and commercialize our product candidates;
- the rate and degree of market acceptance of our product candidates;
- the success of competing products or platform technologies that are or may become available;
- our plans and ability to establish sales, marketing and distribution infrastructure to commercialize any product candidates for which we obtain approval;
- future agreements with third parties in connection with the commercialization of our product candidates;
- the size and growth potential of the markets for our product candidates, if approved for commercial use, and our ability to serve those markets;
- · existing regulations and regulatory developments in the United States and foreign countries;
- the expected potential benefits of strategic collaboration agreements, including our relationships with Roche, AstraZeneca and uniQure, and our ability to attract collaborators with development, regulatory and commercialization expertise;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology;
- · potential claims relating to our intellectual property and third-party intellectual property;

- our ability to contract with third-party suppliers and manufacturers and their ability to perform adequately;
- the pricing and reimbursement of our product candidates, if approved;
- our ability to attract and retain key managerial, scientific and medical personnel;
- the accuracy of our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- · our financial performance;
- · our expectations regarding the period during which we qualify as an emerging growth company under the JOBS Act;
- our anticipated use of the proceeds from this offering; and
- other risks and uncertainties, including those listed under the section titled "Risk Factors."

These forward-looking statements are based on management's current expectations, estimates, forecasts and projections about our business and the industry in which we operate and management's beliefs and assumptions and are not guarantees of future performance or development and involve known and unknown risks, uncertainties and other factors that are in some cases beyond our control. As a result, any or all of our forward-looking statements in this prospectus may turn out to be inaccurate. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under the section titled "Risk Factors" and elsewhere in this prospectus. Potential investors are urged to consider these factors carefully in evaluating the forward-looking statements. These forward-looking statements speak only as of the date of this prospectus. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future. You should, however, review the factors and risks we describe in the reports we will file from time to time with the SEC after the date of this prospectus. See the section titled "Where You Can Find More Information."

## **INDUSTRY AND MARKET DATA**

This prospectus contains estimates, projections and other information concerning our industry, our business, and the markets for our product candidates, including data regarding the estimated patient population and market size for our product candidates, as well as data regarding market research, estimates and forecasts prepared by our management. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties, and actual events or circumstances may differ materially from events and circumstances that are assumed in this information. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources. In some cases, we do not expressly refer to the sources from which this data is derived. In that regard, when we refer to one or more sources of this type of data in any paragraph, you should assume that other data of this type appearing in the same paragraph is derived from the same sources, unless otherwise expressly stated or the context otherwise requires.

### **USE OF PROCEEDS**

We estimate that the net proceeds from the issuance and sale of shares of our common stock in this offering will be approximately \$ million, or approximately \$ million if the underwriters exercise their over-allotment option in full, based on the assumed initial public offering price of \$ per share, the midpoint of the estimated price range set forth on the cover of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Each \$1.00 increase or decrease in the assumed initial public offering price of \$ per share, the midpoint of the estimated price range set forth on the cover of this prospectus, would increase or decrease, as applicable, the net proceeds to us from this offering by approximately \$ million, assuming that the number of shares offered by us, as set forth on the cover of this prospectus, remains the same, and after deducting underwriting discounts and commissions. We may also increase or decrease the number of shares we are offering. Each increase or decrease of 1.0 million shares in the number of shares we are offering would increase or decrease, as applicable, the net proceeds to us from this offering, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, by approximately \$ million, assuming the assumed initial public offering price stays the same. We do not expect that a change in the initial price to the public or the number of shares by these amounts would have a material effect on the uses of the proceeds from this offering, although it may accelerate the time at which we will need to seek additional capital. The pro forma as adjusted information is illustrative only, and we will adjust this information based on the actual initial public offering price and other terms of this offering determined at pricing.

The principal purposes of this offering are to obtain additional capital to support our operations, to create a public market for our common stock and to facilitate our future access to the public equity markets. We intend to use the net proceeds from this offering, together with our cash and cash equivalents, as follows:

- approximately \$ million to fund the ongoing and planned preclinical and clinical development of our product candidates, including the initiation of our planned Phase 1/2 clinical trial for 4D-310 and IND-enabling study activities for 4D-710, as well as the initiation of Phase 1 clinical trials for 4D-125 and 4D-110:
- approximately \$ million to fund the further development and expansion of our pipeline including to complete lead optimization for our research candidates for the treatment of DMD and to complete toxicology studies for our research candidates for the treatment of Wet AMD;
- approximately \$ million to fund the continued expansion of our manufacturing and research and development capabilities;
- · any remaining amounts for working capital and general corporate purposes.

Based on our current operating plan, we estimate that our current cash and cash equivalents, together with the anticipated net proceeds from this offering, will be sufficient to fund our operating expenses and capital expenditure requirements through at least the next months.

The amounts and timing of our actual expenditures and the extent of our research and development activities may vary significantly depending on numerous factors, including the progress of our development efforts, the status of and results from any preclinical or clinical trials we may commence in the future, our ability to take advantage of expedited programs or to obtain regulatory approval for any other product candidates we may identify and pursue, the timing and costs associated with the manufacture and supply of any other product candidates we may identify and pursue for

clinical development or commercialization, and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering.

We may also use a portion of the remaining net proceeds and our existing cash and cash equivalents to in-license, acquire, or invest in complementary businesses, technologies, products or assets. However, we have no current commitments or obligations to do so.

This expected use of the net proceeds from this offering represents our intentions based upon our current plans and business conditions. As of the date of this prospectus, we cannot predict with certainty all of the particular uses for the net proceeds to be received upon the completion of this offering or the amounts that we will actually spend on the uses set forth above.

Pending their use, we intend to invest the net proceeds of this offering in a variety of capital-preservation investments, including short and intermediate-term, interest-bearing, investment-grade securities, and government securities.

# **DIVIDEND POLICY**

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings, if any, to fund the development and expansion of our business, and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination related to dividend policy will be made at the discretion of our board of directors.

#### **CAPITALIZATION**

The following table sets forth our cash and cash equivalents and capitalization as of June 30, 2019:

- · on an actual basis;
- on a pro forma basis to give effect to: (i) the conversion of 7,375,631 shares of our outstanding redeemable convertible preferred stock as of June 30, 2019 into an equivalent number of shares of our common stock immediately prior to the completion of this offering, and (ii) the filing and effectiveness of our amended and restated certificate of incorporation, which will occur, in each case, immediately prior to the completion of this offering; and
- on a pro forma as adjusted basis to give further effect to sale and issuance of shares of our common stock in this offering, assuming an initial public offering price of \$ per share, the midpoint of the estimated price range set forth on the cover of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

You should read this information together with our financial statements and related notes included elsewhere in this prospectus and the information set forth in the sections titled "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations."

		As of June 30, 201	.9
	Actual	Pro Forma	Pro Forma As Adjusted(1)
	(in thous	ands, except share a amounts)	nd per share
Cash and cash equivalents	\$ 72,869	\$	\$
Redeemable convertible preferred stock, \$0.0001 par value: 7,375,638 shares authorized; 7,375,631 shares issued and outstanding, actual; no shares	*****		
authorized, issued or outstanding, pro forma or pro forma as adjusted	\$102,980	\$	\$
Stockholders' (deficit) equity:			
Common stock, \$0.0001 par value: 50,000,000 shares authorized; 5,141,344 shares issued and outstanding, actual; shares			
authorized, pro forma and pro forma as adjusted; shares issued			
and outstanding, pro forma; shares issued and outstanding, pro			
forma as adjusted	1		
Additional paid-in capital	3,930		
Accumulated deficit	(46,235)		
Total stockholders' (deficit) equity	(42,304)		
Total capitalization	\$ 60,676	\$	\$

<sup>(1)</sup> Each \$1.00 increase or decrease in the assumed initial public offering price of \$ per share, the midpoint of the estimated price range set forth on the cover of this prospectus, would increase or decrease, as applicable, each of the amount of our cash and cash equivalents, additional paid-in capital, total stockholders' (deficit) equity and total capitalization by approximately \$ , assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of shares we are offering. Each increase or decrease of 1.0 million in the number of shares offered by us would increase or decrease, as applicable, each of the amount of our cash and cash equivalents, additional paid-in capital, total stockholders' (deficit) equity and total capitalization by approximately \$ , assuming the assumed initial public offering price of \$ per share, the midpoint of the estimated price range set forth on the cover of this prospectus, remains the same, and after deducting the estimated

underwriting discounts and commissions and estimated offering expenses payable by us. The pro forma as adjusted information discussed above is illustrative only and will be adjusted based on the actual public offering price and other terms of this offering determined at pricing.

If the underwriters' exercise their over-allotment option in full, our pro forma as adjusted cash and cash equivalents, and total capitalization as of June 30, 2019, would be \$ million and \$ million, respectively.

The number of shares of our common stock issued and outstanding, pro forma and pro forma as adjusted in the table above is based on 12,516,975 shares of our common stock (including our redeemable convertible preferred stock on an as-converted basis) outstanding as of June 30, 2019, which excludes:

- 2,192,230 shares of our common stock issuable upon the exercise of stock options to purchase common stock that were outstanding as of June 30, 2019, with a weighted-average exercise price of \$6.10 per share;
- 68,669 shares of our common stock issuable upon the exercise of outstanding warrants with a weighted-average exercise price
  of \$1.85 per share;
- 399,886 shares of our common stock reserved for issuance pursuant to future awards under our the Plan and associated amendments as of June 30, 2019;
- shares of our common stock reserved for issuance pursuant to future awards under our 2019 Plan, as well as any
  automatic increases in the number of shares of our common stock reserved for future issuance under this plan, which will
  become effective immediately prior to the completion of this offering; and
- shares of common stock reserved for issuance pursuant to future awards under our ESPP, as well as any automatic
  increases in the number of shares of our common stock reserved for future issuance under this plan, which will become effective
  immediately prior to the completion of this offering.

#### DILUTION

If you invest in our common stock in this offering, your interest will be immediately diluted to the extent of the difference between the initial public offering price per share of our common stock in this offering and the pro forma as adjusted net tangible book value per share of our common stock immediately after the completion of this offering.

As of June 30, 2019, our historical net tangible book value (deficit) was \$(42.7) million, or \$(8.30) per share of our common stock. Our historical net tangible book value (deficit) per share represents total tangible assets less total liabilities and redeemable convertible preferred stock divided by the number of shares of our common stock outstanding on June 30, 2019.

Our pro forma net tangible book value as of June 30, 2019, before giving effect to this offering, was \$ million, or \$ per share of our common stock. Pro forma net tangible book value represents our historical net tangible book value (deficit), before the issuance and sale of shares in this offering, and gives effect to the conversion of 7,375,631 shares of our outstanding redeemable convertible preferred stock as of June 30, 2019 into an equivalent number of shares of our common stock immediately prior to the completion of this offering.

Our pro forma as adjusted net tangible book value represents our pro forma net tangible book value, plus the effect of the sale of shares of our common stock in this offering at an assumed initial public offering price of \$ per share, the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We determine dilution per share to investors participating in this offering by subtracting pro forma as adjusted net tangible book value per share after this offering from the assumed initial public offering price per share paid by investors participating in this offering.

The following table illustrates this per share dilution:

Assumed initial public offering price per share	\$
Historical net tangible book value (deficit) per share as of June 30, 2019 \$(8.30)	
Pro forma increase in historical net tangible book value (deficit) per share	
Pro forma net tangible book value per share as of June 30, 2019	
Increase in pro forma net tangible book value per share attributable to new investors	
Pro forma as adjusted net tangible book value per share	
Dilution per share to new investors participating in this offering	\$

Each \$1.00 increase or decrease in the assumed initial public offering price of \$ per share, the midpoint of the estimated price range set forth on the cover of this prospectus, would increase or decrease, as applicable, our pro forma as adjusted net tangible book value as of June 30, 2019 after this offering by approximately \$ million, or approximately \$ per share, and would decrease or increase dilution to investors in this offering by approximately \$ per share, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of shares we are offering. Each increase or decrease of 1.0 million shares in the number of shares we are offering would increase or decrease, as applicable, our pro forma as adjusted net tangible book value as of June 30, 2019 after this offering by approximately \$ million, or approximately \$ per share, and would

decrease or increase, as applicable, dilution to investors in this offering by approximately \$ per share, assuming the assumed initial public offering price per share remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. The pro forma as adjusted information is illustrative only, and we will adjust this information based on the actual initial public offering price and other terms of this offering determined at pricing.

If the underwriters fully exercise their over-allotment option, pro forma as adjusted net tangible book value after this offering would increase to approximately \$ per share, the increase in pro forma as adjusted net tangible book value per share to existing stockholders would be \$ per share and the dilution to new investors purchasing shares in this offering would be \$ per share

To the extent that outstanding options with an exercise price per share that is less than the pro forma as adjusted net tangible book value per share are exercised, new investors will experience further dilution. In addition, we may choose to raise additional capital due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders.

The following table shows, as of June 30, 2019, on a pro forma as adjusted basis, the number of shares of our common stock purchased from us, the total consideration paid to us and the average price paid per share by existing stockholders and by new investors purchasing common stock in this offering, assuming an initial public offering price of \$ per share, the midpoint of the estimated price range set forth on the cover of this prospectus, before deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us (in thousands, except share and per share amounts and percentages):

	Shares Pu	ırchased	Total Cons	ideration	Weighted- Average Price Per
	Number	Percent	Amount	Percent	Share
Existing stockholders		<del></del> %	\$	<del></del> %	\$
New investors participating in this offering					\$
Total		100%	\$	100%	

If the underwriters were to fully exercise their over-allotment option, the percentage of shares of our common stock held by existing investors would be %, and the percentage of shares of our common stock held by new investors would be %.

The foregoing tables and calculations (other than the historical net tangible book value calculations) are based on 12,516,975 shares of our common stock (including our redeemable convertible preferred stock on an as-converted basis) outstanding as of June 30, 2019, which excludes:

- 2,192,230 shares of our common stock issuable upon the exercise of stock options to purchase common stock that were
  outstanding as of June 30, 2019, with a weighted-average exercise price of \$6.10 per share;
- 68,669 shares of our common stock issuable upon the exercise of outstanding warrants with a weighted-average exercise price of \$1.85 per share;
- 399,886 shares of our common stock reserved for issuance pursuant to future awards under our the Plan and associated amendments as of June 30, 2019;

- shares of our common stock reserved for issuance pursuant to future awards under our 2019 Plan, as well as any automatic increases in the number of shares of our common stock reserved for future issuance under this plan, which will become effective immediately prior to the completion of this offering; and
- shares of common stock reserved for issuance pursuant to future awards under our ESPP, as well as any automatic
  increases in the number of shares of our common stock reserved for future issuance under this plan, which will become effective
  immediately prior to the completion of this offering.

To the extent that outstanding options or warrants are exercised, new options or other securities are issued under our equity incentive plans, or we issue additional shares of our common stock in the future, there will be further dilution to investors participating in this offering. In addition, we may choose to raise additional capital because of market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. If we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders.

### **SELECTED FINANCIAL DATA**

The following tables present our selected financial data for the periods and as of the dates indicated. We derived the selected statements of operations and comprehensive loss data for the years ended December 31, 2017 and 2018 and the selected balance sheet data as of December 31, 2018 from our audited financial statements included elsewhere in this prospectus. We derived the selected statements of operations and comprehensive loss data for the six months ended June 30, 2018 and 2019 and the selected balance sheet data as of June 30, 2019 from our unaudited interim financial statements that are included elsewhere in this prospectus. Our unaudited interim financial statements are prepared on the same basis as our audited financial statements and include, in the opinion of management, all adjustments, consisting of normal recurring adjustments, that are necessary for the fair statement of the financial information set forth in those financial statements. Our historical results are not necessarily indicative of the results that may be expected in the future and our historical results for the six months ended June 30, 2019 are not necessarily indicative of the results that may be expected for the remainder of 2019. You should read this data together with our financial statements and related notes included elsewhere in this prospectus and the information under the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations."

		Year Ended December 31,			Six Months June 3				
		2017		2018	2018		2018		
			(in thou	ısands, except s	hare ar	nd per share (	data)		
Revenue:									
Collaboration and research revenue, related parties	\$	3,176	\$	5,143	\$	143	\$	26	
Collaboration and research revenue		2,614	_	8,987	_	4,926		4,390	
Total revenue		5,790		14,130		5,069		4,416	
Operating expenses:									
Research and development		13,573		18,362		8,208		16,985	
General and administrative		3,489		6,167		2,198		4,884	
Total operating expenses		17,062		24,529		10,406		21,869	
Loss from operations		(11,272)		(10,399)		(5,337)		(17,453)	
Other income (expense):									
Interest income		89		850		147		945	
Other income (expense), net		(40)	_	(2)	_	4		(8)	
Total other income (expense)		49		848		151		937	
Net loss and comprehensive loss	\$	(11,223)	\$	(9,551)	\$	(5,186)	\$	(16,516)	
Net loss per share attributable to common stockholders, basic and ${\sf diluted}^{(1)}$		(2.27)		(1.89)		(1.03)		(3.22)	
Weighted-average shares outstanding used in computing net loss per share attributable to common stockholders, basic and diluted(1)	4,	,953,419		5,049,203	5	5,021,003		5,132,714	
Pro forma net loss per share, basic and diluted(1)			_						
Weighted-average shares outstanding used in computing pro forma net loss per share, basic and diluted(1)			_						

<sup>(1)</sup> See Notes 2 and 14 to our financial statements included elsewhere in this prospectus for an explanation of the calculations of our basic and diluted net loss per share attributable to common stockholders, pro forma net loss per share, and the weighted-average number of common shares used in the computation of the per share amounts.

	Dec	cember 31, 2018 (in thous	June 30, 2019 sands)
Balance Sheet Data:		•	•
Cash and cash equivalents	\$	91,761	\$ 72,869
Working capital(1)		86,014	66,842
Total assets		96,969	80,500
Accumulated deficit		(30,026)	(46,235)
Total stockholders' deficit		(27,587)	(42,304)

<sup>(1)</sup> We define working capital as current assets less current liabilities. See our financial statements and related notes included elsewhere in this prospectus for further details regarding our current assets and current liabilities.

# MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and related notes thereto included elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the section titled "Risk Factors", our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. All amounts are expressed in thousands other than share and per share amounts. Please also see the section of this prospectus titled "Special Note Regarding Forward-Looking Statements."

#### Overview

We are a development stage precision gene therapy company dedicated to pioneering the development of targeted therapies based on our next-generation AAV vectors. Our proprietary Therapeutic Vector Evolution platform enables our "disease first" approach to product discovery and development, thereby allowing us to customize our AAV vectors to target specific tissue types associated with the underlying disease. Our proprietary AAV vectors are designed to provide targeted delivery by routine clinical routes, efficient transduction, reduced immunogenicity and resistance to pre-existing antibodies, which we believe will enable us to develop gene therapies that could overcome known limitations of conventional AAVs. As a result, we believe these key attributes will enable us to develop gene therapies with improved therapeutic profiles, pursue previously untreatable diseases and address a broad range of rare and large market diseases.

Our product candidates that are in or have completed IND-enabling studies include a wholly-owned asset in development for the treatment of Fabry disease, as well as two assets in ophthalmology. In ophthalmology our product candidate for the treatment of XLRP is wholly-owned, subject to an exclusive option for Roche to develop and commercialize the asset, and our product candidate for the treatment of Choroideremia is licensed to Roche. In addition, our wholly-owned product candidate for the treatment of Cystic Fibrosis has completed lead optimization and we expect to initiate IND-enabling studies in the first half of 2020. We have not conducted any clinical trials to date but we expect to initiate the clinical trials for these three product candidates in 2020. We also have a pipeline of product candidates in the lead optimization stage, including for the treatment of DMD and Wet AMD.

We do not have any products approved for sale and have not generated any revenue from product sales since our inception. From inception through June 30, 2019, we funded our operations primarily with an aggregate of \$108.6 million in gross cash proceeds primarily from the sale and issuance of redeemable convertible preferred stock and to a lesser extent from cash received from our collaboration and license agreements. As of June 30, 2019, we had cash and cash equivalents of \$72.9 million. Based on our current operating plan, we estimate that our cash and cash equivalents, together with the anticipated net proceeds from this offering, will be sufficient to fund our operating expenses and capital expenditure requirements through at least the next months.

Since our inception in September 2013, we have devoted substantially all of our resources to discovering and developing product candidates and manufacturing processes, building our Therapeutic Vector Evolution platform and assembling our core capabilities in drug development for genetic therapies.

Our ability to generate product revenue will depend on the successful development and eventual commercialization of one or more of our product candidates. Until such time as we can generate

significant revenue from sales of our product candidates, if ever, we expect to finance our operations through the sale of equity, debt financings or other capital sources, including potential collaborations with other companies or other strategic transactions. Adequate funding may not be available to us on acceptable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back, or discontinue the development and commercialization of our current product candidates or delay our efforts to advance and expand our product pipeline.

We have incurred significant operating losses to date and expect that our operating losses will increase significantly as we advance our product candidates through preclinical and clinical development, seek regulatory approval, and prepare for, and, if approved, proceed to commercialization; broaden and improve our platform; acquire, discover, validate and develop additional product candidates; obtain, maintain, protect and enforce our intellectual property portfolio; and hire additional personnel. In addition, upon the completion of this offering we expect to incur additional costs associated with operating as a public company.

Our net losses were \$11.2 million, \$9.6 million, \$5.2 million and \$16.5 million for the years ended December 31, 2017 and 2018 and the six months ended June 30, 2018 and 2019, respectively. As of June 30, 2019, we had an accumulated deficit of \$46.2 million. We do not expect positive cash flows from operations in the foreseeable future. We expect to continue to incur net operating losses for at least the next several years as we advance our product candidates through clinical development, seek regulatory approval, prepare for and, if approved, proceed to commercialization, continue our research and development efforts and invest in our manufacturing facility.

## **Components of Results of Operations**

#### Revenue

Our revenue to date has been generated through payments from our collaboration and license agreements, primarily from upfront payments and expense reimbursement. We have not generated any revenue from the sale of approved products and do not expect to do so in the near future.

In 2018, we recognized \$14.1 million of revenue from our collaboration and license agreements, principally from our agreements with Roche, Pfizer and AstraZeneca (previously MedImmune). For the six months ended June 30, 2019, we recognized \$4.4 million of revenue, principally from our agreements with Roche and AstraZeneca (previously MedImmune).

- We expect payments from Roche for reimbursement of our internal and third-party costs as well as the recognition of a \$21.0 million upfront payment received in 2017 to be our principal source of revenue in the second half of 2019. Upon initiation of our Phase 1/2 clinical trial for 4D-110 to treat Choroideremia, we expect to receive certain milestone payments. We expect this trial to initiate in the second half of 2020. If Roche continues the development of 4D-110, we will be entitled to further development and approval milestones, and, if the product candidate is approved, royalties on net sales and sales based milestones. Roche also has the option to assume the development and commercialization of 4D-125 to treat XLRP prior to pivotal trial initiation in return for an option payment and assumption of all future expenses. If Roche exercises such option, we would be entitled to future milestones upon development of the product candidate and, if approved, royalties on future net sales and sales-based milestones.
- Pfizer terminated our agreement in 2018, and as a result we recognized \$5.0 million of revenue in 2018 that we had previously deferred. We do not expect to recognize any revenue from Pfizer in 2019.

AstraZeneca (previously MedImmune) is evaluating the results of the development work we performed. If AstraZeneca
(previously MedImmune) decides to continue the program, we will be entitled to an option fee, development milestones, and if
the product candidate is approved, we will be entitled to royalties on net sales.

Future collaboration and license revenue is highly dependent on the successful development and commercialization of products by our collaboration partners, which is uncertain, and revenue may fluctuate significantly from period to period. Additionally, we may never receive the consideration from our license agreements that is contemplated for option fees, development and sales-based milestone payments or royalties on sales of licensed products, given the contingent nature of these payments. We expect that our license revenue in the second half of 2019 will be primarily from Roche. If our agreement with Roche was terminated, it may materially impact the amount of license revenue we recognize in future periods.

## **Operating Expenses**

Our operating expenses consist of research and development and general and administrative expenses. Personnel costs including salaries, benefits, bonuses and stock-based compensation expense, comprise a significant component of research and development and general and administrative expenses. We allocate indirect expenses associated with our facilities, information technology costs, depreciation and other overhead costs between research and development and general and administrative categories based on employee headcount and the nature of work performed by each employee.

#### Research and Development

Our research and development expenses primarily consist of costs incurred for the discovery and development of our product candidates, which include:

- salaries and personnel-related costs, including benefits, stock-based compensation and travel, for our scientific personnel performing research and development activities;
- costs related to executing preclinical studies and clinical trials, including payments to CROs;
- costs related to acquiring, developing and manufacturing materials for preclinical studies and clinical trials including payments to CMOs;
- costs related to laboratory supplies, research materials and other costs related to development and characterization of new AAV vectors:
- fees paid to consultants and other third-parties who support our product candidate development, including CROs, CMOs, and other service providers;
- · other costs in seeking regulatory approval of our product candidates; and
- allocated facility-related costs, depreciation expense and other overhead.

We expense all research and development costs in the periods in which they are incurred. We have entered into various agreements with CROs and CMOs. Costs of certain activities are recognized based on an evaluation of the progress to completion of specific tasks. Payments made prior to the receipt of goods or services that will be used or rendered for future research and development activities are deferred and capitalized as prepaid expenses and other current assets on our balance sheet. The capitalized amounts are recognized as expense as the goods are delivered or the related services are performed.

We do not allocate our costs by product candidate, as a significant amount of research and development expenses includes internal costs, such as payroll and other personnel expenses, laboratory supplies and allocated overhead, and external costs, such as fees paid to third parties to conduct research and development activities on our behalf, none of which are tracked by product candidate. In particular, with respect to internal costs, several of our departments support multiple product candidate research and development programs, and therefore the costs cannot be allocated to a particular product candidate or development program.

At this time, we cannot reasonably estimate or know the nature, timing, and estimated costs of the efforts that will be necessary to complete the development of, and obtain regulatory approval for, any of our product candidates. We expect our research and development expenses to increase substantially for the foreseeable future as we continue to invest in research and development activities related to developing our product candidates, as our product candidates advance into later stages of development, as we begin to conduct larger clinical trials, as we seek regulatory approvals for any product candidates that successfully complete clinical trials, and incur expenses associated with hiring additional personnel to support our research and development efforts. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of our product candidates is highly uncertain.

## General and Administrative

Our general and administrative expenses consist primarily of personnel-related expenses, including salaries, employee benefit costs and stock-based compensation expense, for our personnel in executive, finance and accounting and other administrative functions. General and administrative expenses also include professional fees for legal, patent, consulting, accounting and tax services, allocated overhead, including rent, equipment, depreciation, information technology costs and utilities, and other general operating expenses not otherwise classified as research and development expenses.

We expect our general and administrative expenses to increase in the future as we increase our headcount to support our continued research activities and development of our programs. We also expect increased personnel-related costs, patent costs for our product candidates, consulting, legal and accounting services associated with maintaining compliance with stock exchange listing and requirements of the SEC, investor relations costs, director and officer insurance premiums and other costs associated with being a public company.

## Other Income (Expense)

Our other income (expense) primarily consists of interest income earned on our cash equivalents and adjustments for the change in the fair value of our derivative liability which must be remeasured at each reporting period.

Revenue

## **Results of Operations**

## Comparison of the Six Months Ended June 30, 2018 and 2019

The following table summarizes our results of operations for the periods indicated (dollars in thousands):

		hs Ended e 30,		
	2018	2019	\$ Change	% Change
Revenue:				
Collaboration and license revenue, related party	\$ 143	\$ 26	\$ (117)	(82%)
Collaboration and license revenue	4,926	4,390	(536)	(11%)
Total revenue	5,069	4,416	(653)	(13%)
Operating Expenses:				
Research and development	8,208	16,985	8,777	107%
General and administrative	2,198	4,884	2,686	<u>122</u> %
Total operating expenses	10,406	21,869	11,463	110%
Loss from operations	(5,337)	(17,453)	(12,116)	227%
Other Income (Expense)	151	937	786	521%
Net loss and comprehensive loss	\$ (5,186)	\$(16,516)	\$(11,330)	218%

Revenue decreased from \$5.1 million for the six months ended June 30, 2018 to \$4.4 million for the six months ended June 30, 2019. The decrease of \$0.7 million, or 13%, was primarily due to the following:

- a \$0.3 million decrease in revenue recognized under our Collaboration and License Agreement with Applied Genetic Technologies Corporation;
- a \$0.2 million decrease in revenue recognized under our Collaboration and License Agreement with Roche for the development and treatment of various ophthalmology diseases;
- a \$0.1 million decrease in revenue recognized under our Collaboration and License Agreement with uniQure; and
- a \$0.1 million decrease in revenue recognized under our Collaboration and License Agreement with Benitec Biopharma Limited.

## Research and Development Expenses

The following table provides a breakout of research and development expenses for the periods indicated (dollars in thousands):

		nths Ended ne 30,		
	2018	2019	\$ Change	% Change
External expenses	\$4,415	\$ 9,599	\$ 5,184	117%
Payroll and personnel expenses	2,177	5,236	3,059	141%
Other research and development expenses	1,616	2,150	534	33%
Total research and development expenses	\$8,208	\$16,985	\$ 8,777	107%

Research and development expenses increased from \$8.2 million for the six months ended June 30, 2018 to \$17.0 million for the six months ended June 30, 2019. The increase of \$8.8 million, or 107%, was primarily due to the following:

- a \$5.2 million increase in external costs as we continue to develop novel vectors and identify potential product candidates and progress our preclinical studies; and
- a \$3.1 million increase in payroll and personnel expenses due to increased headcount to support the development of our and our partners' product candidates.

## General and Administrative Expenses

General and administrative expenses increased from \$2.2 million for the six months ended June 30, 2018 to \$4.9 million for the six months ended June 30, 2019. The increase of \$2.7 million, or 122%, was primarily due to the following:

- a \$1.3 million increase for personnel costs as a result of increased headcount of general and administrative personnel, including a \$0.2 million increase in employee stock-based compensation expense; and
- a \$0.9 million increase for professional services, including legal, accounting and other advisory services.

## Other Income (Expense)

Other income (expense) increased from \$0.2 million for the six months ended June 30, 2018 to \$0.9 million for the six months ended June 30, 2019. The increase was due to increased interest income in 2019 as a result of investing the \$84.5 million net proceeds from our Series B redeemable convertible preferred stock financing in August 2018.

## Comparison of the Years Ended December 31, 2017 and 2018

The following table summarizes our results of operations for the periods indicated (dollars in thousands):

	Year E Decem			
	2017	2018	\$ Change	% Change
Revenue:				
Collaboration and license revenue, related party	\$ 3,176	\$ 5,143	\$ 1,967	62%
Collaboration and license revenue	2,614	8,987	6,373	244%
Total revenue	5,790	14,130	8,340	144%
Operating Expenses:				
Research and development	13,573	18,362	4,789	35%
General and administrative	3,489	6,167	2,678	77%
Total operating expenses	17,062	24,529	7,467	44%
Loss from operations	(11,272)	(10,399)	873	(8%)
Other Income (Expense)	49	848	799	<u>1,631</u> %
Net loss and comprehensive loss	\$(11,223)	\$ (9,551)	\$ 1,672	(15%)

#### Revenue

Revenue increased from \$5.8 million for the year ended December 31, 2017 to \$14.1 million for the year ended December 31, 2018. The increase of \$8.3 million, or 144%, was primarily due to the following:

- a \$6.6 million increase in revenue recognized under our Collaboration and License Agreement with Roche for the development and treatment of various ophthalmology diseases;
- a \$5.0 million increase in revenue recognized upon termination of our Collaboration and License Agreement with Pfizer. This
  amount represented recognition of the upfront payment previously made by Pfizer that we had deferred; and
- which were both partially offset by a \$3.0 million decrease in revenue relating to our Collaboration and License Agreement with uniQure.

We do not expect to recognize any revenue from Pfizer in 2019.

## Research and Development Expenses

The following table provides a breakout of research and development expenses for the periods indicated (dollars in thousands):

		Ended iber 31,		
	2017	2018	\$ Change	% Change
External expenses	\$ 4,009	\$ 9,740	\$ 5,731	143%
Payroll and personnel expenses	4,302	4,960	658	15%
Other research and development expenses	5,262	3,662	(1,600)	(30%)
Total research and development expenses	\$13,573	\$18,362	\$ 4,789	35%

Research and development expenses increased from \$13.6 million for the year ended December 31, 2017 to \$18.4 million for the year ended December 31, 2018. The increase of \$4.8 million, or 35%, was primarily due to the following:

- a \$5.7 million increase in external costs as we continue to develop novel vectors and identify potential product candidates and progress our preclinical studies;
- a \$0.7 million increase in payroll and personnel expenses due to increased headcount to support the development of our and our partners' product candidates; and
- which were both partially offset by a \$1.6 million decrease for other research and development expenses, primarily due to a \$2.5 million decrease in stock-based compensation for uniQure, which was fully expensed in the first quarter of 2018. See Note 6 to our financial statements included elsewhere in this prospectus for further discussion of the stock options received from uniQure.

#### General and Administrative Expenses

General and administrative expenses increased from \$3.5 million for the year ended December 31, 2017 to \$6.2 million for the year ended December 31, 2018. The increase of \$2.7 million, or 77%, was primarily due to the following:

 a \$1.0 million increase for personnel costs as a result of increased headcount of general and administrative personnel, including a \$0.2 million increase in employee stock-based compensation expense;

- a \$0.9 million increase for professional services, including legal, accounting and other advisory services; and
- a \$0.2 million increase for allocated overhead, including rent, equipment, depreciation, information technology costs and utilities.

## Other Income (Expense)

Other income (expense) increased from less than \$0.1 million for the year ended December 31, 2017 to \$0.8 million for the year ended December 31, 2018. The increase was due to increased interest income in 2018 as a result of investing the \$84.5 million net proceeds from our Series B redeemable convertible preferred stock financing in August 2018.

## **Liquidity and Capital Resources**

# Sources of Liquidity

As of December 31, 2018 and June 30, 2019, we had cash and cash equivalents of \$91.8 million and \$72.9 million, respectively. We primarily generate cash and cash equivalents from the sale of our equity securities, including from the sale of our Series B redeemable convertible preferred stock, and to a lesser extent from cash received from our collaboration and license agreements.

In August 2018, we completed the private offering of 5,154,632 shares of our Series B redeemable convertible preferred stock at a price of \$17.46 per share. The net proceeds from the offering were \$84.5 million.

## **Future Funding Requirements**

We have incurred net losses since our inception and had an accumulated deficit of \$46.2 million as of June 30, 2019. Our transition to profitability is dependent upon the successful development, approval and commercialization of our product candidates and those of our collaboration partners and achieving a level of revenue adequate to support our cost structure. We do not expect to achieve such revenue and expect to continue to incur losses for the foreseeable future.

We expect that our current cash and cash equivalents will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months from the date of this prospectus.

We expect that our research and development and general and administrative expenses will continue to increase for the foreseeable future. Additionally, we expect our capital expenditures will increase significantly in the future for costs associated with building additional manufacturing capacity. As a result, we will need significant additional capital to fund our operations, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements.

Because of the numerous risks and uncertainties associated with the development and commercialization of gene therapy product candidates, we are unable to estimate the amount of increased capital we will need to raise to support our operations and the outlays and operating expenditures necessary to complete the development of our product candidates and build additional manufacturing capacity, and we may use our available capital resources sooner than we currently expect.

Our future capital requirements will depend on many factors, including:

- the scope, progress, results and costs of vector discovery, laboratory testing, preclinical development and clinical trials for our and our collaboration partner's product candidates;
- · the timing of enrollment, commencement and completion of our clinical trials;
- · the costs associated with building additional laboratory and manufacturing capacity;
- the costs, timing and outcome of regulatory review of our product candidates;
- the cost of manufacturing our product candidates or any future product candidates and any products we successfully commercialize, including costs associated with building-out our manufacturing capabilities
- the costs of future product sales, medical affairs, marketing, manufacturing and distribution activities for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sale of our products, should any of our product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- any product liability or other lawsuits related to our product candidates:
- our efforts to enhance operational systems and our ability to attract, hire and retain qualified personnel, including personnel to support the development of our product candidates;
- · the extent to which we acquire or in-license other technologies;
- · the payment of milestone or royalty payments owed under our existing collaboration and licensing agreements;
- our current collaboration and licensing agreements remaining in effect and our collaboration partners adhering to the terms of such collaboration and licensing agreements;
- our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all; and
- · the costs associated with being a public company.

Many of these factors are outside of our control. Developing novel vectors and identifying potential product candidates and conducting preclinical testing and clinical trials are time-consuming, expensive and uncertain processes that take years to complete, and we and our collaboration partners may never generate the necessary data or results required to obtain regulatory and marketing approval and achieve product sales. In addition, our and our collaboration partner's product candidates, if approved, may not achieve commercial success. Our product revenue, if any, and any commercial milestones or royalty payments under our collaboration agreements, will be derived from or based on sales of products that may not be commercially available for many years, if at all.

Our issuance of additional securities, whether equity or debt, or the possibility of such issuance, may cause the market price of our common stock to decline. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with collaboration partners or otherwise that may require us to relinquish rights to our intellectual property or our product candidates or otherwise agree to terms unfavorable to us. See the section titled "Risk Factors" for additional risks associated with our substantial capital requirements.

#### Summary Statement of Cash Flows

The following is a summary of cash flows for the periods indicated below (in thousands):

	Year	Year Ended		ths Ended	
	Decen	nber 31,	June 30,		
	2017	2018	2018	2019	
Net cash provided by (used in) operating activities	\$ 8,237	\$(16,252)	\$(6,422)	\$(17,168)	
Net cash provided by (used in) in investing activities	7,675	(414)	(33)	(1,662)	
Net cash provided by (used in) financing activities	16	84,577	20	(62)	
Net increase in cash and cash equivalents	<u>\$15,928</u>	\$ 67,911	<u>\$(6,435)</u>	<u>\$(18,892)</u>	

#### Net Cash Provided by (Used in) Operating Activities

Net cash used in operating activities was \$17.2 million for the six months ended June 30, 2019. This was primarily due to net loss of \$16.5 million and a decrease in deferred revenue of \$2.7 million, which were partially offset by stock-based compensation expense of \$1.5 million and depreciation and amortization expense of \$0.4 million.

Net cash used in operating activities was \$6.4 million for the six months ended June 30, 2018. This was primarily due to net loss of \$5.2 million and a decrease in deferred revenue of \$1.4 million.

Net cash used in operating activities was \$16.3 million for the year ended December 31, 2018. This was primarily due to net loss of \$9.6 million and a decrease in deferred revenue of \$8.6 million, which were partially offset by stock-based compensation expense of \$1.4 million and depreciation and amortization expense of \$0.7 million.

Net cash provided by operating activities was \$8.2 million for the year ended December 31, 2017. This was due to an increase in deferred revenue of \$19.0 million, primarily due to a \$21.0 million upfront payment from Roche in December 2017, which was partially offset by net loss of \$11.2 million.

## Net Cash Provided by (Used in) Investing Activities

Net cash used in investing activities was \$1.7 million for the six months ended June 30, 2019, all of which was used to purchase property and equipment.

Net cash used in investing activities was less than \$0.1 million for the six months ended June 30, 2018, all of which was used to purchase property and equipment.

Net cash used in investing activities was \$0.4 million for the year ended December 31, 2018, all of which was used to purchase property and equipment.

Net cash provided by investing activities was \$7.7 million for the year ended December 31, 2017, which was due to the maturity of \$8.2 million of marketable securities, which was partially offset by \$0.6 million used to purchase property and equipment.

#### Net Cash Provided by (Used in) Financing Activities

Net cash used in financing activities was \$0.1 million for the six months ended June 30, 2019, which was primarily due to deferred financing costs.

Net cash provided by financing activities was less than \$0.1 million for the six months ended June 30, 2018, all of which was due to the issuance of our common stock.

Net cash provided by financing activities was \$84.6 million for the year ended December 31, 2018, which was primarily due to \$84.5 million of net proceeds received from the issuance of our Series B redeemable convertible preferred stock in August 2018.

Net cash provided by financing activities was less than \$0.1 million for the year ended December 31, 2017, which was primarily due to the issuance of our common stock.

## **Contractual Obligations, Commitments and Contingencies**

Our commitments include obligations under vendor contracts to provide research services and other purchase commitments with our vendors. In the normal course of business, we enter into services agreements with contract research organizations, contract manufacturing organizations and other third-parties. Generally, these agreements provide for termination upon notice, with specified amounts due upon termination based on the timing of termination and the terms of the agreement. The actual amounts and timing of payments under these agreements are uncertain and contingent upon the initiation and completion of the services to be provided. These amounts are not fixed and determinable and therefore are not included in the table below.

The following table summarizes our contractual obligations, commitments and contingencies as of December 31, 2018 (in thousands):

	Total	Less Than 1 Year	1- 3 Years	3- 5 Years	More Than 5 Years
Operating lease payments	\$13,527	\$ 850	\$3,532	\$3,766	\$ 5,379
Total contractual obligations	\$13,527	\$ 850	\$3,532	\$3,766	\$ 5,379

In October 2018, we entered into a long-term lease for additional office and laboratory space in Emeryville, California, at a cost of \$9.3 million over an 87-month term (the Second Lease). We concurrently amended our existing lease to extend the lease term to end at the same time as the new lease, which has a remaining cost of \$4.2 million.

In May 2019, we amended the Second Lease (the Second Lease Amendment) to add 17,497 square feet to the space being leased pursuant to the Second Lease. The Second Lease Amendment extended the term of the lease to December 31, 2029. We do not have to pay rent until December 2019. The annual rent for the additional space is approximately \$1.0 million per annum and escalates at 3% annually. The incremental increase in the Second Lease cost per annum due to the Second Lease Amendment is not reflected in the above table.

# **Critical Accounting Policies and Significant Judgments and Estimates**

This Management's Discussion and Analysis of Financial Condition and Results of Operations is based on our financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States. The preparation of our financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of our financial statements, as well as the reported revenue and expenses during the reported periods. We evaluate these estimates and assumptions on an ongoing basis. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

Our significant accounting policies and recently announced accounting pronouncements, including the expected impact of such pronouncements, are described in Note 2 to our financial statements included elsewhere in this prospectus. We believe the following accounting policies are critical to the process of making significant judgments and estimates in the preparation of our financial statements and understanding and evaluating our reported financial results.

## Revenue Recognition

Effective January 1, 2019, we adopted ASC 606, using the modified retrospective transition method. As a result, we changed our accounting policies for revenue recognition as detailed below.

We determine revenue recognition for arrangements within the scope of ASC 606 by performing the following five steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations based on estimated selling prices; and (v) recognition of revenue when (or as) we satisfy each performance obligation.

Our revenue is primarily derived through our license, research, development and commercialization agreements. The terms of these types of agreements may include (i) licenses to our technology, (ii) research and development services, and (iii) services or obligations in connection with participation in research or steering committees. Payments to us under these arrangements typically include one or more of the following: nonrefundable upfront and license fees, research funding, milestone and other contingent payments to us for the achievement of defined collaboration objectives and certain preclinical, clinical, regulatory and sales-based events, as well as royalties on sales of any commercialized products. Arrangements that include upfront payments are recorded as deferred revenue upon receipt or when due and may require deferral of revenue recognition to a future period until performance obligations are met. The event-based milestone payments, royalties and cost reimbursements represent variable consideration, and we use the most likely amount method to estimate this variable consideration. Royalty payments are recognized when earned or as the sales occur. We record cost reimbursements as accounts receivable when right to consideration is unconditional.

A performance obligation is a promise in a contract to transfer a distinct good or service to the customer and is the unit of account in ASC 606. We allocate the total transaction price to each performance obligation based on the estimated relative to standalone selling price and recognize revenue when, or as, the performance obligation is satisfied. We include the unconstrained amount of estimated variable consideration in the transaction price. At the end of each reporting period, we re-evaluate the estimated variable consideration included in the transaction price and any related constraint, and if necessary, adjust our estimate of the overall transaction price.

Prior to the adoption of ASC 606 on January 1, 2019, we recognized revenue when all of the following criteria were met: persuasive evidence of an arrangement exists; transfer of technology has been completed or services have been rendered; the price to the customer is fixed or determinable and collectability is reasonably assured.

In arrangements involving the delivery of more than one element, each required deliverable was evaluated to determine whether it qualified as a separate unit of accounting. The determination was based on whether the deliverable had "standalone value" to the customer. If a deliverable did not qualify as a separate unit of accounting, it was combined with the other applicable undelivered item(s) within the arrangement and these combined deliverables were treated as a single unit of accounting.

The arrangement's consideration that was fixed or determinable was allocated to each separate unit of accounting based on the relative selling price methodology in accordance with the selling price hierarchy, which included vendor-specific objective evidence (VSOE) of selling price, if available, or third-party evidence of selling price if VSOE was not available, or the best estimate of selling price, if neither VSOE nor third-party evidence was available.

Payments or reimbursements for our research and development efforts for the arrangements where such efforts were considered as deliverables were recognized as the services were performed and were presented on a gross basis. When upfront payments were received and if there was no discernible pattern of performance, we recognized revenue ratably over the associated period of performance.

## Accrued Research and Development Expenses

We estimate our accrued research and development expenses as of each balance sheet date. This process involves reviewing contracts and purchase orders with service providers, identifying services that have been performed on our behalf and estimating the level of service performed, the expected remaining period of performance and the associated expenses incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. Depending on the timing of payments to the service providers and the estimated expenses incurred, we may record net prepaid or accrued research and development expenses relating to these costs.

Examples of estimated accrued research and development expenses include fees paid to:

- CROs in connection with preclinical development and clinical studies; and
- CMOs and other vendors related to process development and manufacturing of materials for use in preclinical development and clinical studies.

Our understanding of the status and timing of services performed relative to the actual status and timing may vary and may result in us reporting changes in estimates in any particular period. To date, there have been no material differences from our estimates to the amounts actually incurred.

## Stock-Based Compensation

We use a fair value-based method to account for all stock-based compensation arrangements with employees including stock options and stock awards. Our determination of the fair value of stock options on the date of grant utilizes the Black-Scholes option pricing model.

The fair value of the option granted is recognized on a straight-line basis over the period during which an optionee is required to provide services in exchange for the option award, known as the requisite service period, which usually is the vesting period.

We use a fair value-based method to account for stock-based compensation arrangements with non-employees. Our determination of fair value of stock options utilizes the Black-Scholes option pricing model. The compensation expense for these arrangements is subject to remeasurement until the related vesting conditions are met. We account for forfeitures as they occur for both employees and non-employees.

Estimates of the fair value of equity awards as of the grant date using valuation models such as the Black-Scholes option pricing model are affected by assumptions with a number of complex variables.

Changes in the following assumptions can materially affect the estimate of fair value and ultimately how much stock-based compensation expense is recognized; and the resulting change in fair value, if any, is recognized in our statement of operations and comprehensive loss during the period the related services are rendered. These inputs are subjective and generally require significant analysis and judgment to develop:

- Expected Term—The expected term for employee options is calculated using the simplified method as we do not have sufficient historical information to provide a basis for estimate. The simplified method is based on the average of the vesting tranches and the contractual life of each grant. The expected term for nonemployee options is the contractual term of the options.
- Expected Volatility—For all stock options granted to date, the expected volatility was estimated based on a study of publicly traded industry peer companies as we did not have any trading history for our common stock. We selected the peer group based on similarities in industry, stage of development, size and financial leverage with our principal business operations. For each grant, we measured historical volatility over a period equivalent to the expected term.
- *Risk-Free Interest Rate*—The risk-free interest rate is based on the yield available on U.S. Treasury zero-coupon issues whose term is similar in duration to the expected term of the respective stock option.
- Expected Dividend Yield—We have not paid and do not currently anticipate paying any dividends on our common stock. Accordingly, we have estimated the dividend yield to be zero.

As of June 30, 2019, the unrecognized stock-based compensation expense related to stock options was \$9.4 million and is expected to be recognized as expense over a weighted-average period of approximately 3.2 years. The intrinsic value of all outstanding stock options as of June 30, 2019 was approximately \$ million, based on the assumed initial public offering price of \$ per share, the midpoint of the estimated price range set forth on the cover of this prospectus, of which approximately \$ million related to vested options and approximately \$ million relate to invested options.

## **Common Stock Valuations**

The estimated fair value of the common stock underlying our stock options and stock awards was determined at each grant date by our board of directors, with input from management. All options to purchase shares of our common stock are intended to be exercisable at a price per share not less than the per share fair value of our common stock underlying those options on the date of grant.

In the absence of a public trading market for our common stock, on each grant date, we develop an estimate of the fair value of our common stock based on the information known to us on the date of grant, upon a review of any recent events and their potential impact on the estimated fair value per share of our common stock, and in part on input from an independent third-party valuation firm.

Our valuations of our common stock were determined in accordance with the guidelines outlined in the American Institute of Certified Public Accountants Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation* (the Practice Aid).

The assumptions used to determine the estimated fair value of our common stock are based on numerous objective and subjective factors, combined with management's judgment, including:

- external market conditions affecting the pharmaceutical and biotechnology industry and trends within the industry;
- · our stage of development and business strategy;

- the rights, preferences and privileges of our redeemable convertible preferred stock relative to those of our common stock;
- the prices at which we sold shares of our redeemable convertible preferred stock;
- our financial condition and operating results, including our levels of available capital resources;
- · the progress of our research and development efforts;
- · equity market conditions affecting comparable public companies;
- · general U.S. market conditions; and
- · the lack of marketability of our common stock.

The Practice Aid identifies various available methods for allocating enterprise value across classes and series of capital stock to determine the estimated fair value of our common stock at each valuation date. In accordance with the Practice Aid, we considered the following methods:

- Option Pricing Method. Under the option pricing method (OPM) shares are valued by creating a series of call options with exercise prices based on the liquidation preferences and conversion terms of each equity class. The estimated fair values of the preferred and common stock are inferred by analyzing these options.
- Probability-Weighted Expected Return Method. The probability-weighted expected return method (PWERM) is a scenario-based analysis that estimates value per share based on the probability-weighted present value of expected future investment returns, considering each of the possible outcomes available to us, as well as the economic and control rights of each share class.

Based on our early stage of development and other relevant factors, we determined that a hybrid approach of the OPM and the PWERM methods was the most appropriate method for allocating our enterprise value to determine the estimated fair value of our common stock. In determining the estimated fair value of our common stock, our board of directors also considered the fact that our stockholders could not freely trade our common stock in the public markets. Accordingly, we applied discounts to reflect the lack of marketability of our common stock based on the weighted-average expected time to liquidity. The estimated fair value of our common stock at each grant date reflected a non-marketability discount partially based on the anticipated likelihood and timing of a future liquidity event

Following the completion of this offering, our board of directors intends to determine the fair value of our common stock based on the closing quoted market price of our common stock on the date of grant.

#### Redeemable Convertible Preferred Stock

We record all shares of redeemable convertible preferred stock at their respective fair values on the dates of issuance, net of issuance costs. The redeemable convertible preferred stock is recorded outside of permanent equity because while it is not mandatorily redeemable, in certain events considered not solely within our control, such as a merger, acquisition, or sale of all or substantially all of our assets, each of which we refer to as a deemed liquidation event, the convertible preferred stock will become redeemable at the option of the holders of at least a majority of the then outstanding such shares. We have not adjusted the carrying values of the redeemable convertible preferred stock to its liquidation preference because a deemed liquidation event obligating us to pay the liquidation preferences to holders of shares of redeemable convertible preferred stock is not probable of occurring. Subsequent adjustments to the carrying values to the liquidation preferences will be made only when it becomes probable that such a deemed liquidation event will occur.

#### **Income Taxes**

We account for income taxes under the asset and liability method, which requires, among other things, that deferred income taxes be provided for temporary differences between the financial statement reporting and tax basis of our assets and liabilities. In addition, deferred tax assets are recorded for the future benefit of utilizing net operating losses and research and development credit carryforwards and are measured using the enacted tax rates and laws that will be in effect when such items are expected to reverse. A valuation allowance is provided against deferred tax assets unless it is more likely than not that they will be realized.

We account for uncertain tax positions by assessing all material positions taken in any assessment or challenge by relevant taxing authorities. Assessing an uncertain tax position begins with the initial determination of the position's sustainability and is measured at the largest amount of benefit that is greater than fifty percent likely of being realized upon ultimate settlement. Our policy is to recognize interest and penalties related to the underpayment of income taxes as a component of income tax expense or benefit. To date, there have been no interest or penalties charged in relation to the unrecognized tax benefits.

NOLs and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service (IRS) and may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50% as defined under Sections 382 and 383 in the Internal Revenue Code, which could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on our value immediately prior to the ownership change. We have experienced ownership changes in the past. As a result of the ownership changes, we determined that approximately \$0.9 million of our NOLs will expire unutilized for federal income tax purposes and such amounts are excluded from our NOLs as of December 31, 2018. Subsequent ownership changes may result in additional limitations.

# **Off-Balance Sheet Arrangements**

Since our inception, we have not engaged in any off-balance sheet arrangements as defined in the rules and regulations of the SEC.

#### Quantitative and Qualitative Disclosures about Market Risk

#### Interest Rate Sensitivity

The market risk inherent in our financial instruments and in our financial position represents the potential loss arising from adverse changes in interest rates. As of June 30, 2019, we had cash and cash equivalents of \$72.9 million, consisting of bank deposits and interest-bearing money market funds, for which the fair value would be affected by changes in the general level of U.S. interest rates. However, due to the short-term maturities of our cash equivalents, an immediate 10% change in interest rates would not have a material effect on the fair value of our cash equivalents.

### Effects of Inflation

Inflation generally affects us by increasing our cost of labor and development costs. We do not believe that inflation has had a significant impact on our results of operations for any periods presented herein.

# **JOBS Act**

We are an emerging growth company, as defined in the JOBS Act. Under the JOBS Act, emerging growth companies may delay adopting new or revised accounting standards issued

subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. We have elected to avail ourselves of this exemption from new or revised accounting standards and, therefore, while we are an emerging growth company, we will not be subject to new or revised accounting standards at the same time that they become applicable to other public companies that are not emerging growth companies. As a result, our financial statements may not be comparable to companies that comply with new or revised accounting pronouncements as of public company effective dates.

We are an emerging growth company as defined in the Jumpstart Our Business Startups Act of 2012 (the JOBS Act). We will remain an emerging growth company until the earlier of (i) the last day of the year following the fifth anniversary of the completion of this offering, (ii) the last day of the year in which we have total annual gross revenue of at least \$1.07 billion, (iii) the last day of the year in which we are deemed to be a "large accelerated filer" as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended (the Exchange Act), which would occur if the market value of our common stock held by non-affiliates exceeded \$700.0 million as of the last business day of the second fiscal quarter of such year, or (iv) the date on which we have issued more than \$1.0 billion in non-convertible debt securities during the prior three-year period. An emerging growth company may take advantage of specified reduced reporting requirements and is relieved of certain other significant requirements that are otherwise generally applicable to public companies. As an emerging growth company:

- we will present only two years of audited financial statements, plus unaudited financial statements for any interim period, and related management's discussion and analysis of financial condition and results of operations;
- we will avail ourselves of the exemption from the requirement to obtain an attestation and report from our auditors on the assessment of our internal control over financial reporting pursuant to the Sarbanes-Oxley Act of 2002 (the Sarbanes-Oxley Act);
- we will provide less extensive disclosure about our executive compensation arrangements;
- we will not require stockholder non-binding advisory votes on executive compensation or golden parachute arrangements; and
- we will take advantage of extended transition periods to comply with new or revised accounting standards, delaying the adoption
  of these accounting standards until they would apply to private companies.

As a result, the information in this prospectus and that we provide to our investors in the future may be different than what you might receive from other public reporting companies.

# **Recent Accounting Pronouncements**

See Note 2 to our financial statements included elsewhere in this prospectus for information.

#### **BUSINESS**

#### Overview

We are a development stage precision gene therapy company dedicated to pioneering the development of targeted therapies based on our next-generation AAV vectors. Our proprietary Therapeutic Vector Evolution platform enables our "disease first" approach to product discovery and development, thereby allowing us to customize our AAV vectors to target specific tissue types associated with the underlying disease.

Our proprietary AAV vectors are designed to provide targeted delivery by routine clinical routes, efficient transduction, reduced immunogenicity and resistance to pre-existing antibodies, which we believe will enable us to develop gene therapies that could overcome known limitations of conventional AAVs. As a result, we believe these key attributes will enable us to develop gene therapies with improved therapeutic profiles, pursue previously untreatable diseases and address a broad range of rare and large market diseases. Our product candidates that are in or have completed Investigational New Drug (IND)-enabling studies include a wholly-owned asset in development for the treatment of Fabry disease, as well as two assets in ophthalmology. In ophthalmology our product candidate for the treatment of X-linked Retinitis Pigmentosa (XLRP), is wholly-owned subject to an exclusive option for Roche to develop and commercialize the asset, and our product candidate for the treatment of Choroideremia is licensed to Roche. In addition our wholly-owned product candidate for the treatment of Cystic Fibrosis has completed lead optimization and we expect to initiate IND-enabling studies in the first half of 2020. We expect to initiate our first clinical trials for these product candidates in 2020. We also have a pipeline of product candidates in the lead optimization stage, including for the treatment of Duchenne Muscular Dystrophy (DMD) and Wet Age-Related Macular Degeneration (Wet AMD).

While gene therapy holds tremendous promise as a transformative therapeutic class, the majority of gene therapeutic approaches utilize naturally-occurring or conventional AAV vectors that have encountered limitations such as high dose requirements, limited efficacy, inflammation and toxicity, and neutralization by pre-existing antibodies. As a result of these shortcomings, current efforts with conventional AAV vectors have focused on diseases in which a surgical technique is used for direct cell access, such as subretinal injection for the retina, or a low level of gene expression may be sufficient for patient benefit, such as Hemophilia A or B treatment. Rather than using conventional AAV vectors for our products, we instead take a "disease first" approach creating proprietary customized AAV vectors for each disease indication through our Therapeutic Vector Evolution platform. Our Therapeutic Vector Evolution platform is based on directed evolution, a high-throughput biological engineering and screening approach harnessing the power of natural selection to identify novel AAVs with desirable characteristics. As of September 1, 2019 we have a total of 37 distinct libraries, with an estimated over one billion vector sequences. We select customized AAVs based on the Target Vector Profile (TVP) for the disease and we subsequently characterize each lead vector through extensive studies in non-human primates (NHPs) and proprietary human cell and organotypic tissue assays. Based on our preclinical data to date from NHPs and human cell models, including preclinical head to head comparisons with conventional AAVs, we observed that our precision AAVs were well-tolerated and achieved enhanced delivery, increased transgene expression, reduced immunogenicity and/or improved antibody resistance when compared to therapies using conventional AAVs.

Our initial focus is on developing a broad pipeline of transformative gene therapy product candidates designed to treat patients suffering from lysosomal storage diseases, lung diseases, muscular dystrophies and ophthalmological diseases. We are developing a diverse pipeline of wholly-owned and partnered programs for both rare and large market diseases. We have not conducted any clinical trials but expect to initiate our first clinical trials for these three product candidates in 2020. Our pipeline is represented in the diagram below.



- \* Expected timing of milestone. FIH refers to first-in-human, Phase 1 or Phase 1/2, clinical studies.
- \*\* We are responsible for development of this product candidate and Roche has an exclusive option to assume development and commercialization at its sole cost and expense. Such option may be exercised prior to pivotal trial initiation.
- † We are independently optimizing lead assets in this field. If we elect to declare product candidate(s) in the field specified in our collaboration and license agreement with Roche, Roche could exercise the option to license, further develop and commercialize this product candidate at any time prior to pivotal trial initiation. See the section titled "Business—Strategic Collaborations—Collaboration and License Agreement with F. Hoffman Ltd. and Hoffman—La Roche Inc." for further information.
- ‡ Proof of concept studies for 4D-710 were conducted in a pig model of Cystic Fibrosis and utilized a species-specific (i.e., pig-specific) vector rather than the primate-specific 4D-A101 vector, which is the vector for Cystic Fibrosis product candidate 4D-710.

#### Lysosomal Storage Diseases

Our lead product candidate in lysosomal storage diseases, 4D-310, is wholly-owned and under development for the treatment of Fabry disease, a progressive and fatal condition with cardiac complications cited as the leading cause of death. Classic Fabry disease is known to affect at least 5,000 males in the United States. However, we estimate the incidence rate may be higher as recent newborn screening suggests the total number of individuals with Fabry-related genetic mutations in the United States, as well as France, Germany, Italy, Spain and the United Kingdom (the EU-5) could fall between 50,000 and 70,000. Moreover, a recent multicenter study estimated that 57% of patients seen in the clinic are of the classic phenotype. Thus, we estimate the total addressable male Fabry patient population in the United States to be approximately 10,000 individuals, and including the EU-5, we estimate the total addressable male population to be approximately 18,800 individuals. 4D-310 uses a proprietary vector designed for efficient, low dose intravenous delivery to cardiac and other relevant tissues and to have minimal toxicities, and potential resistance to pre-existing antibodies in the Fabry disease population. The approved enzyme replacement therapies (ERT) reportedly do not adequately address Fabry cardiomyopathy, a leading cause of death in this disease. In 2019 we advanced 4D-310 to IND-enabling pharmacology and Good Laboratory Practices (GLP) toxicology and biodistribution studies with 4D-310 and in April 2019, we held a pre-IND meeting with the U.S. Food and Drug Administration (FDA). 4D-310 Phase 1/2 clinical trial initiation is planned for the second half of 2020. We expect initial human safety and biomarker data by

# Lung Diseases

Our lead product candidate in the lung therapeutic area, 4D-710, is wholly-owned and under development for the aerosol treatment of Cystic Fibrosis lung disease. Cystic Fibrosis is the most fatal inherited disease affecting more than 70,000 patients worldwide. Cystic Fibrosis causes impaired lung function, inflammation and bronchiectasis and is commonly associated with repeat and persistent lung infections due to the inability to clear thickened mucus from the lung, often resulting in frequent exacerbations, hospitalizations and eventual end-stage respiratory failure. We produced 4D-710 using a proprietary vector designed for efficient aerosol delivery to the lung airway, enhanced penetration through mucus, efficient airway cell transduction and transgene expression, and resistance to pre-existing antibodies in the patient population. While several products have been approved for the treatment of Cystic Fibrosis, they fall short of restoring normal lung function in most patients, and these chronic therapies require daily dosing for the patient's lifetime. Additionally, approximately 10% of patients remain without a corrective treatment option. We expect to initiate IND-enabling study activities with 4D-710 in the first half of 2020.

#### Muscular Dystrophies

We are currently in the lead optimization phase for our wholly-owned research candidates for the treatment of certain muscular dystrophies, including DMD. DMD, a progressive and fatal muscular-wasting disease, is one of the most prevalent rare genetic diseases, with an estimated prevalence between 10,000 to 15,000 cases in the United States. Our product candidates are engineered from proprietary vectors designed for efficient, low dose intravenous delivery to cardiac and skeletal muscle tissues with minimal toxicities, and potential resistance to pre-existing antibodies in the human patient population. Several companies are developing gene therapies for DMD using conventional AAVs. While early clinical data in some programs have been encouraging, AAV gene therapies using conventional AAV vectors have been subject to inflammation-related toxicity issues and manufacturing challenges due to the relatively high doses required. In addition to requiring high doses, patients have been excluded from clinical trials due to pre-existing antibodies to the conventional vector used. We expect to complete lead optimization in 2020.

# Ophthalmological Disorders

We have two ophthalmology product candidates that utilize our proprietary intravitreal vector for rare monogenic binding diseases: 4D-125 in development for the treatment of XLRP and 4D-110 in development for the treatment of Choroideremia. Our proprietary vector for these ophthalmological diseases is designed for intravitreal injection, potentially leading to transgene expression across the surface area of the retina, and major cell layers of the retina. We have an exclusive partnership in ophthalmology with Roche that includes an option for Roche to license 4D-125 in XLRP, as well as other ophthalmology product candidates we may declare, prior to pivotal trial initiation. 4D-125 is expected to begin clinical trials in mid-2020. In addition, Roche currently holds a license to 4D-110 in Choroideremia. We expect to initiate 4D-110 clinical trials in the second half of 2020. We expect initial human safety and biomarker data for 4D-125 by

We are currently in lead optimization for multiple product candidates that utilize our proprietary intravitreal vector, the same as for 4D-125 and 4D-110 described above, for the treatment of Wet AMD, each of which is engineered to express a different protein to neutralize vascular endothelial growth factor (VEGF). We expect to complete lead optimization in the first half of 2020.

# **Our Team**

We have an experienced team of biotherapeutics developers, innovative gene therapy scientists and clinicians to execute our product design and development strategy. Led by our Chief Executive Officer and co-founder, David Kirn, M.D., our team has more than 100 years of combined experience in

the field of viral vector gene therapy, including leadership of over 30 clinical trials from Phase 1 through Phase 3. Our Chief Technical Officer and Head of Regulatory and Quality, Fred Kamal, Ph.D., has over 20 years of industry experience in product manufacturing and quality, including most recently with AveXis where he was a key contributor to the development and writing of the quality and manufacturing sections of the biologics license application (BLA) for the AAV product Zolgensma. Our Chief Scientific Advisor and cofounder, Dr. David Schaffer, Ph.D., pioneered the application of directed evolution to the capsid of AAV vectors over 15 years ago.

#### **Our Investors**

We have raised \$108.6 million from the sale and issuance of our preferred stock to leading investors including Viking Global Investors, Janus Henderson Investors, The Biotechnology Value Fund, Arrowmark Partners, Pfizer Ventures, Mirae Asset Financial Group, Perceptive Advisors, Ridgeback Capital Investments, Pappas Capital, and Chiesi Ventures.

# **Our Strategy**

To achieve our goal of unlocking the full potential of gene therapy for many patient populations, we strive to achieve the following:

# Develop transformative precision gene therapies using the power of directed evolution and our "disease first" approach.

Our Therapeutic Vector Evolution platform allows us to move beyond naturally-occurring conventional AAV vectors and select proprietary, customized AAV vectors based on a disease-specific Target Vector Profile (TVP). As a result, we believe we will be able to unlock the full potential of gene therapy by creating precision gene therapies with improved characteristics that enable us to treat broader patient populations and previously untreatable diseases, if approved.

We believe our AAV products are modular, in that a single vector can be armed with different transgene payloads in order to treat multiple diseases within the same tissue types or therapeutic areas. As a result, if clinical studies provide us with clinical proof-of-concept for a specific vector, we believe our likelihood of technical success may increase with each subsequent product leveraging the same vector to deliver a different transgene payload.

# Design, develop and commercialize a diverse portfolio of product candidates in a broad range of therapeutic areas for both rare and large market diseases.

We are developing product candidates in diseases and therapeutic areas in which we believe our proprietary AAV vectors may provide differentiating advantages to conventional approaches. Our initial focus is to develop a broad pipeline of potentially transformative gene therapy product candidates designed for patients suffering from lysosomal storage diseases, lung diseases, muscular dystrophies and ophthalmological diseases. Additionally, we believe our customized vectors will allow us to create and, if approved, commercialize transformative therapies for rare and large market diseases that cannot be addressed with conventional AAV vectors.

#### Continue to expand our Good Manufacturing Practice manufacturing capabilities.

We believe a critical component to our success will be to leverage and expand our proprietary manufacturing know-how, expertise, and capacity. We currently operate an internal manufacturing facility in which we intend to produce clinical trial material according to current Good Manufacturing Practices (cGMP) requirements, along with material we have used for Good Laboratory Practices (GLP) toxicology studies, and vectors for characterization studies in non-human primates and other

animal models. We plan to continue expanding our internal manufacturing capabilities, while leveraging the experience of our chemistry, manufacturing and controls (CMC) team to support the development and, if approved, commercialization of our product candidates. We believe that focusing on internal manufacturing activities and control, supplemented by outside activities at CMOs, can mitigate CMC development and commercialization risks, limit reliance on CMOs and increase product development speed. We are dedicated to using the scalable unit operations we have designed for our product and vector manufacturing, which we believe have advantages for large-scale manufacturing versus those developed in academic laboratory settings.

# Protect, defend, and expand our intellectual property portfolio of proprietary AAV vectors discovered through Therapeutic Vector Evolution platform.

We have issued patents and patent application filings on the composition-of-matter for over 300 unique AAV capsid sequences that were identified through our discovery efforts. In addition, we have product-specific patent application claims that include our capsids carrying codon-optimized transgenes. We also hold trade secrets covering vector discovery and manufacturing methodologies. Finally, our intellectual property portfolio includes our vector libraries, consisting of an estimated one billion unique AAV capsid sequences. In addition, we intend to further expand our intellectual property portfolio by pursuing new opportunities, such as potentially developing new promoter and manufacturing-related technologies through our sponsored research agreements (the SRAs) with the UC Regents.

# Selectively partner with leading biopharmaceutical companies to harness the full potential of our platform while retaining product rights in key markets and work closely with patient advocacy groups to inform our programs.

We have retained rights to the majority of therapeutic areas and product opportunities within our proprietary platforms. We have entered into strategic partnerships with biopharmaceutical companies Roche, AstraZeneca (previously MedImmune) and uniQure in order to maximize the potential of the platform and broaden our therapeutic reach through their expertise and commercial capabilities. In addition, we have relationships with the Cystic Fibrosis Foundation, Foundation Fighting Blindness, Choroideremia Research Foundation and CureDuchenne. We will continue to evaluate new opportunities to partner with leading pharmaceutical and biotechnology companies, and with patient disease foundations and advocacy groups, that we believe maximize value and benefits for us and patients.

# **Gene Therapy and Limitations of Current Approaches**

Gene therapy aims to address diseases caused by gene mutations and gene dysregulation, and it holds the promise of delivering transformative and durable benefit to the patient after a single administration. Genetic therapies are designed to address the underlying molecular root cause of genetic diseases, in many cases, by introducing a functional version of the patient's defective gene into their own cells. Gene therapy has shown the potential to halt the progression of rare diseases, as well as to enable or restore critical human functions. These genetic therapies may be delivered to their target cells either *in vivo* or *ex vivo*, and can be paired with other therapeutic approaches including cell therapy and gene editing.

The transformative potential of gene therapy has demonstrated therapeutic utility across multiple disease areas, with a focus on rare diseases. There has been significant progress in the area of gene and cell therapy, including with AAV based gene therapy, as well as chimeric antigen receptor T-cell therapies. Further, the number of companies developing gene and cell therapy products has increased significantly over the last five years. There are currently a number of approved viral vector gene therapy products, including Zynteglo, Zolgensma, Luxturna, Imlygic and Strimvelis.

The physical construct of gene therapies are comprised of two essential components:

- *Vector:* A vehicle that packages and delivers the promoter and transgene into the body, and transports them through the protective cell membrane and ultimately into the cell nucleus. As a result, it plays an essential role in delivery and *transduction*, the process of guiding the transgene into the cell with subsequent expression of the transgene product. The vector is foundational to the efficacy of gene therapy, as the vector must deliver the promoter and transgene to the diseased cells in sufficient quantities to result in clinical benefit. The majority of AAV gene therapy companies use conventional AAV vectors that are comprised of naturally-occurring AAV viruses of a few specific subtypes, including AAV2, AAV5, AAV8, AAV9, AAVrh10, and AAVrh74.
- *Promoter and Transgene:* The promoter is the DNA region that controls and initiates transcription of the transgene, while the transgene is the functional gene intended to be delivered into the cell and expressed at the RNA and protein levels.

### Key Challenges With Conventional AAV Vectors

The fundamental gene therapy components used in current gene therapy candidates originated largely from academia, some as early as the 1960s, with limited improvements since their discovery. While gene therapy holds tremendous promise as a transformative therapeutic class, the demonstrated hurdles with conventional vectors may limit the diseases and patient populations that can be effectively addressed. There are four fundamental challenges that hinder gene therapies that utilize conventional AAV vectors:

- 1. Lack of efficient delivery to specific tissues and/or cell types due to physical barriers. Conventional naturally-occurring AAVs have not been designed or engineered to circumvent natural barriers to viral vector delivery, such as clearance by the liver or the inner-limiting membrane of the retina, and they are not targeted to specific tissues or cells. As a result, products using these vectors may require suboptimal delivery mechanisms (such as subretinal injection compared with intravitreal dosing) or high doses (such as with intravenous administration for the treatment of skeletal muscle diseases) to achieve therapeutic benefit.
- 2. Lack of efficient transduction of specific target cell types. To yield therapeutic benefit, the vector must efficiently deliver its transgene from the cell surface into the target cell nucleus, resulting in subsequent therapeutic transgene expression within the cell. Conventional AAVs are not designed or engineered for efficient transduction of specific target cells. As a consequence, conventional vectors may be associated with inefficient transduction and transgene expression which would limit efficacy.
- 3. Potential to cause toxicity due to inflammation. Conventional AAV vector based products have been associated with inflammation-related toxicities in some patients. Potential contributing factors may include the lack of specificity with current conventional AAV vectors, the high intravenous doses required for delivery to target tissues, and the ability of these AAVs to transduce immune cells. For example, intravenous gene therapy programs in patients with DMD have been associated with acute inflammation and transient kidney dysfunction.
- 4. Neutralization by pre-existing antibodies. The human immune system has evolved to fight viruses, including conventional AAVs that are present in nature. Widespread exposure to these conventional AAVs has resulted in neutralizing antibodies in a large portion of the population (approximately 30% to 70% depending on the vector serotype and patient population); these antibodies can limit gene delivery and the addressable patient population with conventional vectors. In addition, redosing with the same vector is generally not feasible given the induction of neutralizing antibodies to the vector.

Attempts have been made to address some of these limitations by incorporating incremental modifications in naturally-occurring vectors, including through the following methods:

- · engineered mutations to naturally occurring AAVs of a few specific subtypes to create new vectors; and
- acquisition of pools of novel AAV capsids generated using a single methodology, followed by screening and identification of capsids in small animal models (typically in mouse models) or *in vitro*.

While these latter conventional vectors are different from naturally-occurring AAV vectors, we do not believe these attempts at improvements to the naturally-occurring AAV have overcome each of the challenges encountered to date.

# Our Solution: Customized Vectors and "Disease First" Approach

We are pioneering the development of precision gene therapies based on our proprietary AAV vectors. We believe our customized AAV vectors have the potential to enable targeted delivery, efficient transduction, reduced inflammation and resistance to pre-existing antibodies. As a result, we believe we will be able to develop gene therapies with improved therapeutic profiles and also address previously untreatable diseases and patient populations.

Our Therapeutic Vector Evolution platform enables our "disease first" approach to product discovery, design and development. We use our platform to discover custom and proprietary AAVs designed for specific tissues and diseases, which we believe will allow us to overcome known limitations of conventional AAV vectors and potentially address a broad range of both rare and large market diseases. Based on our Target Vector Profile for a disease, we select customized capsids in non-human primates from an estimated over one billion vector capsid sequences in our 37 proprietary and diverse AAV vector libraries as of September 1, 2019. We subsequently characterize each vector through extensive studies in non-human primates and human cell and organotypic tissue assays.

# Key Attributes of Our Proprietary and Customized AAVs

Through our proprietary Therapeutic Vector Evolution platform, we rigorously screen our vectors to select for the optimal AAV for a specific disease, or class of diseases. We focus on discovering and selecting vectors that we believe have the potential to display superiority to conventional vectors with four key attributes:

- Targeted delivery to specific tissues by routine clinical routes of administration;
- · Improved transduction of target cell types and tissues;
- · Lower toxicity with reduced inflammation; and
- Ability to avoid neutralization by pre-existing antibodies in humans.

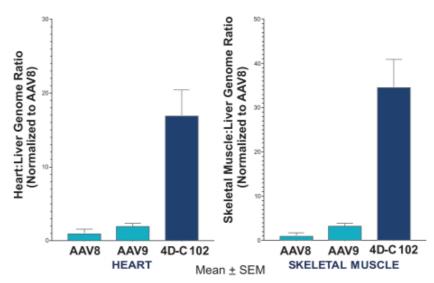
As shown below, while we have not initiated any clinical trials to date, we continue to generate animal proof-of-concept data in both primates *in vivo* and in human cells *in vitro* that we believe provide evidence regarding the potential for our precision AAVs to show superiority over conventional AAVs. As a result, we believe we have the potential to create products that are safer, more efficacious, more efficiently manufactured, and, if approved, more effectively commercialized.

Targeted Delivery to, and Transduction of, Specific Tissues by Routine Clinical Routes of Administration

We select AAVs that are designed to be administered through what we believe to be the optimal method of delivery for a particular disease, with the goal of circumventing physical barriers en route to specific tissues or cell types in the body.

In a first example of targeted delivery in NHP *in vivo*, our investigational, novel and proprietary AAV vector for cardiac and muscle tissues, 4D-C102, was designed for IV administration, which we believe represents the optimal route of administration for systemic delivery. There are diverse barriers to IV delivery to muscle tissues, which include the liver, blood components, immune cells and antibodies. In NHPs we have observed that intravenous delivery of 4D-C102 at relatively low doses (1E13 vg/kg) resulted in genome delivery to 100% of heart and skeletal muscle tissues evaluated in NHPs; 30 of 30 heart biopsies were positive, and 81 of 81 skeletal muscle biopsies were positive for genomes delivery. In addition, 4D-C102 was more efficiently delivered through IV administration to cardiac and skeletal muscle tissues than conventional AAV8 and AAV9. As shown below, 4D-C102 targeted the heart and skeletal muscle tissues (upper and lower extremities plus diaphragm) more efficiently than conventional IV vectors like AAV8 and AAV9, and showed up to 35-fold improvements, on average, in the ratio of vector reaching heart and skeletal muscle tissues versus the liver.

4D-C102 Intravenous Targeting to NHP Skeletal Muscle and Cardiac Tissue Was Superior to AAV8 and AAV9



SEM = standard error of the mean, which denotes the standard deviation of the sampling distribution

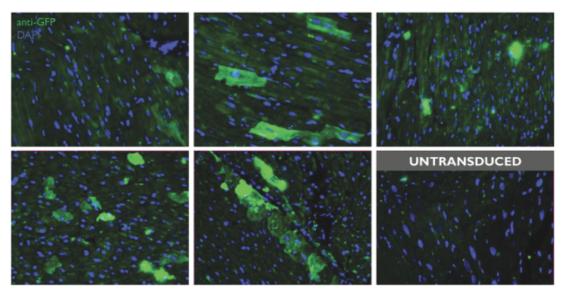
In a second example of targeted delivery and transgene expression in NHP in vivo, 4D-C102 delivered a marker gene (EGFP) that resulted in EGFP protein production in NHP cardiomyocytes in vivo. For example, 30 of 30 heart biopsy samples from three NHPs were positive for genomes, and 29 of 30 samples were positive for EGFP protein expression.

Cardiac Muscle Tissue Transduction and Protein Expression Patterns Observed After Intravenous Injection of 4D-C102 in NHP Cardiomyocytes (EGFP Expression as Detected by Immunohistochemistry).

Five Panels Illustrate Gene Expression Patterns Observed in NHP Cardiac Muscle Tissue After Treatment with 4D-C102. Cardiomyocyte Cell Staining

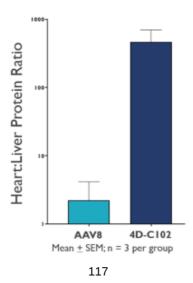
was Visible 8 Weeks Post-Injection.

The Lower-Right Panel Illustrates a Lack of Visible EGFP Staining in an Untransduced Control NHP.



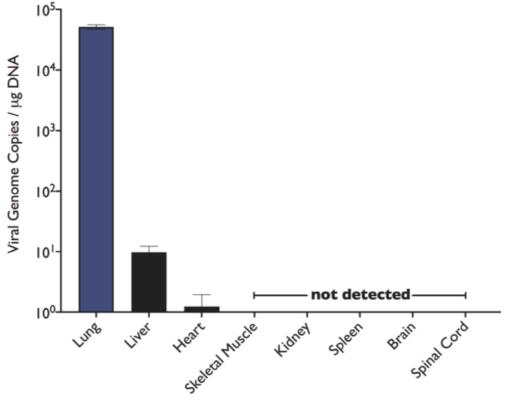
Further to this second example, 4D-C102 selectively induced protein expression in cardiac tissue. Specifically, in NHPs treated with 4D-C102, the ratio of cardiac protein expression to liver protein expression was estimated to be 190 fold higher than that ratio in NHPs treated with AAV8 (420:1 vs 2.2:1 for 4D-C102 vs AAV8, respectively).

Selective 4D-C102 Protein Expression in NHP (IV Administration): Cardiac Tissue Protein Expression Versus Liver Was Superior to AAV8



In a third example of targeted delivery in NHP *in vivo*, our investigational, novel, proprietary AAV for lung tissues, 4D-A101, was designed for aerosol delivery to lung airway cells; this vector was also selected for potential antibody-resistance in humans. In NHPs, we observed that aerosol delivery of 4D-A101 via a standard nebulization device, approved for use in humans, resulted in genome delivery to, and GFP marker gene expression and transduction of, all 48 lung sites evaluated; lung tissue sites evaluated included proximal, medial and distal airways. In addition to efficient lung airway transduction, migration to organs outside the lung was either undetectable (91% of samples tested) or extremely low concentrations (less than 0.1% of the average genomes per microgram of DNA in the lung airway tissues) as shown below.

Aerosol Delivery with 4D-A101 in NHP Resulted in Genome Localization and Protein Expression in All Lung Samples. 4D-A101 Genome Localization Was Limited or Not Present in Other Tissues

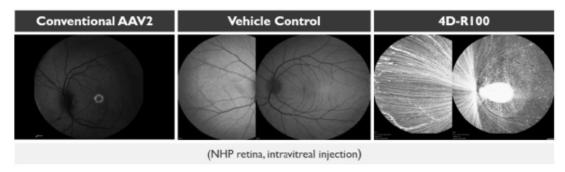


The Y axis is "viral genome copies per ug of DNA"—this refers to the number of viral genomes (a direct measurement of the number of virus particles) that can be quantified per quantity of DNA isolated from each tissue sample. The X axis is the tissue (e.g., lung, heart, liver) from which the samples were obtained.

In a fourth example of targeted delivery in NHP *in vivo*, our investigational, proprietary AAV for the retina, 4D-R100, was designed for intravitreal administration; we believe intravitreal injection is the optimal route of administration for the retina, as evidenced by approved biologics for the treatment of Wet AMD. Conventional AAVs such as AAV2 are not able to reach retinal cells effectively following intravitreal injection due to the inner-limiting membrane barrier between the vitreous and target retinal cells. In NHPs, whose ocular anatomy and physiology closely mirrors that of humans, we have observed that 4D-R100 administered by intravitreal injection covered the area of the retinal surface, and it transduced a high percentage of cells in all layers of the retina, including photoreceptors and retinal pigment epithelial (RPE) cells.

The Images Below Show Retinal Transduction and Protein Expression Patterns following Intravitreal Injection of 4D-R100 and Conventional 1st Generation AAV in NHP (in-life EGFP Expression Captured by Heidelberg Spectralis Camera). The Left Panel Illustrates that Typical Transduction and Protein Expression with Conventional AAV2 was Limited to a Ring of Cells Around the Fovea.

The Right Panel Illustrates Transduction and Protein Expression Pattern Following Intravitreal Dosing with 4D-R100 4D-R100 Exhibited Widespread Transgene Expression Across the Retinal Surface Area in NHP (in Life Images at 6-month Post-Injection)



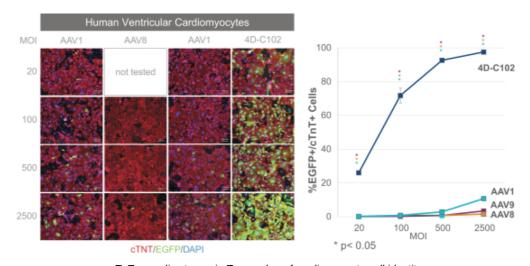
EGFP = enhanced green fluorescent protein

Improved Transduction of Target Human Cell Types and Tissues

We select AAVs for specific diseases that are designed to generate higher levels of gene expression than conventional AAV vectors in an effort to improve long-term efficacy and reduce vector doses required.

For example, our investigational novel AAV, 4D-C102, is designed to efficiently target human muscle cells, including cardiomyocytes. Targeting is especially important when addressing diseases that impact cardiac cells, such as Fabry disease. Conventional AAVs 1, 8 and 9 have been used by competitors to target these cells; however, we believe the limited transduction with these AAVs may limit efficacy and lead to high dose requirements that present safety challenges. In preclinical studies, 4D-C102 exhibited significantly improved transduction of human cardiomyocytes (ventricular phenotype) compared to conventional AAVs across a wide range of concentrations, as shown below.

4D-C102 Exhibited Significantly Higher Transduction in Vitro Relative to Conventional AAV1, 8 and 9, in Human Cardiomyocytes of Ventricular Phenotype



cTnT = cardiac troponin T, a marker of cardiomyocyte cell identity

DAPI = a marker of cell nuclei

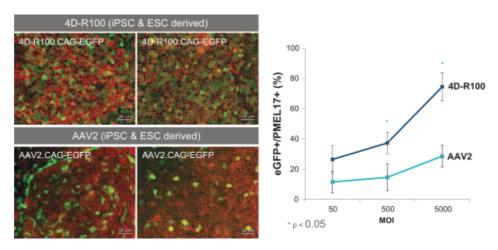
MOI = multiplicity of infection, a description of dose (vg per cell) for in vitro experiments

vg = viral genome, a quantification of the viral particles present

%EGFP+/cTnT+ Cells = the percentage of EGFP-expressing cells within the cTnT-expressing cardiomyocyte population, a quantification of the transduction efficiency of the capsid for the target cell type

In a second example, we have observed that our intravitreal retina vector, 4D-R100, was significantly more efficient at transducing human retina cells *in vitro*, such as RPE cells below, than conventional AAV2, which is commonly used for retina treatment.

# 4D-R100 Exhibited Greater Transduction of Human Retinal Cells in Vitro Compared to Conventional AAV2



CAG = ubiquitous promoter comprised of CMV enhancer, chicken beta actin promoter and intron, and rabbit beta globin intron element iPSC = induced pluripotent stem cell

ESC = embryonic stem cell

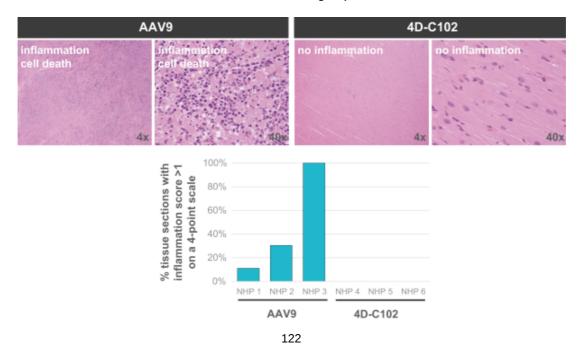
PMEL17 = premelanosome protein, a marker of retinal pigmented epithelial cell identity
%EGFP+/PMEL17+ Cells = the percentage of EGFP-expressing cells within the PMEL17-expressing retinal pigmented epithelia population, a
quantification of the transduction efficiency of the capsid for the target cell type

Lower Toxicity With Reduced Inflammation

We select AAVs that are designed to target specific diseased cells while avoiding non-target cells. We believe that vectors selected in this way have the potential to result in less inflammation, require lower doses and lead to a lower toxicity versus conventional AAVs such as AAV9.

As illustrated below, relative to conventional AAV9, our 4D-C102 vector was associated with lower inflammation and toxicities in NHP heart tissues after IV administration. The two vectors carried the same CAG promoter and EGFP transgene.

NHP Cardiac Muscle Exhibited Reduced Inflammation After Treatment with 4D-C102 Versus AAV9 (IV Administration of Vectors Carrying the EGFP Transgene)

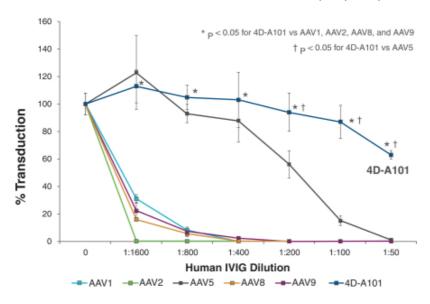


Ability to Avoid Neutralization by Pre-Existing Antibodies in Humans

We can select AAVs designed for resistance to neutralization by pre-existing antibodies in the human population, which we believe may broaden our potential target patient population compared to conventional AAVs. We believe resistance to antibodies may result in less neutralization, and therefore potentially better efficacy, after patient treatment. Finally, we believe we have the potential to re-dose patients when desirable by developing product candidates with different proprietary vectors that target the same tissues; this approach would utilize a proprietary AAV whose antibodies do not cross-react with the second proprietary vector.

For example, our proprietary AAV 4D-A101, which was designed for aerosol delivery, has shown improved antibody resistance when compared to conventional AAV1, 2, 5, 8 and 9 as demonstrated below.

4D-A101 Showed Improved Transduction In Vitro in the Presence of Human Antibodies (IVIG) Compared with Conventional AAV Vectors



IVIG = intravenous immune globulin, a human blood-derived product that contains antibodies (including antibodies against AAV)

# Our Proprietary Therapeutic Vector Evolution Platform

Our proprietary Therapeutic Vector Evolution platform is based on directed evolution. Directed evolution is a high-throughput discovery and optimization approach harnessing the power of evolution in an effort to create biologics with new and desirable characteristics. The first step involves the generation of diverse libraries of biological variants. In the second step, these libraries can be screened to identify improved variants with the desired characteristics. This method has been successfully utilized by other researchers to generate protein therapeutics with enhanced biological activities, antibodies with enhanced binding affinity and enzymes with new specificities. The importance of this biotechnology was demonstrated when the Nobel Prize for Chemistry was awarded in 2018 for work on directed evolution performed by academic investigators; these investigators have no relationship to us.

Our co-founder, Dr. Schaffer, pioneered the use of directed evolution to create improved AAV capsids for use as gene therapy vectors at U.C. Berkeley over 15 years ago. Over the past five years, we have developed our Therapeutic Vector Evolution platform, inspired by Dr. Schaffer's work on AAV directed evolution, to produce improved AAV capsid vectors designed for use in human therapeutic products. Since in-licensing six libraries from the UC Regents, we have created 31 new AAV capsid libraries as of September 1, 2019. In addition, we have developed significant experience in performing discovery in NHPs, with over 10 different discovery efforts completed to date. We believe the investment in our proprietary platform provides us with the largest and most diverse AAV vector library and vector sequences in the field of gene therapy.

### Proprietary AAV Vector Libraries

We have 37 distinct libraries as of September 1, 2019, with an estimated over one billion vector sequences. Each library is defined by its starting material (AAV capsid gene sequences) and genetic diversification methodologies, such as mutagenesis, random peptide sequence insertions and shuffling technologies. We apply bioinformatics, emerging technologies, and experience and know-how resulting from previous discovery programs to continually improve and expand our libraries and improve our ability to select optimized vectors from those libraries.

We believe the size and diversity of our vector libraries represent a differentiating competitive advantage for us in the field of gene therapy.

#### Disease First Approach—Target Vector Profile

We employ a rigorous approach to AAV vector selection based on what we consider an optimal product profile, which we term target vector profile (TVP). For any target disease, or set of diseases that affect the same tissues, this profile includes the following: the optimal route of administration for targeting the specific tissue(s) in humans, the optimal dose range, the desired distribution of vector transduction within the target organs, overall biodistribution and antibody resistance.

We use our Therapeutic Vector Evolution platform to identify the unique vector variant in our libraries that best matches our TVP. We achieve this through serial rounds of "selection", or discovery, with each round of selection filtering down to fewer and fewer capsids from the original library. This funneling process is achieved by applying selective pressures—forcing competition—among all capsid variants in the library to achieve delivery to the target cells as defined in the TVP. Each round is performed in a primate *in vivo*, sometimes in the presence of human IVIG (pooled human antibody preparation). While none of our product candidates have begun clinical trials, we believe this deliberate approach to selection in NHPs and human tissues should lead to identification of vectors with a higher likelihood of success in humans.

By the end of a typical discovery process, we will have identified approximately two to four lead capsids, or hits, based on their frequency in the final pool of capsid sequences, in addition to numerous sequences present at lower frequencies. We then file patent applications disclosing all identified gene sequences from the discovery program.

# Discovery Results to Date

To date, we have completed more than 10 unique vector discovery programs or "selection processes" for specific AAV capsids with specific TVPs. We have utilized four different routes of administration, including intravitreal, intrathecal, aerosol, and intravenous administrations. We have completed discovery programs targeting a diverse array of tissue types including various retinal cell types, heart and skeletal muscle tissues, brain, dorsal root ganglia, different lung cell types, liver, and synovial joints. We have identified and filed patent applications on over 300 unique capsid sequences.

#### Characterization of Novel Vector Variant "Hits"

Vector hits are typically characterized in three major areas: manufacturing, human cell and organotypic model transduction, and delivery to tissues in NHPs by the designated route of administration. Vector hits may also be evaluated for transduction in the presence of human antibodies. In order to perform characterization studies, vectors are armed with marker gene payloads such as EGFPs.

# Development Platform: Human Cell and Disease Modeling

With the goal of optimizing our product development programs prior to entering the clinic, we have developed a robust human cell and disease modeling platform. We evaluate vector transduction, transgene expression and function in target cells of interest in these human models. These cells can be derived from both normal donors or from patients with specific genetic diseases of interest. This approach allows us to perform head-to-head comparisons between different AAV capsids, promoter elements, and transgene payloads. We believe this approach better characterizes and informs the behavior of our vector products in the human body and their potential for clinical success.

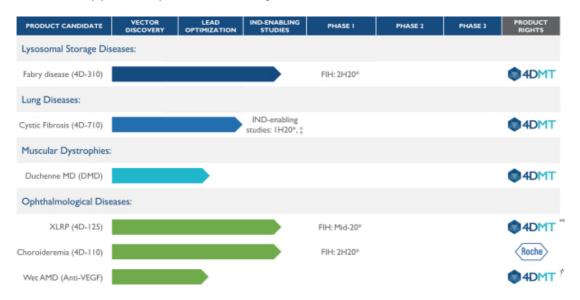
# Therapeutic Areas, Lead Vectors and Product Candidates

Our proprietary platform has enabled broad and diverse AAV programs that we are translating into product candidates with the goal of transformative patient benefit. We currently have a product pipeline that includes product candidates in four therapeutic areas: lysosomal storage diseases, lung diseases, muscular dystrophies and ophthalmological diseases.

For each of these therapeutic areas we have customized proprietary targeted lead vectors that were discovered using our proprietary technology platform. These lead vectors are designed for delivery by optimal clinical routes. We believe that these proprietary vectors will allow us to develop product candidate portfolios within each of these therapeutic areas.

	Cardiac and Skeletal Muscle		Lung	Retina
Lead Vector	4D-C102	4D-STAR100	4D-A101	4D-R100
Tissue and Cell Types Targeted	Diffuse Regions throughout Heart and Skeletal Muscle	Diffuse Regions throughout Heart and Skeletal Muscle	Diffuse Regions throughout Lung	Multiple Layers and Widespread Distribution in Retina
4DMT Product Candidate	4D-310	Lead Optimization	4D-710	4D-125 / 4D-110
Route of Administration	Intravenous	Intravenous	Aerosolized	Intravitreal
Selected for Resistance to Pre-existing Antibodies in Human Serum	Yes	Yes	Yes	N/A (eye is generally immune-privileged)
Conventional AAV Typically Used for Tissue Target in Humans	AAV1, AAV8, AAVrh74, AAV9	AAV1, AAV8, AAVrh74, AAV9	AAV2	AAV2

Our initial focus is on developing a broad pipeline of transformative gene therapy product candidates designed to treat patients suffering from lysosomal storage diseases, lung diseases, muscular dystrophies and ophthalmological diseases. We are developing a diverse pipeline of wholly-owned and partnered programs for both rare and large market diseases. We believe our AAV products are modular, in that a single vector can be armed with different transgene payloads in order to treat multiple diseases within the same tissue types or therapeutic areas. Our pipeline is represented in the diagram below.



- \* Expected timing of milestone. FIH refers to first-in-human, Phase 1 or Phase 1/2, clinical studies.
- \*\* We are responsible for development of this product candidate and Roche has an exclusive option to assume development and commercialization at its sole cost and expense. Such option may be exercised at any time prior to pivotal trial initiation.
- we are independently optimizing lead assets in this field. If we elect to declare product candidates in the field specified in our collaboration and license agreement with Roche, Roche could exercise the option to license, further develop and commercialize this product candidate at any time prior to pivotal trial initiation. See the section title "Business—Strategic Collaborations—Collaboration and License Agreement with F. Hoffman Ltd. and Hoffman-La Roche Inc." for further information.
- <sup>‡</sup> Proof of concept studies for 4D-710 were conducted in a pig model of Cystic Fibrosis and utilized a species-specific (i.e., pig-specific) vector rather than the primate-specific 4D-A101 vector, which is the vector for Cystic Fibrosis product candidate 4D-710.

# 4D-310 for the Treatment of Fabry Disease

# Target Product Profile

4D-310 is our wholly-owned product candidate for the treatment of Fabry disease. 4D-310 is comprised of our proprietary vector 4D-C102, carrying a codon-optimized *GLA* transgene. The product candidate is designed for efficient, single low dose intravenous delivery to cardiac tissue, kidney, liver and skeletal muscle tissues. The intended result is for alpha-galactosidase A (AGA) enzyme expression within cardiac tissues for cardiac disease correction, in addition to expression and secretion from other tissues within the body, such as skeletal muscle and kidney. We believe 4D-310 has the potential to treat "classic" (early onset) Fabry disease, as well as late onset Fabry disease, both of which are often associated with cardiomyopathy. To that end, we believe reducing substrate in cardiomyocytes would represent a strategic advantage and significant opportunity in the treatment of Fabry-associated cardiomyopathy, which we believe remains a significant unmet medical need.

We anticipate completing IND-enabling toxicology and biodistribution studies with 4D-310 in early 2020, and we expect 4D-310 to enter clinical trials in the second half of 2020. We expect initial human data by

Disease Background, Unmet Medical Need and Target Patient Population

Fabry disease is a rare genetic disorder that results in the body's inability to produce an enzyme called AGA, causing the accumulation of globotriaosylceramide-3 (Gb3) in critical organs, including the heart and kidney. Such substrate accumulation can lead to life-threatening heart failure, arrhythmias, vascular blockages, and various degrees of kidney dysfunction. Fabry disease is progressive and fatal, with an average life expectancy of approximately 50 years and cardiac complications being the leading cause of death. Progression of the disease causes significant reduction in the quality of life and significant economic burden associated with greater patient needs for supportive care. Classic Fabry disease is known to affect at least 5,000 males in the United States. However, we estimate the incidence rate may be higher as recent newborn screening suggests the total number of individuals with Fabry-related genetic mutations in the United States and EU-5 could fall between 50,000 and 70,000. Moreover, a recent multicenter study estimated that 57% of patients seen in the clinic are of the classic phenotype. Thus, we estimate the total addressable male Fabry patient population in the United States to be approximately 10,000 individuals, and including the EU-5, we estimate the total addressable male population to be approximately 18,800 individuals.

Fabry disease is currently treated by regular infusions, approximately every two weeks, of replacement AGA enzyme, a class of therapies broadly referred to as ERTs. Two of these approved ERT products are Fabrazyme from Sanofi and Replagal from Takeda, both of which are administered to patients every two weeks. In addition to high burdens of therapy, patients on these ERTs may be exposed to sub-therapeutic levels of AGA for a period of time between infusions, potentially leading to recurrences of disease symptoms and complications. Furthermore, ERTs reportedly lack efficient uptake by cardiac muscle cells; hence the patients remain at risk of cardiac complications, including death. Specifically, a medical officer's review of Fabrazyme which was conducted during the U.S. Food and Drug Administration (FDA)'s evaluation of this drug candidate indicates that consistent increases in Gb3 levels were measured in cardiomyocytes (as opposed to decreases measured in vascular endothelium). Therefore, we believe cardiac-specific treatment of Fabry disease is still an unmet medical need. Despite these shortcomings, ERT for Fabry disease does provide significant benefit for patients.

Competition and Differentiation—Viral Vector Gene Therapy including AAV for Fabry Disease

A number of biotechnology companies are pursuing gene therapy solutions, including with conventional AAV vectors, to treat Fabry disease. In addition to being in early stages of development, these developmental AAV gene therapies are specifically designed to target transduction to the liver only, with liver-specific promoters, which therefore would prevent expression in cardiac muscle cells. When administered as ERT, the AGA protein does not efficiently enter cardiomyocytes; it is therefore unclear whether production of AGA from the liver would result in effective cardiac muscle cell treatment. As a result, we believe 4D-310 is the only gene therapy candidate designed specifically to express the AGA enzyme in cardiac tissues, as well as in other affected tissues in these patients, potentially addressing a major unmet medical need.

We believe that a gene therapy that is specifically targeted to transduce cardiac muscles with high efficiency has the potential to significantly improve patient outcome through the synthesis of AGA directly within the heart tissues, including cardiomyocytes. Our 4D-310 program, for which we expect to initiate clinical trials in the second half of 2020, is designed to take advantage of our proprietary 4D-C102 vector for delivery of transgenes to cardiac muscle cells and other relevant tissues within the body.

Finally, to our knowledge, conventional AAV gene therapy programs in development are limited to treatment of the "classic" (early onset) patient population at this time. In contrast, 4D-310 is designed for the treatment of all patient subpopulations with Fabry disease, including "late onset" and classic population patients with cardiomyopathy. The late onset sub-population is substantially larger than the early onset population.

As we achieve proof-of-concept with the 4D-C102 vector, we intend to advance additional programs for lysosomal storage diseases using the same vector.

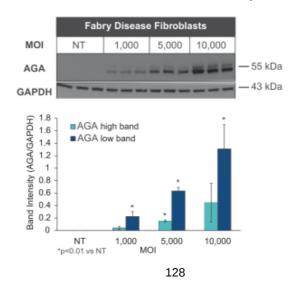
#### Pharmacology and Toxicology Program

In the second quarter of 2019 we initiated a GLP toxicology and biodistribution study of 4D-310 in normal mice. We previously received feedback from the FDA on our preclinical study plans in connection with a pre-IND interaction. Pharmacology studies have been performed in both Fabry disease knock-out mice and Fabry patient-derived cells, including cardiomyocytes differentiated from patient-derived stem cells. We plan to complete IND-enabling preclinical pharmacology and toxicology studies by the first quarter of 2020, and to file an IND for 4D-310 in the second half of 2020.

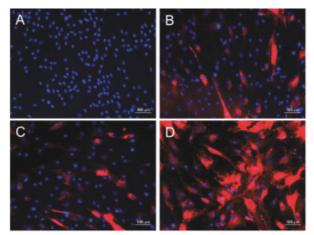
In *in vitro* studies with 4D-310 in Fabry patient-derived fibroblasts and cardiomyocytes we observed dose-related AGA expression and function as shown below.

4D-310 Transduced Fibroblasts Derived from Fabry Patients. These 4D-310-Transduced Fibroblasts Expressed AGA in a Dose-Dependent Manner

Panel A: Fabry-derived fibroblasts expressed AGA after treatment with 4D-310 as measured by western blot.



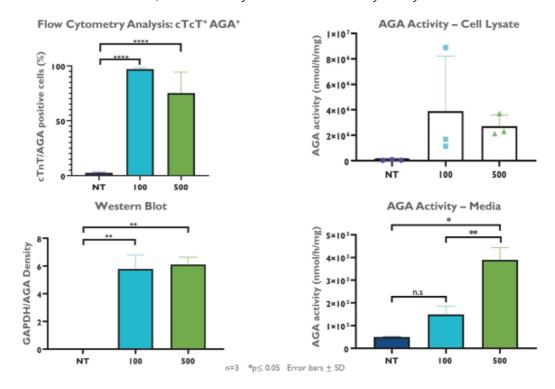
Panel B: Fabry Patient-Derived fibroblasts expressed AGA after treatment with 4D-310 as measured by IHC. AGA is visible in red, cell nuclei are shown in blue.A) vehicle control, B) 4D-310 low dose (MOI 1000), C) 4D-310 medium dose (MOI 5000), and D)4D-310 high dose (MOI 10,000)



Transduced Fabry fibroblasts were stained for AGA and DAPI/Nucleus. Vehicle, MOI 1000, MOI 5000, and MOI 10,000 (A-D, respectively) are shown. Scale bar = 100  $\mu m$ 

AGA Enzyme Expressed in Fabry Patient-Derived Cardiomyocytes After Treatment with 4D-310 Exhibited Enzymatic Activity.

Panel A: Cardiomyocytes that were differentiated from Fabry patient-derived fibroblasts expressed functional AGA after treatment with 4D-310, as measured by western blot and flow cytometry.



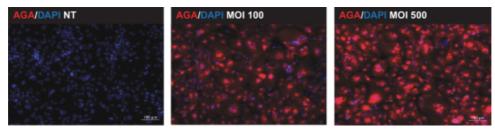
%/cTnT+/AGA+ Cells = the percentage of AGA-expressing cells within the cTnT-expressing cardiomyocyte population nmol = nanomoles

GAPDH = Glyceraldehyde 3-phosphate dehydrogenase, used as a control to show equal amounts of sample are analyzed across conditions

AGA/GAPDH Density = a quantification of the amount of AGA protein expression normalized to the amount of control protein expressed by the same cells

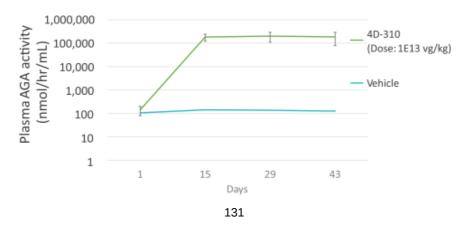
IHC = immunohistochemistry

Panel B: Cardiomyocytes that were differentiated from Fabry patient-derived fibroblasts expressed AGA after treatment with 4D-310, as measured by IHC.



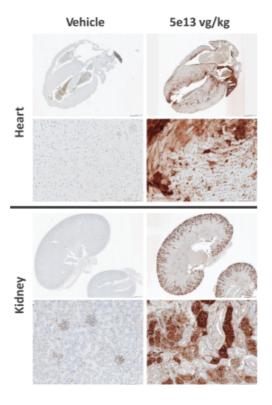
*In vivo* studies with 4D-310 are currently ongoing in both normal and Fabry disease knock-out mouse models. Plasma AGA data have shown that intravenous administration of 4D-310 to normal mice resulted in consistent and stable AGA expression and function as shown in the figure below.

Plasma AGA Function in Normal Mice After Treatment with 4D-310 Was Measured to Be Approximately 1,200-Fold Higher Than in Control Vehicle-Treated Mice on Day 15, 29 and 43 After Treatment with 4D-310. Plasma AGA Function Was Consistently Measured at Similar Levels on Days 15, 29 and 43.



Additionally, IHC data have shown that administration of 4D-310 to a knockout mouse model of Fabry disease resulted in AGA expression in both cardiac and kidney tissue as shown in the illustrative images below.

AGA Expression After Intravenous Treatment with 4D-310 Was Dose-Dependent and Widespread in Both the Heart and Kidneys of Fabry Disease Model Knock-Out Mice



# Clinical Development Plans—Phase 1/2 Trial Design

Our Phase 1/2 clinical trial initiation and the treatment of our first patient is targeted for the second half of 2020. We expect this will be a single dose escalation study carried out at multiple sites within the United States with an estimated 15 to 20 patients enrolled. We expect to treat both early-onset and late-onset patients, including those with cardiomyopathy. We anticipate the primary endpoint will be safety, plus the definition of the maximum-tolerated or -feasible dose. Pharmacodynamic biomarker assessments may include cardiac MRI, a well-established non-invasive diagnostic imaging technique that allows quantitative assessment of Gb3 substrate buildup in cardiac tissues. Additional pharmacodynamic endpoints may include changes in blood levels of Gb3 and AGA.

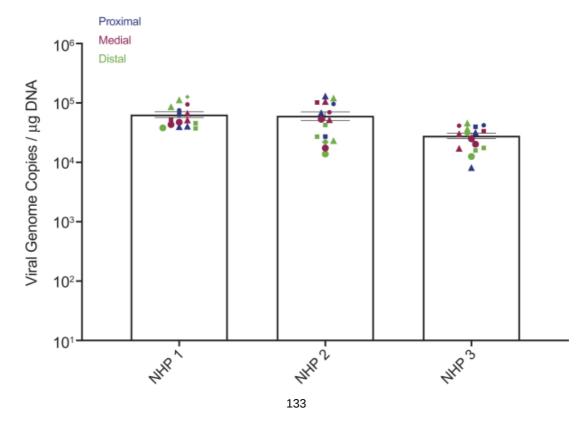
# 4D-710 for the Treatment of Cystic Fibrosis

Target Product Profile

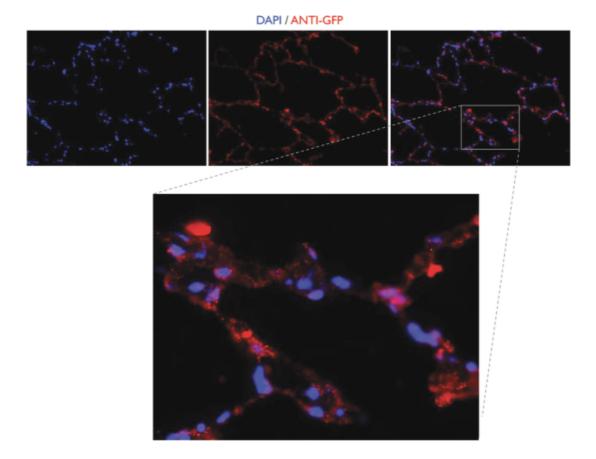
4D-710 is our wholly-owned product candidate for the treatment of Cystic Fibrosis lung disease. 4D-710 is comprised of our proprietary vector 4D-A101, and designed for an efficient, single dose aerosol delivery to the lung airways with resistance to pre-existing antibodies. The intended result is for Cystic Fibrosis transmembrane conductance regulator (CFTR) expression within lung airway cells for lung disease correction. We believe 4D-710 has the potential to treat Cystic Fibrosis patients with a broad range of mutations. Due to the fact that the CFTR coding sequence is larger than can be packaged into AAV gene therapy vectors, we have focused our efforts on assessing codon-optimized versions of a synthetic *CFTR* transgene *deltaR-CFTR*, which we refer to as *microCFTR*, a well-characterized and truncated variant that has shown activity in a large animal model of Cystic Fibrosis. The vector will carry *microCFTR*, a construct that retains the most critical components of the full-size *CFTR* gene yet is small enough to fit within AAV packaging constraints.

While we present preclinical data for components of our cystic fibrosis product candidate 4D-710 below, we have yet to conduct IND-enabling studies with 4D-710. We expect to initiate IND-enabling studies with 4D-710 in the first half of 2020. 4D-A101 is the proprietary 4DMT vector utilized in 4D-710. Its TVP was designed for relatively low dose aerosol delivery to lung in widespread fashion, with subsequent penetration of airway mucus and transduction of airway epithelial cells in the presence of human antibodies. NHP dosing with aerosolized 4D-A101 carrying the *EGFP* transgene resulted in consistent genome delivery (48 of 48 lung sites) and GFP protein expression (48 of 48 lung sites) in 100% of proximal, medial and distal airway samples tested.

Aerosol Delivery with 4D-A101 in NHP Resulted in Genome Localization and Transgene Protein Expression in All Lung Samples



Aerosol Delivery of 4D-A101 Carrying The EGFP Transgene in NHP Associated With GFP Protein Detection by IHC in Lung Airway Cells



Disease Background, Unmet Medical Need and Target Patient Population

Cystic Fibrosis is the most common fatal inherited disease in the United States and results from mutations in the *CFTR* gene. Cystic Fibrosis is a multisystem disorder affecting primarily the pulmonary, gastro-intestinal tract, pancreas and reproductive organs. Cystic Fibrosis causes impaired lung function, inflammation and bronchiectasis and is commonly associated with repeat and persistent lung infections due to the inability to clear thickened mucus from the lung, often resulting in frequent exacerbations and hospitalizations and eventual end stage respiratory failure. There is no cure for Cystic Fibrosis and the median age of death for Cystic Fibrosis patients is 39 years. Cystic Fibrosis is considered a rare, or orphan, disease by both the FDA and the EMA. According to the Cystic Fibrosis Foundation, more than 30,000 people in the United States and more than 70,000 people worldwide are living with Cystic Fibrosis, and approximately 1,000 new cases of Cystic Fibrosis are diagnosed in the United States each year. Cystic Fibrosis patients require lifelong treatment with multiple daily medications, frequent hospitalizations and, ultimately, lung transplants in some end-stage patients. The quality of life for Cystic Fibrosis patients is compromised as a result of spending significant time on self-care every day and frequent outpatient doctor visits and hospitalizations.

Until recently, approved therapies to treat Cystic Fibrosis patients were only designed to treat the symptoms of Cystic Fibrosis, for example by preventing and controlling infections that occur in the

lungs, rather than addressing the underlying cause of the disease. Accordingly, antibiotics are frequently used along with mucus-thinning drugs.

More recently, for patients with certain gene mutations, three therapies from Vertex have been approved for marketing in the United States and the European Union. Kalydeco (ivacaftor—a CFTR potentiator), Orkambi (ivacaftor plus lumacaftor—a CFTR corrector), and Symdeko (ivacaftor plus tezacaftor—also a CFTR corrector) were all approved based on their ability to improve lung function in genetically defined subsets of Cystic Fibrosis patients. Additionally, an NDA was recently filed by Vertex for the triple combination of VX-445 plus Symdeko (ivacaftor plus tezacaftor), which Vertex management believes would be applicable for up to 90% of Cystic Fibrosis patients, leaving at least 10% with no CFTR-targeted options. While these therapies improve lung function, they fall short of restoring it to the normal range in most patients, and these chronic therapies require daily dosing for the patient's lifetime.

We believe there is a clinical need and market opportunity for a durable aerosolized therapy delivered by breath-actuated nebulizer, that can restore normal CFTR function across all Cystic Fibrosis patient subgroups, including patients who are receiving combination CFTR-modulator therapies and/or do not have appreciable CFTR protein expression and are therefore not amenable to CFTR modulators.

Competition and Differentiation—Viral Vector Gene Therapy including AAV for Cystic Fibrosis

A number of biotechnology companies have pursued gene therapy solutions to treat Cystic Fibrosis, most notably Targeted Genetics. We believe these prior attempts to deliver AAV gene therapy to the lungs of cystic fibrosis patients have failed due to an inability of conventional vectors to bypass pulmonary mucus and transduce lung cells efficiently. Moreover, the mucosal immune system actively transports large quantities of antibodies into all mucus secretions, including the lung mucosa.

While a number of companies are currently pursuing other gene therapy solutions to treat Cystic Fibrosis, including with conventional AAV vectors, these product candidates are in early stages of development. Moreover, they are not, to our knowledge, comprised of AAV vectors evolved in NHP for aerosol delivery. In addition, we believe these products were not designed for resistance to pre-existing antibodies to conventional AAVs, which is potentially a key requirement for successful delivery in the lung. As a result, while we have not initiated clinical trials for 4D-710, we believe it is the only AAV gene therapy product candidate in development designed specifically with a vector selected for aerosol delivery and with antibody-resistance.

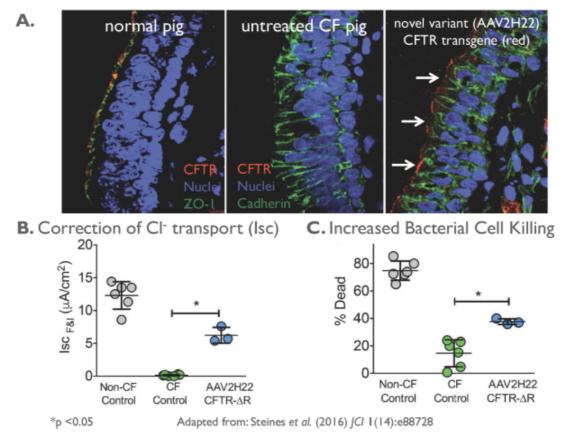
# Preclinical Proof-of-Concept Studies

Our co-founder Dr. Schaffer and his academic colleagues conducted preclinical proof-of-concept (POC) studies for utilizing directed evolution to discover vectors for delivering a corrective *CFTR* gene construct to Cystic Fibrosis lung tissue in a large animal model of Cystic Fibrosis, and in a human Cystic Fibrosis patient lung tissue model. Building on these previous POC studies, our product candidate 4D-710 will utilize a different vector (4D-A101), which has been designed to package the same *microCFTR* gene payload in a customized vector (4D-A101) that was identified through a directed evolution process for aerosol delivery in NHP. We expect to initiate IND-enabling studies with 4D-710 in the first half of 2020.

Dr. Schaffer and his colleagues first observed the potential to treat Cystic Fibrosis via aerosolized delivery of an evolved AAV variant (AAV2H22) in a pig model of Cystic Fibrosis. AAV2H22 was selected for highly efficient transduction of lung epithelial cells in pigs by conducting multiple rounds of directed evolution using aerosolized dosing in pigs. Aerosol delivery of microCFTR using the AAV2H22

vector resulted in CFTR expression in diseased pig lungs with expression patterns that resembled those observed in normal pig lungs as well as in humans (Panel A). In addition to CFTR protein expression, AAV2H22-CFTR gene therapy also resulted in a significant increase in chloride ion transport (Isc) compared to untreated controls (Panel B) as well as a reduction in bacterial colonies within the lungs of treated animals (Panel C).

Illustrative Images in Panel (A) Below Exhibit the Pattern of CFTR Expression Observed by Steines et al. in Normal Pigs, Untreated CF Pigs and CF Pigs Treated with AAV2H22 Carrying the MicroCFTR (also referred to as CFTR-deltaR) Transgene Payload (Same Transgene Utilized in 4D-710, but Different AAV Vector). The Study Involved Six Healthy Pigs, Six Untreated CF Pigs and Three AAV2H22.MicroCFTR-Treated CF Pigs. These Animals Are Represented by the Dots in Each of the Graphs (B) and (C) which Illustrate the Range of Responses Between Animals, and the Significant Difference Between Treated and Untreated CF Pigs.

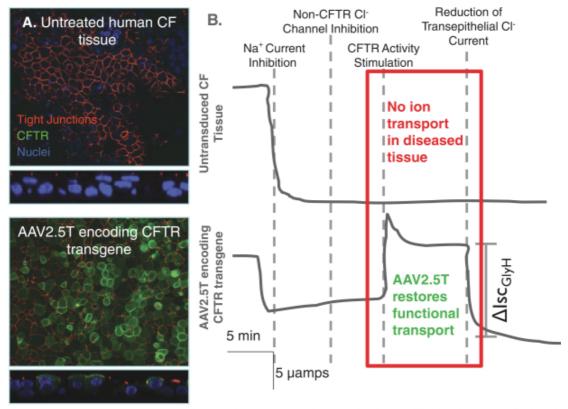


CFTRDR = MicroCFTR, Cystic Fibrosis transmembrane conductance regulator with removal of the R domain, a truncated version of the CFTR transgene engineered to fit within the payload size limitations of AAV

CI= chloride ion Isc = short circuit current, a measurement of CI- movement through cell membranes  $\mu A = microAmp$   $cm^2 = square centimeter.$ 

In addition, Dr. Schaffer and colleagues used directed evolution in an *in vitro* human organotypic air-liquid interface model of Cystic Fibrosis lung epithelium to select AAV2.5T, which we in-licensed and termed 4D-A100. In preclinical studies, AAV2.5T carrying microCFTR transduced human lung epithelial tissue and resulted in expression of functional protein as suggested by increased chloride ion (CI-) transport (Isc) as compared to untreated control (Panel B).

Excoffon et al. Previously Observed Restoration of CFTR Expression and Chloride Transport in Cystic Fibrosis Patient-Derived Airway Epithelium with AAV2.5T Vector Carrying MicroCFTR Payload (Same Payload, but Different Proprietary AAV Vector, Utilized in 4D-710).



Adapted from: Excoffon et al. (2009) PNAS 106(10):3865-70

CF = Cystic Fibrosis
Na+ = sodium ion
IscGlyH = short circuit current, a measurement of CI- movement through cell membranes
μαmps = microAmp
min = minute

We believe that these results provide evidence suggesting that the directed evolution platform enabled isolation of viral vectors that could penetrate the mucus layer of diseased Cystic Fibrosis lungs and deliver functional CFTR protein in a well-validated large animal model of the disease, as well as in human CF patient-derived organotypic lung models.

# Pharmacology and Toxicology Studies

We plan to expand on these results and to conduct dose-ranging and durability studies with 4D-710, and to carry out planned GLP toxicology and biodistribution studies to enable an IND filing and clinical trials in patients. We plan to initiate IND-enabling toxicology and biodistribution studies in the first half of 2020.

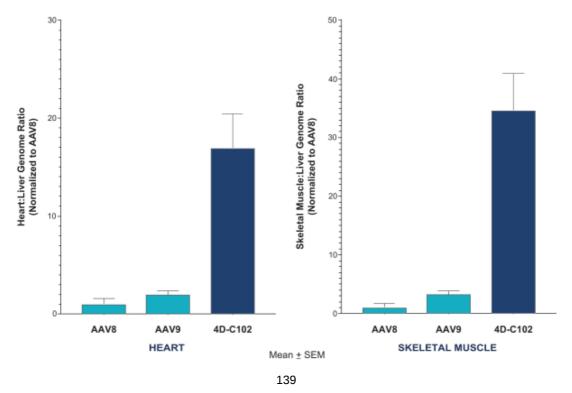
# Product Candidates in Lead Optimization for the Treatment of Duchenne Muscular Dystrophy

# **Target Product Profiles**

We wholly-own our product candidates that are in lead optimization for muscular dystrophies, including for DMD. These product candidates are designed to treat different patient populations within DMD. We are currently in lead optimization for product candidates using our proprietary 4D-C102 vector, which is designed for efficient, low dose intravenous delivery to cardiac and skeletal muscle tissues, to have minimal toxicities, and potential resistance to pre-existing antibodies to conventional AAVs in the human patient population. A second product candidate in lead optimization has been engineered with our proprietary 4D-STAR100 vector, which is designed for resistance to pre-existing antibodies to conventional AAVs in humans. Thus, this product candidate is designed for the treatment of patients who have either been excluded from receiving AAV gene therapy treatments due to their antibody titers, or who have received prior treatment with another AAV gene therapy and we believe would potentially benefit from re-dosing. The promoter used in these product candidates has been observed to be active in both cardiac and skeletal muscle cells. We are evaluating several mini-dystrophin transgene payloads that have preclinical and/or clinical proof-of-concept.

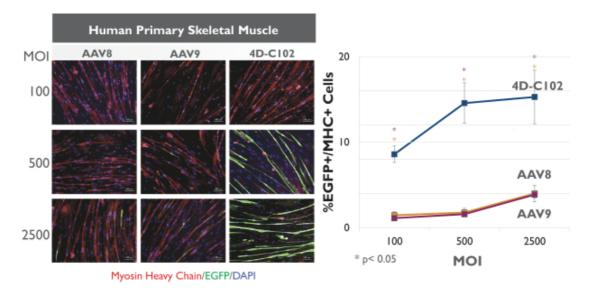
Additionally, in preclinical studies 4D-C102 improved delivery to muscle cells as compared to conventional AAV vectors, and in doing so, did not increase delivery to the liver. 4D-C102 was observed to be significantly more selective for muscle tissue versus liver tissue when compared to both AAV8 and AAV9 as demonstrated below.

4D-C102 Intravenous Targeting to NHP Skeletal Muscle and Cardiac Tissue Versus Liver Tissue was Superior to AAV8 and AAV9



Moreover, 4D-C102 transduced human cardiomyocytes, as well as skeletal muscle with EGFP more efficiently than did AAV8 or AAV9 *in vitro* as demonstrated below.

4D-C102 Exhibited Superior Transduction of Human Primary Skeletal Muscle in Vitro when Compared to Conventional AAV8 and AAV9
Vectors



MHC = myosin heavy chain, a marker of skeletal muscle cell identity

%EGFP+/MHC+ Cells = the percentage of EGFP-expressing cells within the MHC-expressing
skeletal muscle cell population, a quantification of the transduction efficiency of the capsid for the target cell type.

Disease Background, Unmet Medical Need and Target Patient Population

DMD is a progressive and fatal muscular-wasting disease, and one of the most prevalent rare genetic diseases, with an estimated prevalence of 10,000 to 15,000 cases in the United States. DMD is caused by inadequate production of functional dystrophin, a protein necessary for muscle function, as a result of mutations in the dystrophin gene. Early indicators of disease due to muscle weakness include difficulty walking or jumping, frequent falling over and becoming fatigued more easily. By approximately 12 years of age, most DMD patients will need to use a wheelchair on a regular basis. In the later stages of disease progression, life-threatening heart and respiratory conditions become common. Cardiomyopathy, heart disease due to weakened cardiac muscles, which may result in death of patients in their twenties.

Competition and Differentiation—Viral Vector Gene Therapy with Conventional AAV for DMD

Sarepta is commercializing Exondys 51 for patients who have a confirmed mutation of the *DMD* gene amenable to exon 51 skipping, which affects about 13% of the population with DMD. Currently, Pfizer, Sarepta and Solid Biosciences are developing AAV therapy treatments for DMD. All three are in early clinical trials in boys with DMD with products utilizing conventional AAV vectors administered by IV infusion.

Preclinical proof-of-concept has been observed in a dog model of DMD in which a truncated version of the dystrophin gene, microdystrophin, was delivered using conventional AAV leading to stable expression and prevention of dystrophic damage in skeletal muscles; long-term effects on heart

function were not reported. The AAV vectors used in clinical-stage products to date are naturally-occurring conventional AAV capsids AAVrh74 (similar to AAV8) and AAV9 and therefore are not specifically targeted to human cardiac or skeletal muscle tissue. AAV gene therapy in DMD patients treated intravenously by Nationwide Children's Hospital and Sarepta has resulted in transgene expression within skeletal muscle biopsies and a decrease in serum markers of muscle inflammation including creatine kinase. Sarepta has also reported anecdotal improvements in boys' functional performance without signs of adverse effects after nine months. AAV gene therapies for DMD utilizing these conventional vectors have been subject to inflammation-related toxicity issues and manufacturing challenges due to the requirement for relatively high doses.

In addition to requiring high doses, patients have been excluded from clinical trials due to significant pre-existing neutralizing antibody titers to AAVrh74 and AAV9. This antibody-based exclusion from treatment limitation increases in frequency with patient age. Finally, as patients grow and develop more muscle mass following AAV treatment, re-dosing with AAV gene therapy would likely be beneficial. AAV vectors that are not neutralized by antibodies to AAVrh74 and AAV9 would therefore be highly desirable for subsequent treatment. We believe our proprietary vectors that were selected for resistance to antibodies to conventional AAVs have potential in this clinical situation.

Pharmacology and Toxicology Programs

We expect to complete lead optimization and to identify our lead research product candidate in 2020.

#### **Ophthalmological Diseases**

We have a broad partnership with Roche in ophthalmology, excluding ddRNAi delivery where we retain full ownership. Roche is a leader in ophthalmology biotherapeutics development and commercialization. In the ophthalmology field, we and Roche both have the right to declare product candidates. Roche has an option to acquire rights to product candidates declared by us prior to initiation of a pivotal clinical trial by paying an option fee as well as milestone and royalty payments that are higher than for Roche-declared product candidates. If exercised, Roche will assume all commercial and development-related costs.

Approximately 200 rare genetic diseases of the eye have been described, in addition to large market indications such as macular degeneration, diabetic retinopathy and glaucoma. The retina is an attractive tissue to target using gene therapy for the following reasons:

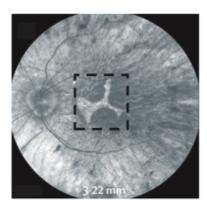
- Therapeutics can be easily administered locally (with intravitreal delivered vectors only)
- · The small volume of retinal tissue requires minimal doses
- The eye is a relatively immune-privileged organ
- A number of clinically-validated endpoints, with rapid readouts, have been defined

Initial AAV gene therapy products for the eye have displayed limitations associated with conventional AAVs. The eye is a complex organ composed of multiple types of cells and tissues such as retina, optic nerve, cornea and the iris. We believe gene therapies are effective only when target cells and tissues receive and express the proper transgene in a widespread fashion. Naturally-occurring conventional AAV vectors such as AAV2 do not transduce the primate retina efficiently after intravitreal injection, likely due to the inner-limiting membrane acting as a barrier to AAV.

As a result, the majority of conventional AAV product candidates studied in humans, including the marketed product Luxturna (voretigene neparvovec-rzyl) from Spark Therapeutics, are administered by subretinal injection surgery, a complex procedure performed in an operating room by highly-specialized

retinal surgeons. This route of administration, typically using AAV2, generally results in a minority "bleb" area of the retina being transduced. Investigators have reported transduction of less than 10% of the retina in primates and humans. Therefore, in most patients, the majority of retinal tissue is left untreated. In addition, retinal detachment can occur, and cataracts routinely result after subretinal injections. Finally, ophthalmic surgeons must be specially trained for this operator-dependent surgical technique, thus limiting the number of sites and ophthalmologists who can perform the surgery.

Representative Bleb Area of the Retina (White Area Inside Black Box) as Treated by Subretinal Injection of an AAV Gene Therapy Candidate.



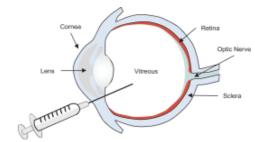
We are developing a product to be administered by intravitreal injection, which includes a vector that tranduces the entire retinal surface area. Intravitreal injection is performed routinely thousands of times every day in the United States and European Union for administration of anti-VEGF protein therapeutics for Wet AMD. Procedure-related complications are uncommon, and the procedure can be carried out in the majority of out-patient eye clinics in the United States.

# Key Aspects of the Intravitreal and Subretinal Ocular Injection Routes Which We Considered when Designing Our Target Product Profile Are Listed Below

# Subretinal Retina Optic Nerve Comea Sclera

Not used in routine clinical practice

# intravitreal



Retinal coverage

Bleb area only (estimated <10% of total retina surface

area)

No

Yes

No

~80%

Simultaneous

Bilateral injection possible

# Injection procedures

performed in U.S.

(daily)

Operating room

required

Routine out-patient procedure

Cataract development risk

due to associated vitrectomy Up to 100% of total retina surface area

Yes

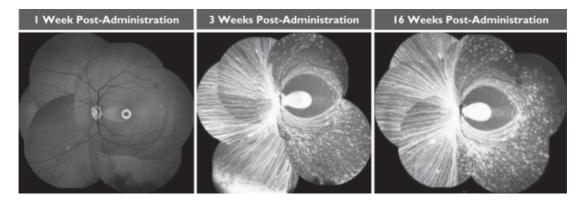
Estimated >5,000 per day

No Yes

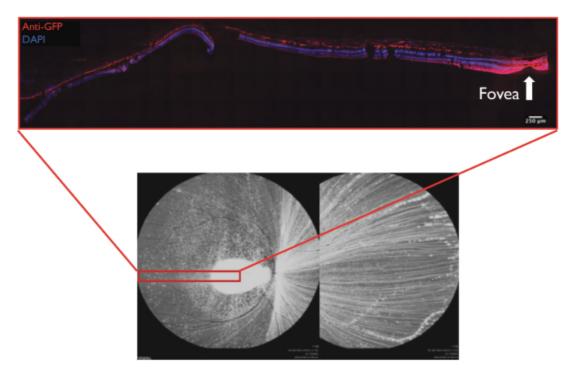
N/A

We utilized our Therapeutic Vector Evolution platform to create and discover proprietary vectors we believed to be suitable for intravitreal injection and transduction of the retina. Assuming our preclinical results are substantiated by our planned clinical trials, we believe the use of intravitreal injection will result both in a more accessible mode of administration and a higher percentage of retinal cells that express our proprietary vectors.

Widespread, Robust Retinal Transduction and Transgene Expression (EGFP) in NHP Retina Observed Following Administration of 4D-R100 Over Time (Heidelberg Spectralis Images, in Life. EGFP Represented in White)



4D-R100 Transduced Multiple Layers of NHP Retina, Including Retinal Pigmental Epithelial Cells, Photoreceptors and Retinal Ganglion Cells, as Exhibited in the Representative Image Below. EGFP Transgene Protein Expression Is Represented in Red.



# 4D-125 for the Treatment of XLRP

Target Product Profile

We are developing 4D-125 for the treatment of XLRP. This product candidate is comprised of our proprietary intravitreal vector 4D-R100 carrying a codon-optimized *RPGR* transgene and designed to address all stages of XLRP. In non-human primate models, we have observed widespread

transduction and transgene expression across the entire retinal surface, in contrast to localized results with subretinal injection of AAV vectors. IND-enabling studies with 4D-125 are expected to be completed in 2019, and 4D-125 is expected to enter clinical trials in mid-2020.

Disease Background, Unmet Medical Need and Target Patient Population

XLRP is a rare inherited X-linked recessive genetic disorder, which causes progressive vision loss and blindness in boys and young men. It is characterized by dysfunction and degeneration of photoreceptors in the retina. Seventy percent of cases are caused by mutations in the *retinitis pigmentosa GTPase regulator* (RPGR) gene. Loss of RPGR function in retinal cells causes the progressive loss of rod and cone photoreceptors, leading to the loss of vision experienced by patients. Symptoms of XLRP are initially characterized by night blindness, followed by loss of peripheral visual field, decreasing visual acuity and eventually blindness. Males are usually the most affected with symptoms starting in the early years of life. The estimated worldwide prevalence of XLRP due to RPGR variants is approximately one in 40,000 people, which represents approximately 20,000 patients in the United States and European Union.

Competition and Differentiation—Viral Vector Gene Therapy including AAV for XLRP

Conventional AAV gene therapy approaches are being developed to treat XLRP by delivering a functional copy of the *RPGR* gene by subretinal injection. Similar to treatment of other rare retinal diseases with AAV gene therapy, these XLRP therapies require delivery by subretinal injections; thus, only a small fraction of the retina is transduced and treated directly. Nightstar Therapeutics, recently acquired by Biogen, is developing NSR-RPGR for XLRP. Investigators reported interim Phase 1 clinical trial data in September 2018. Investigators concluded that microperimetry endpoints, which measure visual field function, improved in a subset of patients; they stated that "clinical proof-of-concept" was achieved with this data on visual improvement. AGTC and MeiraGTx are also developing AAV gene therapy product candidates for XLRP to be administered by subretinal injection.

While 4D-125 also expresses the *RPGR* transgene, in contrast it is designed for intravitreal delivery. In addition, we believe intravitreal dosing may allow for treatment of patients earlier in their disease course, prior to loss of visual field, than those who are typically included in subretinal injection trials. In this patient population, our goal will be to prevent vision loss.

# Pharmacology and Toxicology Studies

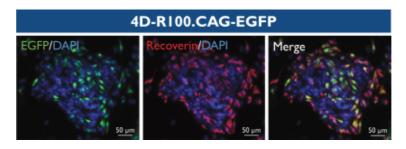
In 2019, we initiated and expect to complete 4D-125 IND-enabling toxicology and biodistribution studies in NHP. We utilized the same GLP toxicology study design that we submitted in the IND application for 4D-110. We plan to complete pharmacology studies in XLRP patient-derived stem cells and photoreceptor cells in 2019; results to date demonstrate RPGR expression and function in XLRP patient-derived cells.

The below images of photoreceptors that were differentiated from fibroblast-derived induced pluripotent stem cells demonstrate that 4D-R100 (the 4D-125 capsid) carrying the CAG promoter and EGFP transgene transduced these photoreceptors to express the EGFP protein.

Human Fibroblast-Induced Pluripotent Stem Cell-Derived Photoreceptor Cultures Were Transduced With 4D-R100.CAG-EGFP (Green).

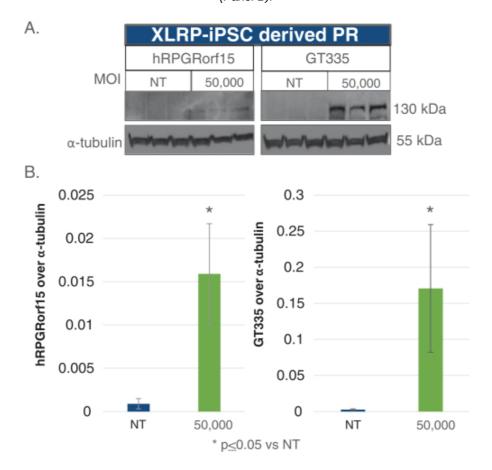
Transduction of Recoverin Positive Cells (Red) – a Marker for Photoreceptors – Following R100.CAG-EGFP Infection at a MOI 10,000 is

Shown as Imaged Fourteen Days After Treatment. Cell Nuclei Are Stained in Blue.



In an in vitro model of the disease, XLRP patient photoreceptors were derived from XLRP diseased fibroblasts which had been reprogrammed into induced pluripotent stem cells. Diseased photoreceptors were transduced with 4D-125 at a MOI of 50,000 and protein lysates were harvested 30 days post transduction. 4D-125 transduced cells expressed significantly more hRPGRorf15 than control cells as illustrated in the figures below. Moreover, this expressed protein was shown to be active as measured by glutamylation (GT335). Additional pharmacology studies in XLRP patient-derived cells are currently ongoing.

4D-125 Treatment-Induced Increase in hRPGRorf15 Protein and Glutamylation of hRPGRorf15 (GT335) Compared to Untreated Controls Was Observed Via Western Blot (Panel A). Quantification of Blot Band Intensity Indicated a Significant Increase (p£0.05) in hRPGRorf15 Protein and Functionality of the Expressed Protein as Measured by Glutamylation (GT335) of hRPGRorf15 for 4D-125 Versus the Control (Panel B).



Clinical Development Plans- Phase 1/2 Clinical Trial Design

We are targeting initiation of our planned 4D-125 Phase 1/2 clinical trial in mid-2020. We expect the Phase 1/2 clinical trial will be a single ascending dose trial of intravitreal injection with 4D-125 in patients with clinically-significant XLRP due to *RPGR* gene mutation. We anticipate the primary endpoint will be safety through six months after treatment, and definition of the maximum-tolerated or -feasible dose. We plan to assess efficacy based on functional visual field of the retina (e.g. with microperimetry) within three to six months after treatment. In addition, changes in retina field area over time will be measured by optical coherence tomography (OCT). Additional efficacy endpoints may include changes in functional endpoints including visual acuity. We expect that the earliest biomarker readouts from the Phase 1/2 clinical trial could be obtained by

#### 4D-110 for the treatment of Choroideremia

# Target Product Profile

We are developing 4D-110, based on our proprietary intravitreal vector 4D-R100, for the treatment of the rare blinding disease Choroideremia. 4D-110 is designed to deliver the *CHM* transgene, the dysfunctional gene in Choroideremia, to RPE cells. We have partnered this program with Roche. We are responsible for filing and maintaining the IND, and for conducting the Phase 1/2 clinical trial(s). We filed an IND application for 4D-110 in 2019. The FDA did not identify any hold issue and the IND was allowed to proceed. Roche will be responsible for conducting any pivotal clinical trials and commercialization, if approved. Our Choroideremia program is also supported by the Choroideremia Research Foundation. We plan to initiate a Phase 1/2 clinical trial in Choroideremia patients in the second half of 2020.

# Disease Background, Unmet Medical Need and Target Patient Population

Choroideremia is an X-linked, slowly-progressive, degenerative disease of the retina and choroid of the eye caused exclusively by mutations in the *CHM* gene which codes for the REP1 protein. It is estimated approximately 10,000 individuals in the United States and the European Union have Choroideremia, with similar prevalence rates globally. While Choroideremia primarily affects men, some heterozygous females also suffer variable visual loss from the condition. No products are approved currently for the treatment of this disease in the United States or European Union.

Choroideremia initially manifests as night-blindness and peripheral visual field defects, usually starting in the first two decades of life. As the disease progresses, the visual field begins to constrict relatively early in the disease's progression, which hinders patients' ability to conduct daily activities. Many Choroideremia patients are blind by the middle decades of life. A patient with advanced disease will be legally blind by virtue of poor visual acuity and minimal preserved visual field.

Almost all mutations in the *CHM* gene result in production of a non-functional REP1, protein. REP1 is essential for the activation (prenylation) of Ras-associated binding (Rab) proteins involved in intracellular vesicle trafficking.

#### Competition and Differentiation—Viral Vector Gene Therapy including AAV for Choroideremia

Conventional AAV gene therapy approaches are being developed to treat Choroideremia. Similar to treatment of other rare retinal diseases with AAV gene therapy, these Choroideremia therapies require delivery by subretinal injection; thus, only a small fraction of the retina is transduced and treated directly. Nightstar Therapeutics, recently acquired by Biogen, is developing a product candidate for Choroideremia called NSR-REP1. In 2018, data was reported from a Phase 1/2 clinical trial in patients with end stage Choroideremia, with reduced visual acuity. In this trial, investigators concluded that best corrected visual acuity improved in a subset of patients. A pivotal randomized Phase 3 clinical trial was initiated in 2018; this trial is enrolling patients with advanced disease with decreased visual acuity.

4D-110, which has not yet begun clinical trials, also expresses the *CHM* transgene. We believe the product candidate's intravitreal delivery data, which showed widespread transduction of the retina in NHPs, suggests that intravitreal treatment of Choroideremia patients with 4D-110 may result in better outcomes than can be achieved with subretinal approaches utilizing conventional AAV vectors. In addition, we believe intravitreal dosing may allow for treatment of earlier stage patients than is typical with subretinal injection trials.

# Pharmacology and Toxicology Program

A total of 43 NHPs have been treated with the 4D-R100 vector, including 30 which were treated with 4D-110 (4D-R100 vector carrying the transgene insert for the *CHM* gene). In all 13 animals treated with 4D-R100 containing an EGFP gene construct (dose range 1E10 to 1E12 vg/eye), widespread and dose-dependent retinal GFP-induced fluorescence was detected in retinal pigmented epithelia, photoreceptors, and retinal ganglion cells across central, macula and peripheral regions of the retina. EGFP was detected as early as one week following vector administration and persisted through to study completion at six months. No meaningful toxicities were reported.

In 2018, we completed an IND-enabling toxicology and biodistribution study in 27 NHPs with 4D-110. 4D-110 was administered at doses of 1E11 vg/eye or 1E12 vg/eye by intravitreal injection. Animals were sacrificed at three weeks, three months or six months. No meaningful toxicities were reported, and retinal transduction and transgene expression was observed at both dose levels. No retinal toxicity was noted; typically mild, transient corticosteroid-responsive anterior uveitis was reported in a minority of treated NHP. In animals treated with 4D-110, we detected vector genomes and CHM/REP1 transgene RNA expression in all non-human primate retinas; the genome and RNA levels were higher in the high dose animals.

Moreover, in preclinical studies involving human CHM patient-derived RPE cells, 4D-110 delivered the *CHM* gene resulting in REP1 protein expression as shown in green in the right panel below. Moreover, this expressed REP1 protein functionally corrected RAB27A trafficking from the cytoplasm to the cell membrane. In similar fashion to normal RPE cells in the leftmost panel below, 4D-110-treated CHM cells in the rightmost panel had RAB27A protein associated with their cell membranes as shown in red in both panels; this finding confirms the functionality of the REP1 protein expressed from 4D-110. By contrast, untreated diseased CHM cells in the center panel did not exhibit a pattern of cell membrane associated RAB27A. In these cells, RAB27A was instead seen diffusely throughout the cytoplasm.

4D-110-Transduced RPE Cells Expressed Functional REP1 and Exhibited Corrected Trafficking of RAB27A

Left Panel: Healthy Human RPE Cells Exhibited Normal REP1 Expression in Green and Trafficking of RAB27A to the Cell Membrane in Red

Center Panel: Un-transduced CHM Patient-Derived RPE Cells Exhibited No Visible REP1 (i.e. No Visible Green Staining) and Un-trafficked RAB17A Diffusely Throughout the Cell in Red. Right Panel: 4D-110-Transduced RPE Cells Exhibited Clear REP1 Expression in Green as well as Corrected RAB17A Trafficking to the Cell Membrane in Red.

Normal Human RPE Cells	CHM RPE Cells	4D-110 Corrected CHM RPE Cells
REP1/RAB27A/DAPI	REP1/RAB27A/DAPI	REP1/RAB27A/DAPI
<u></u>		

Clinical Development Plans—Natural History Patient "Run-In" Study

We have fully enrolled a natural history trial of approximately 50 patients with CHM to document the rate of visual and anatomical decline, and to identify candidates who are most likely to benefit from

participation in our planned Phase 1/2 clinical trial. Patients have been followed for up to two years in most cases; consistent and statistically-significant decreases in OCT and visual function endpoints have been reported by investigators over six and 12 months. We expect that some of these subjects will enroll in our planned Phase 1/2 clinical trial or other future trials we may conduct.

#### Clinical Development Plans—Phase 1/2 Trial Design

We plan to initiate a Phase 1/2 clinical trial for 4D-110 in the second half of 2020. The trial will be a single ascending dose trial of intravitreal injection with 4D-110 in patients with clinically-significant CHM due to *CHM* gene mutation. The primary endpoints will be safety through six months after treatment, and definition of the maximum-tolerated or -feasible dose. Efficacy will be assessed by measuring changes over time in both best corrected visual acuity as well as OCT endpoints. Endpoint changes over time before treatment will be compared to endpoint changes after 4D-110 treatment in the same patients whenever possible.

#### Research Candidates for the Treatment of Wet AMD

#### **Target Product Profiles**

In addition to rare ophthalmic diseases, we are also evaluating AAV gene therapy candidates for large market diseases. We have constructed product candidates in lead optimization, using the 4D-R100 vector, designed to deliver various *anti-VEGF* transgene candidates to treat Wet AMD and other related disorders. We believe these *anti-VEGF* transgene candidates express proteins that have a high degree of similarity to approved anti-VEGF protein therapeutics, which we believe may increase the likelihood of technical success with these product candidates.

# Disease Background, Unmet Medical Need and Target Patient Population

Wet AMD is a type of macular degeneration where abnormal blood vessels (choroidal neovascularization) grow into the macula of the retina. As a consequence, retina swelling (edema) and bleeding can occur and cause visual distortion and reduced acuity. The proliferation of abnormal blood vessels in the retina is stimulated by VEGF. This process distorts and can potentially destroy central vision, and may progress to blindness without treatment. There are on average 200,000 new incidences of Wet AMD per year in the United States alone. Wet AMD accounts for 10% of all diagnosed cases of AMD, but it results in 90% of the legal blindness caused by all types of AMD. High expression levels of VEGF appear to play a causal role in the symptoms of Wet AMD.

The current treatment paradigm for Wet AMD is intravitreal injection of patients with anti-VEGF proteins which inhibit the proliferation of new blood vessels, reducing edema and bleeding and some visual acuity to be recovered. Most anti-VEGF therapies require repeated intravitreal injections every few months to obtain full efficacy. However, based on current clinical experience, after several years of treatment, the early vision gains are frequently lost and acuity declines are observed for reasons that may include variable treatment regimens and patient compliance.

We believe Wet AMD is an ideal candidate application for gene therapy. There are multiple products on the market that validate the anti-vascular endothelial growth factor or anti-VEGF therapeutic approach. Delivering intravitreal gene therapies to the eye is straightforward, and there is an advantage for a single dose therapy that can provide long-term efficacy in patients for whom compliance, or treatment resistance, is a problem.

# Competition and Differentiation—Viral Vector Gene Therapy including AAV for Wet AMD

Conventional AAV gene therapy approaches are being developed to treat Wet AMD by delivering a functional copy of an *anti-VEGF* gene by either subretinal injection or intravitreal administration. To

our knowledge, these assets are in Phase 1 or Phase 1/2 clinical trials. To our knowledge, our product candidate would be the only one to utilize an intravitreal vector (4D-R100) discovered through directed evolution in NHP.

# Pharmacology and Toxicology Program

To date, we have engineered at least four research candidates with potential for anti-VEGF activity. Our *in vitro* experiments have shown expression of the therapeutic transgenes from treated RPE cells, which resulted in VEGF binding and neutralization. We expect to complete pharmacology and pilot toxicology studies for research candidates utilizing 4D-R100 in NHP in 2020.

# Promoter Discovery Platform: Directed Evolution

We are generating next-generation optimized promoter elements through directed evolution. Currently-available promoters may lack sufficient strength of expression and selectivity for clinical benefit of AAV gene therapies. In addition, for some AAV gene therapy products a smaller promoter region may be essential for the gene payload to fit in the AAV. Therefore, we believe there is a need for better promoters for many AAV products to enable or enhance their therapeutic benefit. We generate Target Promoter Profiles for any given product and disease target. This promoter profile includes target cell specificity and strength in order to maximize safety and/or efficacy, as well as any necessary size constraints. Under one of the SRAs with the UC Regents, we are working with our co-founder Dr. Schaffer to create customized and proprietary promoters for use in our products. Libraries of novel and diverse synthetic promoters have been engineered, currently comprising approximately five million unique sequences, and promoter selections will be applied to identify the best promoters within the libraries for any Target Promoter Profile.

# **Human Cell and Disease Modeling**

In order to fulfill our strategy to extensively characterize our vectors and products prior to treatment in patients, we have designed and developed a robust in-house human cell and disease modeling platform. Our group of scientists is experienced in the use of induced pluripotent stem (iPS) cell and embryonic stem (ES) cell techniques to generate and characterize human cell models to evaluate vector transduction, transgene expression and transgene product function. This core resource allows us to perform detailed *in vitro* pharmacology and toxicology evaluations together with head-to-head comparisons between different AAV capsids, promoter elements and transgene payloads. We have differentiated and characterized the following human cell types:

#### Cell Cultures:

- Ventricular phenotype cardiomyocytes (cardiac muscle cells)
- · Skeletal muscle myofibers
- · Lung epithelial cells
- · Retinal cells: RPE, photoreceptors and ganglion cells
- · Hepatocytes (liver cells)
- Neural cells
- · Endothelial cells

# Tissue Cultures and Organotypic Tissues:

- Lung Air Liquid Interface
- · Optic vesicles

Of note, these cells and models can be derived from both normal human donors and from patients with specific genetic diseases of interest. Patient-derived cells used in our preclinical pharmacology studies include RPE cells from Choroideremia patients, retinal cells from patients with XLRP and cardiomyocytes, fibroblasts and endothelial cells from patients with Fabry disease.

#### Manufacturing

In order to fulfill our strategy to maximize the robustness and internal control of our manufacturing processes from discovery and process development through to clinical-grade cGMP manufacturing, we have designed and are continually developing a robust in-house manufacturing platform. Some companies in the field in-license clinical trial material or manufacturing technologies from other companies or academic manufacturing centers. In contrast, our manufacturing processes were developed internally using internal technology transfers from our own process development labs. Our current in-house manufacturing capabilities include GLP production capabilities for IND-enabling studies and GMP production for early stage clinical trials. We intend to further scale these capabilities to support later stage programs and indications requiring higher doses. In addition to our internal activities, we also work with CMOs with person-in-plant CMO production oversight, with work currently ongoing at Paragon.

# Process and Capabilities

We use robust, scalable and transferable manufacturing unit operations throughout the vector characterization process and product development, which are both platform and product-specific. The upstream manufacturing step involves triple plasmid transfections in an adherent HEK293 mammalian production cell line. Downstream manufacturing steps for purification and concentration include multiple orthogonal column chromatography steps and tangential flow filtration. The columns used in our process are from stable sources including General Electric. Using internally developed manufacturing processes and testing, we characterize our novel capsids and payloads. In addition, leveraging internal expertise and capabilities, we package and test our novel capsids with payloads using non-GLP manufacturing processes.

Our team has extensive experience with the manufacturing and analytical testing of numerous unique AAV capsids. We have developed and qualified assays for characterization, in-process testing and release and stability testing of our internally-manufactured novel AAV vectors. To date, our team has internally manufactured approximately 50 unique AAV vector capsids, including both proprietary evolved 4DMT capsid variants and naturally-occurring capsids. Our team has manufactured over 100 total lots of AAV vectors. This total includes 9 lots of material for GLP toxicology and biodistribution studies. We initiated our first in-house manufacturing run for clinical trial material for use in patients, according to cGMP, in 2019. Leveraging internal testing capabilities in addition to qualified contract testing laboratories, we fully test and release our GLP and GMP lots used for toxicology and clinical trials studies, respectively.

# **Facilities**

Our manufacturing facilities are on site at company headquarters in Emeryville, California and include a process development lab, an analytical development lab, and a larger-scale manufacturing facility. Our manufacturing facility is approximately 800 square feet, and we intend to build a second cGMP facility that is approximately 8,000 square feet. In this new facility, we expect to utilize large-scale bioreactors that are designed to enable higher titer clinical trial material lots for clinical trials. These manufacturing facilities are also designed for production of material for GLP toxicology and biodistribution studies.

#### Team

Our team of approximately 20 highly-trained individuals is led by our Chief Technical Officer, Dr. Kamal. Collectively, they have significant experience in viral vector manufacturing, chemistry-manufacturing-controls (CMC), regulatory affairs, analytical and process development, and quality assurance and controls. Our team also has experience with manufacturing multiple AAV vectors from preclinical studies through to multiple Phase 3 trials. For example, Dr. Kamal helped to write and compile the AAV gene therapy BLA for AVXS-101 or Zolgensma (Novartis).

# **Intellectual Property**

Our commercial success depends in part on our ability to obtain and maintain proprietary protection for our product candidates, manufacturing and process discoveries, and other know-how, to operate without infringing the proprietary rights of others and to prevent others from infringing our proprietary rights. Our policy is to seek to protect our proprietary position by, among other methods, filing U.S. and foreign patent applications related to our proprietary technology, inventions and improvements that are important to the development and implementation of our business. We also rely on trade secrets, know-how, continuing technological innovation and potential inlicensing opportunities to develop and maintain our proprietary position.

Our product and lead optimization candidates were discovered by us utilizing our proprietary technology. We have filed several non-provisional and provisional patent applications, all owned by us, relating to our product and lead optimization candidates in the United States, certain foreign countries, and the World Intellectual Property Organization that are directed to compositions-of-matter, dosage unit forms, methods-of-treatment and medical use. We have also licensed several non-provisional patent applications, granted patents and international patent applications relating to our product and lead optimization candidates from U.C. Berkeley.

Our solely owned patent portfolio includes one pending U.S. non-provisional application, thirty-one pending foreign applications, one of which has been allowed, one granted foreign patent and two pending international applications. We expect that United States and European patents and the patent applications in this portfolio, if issued, would expire between May 12, 2037 and November 26, 2038, excluding any additional term from patent term adjustment or patent term extension if appropriate maintenance and other governmental fees are paid. Additional patent term for the presently-issued or later issued U.S. patents may be awarded as a result of the patent term extension provision of the Hatch-Waxman Amendments of 1984. In the European Union member countries, a supplementary protection certificate, if obtained, provides a maximum five years of market exclusivity.

In other jurisdictions (currently, Australia, Bahrain, Brazil, Canada, Chile, China, Colombia, Costa Rica, Egypt, India, Indonesia, Iran, Israel, Japan, Korea, Kuwait, Malaysia, Mexico, New Zealand, Oman, Peru, Philippines, Qatar, Russia, Saudi Arabia, Singapore, Thailand, United Arab Emirates, Ukraine, Vietnam and South Africa), the patent applications relating to our product and lead optimization candidates, including composition of matter and various other patents, including dosage unit form, method-of-treatment and medical use patents, where applicable, are expected to expire between May 12, 2037 and November 26, 2038, if the appropriate maintenance, renewal, annuity, and other government fees are paid. These patents and patent applications (if applicable), depending on the national laws, may benefit from extension of patent term in individual countries if regulatory approval of any of our product or lead optimization candidates is obtained in those countries. For example, in Japan, the term of a patent may be extended by a maximum of five years in certain circumstances.

Our in-licensed patent portfolio includes four granted U.S. patents and six granted foreign patents; each of these patents is expected to expire between June 28, 2024. and June 13, 2029. Our

in-licensed patent portfolio also includes three pending U.S. non-provisional patent applications, five pending foreign patent applications and one pending international patent application.

In other jurisdictions (currently, Canada, China, France, Germany, Great Britain, Hong Kong, Japan and Italy), the term and patent applications relating to our product and lead optimization candidates, including composition of matter and various other patents, including dosage unit form, method-of-treatment and medical use patents, where applicable, are expected to expire between June 29, 2024 and June 28, 2038, if the appropriate maintenance, renewal, annuity, and other government fees are paid. These patents and patent applications (if applicable), depending on the national laws, may benefit from extension of patent term in individual countries if regulatory approval of any of our product or lead optimization candidates is obtained in those countries. For example, in Japan, the term of a patent may be extended by a maximum of five years in certain circumstances.

Individual patents extend for varying periods depending on the date of filing of the patent application or the date of patent issuance and the legal term of patents in the countries in which they are obtained. Generally, patents issued for regularly filed applications in the United States are effective for 20 years from the earliest effective non-provisional filing date. In addition, in certain instances, a patent term can be extended to recapture a portion of the U.S. Patent and Trademark Office (USPTO) delay in issuing the patent as well as a portion of the term effectively lost as a result of the FDA regulatory review period. However, as to the FDA component, the restoration period cannot be longer than five years and the total patent term including the restoration period must not exceed 14 years following FDA approval. The duration of foreign patents varies in accordance with provisions of applicable local law, but typically is also 20 years from the earliest effective filing date. The actual protection afforded by a patent varies on a product by product basis, from country to country and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent.

We also protect our proprietary technology and processes, in part, by confidentiality and invention assignment agreements with our employees, consultants, scientific advisors and other contractors. These agreements may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our employees, consultants, scientific advisors or other contractors use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Our commercial success will also depend in part on not infringing the proprietary rights of third parties. It is uncertain whether the issuance of any third-party patent would require us to alter our development or commercial strategies, alter our drugs or processes, obtain licenses or cease certain activities. Our breach of any license agreements or failure to obtain a license to proprietary rights that we may require to develop or commercialize our future drugs may have a material adverse impact on us.

#### **Strategic Collaborations**

# Collaboration and License Agreement with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc.

In November 2017, we entered into a Collaboration and License Agreement (the Roche Agreement), with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., collectively referred to as Roche. Under the Roche Agreement, we granted Roche an exclusive, sublicenseable, worldwide license under certain intellectual property rights to research, develop, make, use, import, export, and sell products and constructs using our proprietary AAV vectors to treat ophthalmological diseases and

disorders, excluding treatment and prevention of cancer and central nervous system conditions (but not retinal nerves) and delivery of DNA-directed RNA interference (the Roche Field).

Under the terms of the Roche Agreement, we and Roche will engage in collaboration programs to develop one or more products, and Choroideremia has been designated as the first collaboration program. We are primarily responsible for the initial development of such collaboration programs and Roche agreed to reimburse us for our development costs and expenses in accordance with the terms of the agreement. Upon completion of such initial development, we will transfer data, know-how and regulatory filings to the applicable collaboration program to Roche and Roche will be responsible for the development and commercialization of such program at its own cost and expense.

Subject to the terms of the Roche Agreement, either party may also develop one or more programs in the Roche Field independent of the other party at such party's own cost and expense. Roche has an option to elect one or more of the programs that we may independently develop under the agreement, including XLRP, which we have designated as our initial independent program. If Roche exercises its option, and subject to its payment of the applicable option exercise fee, we will transfer our data, know-how and regulatory filings related to such programs. If Roche does not exercise its option within the applicable option period, we will have the sole right to commercialization of such product. Each party agreed to various diligence obligations under the agreement.

Pursuant to the Roche Agreement, we received an upfront payment from Roche of \$21.0 million. In addition, we are entitled to contingent payments including (i) \$1.0 million for each Roche nominated product beyond the first three, (ii) up to \$30.0 million upon exercise of the option to convert a product we nominated and developed prior to pivotal clinical studies, (iii) up to \$223.0 million in specified development milestones in connection with the licensed products, \$86.0 million of which relate to Choroideremia; and (iv) sales-based milestones of up to \$123.0 million based on worldwide calendar year net sales in connection with licensed products. On a product-by-product basis, Roche will also be required to pay us tiered royalties for worldwide calendar year net sales of products at percentages ranging from the mid-single digit to mid-teens, in each case subject to reductions in accordance with the terms of the agreement. The royalties are payable on a product-by-product and country-by-country basis until the later of ten years after the date of first commercial sale of such product in such country and the expiration of the last-to-expire licensed patent right covering such product.

The Roche Agreement will expire on the later of expiration of all payment obligations and the date when no products are actively developed by either party or both parties in accordance with the terms of the agreement. Either party may terminate the agreement in its entirety or on a country-by-country basis if the other party fails to cure its material breach within 90 days of receiving notice. Roche may terminate the agreement in its entirety, on a product-by-product basis or on a country-by-country basis upon 90 days' prior written notice. If we terminate the agreement for Roche's material breach or if Roche terminates the agreement without cause, the rights to the products generally revert back us. If we commercialize reverted products after such termination, we may be required to pay Roche tiered royalties for worldwide calendar year net sales of such products at percentages ranging from zero to the low-teens, in each case subject to reductions in accordance with the terms of the agreement. If Roche terminates the agreement for our material breach, Roche may retain its rights under the license that we grant to Roche under our intellectual property rights and Roche's payment obligations will survive.

#### Collaboration and Option Agreement with AstraZeneca (previously MedImmune).

In December 2017, we entered into a Collaboration and Option Agreement (the MedImmune Agreement), with MedImmune, LLC (MedImmune). Under the MedImmune Agreement, we and MedImmune will collaborate to optimize AAV capsid variants for the delivery of certain genes to the lung for the treatment of chronic lung disease (the MedImmune Field), using our AAV technology. We are primarily responsible for the initial optimization and MedImmune has an option to select up to 3 capsid variants.

Upon MedImmune's exercise of its option, and subject to its payment of the applicable option exercise fee, we will automatically grant MedImmune an exclusive, sublicenseable, worldwide license under certain of our intellectual property rights to make, use, sell, import, export or otherwise utilize the selected capsid variants and products containing such selected capsid variants and certain genes in the MedImmune Field. Following the exercise of its option, MedImmune will be responsible for the development and commercialization of the licensed products and has agreed to certain diligence obligations.

We received an upfront payment from MedImmune of \$1.5 million. Upon MedImmune's exercise of its option, it will be required to pay us a one-time option exercise fee of \$2 million. Upon the achievement of specified development milestones, MedImmune will be required to pay us up to \$45.0 million on a licensed product-by-licensed product basis. MedImmune will be required to pay us tiered royalties on worldwide annual net sales of licensed products between low-single digit and mid-single digit percentage rates, subject to certain specified reductions. These royalties are payable on a product-by-product and country-by-country basis until the later of ten years after the date of first commercial sale of such licensed product in such country and the expiration of the last-to-expire licensed patent right covering such licensed product (the MedImmune Royalty Term).

The MedImmune Agreement will expire on a product-by-product basis on the earlier of the expiration of the option period if the option for such product is unexercised by MedImmune or the MedImmune Royalty Term for the product. Either party may terminate the agreement in its entirety if the other party fails to cure its material breach within 90 days of receiving notice, subject to additional cure period in accordance with the terms of the agreement. Prior to its option exercise, MedImmune may terminate the agreement upon 30 days' prior written notice. Following its option exercise, MedImmune may terminate the agreement in its entirety or on a licensed product-by-licensed product basis upon 90 days' prior written notice.

If we terminate the agreement for MedImmune's material breach or if MedImmune terminates the agreement for convenience, our rights to the licensed products generally revert back to us. If MedImmune terminates the agreement for our material breach, MedImmune may retain its rights under the license that we grant to MedImmune under our intellectual property rights and MedImmune's payment obligations will survive.

# Collaboration and License Agreements with uniQure biopharma B.V.

In August 2019, we entered into an Amended and Restated Collaboration and License Agreement (the Amended and Restated uniQure Agreement) with uniQure biopharma B.V., now uniQure N.V. (uniQure), which amended and restated the Collaboration and License Agreement that we entered into with uniQure in January 2014.

Under the Amended and Restated uniQure Agreement, we granted uniQure an exclusive, sublicenseable, worldwide license under certain of our intellectual property rights, and other rights, to research, develop, make, use, and commercialize pre-selected AAV capsid variants (Selected Variants), and compounds and products containing such Selected Variants, using our proprietary AAV technology for delivery of gene therapy constructs to cells in the central nervous system and the liver (the uniQure Field). uniQure is solely responsible, at its cost and expense, to develop and commercialize the compounds and products containing the Selected Variants in accordance with the terms of the Amended and Restated uniQure Agreement. We retain all rights to all other AAV capsid variants, and compounds and products containing such AAV capsid variants, in the uniQure Field.

Also in August 2019, we entered into a separate Collaboration and License Agreement with uniQure (Second uniQure Agreement). Under the Second uniQure Agreement, the parties agreed to research and develop new AAV capsid variants that are not Selected Variants (New Variants) using our proprietary AAV technology for delivery of transgene constructs that affect certain targets (uniQure Targets) in the uniQure Field. We are responsible for the research of the New Variants, and uniQure is

responsible for the development and commercialization of a certain number of compounds and products containing New Variants, that affect the uniQure Targets (Licensed Products). We granted uniQure an exclusive, sublicenseable, worldwide license under certain of our intellectual property rights, and other rights, to research, develop, make, use, and commercialize the Licensed Products. We retain all rights to New Variants in the uniQure Field that affect targets other than the uniQure Targets. We also retain all rights to any new AAV capsid variants developed under the agreements that are not New Variants, and compounds and products containing such variants.

Under both the Amended and Restated uniQure Agreement and the Second uniQure Agreement, uniQure will be required to pay us royalties on worldwide annual net sales of licensed products at a mid-single digit percentage rate, subject to certain specified reductions. These royalties are payable on a product-by-product and country-by-country basis until the latest of ten years after the date of the first commercial sale of such product in such country, the expiration of the last-to-expire licensed patent right covering such product in such country, and the expiration of any applicable exclusivity granted by a regulatory authority in such country for such product (the uniQure Royalty Term). uniQure will also be required to pay us a portion of the amounts it receives for licensing or sublicensing to third parties our intellectual property rights licensed or other rights otherwise granted under the Amended and Restated uniQure Agreement, and a portion of the amounts it receives for licensing to third parties our intellectual property rights granted under the or the Second uniQure Agreement, each at a rate between mid-single digit to mid-twenties percentages, depending on the stage of development at which such third party grant occurs.

Under both the Amended and Restated uniQure Agreement and the Second uniQure Agreement, under certain circumstances, we may propose to uniQure, and uniQure may grant to us, a non-exclusive right for us to develop and commercialize certain licensed products based on Selected Variants in the uniQure Field, or the New Variants in the uniQure Field to deliver transgene constructs that affect the uniQure Targets (4DMT Proposed Products). Pursuant to the Second uniQure Agreement, under certain circumstances, uniQure may propose to us, and we may grant to uniQuire a non-exclusive right for uniQure to develop and commercialize certain licensed products using any new AAV capsid variants developed under the agreement that are not New Variants in the uniQure Field to deliver transgene constructs that affect targets other than the uniQure Targets (uniQure Proposed Products). If either party obtains the rights to develop and commercialize a 4DMT Proposed Product or a uniQure Proposed Product, as applicable, such party will be required to pay the other party royalties on worldwide annual net sales of such products at a mid-single digit percentage rate, subject to specified reductions. These royalties will be payable on a product-by-product basis during the uniQure Royalty Term for such products. The party receiving such license will also be required to pay the other party a portion of the amounts that it may receive for licensing or sublicensing to third parties rights for such 4DMT Proposed Products or uniQure Proposed Products, as applicable, at a rate between mid-single digit to mid-twenties percentages depending on the stage of development at which the sublicense is granted.

Each of the Amended and Restated uniQure Agreement and the Second uniQure Agreement will expire on the expiration of all payment obligations of the parties under such agreement. Each party may terminate either agreement for the other party's insolvency or bankruptcy. Each party may also terminate either agreement in its entirety in some circumstances or on an indication-by-indication basis if the other party fails to cure its material breach under the applicable agreement within 90 days of receiving notice, subject to an additional cure period in accordance with the terms of such agreement. uniQure may terminate either agreement upon 90 days' prior written notice. In addition, uniQure may terminate the Second uniQure Agreement at any point prior to the first anniversary of the effective date if the joint research committee determines that it would be futile to continue the research program under the agreement, including if such committee determines that certain agreed-upon development

success criteria will not be able to be met, or if we are not making bona fide efforts to achieve the mutually agreed timelines set forth in the research plan. If we terminate either agreement for uniQure's material breach, insolvency or bankruptcy or if uniQure terminates either agreement for convenience or due to its determination of futility, the rights to the Selected Variants, and compounds and products containing such Selected Variants, or the uniQure New Variants, and compounds and products containing such uniQure New Variants, as applicable, generally revert back to us. If uniQure terminates either agreement for our material breach under the applicable agreement, insolvency or bankruptcy, uniQure may retain its rights to the intellectual property license grant under such agreement and uniQure's payment obligations will survive.

# Exclusive License and Bailment Agreements with The Regents of the University of California

In December 2013, we entered into two Exclusive License and Bailment Agreements (the UC Agreements) with The Regents of the University of California (the UC Regents). Under both UC Agreements, the UC Regents granted us an exclusive, sublicenseable license under certain patent rights to make, use, sell, offer to sell, and import products and services, and to practice methods in the United States and foreign countries where the licensed patent rights exist. The license grant under one UC Agreement is in all fields of use and the license grant under the other UC Agreement is in all fields of use, with the exception of the ophthalmic field. We agreed to certain general and specific diligence obligations under both UC Agreements in connection with the development, manufacture and sales of the licensed products, services and methods, in accordance with the terms of the UC Agreements.

Under each UC Agreement, we paid the UC Regents an upfront payment of \$5,000, Further, at the closing of our Series A financing that was a qualified financing pursuant to the UC Agreements, we issued 311,812 shares of our common stock in aggregate under both agreements. Under each UC Agreement, we agreed to pay the UC Regents a specified annual license maintenance fee in each year in which we do not owe royalties to the UC Regents. We also agreed to pay the UC Regents a mid-teens to mid-twenties percentage range of any consideration, including royalties (Sublicense Consideration), we receive for the grant of a sublicense under the licensed patent rights under each UC Agreement, with the consideration payable to the UC Regents to not exceed such percentage range in the aggregate under both UC Agreements for the same sublicense grant. We may reduce any Sublicense Consideration if we sublicense any of our own or third party patent rights under the sublicense grant based on the relative value of the sublicensed patents. Upon the achievement of specified development and regulatory milestones by the first licensed product or method, we will be required to pay the UC Regents up to \$3.1 million under each UC Agreement. We will also be required to pay the UC Regents a royalty on net sales of licensed products, services and methods covered by the patents licensed under the UC Agreements at a percentage in the low single-digit percentage rate, subject to certain specified reductions. Under the UC Agreements, a specified minimum annual royalty will also be due to the UC Regents beginning the first calendar year after the year in which any net sales of a licensed product first occur, such minimum royalty amount to increase on an annual basis, but not to exceed \$0.1 million in the aggregate under both UC Agreements. Under each UC Agreement, royalties are payable until the expiration of the last-to-expire licensed patent right covering the licensed product, service or method (the UC Royalty Term). Milestone, royalty and sublicense revenue payments will be due to the UC Regents under only one of the UC Agreements covering any licensed product, regardless of the number of patents covering a given licensed product.

Each UC Agreement will expire at the end of the UC Royalty Term. The UC Regents may terminate each of the UC Agreements if we fail to cure a breach of such UC Agreement within 60 days of notice. If we fail to meet our diligence obligations, the UC Regents has the right to either terminate the UC Agreement or to reduce our exclusive license to a non-exclusive license, after giving us 60 days to cure or request arbitration. We may terminate either UC Agreement at-will in its entirety or with respect to any portion of the licensed patent rights upon 90 days prior written notice. Each UC

Agreement will terminate immediately if we or a third party on our behalf files a claim asserting that the licensed patent rights are invalid or unenforceable.

# **Government Regulation**

The FDA and other regulatory authorities at federal, state, and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, advertising, promotion, marketing, post-approval monitoring, and post-approval reporting of biological product candidates such as those we are developing. We, along with third-party contractors, will be required to navigate the various preclinical, clinical and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval or licensure of our product candidates. The process of obtaining regulatory approvals and the subsequent compliance with applicable federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources.

# U.S. Biologics Regulation

The process required by the FDA before biologic product candidates may be marketed in the United States generally involves the following:

- · completion of preclinical laboratory tests and animal studies performed in accordance with the FDA's GLPs;
- submission to the FDA of an IND which must become effective before clinical trials may begin;
- approval by an IRB or ethics committee at each clinical site before the trial is commenced;
- performance of adequate and well-controlled human clinical trials to establish the safety, purity and potency of the proposed biologic product candidate for its intended purpose;
- preparation of and submission to the FDA of a BLA after completion of all pivotal clinical trials;
- · satisfactory completion of an FDA Advisory Committee review, if applicable;
- a determination by the FDA within 60 days of its receipt of a BLA to file the application for review;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the proposed product is produced to assess compliance with cGMP and to assure that the facilities, methods and controls are adequate to preserve the biological product's continued safety, purity and potency, and of selected clinical investigation sites to assess compliance with Good Clinical Practices (GCPs); and
- FDA review and approval of the BLA to permit commercial marketing of the product for particular indications for use in the United States.

Prior to beginning the first clinical trial with a product candidate in the United States, we must submit an IND to the FDA. An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for clinical studies. The IND also includes results of animal and *in vitro* studies assessing the toxicology, pharmacokinetics, pharmacology, and pharmacodynamic characteristics of the product; chemistry, manufacturing, and controls information; and any available human data or literature to support the use of the investigational product. An IND must become effective before human clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises safety concerns or questions about the proposed clinical trial. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before the clinical trial can begin. Submission of an IND therefore may or may not result in FDA authorization to begin a clinical trial.

In addition to the submission of an IND to the FDA, supervision of certain human gene transfer trials may also require evaluation and assessment by an institutional biosafety committee (IBC), a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to the public health or the environment, and such assessment may result in some delay before initiation of a clinical trial.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND must be made for each successive clinical trial conducted during product development and for any subsequent protocol amendments. Furthermore, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site and must monitor the study until completed. Regulatory authorities, the IRB or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk or that the trial is unlikely to meet its stated objectives. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study and may halt the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy. There are also requirements governing the reporting of ongoing clinical studies and clinical study results to public registries.

For purposes of BLA approval, human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- Phase 1—The investigational product is initially introduced into healthy human subjects or patients with the target disease or condition. These studies are designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness.
- Phase 2—The investigational product is administered to a limited patient population with a specified disease or condition to
  evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify possible adverse side effects and safety
  risks. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning larger and more expensive Phase
  3 clinical trials.
- Phase 3—The investigational product is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval.

In some cases, the FDA may require, or companies may voluntarily pursue, additional clinical trials after a product is approved to gain more information about the product. These so-called Phase 4 studies may also be made a condition to approval of the BLA.

Concurrent with clinical trials, companies may complete additional animal studies and develop additional information about the biological characteristics of the product candidate, and must finalize a

process for manufacturing the product in commercial quantities in accordance with cGMP. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

# BLA Submission and Review by the FDA

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development, nonclinical studies and clinical trials are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications. The BLA must include all relevant data available from preclinical and clinical studies, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls, and proposed labeling, among other things. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of a use of the product, or from a number of alternative sources, including studies initiated by investigators. The submission of a BLA requires payment of a substantial user fee to FDA, and the sponsor of an approved BLA is also subject to an annual program fee. A waiver of user fees may be obtained under certain limited circumstances. Additionally, no user fees are assessed on BLAs for products designated as orphan drugs, unless the product also includes a non-orphan indication.

Once a BLA has been submitted, the FDA's goal is to review standard applications within ten months after it accepts the application for filing, or, if the application qualifies for priority review, six months after the FDA accepts the application for filing. Priority review designation will direct overall attention and resources to the evaluation of applications for products that, if approved, would be significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions. In both standard and priority reviews, the review process is often significantly extended by FDA requests for additional information or clarification. The FDA reviews a BLA to determine, among other things, whether a product is safe, pure and potent and the facility in which it is manufactured, processed, packed, or held meets standards designed to assure the product's continued safety, purity and potency. The FDA may also convene an advisory committee to provide clinical insight on application review questions. The FDA is not bound by recommendations of an advisory committee, but it considers such recommendations when making decisions regarding approval.

Before approving a BLA, the FDA will typically inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

After the FDA evaluates a BLA and conducts inspections of manufacturing facilities where the investigational product and/or its drug substance will be produced, the FDA may issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A Complete Response Letter will describe all of the deficiencies that the FDA has identified in the BLA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may

issue the Complete Response Letter without first conducting required inspections, testing submitted product lots, and/or reviewing proposed labeling. In issuing the Complete Response Letter, the FDA may recommend actions that the applicant might take to place the BLA in condition for approval, including requests for additional information or clarification. The FDA may delay or refuse approval of a BLA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor safety or efficacy of a product.

If regulatory approval of a product is granted, such approval will be granted for particular indications and may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the BLA with a Risk Evaluation and Mitigation Strategy (REMS), to ensure the benefits of the product outweigh its risks. A REMS is a safety strategy to manage a known or potential serious risk associated with a medicine and to enable patients to have continued access to such medicines by managing their safe use, and could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries, and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling or the development of adequate controls and specifications. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing requirements is not maintained or if problems occur after the product reaches the marketplace. The FDA may also require one or more Phase IV post-market studies and surveillance to further assess and monitor the product's safety and effectiveness after commercialization, and may limit further marketing of the product based on the results of these post-marketing studies. In addition, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could impact the timeline for regulatory approval or otherwise impact ongoing development programs.

#### **Expedited Development and Review Programs**

A sponsor may seek approval of its product candidate under programs designed to accelerate FDA's review and approval of new drugs and biological products that meet certain criteria. Specifically, new drugs and biological products are eligible for fast track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. For a fast track product, the FDA may consider sections of the BLA for review on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable and the sponsor pays any required user fees upon submission of the first section of the application. A fast track designated product candidate may also qualify for priority review, under which the FDA sets the target date for FDA action on the BLA at six months after the FDA accepts the application for filing. Priority review is granted when there is evidence that the proposed product would be a significant improvement in the safety or effectiveness of the treatment, diagnosis, or prevention of a serious disease or condition. If criteria are not met for priority review, the application is subject to the standard FDA review period of 10 months after FDA accepts the application for filing. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Under the accelerated approval program, the FDA may approve a BLA on the basis of either a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Post-marketing studies or completion of ongoing studies after marketing approval are generally required to verify the biologic's clinical benefit in relationship to the surrogate endpoint or ultimate outcome in relationship to the clinical benefit. In addition, the FDA currently requires as a condition for accelerated approval pre-

approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. FDA may withdraw approval of a drug or indication approved under accelerated approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product.

In addition, a sponsor may seek FDA designation of its product candidate as a breakthrough therapy if the product candidate is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. If the FDA designates a product as a breakthrough therapy, it may take actions appropriate to expedite the development and review of the application, which may include holding meetings with the sponsor and the review team throughout the development of the therapy; providing timely advice to, and interactive communication with, the sponsor regarding the development of the drug to ensure that the development program to gather the nonclinical and clinical data necessary for approval is as efficient as practicable; involving senior managers and experienced review staff, as appropriate, in a collaborative, cross-disciplinary review; assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor; and considering alternative clinical trial designs when scientifically appropriate, which may result in smaller trials or more efficient trials that require less time to complete and may minimize the number of patients exposed to a potentially less efficacious treatment. Breakthrough therapy designation comes with all of the benefits of fast track designation.

Fast Track designation, priority review, accelerated approval and breakthrough therapy designation do not change the standards for approval but may expedite the development or approval process. Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

#### Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, defined as a disease or condition with a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 individuals in the United States and when there is no reasonable expectation that the cost of developing and making available the drug or biologic in the United States will be recovered from sales in the United States for that drug or biologic. Orphan drug designation must be requested before submitting a BLA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA.

If a product that has orphan drug designation subsequently receives the first FDA approval for a particular active ingredient for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications, including a full BLA, to market the same biologic for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or if the FDA finds that the holder of the orphan drug exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated. Orphan drug exclusivity does not prevent the FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the BLA application user fee.

A designated orphan drug many not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or, as noted above, if the second applicant demonstrates that its product is clinically superior to the approved product with orphan exclusivity or the manufacturer of the approved product is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. We have obtained orphan drug designation for 4D-110 for the treatment of Chroideremia, and we plan to seek additional orphan drug designations for some or all of our product candidates in specific orphan indications in which there is a medically plausible basis for the use of these products.

#### Post-Approval Requirements

Any products manufactured or distributed by us pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to record-keeping, reporting of adverse experiences, periodic reporting, product sampling and distribution, and advertising and promotion of the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing, annual program fees for any marketed products. Biologic manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP, which impose certain procedural and documentation requirements upon us and our third-party manufacturers. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

The FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- · fines, warning letters, or untitled letters;
- · clinical holds on clinical studies;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information;

- the issuance of safety alerts, Dear Healthcare Provider letters, press releases and other communications containing warnings or other safety information about the product; or
- · injunctions or the imposition of civil or criminal penalties.

The FDA closely regulates the marketing, labeling, advertising and promotion of biologics. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

# Biosimilars and Exclusivity

The Affordable Care Act, signed into law in 2010, includes a subtitle called the BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars. Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product in any given patient and, for products that are administered multiple times to an individual, the biologic and the reference biologic may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. However, complexities associated with the larger, and often more complex, structures of biological products, as well as the processes by which such products are manufactured, pose significant hurdles to implementation of the abbreviated approval pathway that are still being worked out by the FDA.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing that applicant's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of its product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

A biological product can also obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

The BPCIA is complex and continues to be interpreted and implemented by the FDA. In addition, government proposals have sought to reduce the 12-year reference product exclusivity period. Other

aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. As a result, the ultimate impact, implementation, and impact of the BPCIA is subject to significant uncertainty.

#### Other Healthcare Laws

Pharmaceutical companies are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which they conduct their business. Such laws include, without limitation, U.S. federal and state anti-kickback, fraud and abuse, false claims, pricing reporting, data privacy and security, and transparency laws and regulations as well as similar foreign laws in the jurisdictions outside the U.S. For example, the federal Anti-Kickback Statute prohibits, among other things, individuals or entities from knowingly and willfully offering, paying, soliciting or receiving remuneration, directly or indirectly, overtly or covertly, in cash or in kind to induce or in return for purchasing, leasing, ordering or arranging for or recommending the purchase, lease or order of any item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. A person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act and the civil monetary penalties statute. The federal civil and criminal false claims laws, including the civil False Claims Act, prohibit, among other things, any individual or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government or knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. The federal Health Insurance Portability and Accountability Act of 1996 (HIPAA), created additional federal civil and criminal statutes that prohibit, among other things, knowingly and willfully executing a scheme to defraud any healthcare benefit program. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the healthcare fraud statute implemented under HIPAA or specific intent to violate it in order to have committed a violation. The federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to CMS information related to payments or other transfers of value made to physicians and teaching hospitals, and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by physicians and their immediate family members.

Similar state and local laws and regulations may also restrict business practices in the pharmaceutical industry, such as state anti-kickback and false claims laws, which may apply to business practices, including but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, or by patients themselves; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws and regulations that require drug manufacturers to file reports relating to pricing and marketing information or which require tracking gifts and other remuneration and items of value provided to physicians, other healthcare providers and entities; state and local laws that require the registration of pharmaceutical sales representatives. Violation of any of such laws or any other governmental regulations that apply may result in significant penalties, including, without limitation, administrative civil and criminal penalties, damages, disgorgement fines, additional reporting requirements and oversight obligations, contractual damages, the curtailment or restructuring of operations, exclusion from participation in governmental healthcare programs and imprisonment.

#### Data Privacy and Security Laws

Pharmaceutical companies may be subject to domestic and foreign privacy, security and data breach notification laws, which are rapidly evolving in many jurisdictions worldwide. In the United States, federal and state health information laws may govern the collection, use, disclosure and protection of health-related and other personal information. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 (HITECH), and their respective implementing regulations, which impose obligations on "covered entities," including certain healthcare providers, health plans, and healthcare clearinghouses, as well as their respective "business associates" that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to safeguarding the privacy, security and transmission of individually identifiable health information. Additionally, HITECH created four new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in U.S. federal courts to enforce HIPAA and seek attorneys' fees and costs associated with pursuing federal civil actions. State laws may be more stringent. broader in scope or offer greater individual rights with respect to protected health information (PHI) than HIPAA and state laws may differ from each other, which may complicate compliance efforts. For example, California enacted the California Consumer Privacy Act (the CCPA) on June 28, 2018, which takes effect on January 1, 2020 and has been dubbed the first "GDPR-like" law in the United States (referring to the EU's General Data Protection Regulation, described below). The CCPA gives California residents expanded rights regarding their personal information. Although CCPA contains a HIPAA exemption, uncertainties over how it applies and how our treatment of non-PHI personal information may be interpreted mean that the CCPA may ultimately increase our compliance costs and potential liability. Entities that are found to be in violation of HIPAA as the result of a breach of unsecured PHI, a complaint about privacy practices or an audit by the Department of Health and Human Services (HHS) may be subject to significant civil, criminal and administrative fines and penalties and/or additional reporting and oversight obligations if required to enter into a resolution agreement and corrective action plan with HHS to settle allegations of HIPAA non-compliance.

European Union member states, the United Kingdom, Switzerland and other jurisdictions have also adopted data protection laws and regulations, which impose significant compliance obligations. In the EEA and the United Kingdom, the collection and use of personal data, including clinical trial data, is governed by the provisions of the General Data Protection Regulation (GDPR). The GDPR became effective on May 25, 2018, repealing its predecessor directive and increasing responsibility and liability of pharmaceutical companies in relation to the processing of personal data of European Union data subjects. The GDPR, together with national legislation, regulations and guidelines of the European Union member states and the United Kingdom governing the processing of personal data, impose strict obligations and restrictions on the ability to collect, analyze and transfer personal data, including health data from clinical trials and adverse event reporting. In particular, these obligations and restrictions concern the consent of the individuals to whom the personal data relates, the information provided to the individuals, the transfer of personal data out of the European Economic Area (EEA) or the United Kingdom, security breach notifications, security and confidentiality of the personal data and imposition of substantial potential fines for breaches of the data protection obligations. European data protection authorities may interpret the GDPR and national laws differently and impose additional requirements, which add to the complexity of processing personal data in or from the EEA or United Kingdom. Guidance on implementation and compliance practices are often updated or otherwise revised.

# Coverage and Reimbursement

Sales of any pharmaceutical product depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state and foreign government healthcare programs,

commercial insurance and managed healthcare organizations, and the level of reimbursement for such product by third-party payors. Significant uncertainty exists as to the coverage and reimbursement status of any newly approved product, particularly for gene therapy products where the Centers for Medicare & Medicaid Services (CMS) and other third-party payors in the United States have not yet established a uniform policy of coverage and reimbursement. Therefore, decisions. Decisions regarding the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. One third-party payor's decision to cover a particular product does not ensure that other payors will also provide coverage for the product. As a result, the coverage determination process can require manufactures to provide scientific and clinical support for the use of a product to each payor separately and can be a time-consuming process, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization.

In addition, third-party payors are increasingly reducing reimbursements for pharmaceutical products and services. The U.S. government and state legislatures have continued implementing cost-containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. Third-party payors are more and more challenging the prices charged, examining the medical necessity and reviewing the cost effectiveness of pharmaceutical products, in addition to questioning their safety and efficacy. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit sales of any product. Decreases in third-party reimbursement for any product or a decision by a third-party payor not to cover a product could reduce physician usage and patient demand for the product.

In international markets, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. For example, the European Union provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of us placing the medicinal product on the market. Pharmaceutical products may face competition from lower-priced products in foreign countries that have placed price controls on pharmaceutical products and may also compete with imported foreign products. Furthermore, there is no assurance that a product will be considered medically reasonable and necessary for a specific indication, will be considered cost-effective by third-party payors, that an adequate level of reimbursement will be established even if coverage is available or that the third-party payors' reimbursement policies will not adversely affect the ability for manufacturers to sell products profitably.

# Healthcare Reform

In the United States and certain foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system. In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (collectively the ACA) was signed into law, which substantially changed the way healthcare is financed by both governmental and private insurers in the United States. The ACA contains a number of provisions, including those governing enrollment in federal healthcare programs, reimbursement adjustments and fraud and abuse changes. Additionally, the ACA increased the minimum level of Medicaid rebates payable by manufacturers of brand name drugs from 15.1% to 23.1%; required collection of rebates for drugs paid by Medicaid managed care organizations; imposed

a non-deductible annual fee on pharmaceutical manufacturers or importers who sell certain "branded prescription drugs" to specified federal government programs, implemented a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted, or injected; expanded eligibility criteria for Medicaid programs; created a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and established a Center for Medicare & Medicaid Innovation at CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

Since its enactment, there have been judicial and Congressional challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. For example, in 2017, Congress enacted the Tax Cuts and Jobs Act, which eliminated the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas (the Texas District Court Judge), ruled that the individual mandate is a critical and inseverable feature of the ACA, and therefore, because it was repealed as part of the Tax Cuts and Jobs Act, the remaining provisions of the ACA are invalid as well. While the Texas U.S. District Court Judge, as well as the Trump Administration and CMS, have stated that the ruling will have no immediate effect, and on December 30, 2018 the Texas District Court Judge issued an order staying the judgment pending appeal, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the ACA will impact the ACA.

Other legislative changes have been proposed and adopted since the ACA was enacted, including aggregate reductions of Medicare payments to providers of 2% per fiscal year and reduced payments to several types of Medicare providers, which will remain in effect through 2027 absent additional congressional action. Moreover, there has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted legislation designed, among other things, to bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for pharmaceutical products. At the federal level, the Trump administration's budget proposals for fiscal years 2019 and 2020 contain further drug price control measures that could be enacted during the budget process or in other future legislation. Further, the Trump administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers. While some existing measures may require additional authorization to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. Individual states in the United States have also become increasingly active in implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures and, in some cases, mechanisms to encourage importation from other countries and bulk purchasing. Furthermore, there has been increased interest by third party payors and governmental authorities in reference pricing systems and publication of discounts and list prices.

# **Employees**

As of June 30, 2019, we had 57 full-time employees. Of these employees, 46 are engaged in research and development. Our employees are not represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

# **Facilities**

We lease approximately 51,000 square feet of office and laboratory space in Emeryville, California under leases that expire in September 2026 and December 2029. We believe that our facilities are adequate to meet our current needs, and that suitable additional alternative spaces will be available in the future on commercially reasonable terms, if required.

# **Legal Proceedings**

We are not currently a party to any material legal proceedings. We may, however, in the ordinary course of business face various claims brought by third parties and we may, from time to time, make claims or take legal actions to assert our rights, including intellectual property rights as well as claims relating to employment matters and the safety or efficacy of our products. Any of these claims could subject us to costly litigation and, while we generally believe that we have adequate insurance to cover many different types of liabilities, our insurance carriers may deny coverage, may be inadequately capitalized to pay on valid claims, or our policy limits may be inadequate to fully satisfy any damage awards or settlements. If this were to happen, the payment of any such awards could have a material adverse effect on our operations, cash flows and financial position. Additionally, any such claims, whether or not successful, could damage our reputation and business.

#### MANAGEMENT

#### **Executive Officers and Directors**

The following table sets forth information regarding our executive officers and directors as of June 30, 2019:

<u>Name</u>	Age	Position(s)
Executive Officers and Employee Directors		
David Kirn, M.D.	57	Chairman and Chief Executive Officer
August Moretti	68	Chief Financial Officer
Theresa Janke	44	Chief Operating Officer
Peter Francis, M.D., Ph.D.	50	Chief Medical Officer
Fred Kamal, Ph.D.	56	Chief Technical Officer, Head of Regulatory and Quality
Non-Employee Directors		
David Schaffer, Ph.D.	48	Director, Chief Scientific Advisor
William Burkoth, MBA	42	Director
Jacob Chacko, M.D., MBA	41	Director
Charles Theuer, M.D., Ph.D.	55	Director
Tony Yao, M.D., Ph.D.	47	Director

<sup>(1)</sup> Member of compensation committee.

# **Executive Officers and Employee Directors**

David Kirn, M.D., is our co-founder and has served as our Chief Executive Officer and Chairman of our board of directors since our inception in 2013. Dr. Kirn is an Adjunct Professor of Bioengineering at U.C. Berkeley. He currently serves as Executive Chairman of the board of the following privately held company: Ignite Immunotherapy Inc., where he is a co-founder. Dr. Kirn held senior development positions at Onyx Pharmaceuticals and Celgene, and he was a senior advisor on viral vector gene therapeutics and cancer immunotherapy for over 10 years with numerous companies, including Biogen Idec, Novartis, Cell Genesys, Pfizer and Bayer. Dr. Kirn received a B.A. in Physiology (Departmental Citation; Phi Beta Kappa) from U.C. Berkeley in 1985, an M.D. (Alpha Omega Alpha) from U.C. San Francisco Medical School in 1989 and completed internal medicine residency training at Harvard Medical School, Brigham and Women's Hospital (including a term as Chief Medical Resident at affiliated VA hospital). He has also completed hematology-oncology and clinical research fellowships at U.C. San Francisco and completed a certificate of business excellence from the Haas Business School at U.C. Berkeley. In 2013, he was awarded the Johnson & Johnson Entrepreneur Innovator award from the J&J Innovation Center. We believe that Dr. Kirn is qualified to serve as a member of our board of directors based on his perspective and the experience he brings as one of our founders and Chief Executive Officer, and because of his extensive experience at other life science companies.

August Moretti has served as our Chief Financial Officer since January 2019. Mr. Moretti previously served as Chief Financial Officer at Assertio Therapeutics (formerly Depomed, Inc.), a publicly held specialty pharmaceuticals company focused in pain and neurology, from January 2012 until August 2018. Mr. Moretti received his B.A. in Economics from Princeton University in 1972. He received his J.D. from Harvard Law School in 1975.

<sup>(2)</sup> Member of audit committee.

<sup>(3)</sup> Member of nominating and corporate governance committee.

Theresa Janke is a co-founder and has served in positions of increasing responsibility since our inception. She has served as our Chief Operating Officer since April 2018. Ms. Janke previously served in various consulting roles in 2013-2014 including: Senior Director of Corporate Projects & Alliance Management at SillaJen, Inc. (formerly Jennerex Biotherapeutics Inc.), a biotech company focused on engineering and developing oncolytic immunotherapeutics, and Director, Clinical Research & Development—Strategy and Alliances at Celgene Corporation, a global biopharmaceutical company. Prior to that, she served in positions of increasing responsibility, including Director of Clinical Operations, at Jennerex Biotherapeutics Inc., an oncolytic immunotherapy biotech company, from 2007 through 2013. She is a co-founder and currently serves on the board of directors of Ignite Immunotherapy Inc., a private biotech company focused on oncolytic cancer vaccine discovery and development. Ms. Janke received a B.S. in Biopsychology from the U.C. Santa Barbara in 1996.

Peter Francis, M.D., Ph.D., has served as our Chief Medical Officer since January 2019. Dr. Francis previously served as our Senior Vice President, Clinical Translational R&D, and Retina Therapeutic Area Head from August 2018 to January 2019. Dr. Francis previously served as Chief Medical Officer at RetroSense Therapeutics from February 2012 until August 2016 when it was purchased by Allergan Inc. Dr. Francis practices as a physician at Orion Eye Center. Dr. Francis received his B.Sc. in Molecular Cell Biology from the University of Southampton, England in 1991. He earned his Ph.D. in ophthalmic genetics from University College, London in 2000.

Fred Kamal, Ph.D., has served as our Chief Technical Officer since October 2018. Dr. Kamal previously served as Senior Vice President of Quality and Regulatory CMC for AveXis Inc., a gene therapy company, from May 2017 through August 2018. Prior to AveXis, Dr. Kamal served as the Vice President of Quality for Juno Therapeutics from May 2015 through April 2017 and prior to that Dr. Kamal served as the Vice President of Quality and Regulatory CMC for Intermune Inc. from January 2013 through March 2015. Dr. Kamal received his B.S. in Chemistry from San Jose State University in 1986. Dr. Kamal received his M.Sc. in Chemistry from The American University in 2000. He received his Ph.D. in Chemistry from The American University in 2003.

#### **Non-Employee Directors**

David Schaffer, Ph.D. is our co-founder and has served as our Chief Scientific Advisor and a member of our board of directors since our inception in 2013. Dr. Schaffer has served as a Professor of Chemical and Biomolecular Engineering, Bioengineering, Molecular and Cell Biology, and the Helen Wills Neuroscience Institute at the U.C. Berkeley since 1999 and has served as the Director of the Berkeley Stem Cell Center since 2011. He current serves on the board of directors of the following publicly-held company: uniQure NV since January 2014. Dr. Schaffer received a B.S. in Chemical Engineering from Stanford University in 1993. He earned his Ph.D. in Chemical Engineering from the Massachusetts Institute of Technology in 1998. We believe Dr. Schaffer is qualified to serve as a member of our board of directors based on his prospective and the experience he brings as one of our founders, and because of his scientific expertise and leading work in directed evolution.

William Burkoth, MBA, has served as a member of our board of directors since March 2019. Mr. Burkoth has served on the venture investments team of Pfizer Inc. since 2004 and is currently Executive Director at Pfizer Inc. and Senior Partner at Pfizer Ventures (US) LLC. Mr. Burkoth worked in business development at Galileo Pharmaceuticals, Inc. and at IntraBiotics Pharmaceuticals, Inc. from 2002 to 2004. Mr. Burkoth worked as an analyst at Bay City Capital, a life sciences venture capital firm, from 1999 to 2002. He previously served on the board of directors of the following publicly-held company: NovoCure Limited from July 2009 to May 2019. Mr. Burkoth received a B.A. in Chemistry from Whitman College in 1999 and an M.B.A. from Columbia Business School in 2011. We believe Mr. Burkoth is qualified to serve as a member of our board of directors based on his finance background and his extensive investment experience in the life science industry.

Jacob Chacko, M.D., MBA, has served as a member of our board of directors since March 2019. Dr. Chacko has served as Chief Executive Officer of ORIC Pharmaceuticals, Inc., a clinical-stage oncology company focused on discovery and development of novel therapies against treatment-resistant cancers, since May 2018. Prior to ORIC, Dr. Chacko served as Chief Financial Officer of Ignyta, Inc., a publicly traded precision oncology company, from May 2014 until February 2018 when Ignyta was acquired by Roche Holdings, Inc. Prior to Ignyta, Dr. Chacko was Vice President at TPG Capital from August 2008 until May 2014. From 2002 until 2003, Dr. Chacko was a consultant serving healthcare clients at McKinsey & Company. Dr. Chacko currently serves on the board of directors of Turning Point Therapeutics, Inc., a publicly-traded biotechnology company, from November 2018. Dr. Chacko served on the board of directors of RentPath Inc., a digital media company, from 2011 until 2014, Envision Pharmaceutical Services, LLC from 2013 until 2014, Bonti, Inc., a biotechnology company, from February 2018 until October 2018 and the Packard Children's Health Alliance at the Lucile Packard Children's Hospital Stanford from 2013 until June 2017. Dr. Chacko currently chairs the Western Regional Selection Committee for the Marshall Scholarship. Dr. Chacko concurrently received his M.D. from the U.C. Los Angeles and his M.B.A. from Harvard Business School. Dr. Chacko received a M.Sc. from Oxford University as a Marshall Scholar. We believe Dr. Chacko is qualified to serve as a member of our board of directors based on his medical and finance background, his experience in investing in life science companies and his service on the boards of public and private companies.

Charles Theuer, M.D., Ph.D., has served as a member of our board of directors since December 2015. Dr. Theuer has served as President and Chief Executive Officer at Tracon Pharmaceuticals, Inc., since June 2006. He previously served as Chief Medical Officer at TargeGen Pharmaceuticals, Inc. until June 2006. He current serves on the board of directors of the following publicly-held companies: Tracon Pharmaceuticals Inc., since June 2006 and Oncternal Therapeutics Inc. since May 2018, where he serves on the Science and Development and Nominating and Corporate Governance committees. Dr. Theuer received a B.S. in Life Sciences from the Massachusetts Institute of Technology in 1985. He received his M.D. from U.C. San Francisco in 1989. He received his Ph.D. in Environmental Health Science from U.C. Irvine in 2002. We believe that Dr. Theuer is qualified to serve as a member of our board of directors based on his medical and scientific background and because of his experience in leading and serving on the boards of public and private life science companies.

Tony Yao, M.D., Ph.D. has served as a member of our board of directors since August 2018. Dr. Yao has served as portfolio manager of life science strategy at ArrowMark Partners since April 2012. He previously served as an equity analyst at Janus Capital Group until March 2012. He currently serves on the board of directors of the following publicly-held company: Precision Biosciences since June 2018, where he serves on the compensation and audit committee. Dr. Yao received a B.S. in biochemistry from Brown University in 1994. He earned an M.D. and a Ph.D. in Immunology from Stanford University in 2002. We believe that Dr. Yao is qualified to serve as a member of our board of directors based on his scientific background and his extensive experience in investing in life science companies.

# **Family Relationships**

There are no family relationships among any of our executive officers or directors.

# **Board Composition**

# **Director Independence**

Our board of directors currently consists of six members. Our board of directors has determined that all of our directors, other than Dr. Kirn, qualify as "independent" directors in accordance with The

Nasdaq Global Market listing requirements. Dr. Kirn is not considered independent because he is an employee. The Nasdaq Global Market's independence definition includes a series of objective tests, such as that the director is not, and has not been for at least three years, one of our employees and that neither the director nor any of his or her family members has engaged in various types of business dealings with us. In addition, as required by The Nasdaq Global Market rules, our board of directors has made a subjective determination as to each independent director that no relationships exists that, in the opinion of our board of directors, would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In making these determinations, our board of directors reviewed and discussed information provided by the directors and us with regard to each director's business and personal activities and relationships as they may relate to us and our management. There are no family relationships among any of our directors or executive officers.

#### Classified Board of Directors

held in 2022.

In accordance with our amended and restated certificate of incorporation to be in effect immediately prior to the consummation of this offering, our board of directors will be divided into three classes with staggered, three-year terms. At each annual meeting of stockholders, the successors to directors whose terms then expire will be elected to serve from the time of election and qualification until the third annual meeting following election. Effective upon the consummation of this offering, we expect that our directors will be divided among the three classes as follows:

•	the Class I directors will be in 2020;	and	, and their terms will expire at the annual meeting of stockholders to be held
•	the Class II directors will be in 2021; and	and	, and their terms will expire at the annual meeting of stockholders to be held
•	the Class III directors will be	and	, and their terms will expire at the annual meeting of stockholders to be

Our amended and restated certificate of incorporation will provide that the authorized number of directors may be changed only by resolution of our board of directors. Any additional directorships resulting from an increase in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors. The division of our board of directors into three classes with staggered three-year terms may delay or prevent a change of our board of directors or a change in control.

# **Voting Arrangements**

The election of the members of our board of directors is governed by the Second Amended and Restated Investors' Rights Agreement, dated as of August 27, 2018, that we entered into with certain holders of our common stock and certain holders of our redeemable convertible preferred stock (the Investors' Rights Agreement) and the related provisions of our amended and restated certificate of incorporation.

Pursuant to the Investors' Rights Agreement, the holders of our common stock and redeemable convertible preferred stock who are parties to the Investors' Rights Agreement are obligated to vote for the election of certain designees of our board of directors which are as follows:

- one member designated by Pfizer, for which Mr. Burkoth has been designated;
- one member designated by the holders of a majority of our Series B redeemable convertible preferred stock, voting exclusively
  and as a separate class, for which Dr. Yao has been designated; and

- two members designated by the holders of a majority of our common stock (other than any common stock issued or issuable upon the conversion of the redeemable convertible preferred stock), voting exclusively and together as a single class, for which Dr. Kirn and Dr. Shaffer have been designated; and
- three members, who are not otherwise affiliated with any of our investors, the first of whom shall be mutually agreed upon by a majority of the other members of our board of directors, which is currently vacant; the second of whom shall be proposed by our management subject to the approval of a majority of the members of our board of directors, for which Dr. Theuer has been designated; and the third of whom shall be an individual that satisfies the independence, financial literacy and financial expertise requirements to serve as an audit committee chairperson pursuant to relevant SEC and Nasdaq laws and regulations, and mutually acceptable to a majority of the other members of our board of directors, for which Jacob Chacko has been designated.

The above provisions of Investors' Rights Agreement will terminate upon the consummation of this offering and our amended and restated certificate of incorporation will be amended and restated, after which there will be no further contractual obligations or charter provisions regarding the election of our directors. Our directors hold office until their successors have been elected and qualified or appointed, or the earlier of their death, resignation or removal.

# Leadership Structure of our Board

Our amended and restated bylaws and corporate governance guidelines will provide our board of directors with flexibility to combine or separate the positions of Chairman of our board of directors and Chief Executive Officer and to implement a lead director in accordance with its determination that utilizing one or the other structure would be in our best interest. Dr. Kirn currently serves as the chairman of our board of directors. In that role, Dr. Kirn presides over the executive sessions of our board of directors and as a liaison between management and our board of directors.

Our board of directors has concluded that our current leadership structure is appropriate at this time. However, our board of directors will continue to periodically review our leadership structure and may make such changes in the future as it deems appropriate.

# Role of our Board in Risk Oversight Process

Risk assessment and oversight are an integral part of our governance and management processes. Our board of directors encourages management to promote a culture that incorporates risk management into our corporate strategy and day-to-day business operations. Management discusses strategic and operational risks at regular management meetings, and conducts specific strategic planning and review sessions during the year that include a focused discussion and analysis of the risks facing us. Throughout the year, senior management reviews these risks with our board of directors at regular board meetings as part of management presentations that focus on particular business functions, operations or strategies, and presents the steps taken by management to mitigate or eliminate such risks.

Our board of directors does not have a standing risk management committee, but rather administers this oversight function directly through our board of directors as a whole, as well as through various standing committees of our board of directors that address risks inherent in their respective areas of oversight. While our board of directors is responsible for monitoring and assessing strategic risk exposure, our audit committee is responsible for overseeing our major financial risk

exposures and the steps our management has taken to monitor and control these exposures. The audit committee also monitors compliance with legal and regulatory requirements and considers and approves or disapproves any related person transactions. Our nominating and corporate governance committee monitors the effectiveness of our corporate governance guidelines. Our compensation committee assesses and monitors whether any of our compensation policies and programs has the potential to encourage excessive risk-taking.

#### **Board Committees**

Our board of directors has the following standing committees: an audit committee, a compensation committee and a nominating and corporate governance committee. Our board of directors may establish other committees to facilitate the management of our business. The composition and functions of each committee are described below.

#### **Audit Committee**

Our audit committee oversees our corporate accounting and financial reporting process. Among other matters, the audit committee:

- · appoints our independent registered public accounting firm;
- evaluates the independent registered public accounting firm's qualifications, independence and performance;
- · determines the engagement of the independent registered public accounting firm;
- reviews and approves the scope of the annual audit and pre-approves the audit and non-audit fees and services;
- · reviews and approves all related party transactions on an ongoing basis;
- establishes procedures for the receipt, retention and treatment of complaints received by us regarding accounting, internal
  accounting controls or auditing matters
- discusses with management and the independent registered public accounting firm the results of the annual audit and the review
  of our quarterly financial statements;
- approves the retention of the independent registered public accounting firm to perform any proposed permissible non-audit services;
- monitors the rotation of partners of the independent registered public accounting firm on our engagement team in accordance with requirements established by the SEC;
- discusses on a periodic basis, or as appropriate, with management our policies and procedures with respect to risk assessment and risk management;
- is responsible for reviewing our financial statements and our management's discussion and analysis of financial condition and results of operations to be included in our annual and quarterly reports to be filed with the SEC;
- annually reviews and assesses internal controls and treasury functions including cash management procedures;
- investigates any reports received through the ethics helpline and report to our board of directors periodically with respect to the information received through the ethics helpline and any related investigations;
- · reviews our critical accounting policies and estimates; and
- · reviews the audit committee charter and the committee's performance at least annually.

Effective upon the consummation of this offering, the members of our audit committee will be , will serve as the chairperson of the committee. All members of our audit committee will meet the requirements for financial literacy under the applicable rules and regulations of the SEC and The Nasdaq Global Market. Our board of directors will have determined that is an audit committee financial expert as defined under the applicable rules of the SEC and has the requisite financial sophistication as defined under the applicable rules and regulations of The Nasdaq Global Market. Under the rules of the SEC, members of the audit committee must also meet heightened independence standards. Our board of directors will have determined that each of , and are independent under the applicable rules of the SEC and The Nasdaq Global Market. The audit committee will operate under a written charter that satisfies the applicable standards of the SEC and The Nasdaq Global Market.

#### **Compensation Committee**

Our compensation committee oversees policies relating to compensation and benefits of our officers and employees. The compensation committee reviews and approves or recommends corporate goals and objectives relevant to compensation of our executive officers (other than our Chief Executive Officer), evaluates the performance of these officers in light of those goals and objectives and approves the compensation of these officers based on such evaluations. The compensation committee also reviews and approves or makes recommendations to our board of directors regarding the issuance of stock options and other awards under our stock plans to our executive officers (other than our Chief Executive Officer). The compensation committee reviews the performance of our Chief Executive Officer and makes recommendations to our board of directors with respect to his compensation and our board of directors retains the authority to make compensation decisions relative to our Chief Executive Officer. The compensation committee will review and evaluate, at least annually, the performance of the compensation committee and its members, including compliance by the compensation committee with its charter.

Effective upon the consummation of this offering, the members of our compensation committee will be , and . will serve as the chairman of the committee. Each of the members of our compensation committee will be independent under the applicable rules and regulations of The Nasdaq Global Market, will be a "non-employee director" as defined in Rule 16b-3 promulgated under the Exchange Act and will be an "outside director" as that term is defined in Section 162(m) of the U.S. Internal Revenue Code of 1986, as amended (Section 162(m)). The compensation committee will operate under a written charter that satisfies the applicable standards of the SEC and The Nasdaq Global Market.

# Nominating and Corporate Governance Committee

The nominating and corporate governance committee is responsible for making recommendations to our board of directors regarding candidates for directorships and the size and composition of our board of directors. In addition, the nominating and corporate governance committee is responsible for overseeing our corporate governance policies and reporting and making recommendations to our board of directors concerning governance matters.

Effective upon the consummation of this offering, the members of our nominating and corporate governance committee will be , and . will serve as the chairman of the committee. Each of the members of our nominating and corporate governance committee will be an independent director under the applicable rules and regulations of The Nasdaq Global Market relating to nominating and corporate governance committee independence. The nominating and corporate governance committee will operate under a written charter that satisfies the applicable standards of the SEC and The Nasdaq Global Market.

## **Compensation Committee Interlocks and Insider Participation**

During the year ended December 31, 2018, our compensation committee consisted of Dr. Margi McLoughlin, who resigned from our board of directors on March 20, 2019, Dr. Huh, who resigned from our board of directors on December 31, 2018 and Dr. Theuer. None of the members of our compensation committee during 2018 nor any of the current members of our compensation committee has at any time been one of our officers or employees. None of our executive officers currently serves, or in the past fiscal year has served, as a member of our board of directors or compensation committee of any entity that has one or more executive officers on our board of directors or compensation committee.

## **Board Diversity**

Upon consummation of this offering, our nominating and corporate governance committee will be responsible for reviewing with our board of directors, on an annual basis, the appropriate characteristics, skills and experience required for our board of directors as a whole and its individual members. In evaluating the suitability of individual candidates (both new candidates and current members), the nominating and corporate governance committee, in recommending candidates for election, and our board of directors, in approving (and, in the case of vacancies, appointing) such candidates, may take into account many factors, including but not limited to the following:

- · personal and professional integrity;
- · ethics and values;
- · experience in corporate management, such as serving as an officer or former officer of a publicly held company;
- experience in the industries in which we compete;
- experience as a board member or executive officer of another publicly held company;
- diversity of expertise and experience in substantive matters pertaining to our business relative to other board members;
- · conflicts of interest; and
- · practical and mature business judgment.

Currently, our board of directors evaluates, and following the consummation of this offering will evaluate, each individual in the context of our board of directors as a whole, with the objective of assembling a group that can best maximize the success of the business and represent stockholder interests through the exercise of sound judgment using its diversity of experience in these various areas.

## **Code of Business Conduct and Ethics**

Prior to the consummation of this offering, we will adopt a code of business conduct and ethics that will apply to all of our employees, officers and directors, including those officers responsible for financial reporting. Following the consummation of this offering, the code of business conduct and ethics will be available on our website. We expect that any amendments to the code, or any waivers of its requirements, will be disclosed on our website.

## Limitation on Liability and Indemnification Matters

Our amended and restated certificate of incorporation, which will become effective immediately prior to the consummation of this offering, will contain provisions that limit the liability of our directors

for monetary damages to the fullest extent permitted by Delaware law. Consequently, our directors will not be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duties as directors, except liability for:

- any breach of the director's duty of loyalty to us or our stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the Delaware General Corporation Law; or
- any transaction from which the director derived an improper personal benefit.

Each of our amended and restated certificate of incorporation and amended and restated bylaws, which will become effective immediately prior to the consummation of this offering, will provide that we are required to indemnify our directors and officers, in each case to the fullest extent permitted by Delaware law. Our amended and restated bylaws will also obligate us to advance expenses incurred by a director or officer in advance of the final disposition of any action or proceeding, and permit us to secure insurance on behalf of any officer, director, employee or other agent for any liability arising out of his or her actions in that capacity regardless of whether we would otherwise be permitted to indemnify him or her under Delaware law. We have entered and expect to continue to enter into agreements to indemnify our directors, executive officers and other employees as determined by our board of directors. With specified exceptions, these agreements provide for indemnification for related expenses including, among other things, attorneys' fees, judgments, fines and settlement amounts incurred by any of these individuals in any action or proceeding. We believe that these bylaw provisions and indemnification agreements are necessary to attract and retain qualified persons as directors and officers. We also maintain directors' and officers' liability insurance.

The limitation of liability and indemnification provisions in our amended and restated certificate of incorporation and amended and restated bylaws may discourage stockholders from bringing a lawsuit against our directors and officers for breach of their fiduciary duty. They may also reduce the likelihood of derivative litigation against our directors and officers, even though an action, if successful, might benefit us and our stockholders. Further, a stockholder's investment may be adversely affected to the extent that we pay the costs of settlement and damages.

## **Director Compensation**

Historically, we have not had a formalized non-employee director compensation program. However, we have generally adopted a practice of paying director fees of \$35,000 per year and issuing options to purchase shares of our common stock targeted at certain percentages of our fully diluted capitalization to our independent directors. We paid director fees in the amount of \$35,000 to both of our independent directors, Dr. Theuer and Dr. Huh, for their board service in fiscal year 2018. In addition, during April 2018, we issued to Dr. Theuer two options to purchase 13,684 shares of our common stock each, with per share exercise prices of \$3.19, one of which was vested as to 100% of its shares at time of grant, and the other of which vested as to 100% of its shares in March 2019, subject to Dr. Theuer's continued service to us through such date. In addition, during April 2018, we issued to Dr. Huh two options to purchase 13,684 shares of our common stock each, with per share exercise prices of \$3.19, one of which was vested as to 100% of its shares in at time of grant, and the other of which vested as to 100% of its shares in March 2019, subject to Dr. Huh's continued service to us through such date. All unvested options accelerate in full on a change in control. We did not make any other equity grants to our non-employee directors in 2018. In addition, we paid consulting fees in the amount of \$50,000 to Dr. Schaffer for research services he provided to us in addition to his service

as a member of the board of directors. We also provide reimbursement to our non-employee directors for their reasonable expenses incurred in attending meetings of our board of directors and committees of our board of directors.

We intend to approve and implement a compensation policy for our non-employee directors, or the Director Compensation Program, to be effective in connection with the consummation of this offering. Pursuant to the Director Compensation Program, our non-employee directors, other than Mr. Burkoth, will receive cash compensation as follows:

- Each non-employee director will receive an annual cash retainer in the amount of \$40,000 per year.
- The chairperson of the audit committee will receive additional annual cash compensation in the amount of \$20,000 per year for such chairperson's service on the audit committee. Each non-chairperson member of the audit committee will receive additional annual cash compensation in the amount of \$10,000 per year for such member's service on the audit committee.
- The chairperson of the compensation committee will receive additional annual cash compensation in the amount of \$12,000 per year for such chairperson's service on the compensation committee. Each non-chairperson member of the compensation committee will receive additional annual cash compensation in the amount of \$6,000 per year for such member's service on the compensation committee.
- The chairperson of the nominating and corporate governance committee will receive additional annual cash compensation in the amount of \$10,000 per year for such chairperson's service on the nominating and corporate governance committee. Each non-chairperson member of the nominating and corporate governance committee will receive additional annual cash compensation in the amount of \$5,000 per year for such member's service on the nominating and corporate governance committee.

Under the Director Compensation Program, each non-employee director, other than Dr. Schaffer, will automatically be granted an option to purchase 9,106 shares of our common stock upon the director's initial appointment or election to our board of directors, referred to as the Initial Grant, and an option to purchase 4,553 shares of our common stock automatically on the date of each annual shareholder meeting thereafter, referred to as the Annual Grant, provided that the non-employee director has, for at least 120 days, been serving on the board of directors as of the date of the shareholder meeting. The Initial Grant will vest as to 1/36th of the underlying shares on a monthly basis over three years, subject to continued service through each applicable vesting date. The Annual Grant will vest on the earlier of the first anniversary of the date of grant or immediately prior to the next annual stockholder's meeting to the extent unvested as of such date, subject to continued service through each applicable vesting date. The exercise price per share of director options is equal to the fair market value of a share of our common stock on the grant date, and the director options will vest in full upon the consummation of a change in control. Upon a change in control (as defined in the 2019 Plan), all outstanding equity awards granted under the 2019 Plan or any other equity incentive plan we maintain that are held by non-employee directors shall become fully vested and/or exercisable, irrespective of any other provisions of the non-employee director's award agreement.

The following table summarizes the total compensation earned during the year ended December 31, 2018 for our non-employee directors.

<u>Name</u>	Fees Earned or Paid in Cash (\$)	Option Awards (\$)(1)	All Other Compensation (\$)(2)	Total (\$)
David Schaffer, Ph.D.	0	0	50,000	50,000
Charles Theuer, M.D., Ph.D.	35,000	60,757	0	95,757
Jacob Chacko, M.D.	0	0	0	0
Hoyoung Huh, M.D., Ph.D.	35,000	60,757	0	95,757
Tony Yao, M.D., Ph.D.	0	0	0	0
William Burkoth	0	0	0	0

Amounts shown represent the grant date fair value of options granted during fiscal year 2018 as calculated in accordance with ASC Topic 718. See Note 12 of the financial statements included in this prospectus for the assumptions used in calculating this amount. As of December 31, 2018, Dr. Theuer held options to purchase 23,017 shares of our common stock, Dr. Huh held options to purchase 101,351 shares of our common stock and no other non-employee directors held any (1) outstanding options or other equity awards.

Amount represents \$50,000 consulting payments paid to Dr. Schaffer in consideration for research services provided us in 2018.

#### **EXECUTIVE COMPENSATION**

The following is a discussion and analysis of compensation arrangements of our named executive officers (NEOs). This discussion contains forward looking statements that are based on our current plans, considerations, expectations and determinations regarding future compensation programs. Actual compensation programs that we adopt may differ materially from currently planned programs as summarized in this discussion. As an "emerging growth company" as defined in the JOBS Act, we are not required to include a Compensation Discussion and Analysis section and have elected to comply with the scaled disclosure requirements applicable to emerging growth companies.

We seek to ensure that the total compensation paid to our executive officers is reasonable and competitive. Compensation of our executives is structured around the achievement of individual performance and near-term corporate targets as well as long-term business objectives.

Our NEOs for fiscal year 2018 were as follows:

- · David Kirn, M.D., our Chief Executive Officer;
- Peter Francis, M.D., Ph.D., our Chief Medical Officer (and formerly our Senior Vice President, Clinical Translational R&D Program Leader, Retina Therapeutic Area for the entirety of fiscal year 2018); and
- · Fred Kamal, Ph.D., our Chief Technical Officer.

# 2018 Summary Compensation Table

The following table sets forth information concerning the compensation of our NEOs for the year ended December 31, 2018.

Name and Principal Position	Year	Salary (\$)( <sup>1</sup> )	Bonus (\$) <sup>(7)</sup>	Option Awards (\$)(2)	Non-Equity Incentive Plan Compensation (\$)(3)	All Other Compensation (\$)(4)	Total (\$)
David Kirn, M.D.  Chief Executive Officer	2018	396,445	0	0	137,700	12,250	546,395
Peter Francis, M.D., Ph.D.  Chief Medical Officer(5)	2018	270,000	24,300	200,024	65,610	46,204	606,138
Fred Kamal, Ph.D.  Chief Technical Officer(6)	2018	70,303	9,552	1,855,340	19,931	9,439	1,964,565

<sup>(1)</sup> Amounts represent (i) for Dr. Kirn, \$382,500 base salary earned in 2018 and \$13,945 paid to him in 2018 in consideration for accrued vacation benefits lost as a result of a decrease in the maximum amount of vacation benefits that employees are able to accrue; (ii) for Dr. Francis, \$157,500 consulting fee for services performed in 2018 prior to Dr. Francis' hire as a full-time employee in August 2018 and \$112,500 base salary earned by Dr. Francis in 2018 as a full-time employee; and (iii) for Dr. Kamal, \$70,303 base salary earned in 2018.

<sup>(2)</sup> Amounts shown represents the grant date fair value of options granted during fiscal year 2018 as calculated in accordance with ASC Topic 718. See Note 12 of the financial statements included in this registration statement for the assumptions used in calculating this amount.

<sup>(3)</sup> Amounts represent the annual performance-based cash bonuses earned by our NEOs based on the achievement of certain corporate performance objectives during 2018. These amounts were paid to the NEOs in early 2019. Please see the descriptions of the annual performance bonuses paid to our named executive officers under "2018 Bonuses" below.

<sup>(4)</sup> Amounts represent: (i) for Dr. Kirn, \$12,250 for matching contributions made by us under our 401(k) plan; (ii) for Dr. Francis, \$43,069 for reimbursements of travel expenses and \$3,135 for matching contributions made by us under our 401(k) plan; and (iii) for Dr. Kamal, \$9,439 for relocation expenses.

<sup>(5)</sup> Dr. Francis commenced employment with us in August 2018, and prior to such time served as a consultant to us.

- (6) Dr. Kamal commenced employment with us in October 2018.
- (7) Amounts represents discretionary, one-time cash bonuses paid to Drs. Francis and Kamal in late 2018 and early 2019 for their performance during 2018.

#### **Outstanding Equity Awards at 2018 Fiscal Year End**

The following table lists all outstanding equity awards held by our NEOs as of December 31, 2018. Dr. Kirn does not hold any outstanding equity awards as of December 31, 2018.

		Option Awards			
	Vesting Commencement	Number of Securities Underlying Unexercised Options Exercisable	Number Of Securities Underlying Unexercised Options Unexercisable	Option Exercise Price	Option Expiration
<u>Name</u>	Date(1)	(#)	(#)	(\$)	Date
Peter Francis, M.D., Ph.D.	9/30/2016(2)	37,830	37,170	3.19	4/19/2028
Fred Kamal, Ph.D.	10/11/2018	0	214,887	8.27	10/11/2028
	10/11/2018	0	71,930	8.27	12/19/2028

<sup>(1)</sup> Except as otherwise noted, options vest as to 25% of the shares on the one year anniversary of the vesting commencement date and vest as to 1/48<sup>th</sup> of the shares monthly thereafter, such that all awards will be vested on the four year anniversary of the vesting commencement date, subject to the holder continuing to provide services to us through such vesting date.

#### **Narrative to Summary Compensation Table**

#### 2018 Salaries

Our NEOs each receive a base salary to compensate them for services rendered to our company. The base salary payable to each NEO is intended to provide a fixed component of compensation reflecting the executive's skill set, experience, role and responsibilities.

For fiscal year 2018, Dr. Kirn's annual base salary was \$375,000 from January 1, 2018 to March 31, 2018, and \$385,000 from April 1, 2018 to December 31, 2018. Drs. Francis' and Kamal's base salaries were prorated for their start dates in August 2018 and October 2018, respectively.

## 2018 Bonuses

We maintain an annual performance-based cash bonus program in which each of our NEOs participated in 2018. Each NEO's target bonus is expressed as a percentage of base salary which can be achieved by meeting company goals at target level. The 2018 annual bonuses for Drs. Kirn, Francis and Kamal were targeted at 40%, 30% and 35%, respectively, of their respective base salaries. Our board of directors has historically reviewed these target percentages to ensure they are adequate, but does not follow a formula. Instead, our board of directors set these rates based on each NEO's experience in their role with us and the level of responsibility held by the NEO, which we believe directly correlates to their ability to influence corporate results.

For determining performance bonus amounts, our board of directors set certain corporate performance goals after receiving input from our Chief Executive Officer. The performance goals generally relate to product development and other goals relating to our business.

Following its review and determinations of corporate performance for 2018, our board of directors determined an achievement level of 90% for each NEO. The actual amount of the cash bonuses

<sup>(2)</sup> The options vested as to 11,280 of the shares on the one year anniversary of the vesting commencement date and vest as to 1,770 of the remaining shares monthly thereafter, subject to the holder continuing to provide services through such vesting date.

awarded to each NEO for 2018 performance are set forth above in the Summary Compensation Table in the column titled "Non-Equity Incentive Plan Compensation."

In addition, in late 2018 and early 2019, we awarded to Drs. Francis and Kamal discretionary bonuses, in consideration for their services to us through 2018. No other NEO received any discretionary bonus for their service in 2018. The one-time cash bonuses awarded to Drs. Francis and Kamal are set forth above in the Summary Compensation Table in the column titled "Bonus."

## **Equity-Based Compensation**

In October and December 2018, we granted to Dr. Kamal options to purchase an aggregate of 286,817 shares of our common stock. Each option vests and becomes exercisable as to 25% of the shares on the one year anniversary of October 11, 2018 and vest as to 1/48th of the shares monthly thereafter, subject to his continued service through the applicable vesting date. The exercise price per share for each option was \$8.27, which was the fair market value of our common stock as of the date of grant. In April 2018, we granted to Dr. Francis options to purchase an aggregate of 75,000 shares of our common stock. The option vests and becomes exercisable as to 11,280 of the shares on the one year anniversary of September 30, 2016 and vest as to 1,770 of the remaining shares monthly thereafter, subject to his continued service through the applicable vesting date. The exercise price per share for each option was \$3.19, which was the fair market value of our common stock as of the date of grant.

We intend to adopt the 2019 Plan, in order to facilitate the grant of cash and equity incentives to directors, employees (including our NEOs) and consultants of our company and certain of its affiliates and to enable us to obtain and retain services of these individuals, which is essential to our long-term success. We expect that the 2019 Plan will be effective on the date on which it is adopted by our board of directors, subject to approval of such plan by our stockholders. For additional information about the 2019 Plan, please see the section titled "Equity Compensation Plans" below.

## Other Elements of Compensation

## Retirement Savings and Health and Welfare Benefits

We currently maintain a 401(k) retirement savings plan for our employees, including our NEOs, who satisfy certain eligibility requirements. Our NEOs are eligible to participate in the 401(k) plan on the same terms as other full-time employees. We make matching contributions equal to 50% of employee contributions of the first ten percent of compensation. Matching contributions will vest annually over 4 years. We believe that providing a vehicle for tax-deferred retirement savings though our 401(k) plan adds to the overall desirability of our executive compensation package and further incentivizes our employees, including our NEOs, in accordance with our compensation policies.

All of our full-time employees, including our NEOs, are eligible to participate in our health and welfare plans, including medical, dental and vision benefits; medical and dependent care flexible spending accounts; short-term and long-term disability insurance; and life and AD&D insurance.

## Perquisites and Other Personal Benefits

We provide limited perquisites to our NEOs, such as the reimbursement of Dr. Kamal's relocation expenses and Dr. Francis' travel reimbursements, when our compensation committee determines that such perquisites are necessary or advisable to fairly compensate or incentivize our employees. We reimbursed Dr. Kamal in the amount of \$9,439 in 2018 for expenses he incurred in connection with his relocation to California related to his commencement of employment. In addition, we reimbursed Dr. Francis in the amount of \$43,069 in 2018 for travel-related expenses incurred in connection with his travel between his home office in Oregon and our headquarters in California.

#### **Executive Compensation Arrangements**

## **Employment Agreements**

As of December 31, 2018, we were party to offer letters with each of our NEOs, which set forth their initial base salary, annual bonus opportunity, initial stock option grant, benefit plans participation and the other benefits noted below for each NEO. In addition, as described below under the heading "—Severance Agreements," we intend to enter into new severance agreements with all of our named executive officers, which agreements supersede and replace the severance benefits set forth in their respective offer letters described herein.

David Kirn, M.D. In addition to the above, Dr. Kirn is eligible to receive certain severance benefits upon qualifying terminations under the offer letter. In the event we terminate Dr. Kirn other than for cause or Dr. Kirn resigns for good reason, then (i) he will receive a lump sum payment equal to 12 months of his base salary, (ii) a lump sum cash payment equal to his prorated annual target bonus for the year in which the termination occurs and (iii) reimbursement of COBRA premiums for up to 12 months. The severance benefits set forth above are subject to his timely execution and non-revocation of a general release of claims against us. In addition, effective as of late 2018, the board approved that Dr. Kirn is also eligible for 100% accelerated vesting of any of his outstanding equity awards upon a change in control, subject to his continued employment through such date.

Peter Francis, M.D., Ph.D. In addition to the above, effective as of Dr. Francis' promotion in early 2019, Dr. Francis is eligible to receive certain severance benefits upon qualifying terminations under the offer letter. In the event we terminate Dr. Francis other than for cause or Dr. Francis resigns for good reason, then (i) he will receive a lump sum payment equal to 9 months of his base salary and (ii) reimbursement of COBRA premiums for up to 9 months. In addition to the severance benefits above, in the event we undergo a change in control, he will receive accelerated vesting of 100% of the then-unvested equity awards held by him. The severance benefits set forth above are subject to his timely execution and non-revocation of a general release of claims against us.

Prior to Dr. Francis becoming an employee in August 2018, he was party to a consulting agreement. The consulting agreement provided for Dr. Francis to provide research and advisory services on our internal retinal program. In consideration for these services, Dr. Francis was entitled to a fixed fee of \$22,500 per month, as well as a discretionary performance bonus, the payment and amount of which was subject to approval by our board of directors. The consulting agreement was superseded in full by his offer letter when he became an employee.

Fred Kamal, Ph.D. In addition to the above, Dr. Kamal is eligible to receive certain severance benefits upon qualifying terminations under the offer letter. In the event we terminate Dr. Kamal other than for cause or Dr. Kamal resigns for good reason, in any case, outside of 1 month prior to, or 12 months following a change in control (as defined in the 2015 Plan), then he will receive (i) a lump sum payment equal to 9 months of his base salary and (ii) reimbursement of COBRA premiums for up to 9 months. In the event we terminate Dr. Kamal other than for cause or Dr. Kamal resigns for good reason, in any case, within the 1 month prior to or the 12 months following a change in control, then, in addition to the benefits in (i) and (ii) above, he will receive accelerated vesting of 100% of the then-unvested equity awards held by him. The severance benefits set forth above are subject to his timely execution and non-revocation of a general release of claims against us and continued compliance with the Confidential Information and Invention Assignment Agreement.

For the purposes of Drs. Kirn's and Francis' offer letters, "cause" is defined as (i) their material failure to perform their principally assigned duties or responsibilities as an employee, director or consultant (other than a failure resulting from disability (as defined under Section 22(e)(3) of the Code); provided that, the failure to achieve certain results, such as the our business plan, in and of itself,

would not constitute "cause"; (ii) their engaging in any act of dishonesty, fraud or material misrepresentation; (iii) their violation of any federal or state law or regulation applicable to our business that results in or could reasonably be expected to result in harm or creates material risk, as determined by the board of directors; (iv) their breach of any confidentiality agreement or invention assignment agreement, or any other material contract made between us and them or violation of any of our written policies; or (v) their being convicted of, or entering a plea of nolo contendere to, any crime or committing any act of moral turpitude. In the case of (i) above, we shall not terminate Drs. Kirn or Francis without first providing them with written notice of the acts or omissions constituting the grounds for such termination and expiration of a reasonable cure period not to exceed thirty 30 days following the date of such notice if we reasonably judge that such failure may be cured within 30 days.

For the purposes of Dr. Kamal's offer letter, "cause" is defined as (i) his material failure to perform his principally assigned duties or responsibilities as an employee, director or consultant (other than a failure resulting from disability (as defined under Section 22(e)(3) of the Code); provided that, the failure to achieve certain results, such as our business plan, in and of itself, would not constitute "cause"; (ii) his engaging in any act of dishonesty, fraud or material misrepresentation; (iii) his violation of any federal or state law or regulation applicable to our business that results in or could reasonably be expected to result in harm, or creates material risk, as determined by the board of directors; (iv) his breach of any confidentiality agreement or invention assignment agreement, or any other material contract between him and us or his violation of any of our written policies (or any of our affiliates); (v) his being convicted of, or entering a plea of nolo contendere to, any crime or committing any act of moral turpitude; or (vi) his commission of any act or involvement in any situation, or occurrence, which brings him into widespread public disrepute, contempt, scandal or ridicule, or which justifiably shocks, insults or offends a significant portion of the community, or his being subject to publicity for any such conduct or involvement in such conduct. In the case of (i) above, we shall not terminate Dr. Kamal without first providing him with written notice of the acts or omissions constituting the grounds for such termination and expiration of a reasonable cure period not to exceed thirty 30 days following the date of such notice if we reasonably judge that such failure may be cured within 30 days.

For the purposes of Dr. Kamal's offer letter, "good reason" is defined as (i) a material diminution in his salary except for across-the-board salary reductions similarly affecting all or substantially all similarly situated employees, (ii) a material diminution in his authority, duties or responsibilities or (iii) a change of more than 50 miles in the geographic location at which he provides services, provided, however, that in the event of the occurrence of a good reason condition listed above, he must provide notice to us within 30 days of the initial occurrence of such condition and allow us 30 days in which to cure such condition. Additionally, in the event we fail to cure the condition within the cure period provided, he must terminate employment with us within sixty (60) days of the end of the cure period.

For the purposes of Dr. Francis' offer letter, "good reason" is defined as the occurrence, without his written consent, of (i) a material diminution in his salary except for across-the-board salary reductions similarly affecting all or substantially all similarly situated employees, (ii) any material and adverse change, including any material diminution in his title, duties, authority or responsibilities, but excluding any such changes in the event of a change of control; provided his remaining duties and responsibilities are consistent with industry norms for the title, (iii) a change of more than 50 miles in the geographic location of our offices, (iv) assignment of duties materially inconsistent with his position, or (v) any material breach by us of the offer letter; provided, however, that in the event of the occurrence of a good reason condition listed above, he must provide written notice to us within 20 days of the initial occurrence of such condition and allow us 30 days in which to cure such condition. His termination will be effective once the 30 day period has lapsed and we have failed to materially cure such acts, failures or failures to act that gave rise to the good reason. We may, in our sole election, waive any cure period such that his termination will be effective on such earlier date determined by our board of directors.

For the purposes of Dr. Kirn's offer letter, "good reason" is defined as (i) a change of more than 50 miles in the geographic location of our offices; (ii) his removal from the our board of directors or (iii) any material and adverse change, including any material diminution in his title, duties, authority or responsibilities, but excluding any such changes or changes in reporting relationships in the event of a Change of Control; provided his remaining duties and responsibilities are consistent with industry norms for the title.

For the purposes of Drs. Kirn's offer letter, a "change in control" occurs when any "person" (as such term is used in Sections 13(d) and 14(d) of the Securities Exchange Act of 1934, as amended) is or becomes the "beneficial owner" (as defined in Rule 13d-3 under the Securities Exchange Act of 1934), directly or indirectly, of our securities representing more than 50% of the total voting power or (ii) on the date of the consummation of a merger or consolidation with any other corporation that has been approved by our stockholders, other than a merger or consolidation which would result in our voting securities outstanding immediately prior to the merger or consolidation continuing to represent at least 50% of the total voting power represented by our voting securities or the voting securities such surviving entity or its parent outstanding immediately after such merger or consolidation; or (iii) the date of the consummation of the sale or disposition by us of all or substantially all the our assets. A transaction will not be deemed a change in control under Dr. Kirn's offer letter unless the transaction qualifies as a "change in control event" within the meaning of Section 409A of the Code.

## Severance Agreements

Pending approval by our board of directors, we intend to enter into new severance agreements with all of our named executive officers, which agreements will supersede and replace the severance benefits set forth in their respective offer letters described above under the heading "—Employment Agreements."

*Dr. Kirn.* Under our proposed new severance agreement with Dr. Kirn, if his employment with us is terminated without "cause" or he resigns for "good reason" (as each is defined below), he will be entitled to receive: (i) 12 months of continued base salary, (ii) payment or reimbursement of the cost of continued healthcare coverage for 12 months and (iii) (A) any earned, but unpaid annual performance bonus for the fiscal year prior to the termination date and (B) a pro-rated (based on days of actual service) amount of the annual bonus that he would have earned had he remained employed by us through the end of the calendar year in which his termination occurs. In lieu of the foregoing benefits, if Dr. Kirn's employment with us is terminated without "cause" or he resigns for "good reason" during the three-month period prior to, or the twelve-month period following, a change in control (as defined in the 2019 Plan), he will be entitled to receive: (i) 18 months of continued base salary, (ii) payment or reimbursement of the cost of continued healthcare coverage for 18 months, (iii) an amount equal to 18 months of his annual bonus for the year of termination assuming 100% of target performance, (iv) full accelerated vesting of any of his unvested equity awards (except for any performance awards) and (v) (A) any earned, but unpaid annual performance bonus for the fiscal year prior to the termination date and (B) a pro-rated (based on days of actual service) amount of the annual bonus that he would have earned had he remained employed by us through the end of the calendar year in which his termination occurs. The foregoing severance benefits are subject to his delivery of an executed release of claims against us and continued compliance with his confidentiality agreement with us.

*Dr. Francis and Dr. Kamal.* Under our proposed new severance agreements with Dr. Francis and Dr. Kamal, if their employment with us is terminated without "cause" or they resign for "good reason" (as each is defined below), they will be entitled to receive: (i) nine months of continued base salary, (ii) payment or reimbursement of the cost of continued healthcare coverage for nine months and (iii) (A) any earned, but unpaid annual performance bonus for the fiscal year prior to the termination date and (B) a pro-rated (based on days of actual service) amount of the annual bonus that they would have

earned had they remained employed by us through the end of the calendar year in which their respective terminations occur. In lieu of the foregoing benefits, if Their employment with us is terminated without "cause" or they resign for "good reason" during the three-month period prior to, or the twelve-month period following, a change in control (as defined in the 2019 Plan), they will be entitled to receive: (i) 12 months of continued base salary, (ii) payment or reimbursement of the cost of continued healthcare coverage for 12 months, (iii) an amount equal to 12 months of their annual bonus for the year of termination assuming 100% of target performance, (iv) full accelerated vesting of any of their unvested equity awards (except any performance awards) and (v) (A) any earned, but unpaid annual performance bonus for the fiscal year prior to the termination date and (B) a pro-rated (based on days of actual service) amount of the annual bonus that they would have earned had they remained employed through the end of the calendar year in which their respective terminations occur. The foregoing severance benefits are subject to their delivery of executed releases of claims against us and continued compliance with their confidentiality agreements with us.

Under Drs. Kirn's, Francis' and Kamal's proposed new severance agreements, "good reason" is defined as the occurrence, without their written consent, of (i) a material and adverse change, including any reduction in their title, duties, authority or responsibilities but excluding any such reduction in connection with a change in control in which their duties and responsibilities remain consistent with industry norms for their title immediately before the change in control, (ii) a reduction by us of their annual base salary or annual bonus or incentive compensation opportunity, other than pursuant to a reduction in compensation that applies to all similarly situated employees, (iii) a requirement by us that their principal place of employment relocate to a location more than 20 miles, (iv) any material breach by us of any employment agreement between them and us and (v) the assignment to them of duties materially inconsistent with their positions with us.

Under Drs. Kirn's, Francis', and Kamal's proposed new severance agreements, "cause" is defined as the occurrence of (i) their material failure to perform their principal assigned duties or responsibilities that goes uncured after notice is provided, provided the failure is not due to their disability, (ii) their engaging in any act of dishonesty, fraud or material misrepresentation, (iii) their violation of any federal or state law or regulation applicable to our business that results in or which we may reasonably expect to result in harm or create material risk to us, (iv) their breach of any confidentiality or invention assignment agreement or other material contract between them and us or violation of our written policies or (v) their being convicted of, or entering a plea of nolo contendere to, any crime or committing any act of moral turpitude.

## **Equity Compensation Plans**

The following summarizes the material terms of the long-term incentive compensation plan in which our NEOs will be eligible to participate following the consummation of this offering and our 2015 Equity Incentive Plan, referred to as the 2015 Plan, under which we have previously made periodic grants of equity and equity-based awards to our NEOs and other key employees. We intend to file with the SEC a registration statement on Form S-8 covering the shares of our common stock issuable under the 2019 Plan, the 2015 Plan and the ESPP.

## 2019 Incentive Award Plan

We intend to adopt the 2019 Plan, which we expect will become effective upon the day prior to the effectiveness of the registration statement to which this prospectus relates, subject to approval of such plan by our stockholders. The principal purpose of the 2019 Plan is to attract, retain and motivate selected employees, consultants and directors through the granting of stock-based compensation awards and cash-based performance bonus awards. The material terms of the 2019 Plan, as it is currently contemplated, are summarized below.

Share reserve. Under the 2019 Plan, 1,214,220 shares of our common stock will be initially reserved for issuance pursuant to a variety of stock-based compensation awards, including stock options, stock appreciation rights (SARs) restricted stock awards, restricted stock unit awards and other stock-based awards. The number of shares initially reserved for issuance or transfer pursuant to awards under the 2019 Plan will be increased by an annual increase on the first day of each fiscal year beginning in 2020 and ending in 2029, equal to the lesser of (i) five percent of the shares of stock outstanding (on an as converted basis) on the last day of the immediately preceding fiscal year and (ii) such smaller number of shares of stock as determined by our board of directors; provided, however, that no more than 13,000,000 shares of stock may be issued upon the exercise of incentive stock options.

The following counting provisions will be in effect for the share reserve under the 2019 Plan:

- to the extent that an award terminates, expires or lapses for any reason or an award is settled in cash without the delivery of shares, any shares subject to the award at such time will be available for future grants under the 2019 Plan;
- to the extent shares are tendered or withheld to satisfy the grant, exercise price or tax withholding obligation with respect to any award under the 2019 Plan, such tendered or withheld shares will be available for future grants under the 2019 Plan;
- to the extent shares subject to SARs are not issued in connection with the stock settlement of SARs on exercise thereof, such shares will be available for future grants under the 2019 Plan;
- to the extent that shares of our common stock are repurchased by us prior to vesting so that shares are returned to us, such shares will be available for future grants under the 2019 Plan;
- the payment of dividend equivalents in cash in conjunction with any outstanding awards will not be counted against the shares available for issuance under the 2019 Plan; and
- to the extent permitted by applicable law or any exchange rule, shares issued in assumption of, or in substitution for, any outstanding awards of any entity acquired in any form of combination by us or any of our subsidiaries will not be counted against the shares available for issuance under the 2019 Plan.

Administration. The compensation committee of our board of directors is expected to administer the 2019 Plan unless our board of directors assumes authority for administration. The compensation committee must consist of at least three members of our board of directors, each of whom is intended to qualify as a "non-employee director" for purposes of Rule 16b-3 under the Exchange Act and an "independent director" within the meaning of the rules of the applicable stock exchange, or other principal securities market on which shares of our common stock are traded. The 2019 Plan provides that the board or compensation committee may delegate its authority to grant awards to employees other than executive officers and certain senior executives to a committee consisting of one or more members of our board of directors or one or more of our officers, other than awards made to our non-employee directors, which must be approved by our full board of directors.

Subject to the terms and conditions of the 2019 Plan, the administrator has the authority to select the persons to whom awards are to be made, to determine the number of shares to be subject to awards and the terms and conditions of awards, and to make all other determinations and to take all other actions necessary or advisable for the administration of the 2019 Plan. The administrator is also authorized to adopt, amend or rescind rules relating to administration of the 2019 Plan. Our board of directors may at any time remove the compensation committee as the administrator and revest in itself the authority to administer the 2019 Plan. The full board of directors will administer the 2019 Plan with respect to awards to non-employee directors.

*Eligibility.* Options, SARs, restricted stock and all other stock-based and cash-based awards under the 2019 Plan may be granted to individuals who are then our officers, employees or consultants or are the officers, employees or consultants of certain of our subsidiaries. Such awards also may be granted to our directors. Only employees of our company or certain of our subsidiaries may be granted incentive stock options (ISOs).

Awards. The 2019 Plan provides that the administrator may grant or issue stock options, SARs, restricted stock, restricted stock units, other stock- or cash-based awards and dividend equivalents or any combination thereof. Each award will be set forth in a separate agreement with the person receiving the award and will indicate the type, terms and conditions of the award.

- Nonstatutory stock options. Nonstatutory Stock Options (NSOs) will provide for the right to purchase shares of our common stock at a specified price which may not be less than fair market value on the date of grant, and usually will become exercisable (at the discretion of the administrator) in one or more installments after the grant date, subject to the participant's continued employment or service with us and/or subject to the satisfaction of corporate performance targets and individual performance targets established by the administrator. NSOs may be granted for any term specified by the administrator that does not exceed ten years.
- Incentive stock options. ISOs will be designed in a manner intended to comply with the provisions of Section 422 of the Code and will be subject to specified restrictions contained in the Code. Among such restrictions, ISOs must have an exercise price of not less than the fair market value of a share of common stock on the date of grant, may only be granted to employees and must not be exercisable after a period of ten years measured from the date of grant. In the case of an ISO granted to an individual who owns (or is deemed to own) at least 10% of the total combined voting power of all classes of our capital stock, the 2019 Plan provides that the exercise price must be at least 110% of the fair market value of a share of common stock on the date of grant and the ISO must not be exercisable after a period of five years measured from the date of grant.
- Restricted stock. Restricted stock may be granted to any eligible individual and made subject to such restrictions as may be
  determined by the administrator. Restricted stock, typically, may be forfeited for no consideration or repurchased by us at the
  original purchase price if the conditions or restrictions on vesting are not met. In general, restricted stock may not be sold or
  otherwise transferred until restrictions are removed or expire. Purchasers of restricted stock, unlike recipients of options, will
  have voting rights and will have the right to receive dividends, if any, prior to the time when the restrictions lapse, however,
  extraordinary dividends will generally be placed in escrow, and will not be released until restrictions are removed or expire.
- Restricted stock units. Restricted stock units may be awarded to any eligible individual, typically without payment of
  consideration, but subject to vesting conditions based on continued employment or service or on performance criteria
  established by the administrator. Like restricted stock, restricted stock units may not be sold, or otherwise transferred or
  hypothecated, until vesting conditions are removed or expire. Unlike restricted stock, stock underlying restricted stock units will
  not be issued until the restricted stock units have vested, and recipients of restricted stock units generally will have no voting or
  dividend rights prior to the time when vesting conditions are satisfied.
- Stock appreciation rights. SARs may be granted in connection with stock options or other awards, or separately. SARs granted
  in connection with stock options or other awards typically will provide for payments to the holder based upon increases in the
  price of our common stock over a set exercise price. The exercise price of any SAR granted under the 2019 Plan must be at
  least 100% of the fair market value of a share of our common stock on the date of grant. SARs under the 2019 Plan will be
  settled in cash or shares of our common stock, or in a combination of both, at the election of the administrator.

- Other stock or cash based awards. Other stock or cash based awards are awards of cash, fully vested shares of our common stock and other awards valued wholly or partially by referring to, or otherwise based on, shares of our common stock. Other stock or cash based awards may be granted to participants and may also be available as a payment form in the settlement of other awards, as standalone payments and as payment in lieu of base salary, bonus, fees or other cash compensation otherwise payable to any individual who is eligible to receive awards. The plan administrator will determine the terms and conditions of other stock or cash based awards, which may include vesting conditions based on continued service, performance and/or other conditions.
- Dividend equivalents. Dividend equivalents represent the right to receive the equivalent value of dividends paid on shares of our common stock and may be granted alone or in tandem with awards other than stock options or SARs. Dividend equivalents are credited as of dividend payments dates during the period between a specified date and the date such award terminates or expires, as determined by the plan administrator. In addition, dividend equivalents with respect to shares covered by a performance award will only be paid to the participant at the same time or times and to the same extent that the vesting conditions, if any, are subsequently satisfied and the performance award vests with respect to such shares.

Any award may be granted as a performance award, meaning that the award will be subject to vesting and/or payment based on the attainment of specified performance goals.

Change in control. In the event of a change in control, unless the plan administrator elects to terminate an award in exchange for cash, rights or other property, or cause an award to accelerate in full prior to the change in control, such award will continue in effect or be assumed or substituted by the acquirer, provided that any performance-based portion of the award will be subject to the terms and conditions of the applicable award agreement. In the event the acquirer refuses to assume or replace awards granted, prior to the consummation of such transaction, awards issued under the 2019 Plan will be subject to accelerated vesting such that 100% of such awards will become vested and exercisable or payable, as applicable. The administrator may also make appropriate adjustments to awards under the 2019 Plan and is authorized to provide for the acceleration, cash-out, termination, assumption, substitution or conversion of such awards in the event of a change in control or certain other unusual or nonrecurring events or transactions.

Adjustments of awards. In the event of any stock dividend or other distribution, stock split, reverse stock split, reorganization, combination or exchange of shares, merger, consolidation, split-up, spin-off, recapitalization, repurchase or any other corporate event affecting the number of outstanding shares of our common stock or the share price of our common stock that would require adjustments to the 2019 Plan or any awards under the 2019 Plan in order to prevent the dilution or enlargement of the potential benefits intended to be made available thereunder, the administrator will make appropriate, proportionate adjustments to: (i) the aggregate number and type of shares subject to the 2019 Plan; (ii) the number and kind of shares subject to outstanding awards and terms and conditions of outstanding awards (including, without limitation, any applicable performance targets or criteria with respect to such awards); and (iii) the grant or exercise price per share of any outstanding awards under the 2019 Plan.

Amendment and termination. The administrator may terminate, amend or modify the 2019 Plan at any time and from time to time. However, we must generally obtain stockholder approval to the extent required by applicable law, rule or regulation (including any applicable stock exchange rule). Notwithstanding the foregoing, an option may be amended to reduce the per share exercise price below the per share exercise price of such option on the grant date and options may be granted in exchange for, or in connection with, the cancellation or surrender of options having a higher per share exercise price without receiving additional stockholder approval.

No incentive stock options may be granted pursuant to the 2019 Plan after the tenth anniversary of the effective date of the 2019 Plan, and no additional annual share increases to the 2019 Plan's aggregate share limit will occur from and after such anniversary. Any award that is outstanding on the termination date of the 2019 Plan will remain in force according to the terms of the 2019 Plan and the applicable award agreement.

## 2015 Equity Incentive Plan

On March 20, 2015, our board of directors adopted, and our stockholders approved, the 2015 Plan. Following the offering, and in connection with the effectiveness of our 2019 Plan, the 2015 Plan will terminate, and no further awards will be granted under the 2015 Plan. However, all outstanding awards will continue to be governed by their existing terms.

Administration. Our board of directors, or a committee thereof appointed by our board of directors, has the authority to administer the 2015 Plan and the awards granted under it. The plan administrator has broad authority to make determinations and interpretations under, prescribe forms for use with and adopt rules for the administration of, the 2015 Plan, subject to its express terms and conditions. The plan administrator also sets the terms and conditions of all awards under the 2015 Plan, including any vesting and acceleration conditions.

Limitation on awards and shares available. The aggregate number of shares of our common stock that is authorized pursuant to the 2015 Plan is 2,694,528, which shares may be authorized but unissued shares, reacquired common stock or represent shares underlying forfeited awards. Shares tendered or withheld to satisfy grant or exercise price or tax withholding obligations associated with an award granted under the 2015 Plan and shares issued pursuant to awards of restricted stock or restricted stock units that are repurchased by us or are forfeited due to the failure to vest may be used again for new grants under the 2015 Plan.

Awards. The 2015 Plan provides that the administrator may grant or issue ISOs, NSOs, SARs, restricted stock and restricted stock units to our employees, directors and consultants, provided that only employees may be granted ISOs. Awards under the 2015 Plan are set forth in award agreements, which detail the terms and conditions of the awards, including any applicable vesting and payment terms and post-termination exercise limitations. Awards are generally settled in shares of our common stock.

- Stock Options. Stock options provide for the purchase of shares of our common stock in the future at an exercise price set on the grant date. ISOs, by contrast to NSOs, may provide tax deferral beyond exercise and favorable capital gains tax treatment to their holders if certain holding period and other Internal Revenue Code requirements are satisfied. The exercise price of a stock option may not be less than 100% of the fair market value of the underlying share on the date of grant (or 110% in the case of ISOs granted to certain significant shareholders), except with respect to certain substitute options granted in connection with a corporate transaction. The term of a stock option may not be longer than ten years (or five years in the case of ISOs granted to certain significant shareholders). Vesting conditions determined by the plan administrator may apply to stock options and may include continued service, performance and/or other conditions. A stock option may provide for "early exercise" prior to vesting in exchange for shares of restricted shares that vest on the option's vesting schedule.
- Stock appreciation rights. SARs may be granted in connection with stock options or other awards, or separately. SARs granted in connection with stock options or other awards typically will provide for payments to the holder based upon increases in the price of our common stock over a set exercise price. The exercise price of any SAR granted under the 2015 Plan must be at least 100% of the fair market value of a share of our common stock on the date of grant.

SARs under the 2015 Plan will be settled in cash or shares of our common stock, or in a combination of both, as set forth in the applicable award agreement.

- Restricted stock. Restricted stock may be granted to any eligible individual and made subject to such restrictions as may be
  determined by the administrator. Restricted stock, typically, may be forfeited for no consideration or repurchased by us at the
  original purchase price if the conditions or restrictions on vesting are not met. In general, restricted stock may not be sold or
  otherwise transferred until restrictions are removed or expire. Purchasers of restricted stock, unlike recipients of options, will
  have voting rights and will have the right to receive dividends, if any, prior to the time when the restrictions lapse.
- Restricted stock units. Restricted stock units may be awarded to any eligible individual, typically without payment of consideration, but subject to vesting conditions continuing service with us or our affiliates, the attainment of performance goals and/or such other conditions as the plan administrator may determine. Like restricted stock, restricted stock units may not be sold, or otherwise transferred or hypothecated, until vesting conditions are removed or expire. Unlike restricted stock, stock underlying restricted stock units will not be issued until the restricted stock units have vested, and recipients of restricted stock units generally will have no voting or dividend rights prior to the time when vesting conditions are satisfied.

Certain transactions. The plan administrator has broad discretion to equitably adjust the provisions of the 2015 Plan, as well as the terms and conditions of existing and future awards, to prevent the diminution or enlargement of intended benefits and facilitate necessary or desirable changes in the event of certain transactions and events affecting our common stock, such as a dividend or other distribution (whether in the form of cash, Shares, other securities or other property), recapitalization, reincorporation, stock split, reverse stock split, reorganization, merger, consolidation, split-up, spin-off, combination, reclassification, repurchase or exchange of our shares or our other securities, or other change in our corporate structure. In the event of a merger with or into another corporation or other entity or a change in control (as defined in the 2015 Plan), the plan administrator may provide, in any combination hereof, subject to the applicable award agreement and without the participant's consent, that (i) the surviving entity assume outstanding awards or substitute economically equivalent awards for such outstanding awards; (ii) that the participant's awards will terminate upon or immediately prior to the consummation of the merger or change in control; (iii) outstanding awards will vest and become exercisable, realizable or payable, or restrictions applicable to an award will lapse, in whole or in part prior to or upon consummation of such merger or change in control; and (iv) the termination of an award in exchange for any combination of cash, property or other rights, if any, selected by the administrator in its sole discretion, equal in value to the cash or other property that would have been attained upon the exercise of such award or realization of the participant's rights as of the date of the occurrence of the transaction. The administrator may also make appropriate adjustments to awards under the 2015 Plan and is authorized to provide for the acceleration, cash-out, termination, assumption, substitution or conversion of such awards in the event of a change in control or certain other unusual or nonrecurring events or transactions.

Foreign participants, transferability and participant payments. The plan administrator may establish sub-plans under the 2015 Plan, subject to the share limits described above, containing such limitations and other terms and conditions that the plan administrator determines is necessary or desirable to satisfy blue sky, securities, tax or other laws of various jurisdictions in which we intend to grant awards or qualifying for favorable tax treatment under applicable foreign laws. With limited exceptions for gifts or executors of the participant's estate upon the participant's death, in connection with certain acquisitions or a change in control, and transfers to us, awards under the 2015 Plan are generally non-transferable prior to exercise or delivery and are exercisable only by the participant. With regard to tax withholding, exercise price and purchase price obligations arising in connection with awards under the 2015 Plan, as applicable, the plan administrator may, in its discretion, accept cash,

check, promissory note, shares of our common stock that meet specified conditions, such other consideration and method of payment for the issuance of shares, to the extent permitted by applicable laws, by "net exercise." a "market sell order" or any combination thereof.

Amendment; termination. Our board of directors may amend or terminate the 2015 Plan at any time; however, (i) no amendment or termination may adversely affect an outstanding award without the affected participant's written consent and (ii) except in connection with certain changes in our capital structure, stockholder approval will be required for (A) any amendment that increases the number of shares available under the 2015 Plan or extends the term of the 2015 Plan, or (B) as required under applicable law. No award may be granted pursuant to the 2015 Plan after the ten year anniversary of the date the 2015 Plan, as amended or restated, was approved by our board of directors or our stockholders (whichever was earlier), however, we will cease granting awards under the 2015 Plan upon effectiveness of the 2019 Plan.

We intend to file with the SEC a registration statement on Form S-8 covering our shares of common stock issuable under the 2015 Plan.

## 2019 Employee Stock Purchase Plan

We intend to adopt and ask our stockholders to approve the ESPP, which will be effective upon the day prior to the effectiveness of the registration statement to which this prospectus relates. The ESPP is designed to allow our eligible employees to purchase shares of our common stock, at semi-annual intervals, with their accumulated payroll deductions. The ESPP is intended to qualify under Section 423 of the Code. The material terms of the ESPP, as it is currently contemplated, are summarized below.

Administration. Subject to the terms and conditions of the ESPP, our compensation committee will administer the ESPP. Our compensation committee can delegate administrative tasks under the ESPP to the services of an agent and/or employees to assist in the administration of the ESPP. The administrator will have the discretionary authority to administer and interpret the ESPP. Interpretations and constructions of the administrator of any provision of the ESPP or of any rights thereunder will be conclusive and binding on all persons. We will bear all expenses and liabilities incurred by the ESPP administrator.

Share reserve. The maximum number of shares of our common stock which will be authorized for sale under the ESPP is equal to the sum of (i) 151,777 shares of common stock and (ii) an annual increase on the first day of each year beginning in 2020 and ending in 2029, equal to the lesser of (A) one percent of the shares of common stock outstanding (on an as converted basis) on the last day of the immediately preceding fiscal year and (B) such number of shares of common stock as determined by our board of directors; provided, however, no more than 3,000,000 shares of our common stock may be issued under the ESPP. The shares reserved for issuance under the ESPP may be authorized but unissued shares or reacquired shares.

Eligibility. Employees eligible to participate in the ESPP for a given offering period generally include employees who are employed by us or one of our subsidiaries on the first day of the offering period, or the enrollment date. Our employees (and, if applicable, any employees of our subsidiaries) who customarily work less than five months in a calendar year or are customarily scheduled to work less than 20 hours per week will not be eligible to participate in the ESPP. Finally, an employee who owns (or is deemed to own through attribution) 5% or more of the combined voting power or value of all our classes of stock or of one of our subsidiaries will not be allowed to participate in the ESPP.

Participation. Employees will enroll under the ESPP by completing a payroll deduction form permitting the deduction from their compensation of at least 1% of their compensation but not more

than 15% of their compensation. Such payroll deductions may be expressed as either a whole number percentage or a fixed dollar amount, and the accumulated deductions will be applied to the purchase of shares on each purchase date. However, a participant may not purchase more than 60,000 shares in each offering period and may not subscribe for more than \$25,000 in fair market value of shares of our common stock (determined at the time the option is granted) during any calendar year. The ESPP administrator has the authority to change these limitations for any subsequent offering period.

Offerings. Generally, the ESPP will offer employees the option to purchase shares through a series of overlapping 24-month offering periods, and each offering period will generally be comprised of four 6-month purchase periods. The initial offering period under the ESPP will be longer than 24 months, commencing upon the effectiveness of this registration statement and ending on November 14, 2021. The initial purchase period under the ESPP will commence on the first day of the offering period and end on May 15, 2020, with the remaining purchase periods in the initial offering period to be comprised of consecutive 6 month periods. In no event may an offering period be longer than 27 months in length.

The option purchase price will be the lower of 85% of the closing trading price per share of our common stock on the trading date immediately preceding the first day of an offering period in which a participant is enrolled or 85% of the closing trading price per share on the trading day immediately preceding the last day of each purchase period.

Unless a participant has previously canceled his or her participation in the ESPP before the purchase date, the participant will be deemed to have exercised his or her option in full as of each purchase date. Upon exercise, the participant will purchase the number of whole shares that his or her accumulated payroll deductions will buy at the option purchase price, subject to the participation limitations listed above.

A participant may cancel his or her payroll deduction authorization at any time prior to the end of the offering period. Upon cancellation, the participant will have the option to either (i) receive a refund of the participant's account balance in cash without interest or (ii) exercise the participant's option for the current offering period for the maximum number of shares of common stock on the applicable purchase date, with the remaining account balance refunded in cash without interest. Following at least one payroll deduction, a participant may also decrease (but not increase) his or her payroll deduction authorization once during any offering period. If a participant wants to increase or decrease the rate of payroll withholding, he or she may do so effective for the next offering period by submitting a new form before the offering period for which such change is to be effective.

A participant may not assign, transfer, pledge or otherwise dispose of (other than by will or the laws of descent and distribution) payroll deductions credited to a participant's account or any rights to exercise an option or to receive shares of our common stock under the ESPP, and during a participant's lifetime, options in the ESPP shall be exercisable only by such participant. Any such attempt at assignment, transfer, pledge or other disposition will not be given effect.

Adjustments upon changes in recapitalization, dissolution, liquidation, merger or asset sale. In the event of any increase or decrease in the number of issued shares of our common stock resulting from a stock split, reverse stock split, stock dividend, combination or reclassification of the common stock, or any other increase or decrease in the number of shares of common stock effected without receipt of consideration by us, we will proportionately adjust the aggregate number of shares of our common stock offered under the ESPP, the number and price of shares which any participant has elected to purchase under the ESPP and the maximum number of shares which a participant may elect to purchase in any single offering period. If there is a proposal to dissolve or liquidate us, then the ESPP will terminate immediately prior to the consummation of such proposed dissolution or liquidation, and any offering period and purchase period then in progress will be shortened by setting a new

purchase date to take place before the date of our dissolution or liquidation. We will notify each participant of such change in writing at least ten business days prior to the new exercise date. If we undergo a merger with or into another corporation or sell all or substantially all of our assets, each outstanding option will be assumed or an equivalent option substituted by the successor corporation or the parent or subsidiary of the successor corporation. If the successor corporation refuses to assume the outstanding options or substitute equivalent options, then any offering period and purchase period then in progress will be shortened by setting a new purchase date to take place before the date of our proposed sale or merger. We will notify each participant of such change in writing at least ten business days prior to the new exercise date.

Amendment and termination. Our board of directors may amend, suspend or terminate the ESPP at any time. However, the board of directors may not amend the ESPP without obtaining stockholder approval within 12 months before or after such amendment to the extent required by applicable laws.

#### CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

The following is a description of transactions since January 1, 2016 to which we have been a party, in which the amount involved exceeds \$120,000, and in which any of our directors, executive officers or beneficial owners of more than 5% of our capital stock, or an affiliate or immediate family member thereof, had or will have a direct or indirect material interest.

#### Sales and Purchases of Securities

## Series B Convertible Preferred Stock Financing

In August 2018, we issued an aggregate of 5,154,632 shares of our Series B convertible preferred stock at \$17.46 per share for aggregate proceeds to us of approximately \$90.0 million.

The table below sets forth the aggregate number of shares of Series B convertible preferred stock sold to our directors, executive officers or owners of more than 5% of a class of our capital stock, or an affiliate or immediate family member thereof:

	Number of	Aggregate
	Shares of	Purchase
	Series B Convertible	Price
<u>Name</u>	Preferred Stock	(\$)
Pfizer Inc.(1)	343,642	5,999,989.32

<sup>(1)</sup> William Burkoth, a member of our board of directors, is an employee of Pfizer Inc.

#### **Director and Executive Officer Compensation**

Please see the sections titled "Director Compensation" and "Executive Compensation" for information regarding the compensation of our directors and executive officers.

# **Employment Agreements**

We have entered into employment agreements with our executive officers. For more information regarding these agreements, see the section titled "Executive Compensation—Narrative to Summary Compensation Table and Outstanding Equity Awards at 2018 Fiscal Year End."

# Indemnification Agreements and Directors' and Officers' Liability Insurance

We have entered into or intend to enter into indemnification agreements with each of our directors and executive officers. These agreements will require us to, among other things, indemnify each director and executive officer to the fullest extent permitted by Delaware law, including indemnification of expenses such as attorneys' fees, judgments, penalties fines and settlement amounts incurred by the director or executive officer in any action or proceeding, including any action or proceeding by or in right of us, arising out of the person's services as a director or executive officer. We have obtained an insurance policy that insures our directors and officers against certain liabilities, including liabilities arising under applicable securities laws. For additional information see the section titled "Management—Limitation of Liability and Indemnification Matters."

## **Investors' Rights Agreement**

We entered into an amended and restated investors' rights agreement with the purchasers of our outstanding redeemable convertible preferred stock, including entities with which certain of our

directors are affiliated. As of June 30, 2019, the holders of approximately 7.4 million shares of our common stock, including the shares of our common stock issuable upon the conversion of our Series A, Series A-1 and Series B redeemable convertible preferred stock, are entitled to rights with respect to the registration of their shares under the Securities Act. For a more detailed description of these registration rights, see the section titled "Description of Capital Stock—Registration Rights." The investors' rights agreement also provides for a right of first refusal in favor of certain holders of redeemable convertible preferred stock with regard to certain issuances of our capital stock. The rights of first refusal will not apply to, and will terminate upon the consummation of, this offering. The investors' right agreement also provides for certain voting arrangements. For a description of these voting arrangements, see the section titled "Management—Board Composition—Voting Arrangements."

## Right of First Refusal and Co-Sale Agreement

We entered into an amended and restated right of first refusal and co-sale agreement with certain holders of our common stock and redeemable convertible preferred stock. This agreement provides for rights of first refusal and co-sale relating to the shares of our common stock held by the parties to the agreement. Upon the consummation of this offering, the amended and restated right of first refusal and co-sale agreement will terminate.

#### **Other Transactions**

In April 2019, we entered into the SRAs with the UC Regents to conduct research in a lab on the Berkeley campus that is under the direction of Dr. Schaffer. Pursuant to the SRAs, we have committed to pay the UC Regents a total of \$1.5 million, of which \$0.4 million was paid upon execution of the SRAs. The SRAs have a three year term ending in 2022. While the SRAs are between us and the UC Regents, the payments under the SRAs may be used to fund the lab under the direction of Dr. Schaffer.

Our former Chief Operating Officer, Anthony Davies, who was employed through November 2017, was the Executive Chairman of Dark Horse Consulting. During the year ended December 31, 2017, we paid \$0.2 million to Dark Horse Consulting under a consulting agreement to design and implement pharmaceutical quality manufacturing of and controls for drug products. The consulting agreement terminated in December 2017.

In 2016, we entered into a consulting agreement with one our former directors, Hoyoung Huh, who resigned as a director in December 2018, to provide business development strategy services. In connection with this agreement, Dr. Huh was granted stock options. In the years ended December 31, 2018 and 2017, we recorded \$219,500 and \$14,700, respectively, of stock-based compensation expense related to such stock options.

In 2016, Dr. Kirn, our Chief Executive Officer and several other of our employees founded Ignite Immunotherapy (Ignite). Dr. Kirn also serves as the Chief Executive Officer and Executive Chairman of Ignite. Certain of our executives hold ownership interests in Ignite and are members of the board of directors of Ignite. Additionally, Pfizer, who is one of our related parties, holds a significant equity stake in Ignite. There have been no transactions between us and Ignite for the years ended December 31, 2018 and 2017.

Other than as described above, since January 1, 2016, we have not entered into any transactions, nor are there any currently proposed transactions, between us and a related party where the amount involved exceeds, or would exceed, \$120,000, and in which any related person had or will have a direct or indirect material interest. We believe the terms of the transactions described above were comparable to terms we could have obtained in arms-length dealings with unrelated third parties.

# **Policies and Procedures for Related Party Transactions**

Prior to the consummation of this offering, our board of directors will adopt a written related person transaction policy, to be effective upon the consummation of this offering, setting forth the policies and procedures for the review and approval or ratification of related person transactions. This policy will cover, with certain exceptions set forth in Item 404 of Regulation S-K under the Securities Act, any transaction, arrangement or relationship, or any series of similar transactions, arrangements or relationships in which we were or are to be a participant, where the amount involved exceeds \$120,000 and a related person had or will have a direct or indirect material interest, including without limitation purchases of goods or services by or from the related person or entities in which the related person has a material interest, indebtedness, guarantees of indebtedness and employment by us of a related person. In reviewing and approving any such transactions, our audit committee is tasked to consider all relevant facts and circumstances, including but not limited to whether the transaction is on terms comparable to those that could be obtained in an arm's length transaction with an unrelated third party and the extent of the related person's interest in the transaction. All of the transactions described in this section occurred prior to the adoption of this policy.

#### PRINCIPAL STOCKHOLDERS

The following table sets forth information relating to the beneficial ownership of our common stock as of July 31, 2019, by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our outstanding shares of our common stock;
- · each of our directors;
- · each of our named executive officers; and
- · all directors and executive officers as a group.

The number of shares beneficially owned by each entity, person, director or executive officer is determined in accordance with the rules of the SEC, and the information is not necessarily indicative of beneficial ownership for any other purpose. Under such rules, beneficial ownership includes any shares over which the individual has sole or shared voting power or investment power as well as any shares that the individual has the right to acquire within 60 days after July 31, 2019 through the exercise of any stock option, warrants or other rights. Except as otherwise indicated, and subject to applicable community property laws, the persons named in the table have sole voting and investment power with respect to all shares of our common stock held by that person.

The percentage of shares beneficially owned is computed on the basis of 12,516,975 shares of our common stock outstanding as of July 31, 2019, which reflects the automatic conversion of 7,375,631 shares of our outstanding shares of redeemable convertible preferred stock into an equivalent number of shares of our common stock. Shares of our common stock that a person has the right to acquire within 60 days after July 31, 2019 are deemed outstanding for purposes of computing the percentage ownership of the person holding such rights, but are not deemed outstanding for purposes of computing the percentage ownership of any other person, except with respect to the percentage ownership of all directors and executive officers as a group. Unless otherwise indicated below, the address for each beneficial owner listed is c/o 4D Molecular Therapeutics, Inc., 5858 Horton Street #455, Emeryville, California 94608.

	Number of Outstanding Shares	Number of Shares Exercisable	Number of Shares	Number of Shares Owned	Percentage of Beneficial Ownership	
Name of Beneficial Owner	Beneficially Owned	Within 60 Days	Beneficially Owned	After the Offering	Before Offering	After Offering
5% and Greater Stockholders:						
Viking Global Opportunities Illiquid Investments Sub-Master						
LP(1)	2,004,581	0	2,004,581	2,004,581	16.0%	
Entities Affiliated with Pfizer(2)	1,474,992	0	1,474,992	1,474,992	11.8%	
Repleon, LLC(3)	909,312	0	909,312	909,312	7.3%	
Executive Officers and Directors:						
David Kirn, M.D.(4)	2,000,000	0	2,000,000	2,000,000	16.0%	
Fred Kamal, Ph.D.	0	0	0	0	_	
Peter Francis, M.D., Ph.D.(5)	0	78,206	78,206	78,206	*	
David Schaffer, Ph.D.(6)	2,000,000	0	2,000,000	2,000,000	16.0%	
Charles Theuer, M.D., Ph.D.(7)	32,351	23,017	55,368	55,368	*	
Jacob Chacko, M.D.	0	0	0	0	_	
Tony Yao, M.D., Ph.D.(8)	458,190	0	458,190	458,190	3.7%	
William Burkoth, MBA	0	0	0	0	_	
All executive officers and directors as a group (8 persons)	4,490,541	101,223	4,591,764	4,591,764	36.4%	
(0 po.00)	.,,		.,552,151	.,001,101	23.170	

- Indicates beneficial ownership of less than 1% of our total outstanding common stock.
- (1) Consists of 2,004,581 shares of our common stock issuable upon the conversion of our Series B redeemable convertible preferred stock held by Viking Global Opportunities Illiquid Investments Sub-Master LP (Opportunities Fund). Opportunities Fund has the authority to dispose of and vote the shares directly owned by it, which power may be exercised by its general partner, Viking Global Opportunities Portfolio GP LLC (Opportunities GP), and by Viking Global Investors LP (VGI), which provides managerial services to Opportunities Fund. O. Andreas Halvorsen, David C. Ott and Rose Shabet, as Executive Committee members of Viking Global Partners LLC (the general partner of VGI) and Opportunities GP, have shared authority to direct the voting and disposition of investments beneficially owned by the Opportunities Fund and Opportunities GP. The business address of each of the entities is c/o Viking Global Investors LP, 55 Railroad Avenue, Greenwich, CT 06830
- Consists of (i) 1,131,350 shares of our common stock issuable upon the conversion of our Series A-1 redeemable convertible preferred stock directly held by Pfizer (2)Ventures (US) LLC and (ii) 343,642 shares of our common stock issuable upon the conversion of our Series B redeemable convertible preferred stock directly held by Pfizer Inc. Pfizer Inc. is the parent company to Pfizer Ventures (US) LLC and may be deemed to beneficially own the shares directly owned by Pfizer Ventures (US) LLC. William Burkoth, a member of our board of directors, is an employee of Pfizer Inc. The address for these entities is 235 East 42nd Street, New York, NY
- (3) Consists of 909,312 shares of our common stock issuable upon the conversion of our Series A redeemable convertible preferred stock. These shares are directly owned by Repleon, LLC. The address for Repleon, LLC is 735 E. Venice Avenue, Suite 200, Venice, FL 34285.
- Consists of 2,000,000 shares of our common stock
- (5) (6) (7) Consists of 78,206 shares of our common stock that may be acquired pursuant to the exercise of stock options within 60 days of July 31, 2019.
  - Consists of 2,000,000 shares of our common stock directly held by the Shaffer-Hinh Family Trust.
- Consists of (i) 32,351 shares of our common stock and (ii) 23,017 shares of our common stock that may be acquired pursuant to the exercise of stock options within 60 days of July 31, 2019.
- (8) Consists of 2,864 shares of our common stock issuable upon the conversion of our Series B redeemable convertible preferred stock held directly by Dr. Yao and, (i) 1,432 shares of our common stock issuable upon the conversion of our Series B redeemable convertible preferred stock directly held by CF Ascent LLC, (ii) 50,830 shares of our common stock issuable upon the conversion of our Series B redeemable convertible preferred stock directly held by Iron Horse Investments, LLC, (iii) 5,727 shares of our common stock issuable upon the conversion of our Series B redeemable convertible preferred stock directly held by Lookfar Investments, LLC, (iv) 229,095 shares of our common stock issuable upon the conversion of our Series B redeemable convertible preferred stock directly held by Meridian Small Cap Growth Fund, (v) 50,830 shares of our common stock issuable upon the conversion of our Series B redeemable convertible preferred stock directly held by THB Iron Rose LLC, (vi) 2,864 shares of our common stock issuable upon the conversion of our Series B redeemable convertible preferred stock directly held by THB Iron Rose LLC, Life Sciences Portfolio, (vii) 57,274 shares of our common stock issuable upon the conversion of our Series B redeemable convertible preferred stock directly held by Arrowmark Fundamental Opportunity Fundl L.P., and (viii) 57,274 shares of our common stock issuable upon the conversion of our Series B redeemable convertible preferred stock directly held by Arrowmark Life Science Fund, LP, which are referred to collectively as the ArrowMark Funds. ArrowMark Colorado Holdings LLC (ArrowMark Colorado) is investment advisor to ArrowMark Funds. Dr. Yao, one of our directors, is employed as a portfolio manager for ArrowMark Colorado and has direct voting and dispositive control over the shares held by the ArrowMark Funds. Dr. Yao may be considered the beneficial owner of the shares held by ArrowMark Funds and disclaims beneficial ownership of such shares, except to the extent of any pecuniary interest therein. The principal business address of the ArrowMark Funds is 100 Fillmore Street, Suite 325, Denver, Colorado 80206.

#### DESCRIPTION OF CAPITAL STOCK

The following summary describes our capital stock and the material provisions of our amended and restated certificate of incorporation and our amended and restated bylaws, which will become effective immediately prior to the consummation of this offering, the amended and restated investors' rights agreement to which we and certain of our stockholders are parties and of the Delaware General Corporation Law. Because the following is only a summary, it does not contain all of the information that may be important to you. For a complete description, you should refer to our amended and restated certificate of incorporation, amended and restated bylaws and amended and restated investors' rights agreement, copies of which have been filed as exhibits to the registration statement of which this prospectus is part.

#### General

Immediately prior to the consummation of this offering, we will file our amended and restated certificate of incorporation that authorizes shares of common stock, \$0.0001 par value per share, and shares of preferred stock, \$0.0001 par value per share. As of June 30, 2019, there were outstanding:

- · 12,516,975 shares of our common stock, on an as-converted basis, held by approximately 46 stockholders of record; and
- 2,192,230 shares of our common stock issuable upon exercise of outstanding stock options.

In connection with this offering, we expect to consummate a forward stock split of our outstanding common stock at a ratio to be determined.

## **Common Stock**

## **Voting Rights**

Each holder of our common stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders, including the election of directors. Our stockholders do not have cumulative voting rights in the election of directors. Accordingly, holders of a majority of the voting shares are able to elect all of the directors. In addition, the affirmative vote of holders of 66 2/3% of the voting power of all of the then outstanding voting stock will be required to take certain actions, including amending certain provisions of our amended and restated certificate of incorporation, such as the provisions relating to amending our amended and restated bylaws, the classified board and director liability.

#### **Dividends**

Subject to preferences that may be applicable to any then outstanding preferred stock, holders of our common stock are entitled to receive dividends, if any, as may be declared from time to time by our board of directors out of legally available funds.

## Liquidation

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities and the satisfaction of any liquidation preference granted to the holders of any then outstanding shares of preferred stock.

## Rights and Preferences

Holders of our common stock have no preemptive, conversion, subscription or other rights, and there are no redemption or sinking fund provisions applicable to our common stock. The rights, preferences and privileges of the holders of our common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of our preferred stock that we may designate in the future.

# Fully Paid and Nonassessable

All of our outstanding shares of common stock are, and the shares of our common stock to be issued in this offering will be, fully paid and nonassessable.

# **Redeemable Convertible Preferred Stock**

Immediately prior to the consummation of this offering, all outstanding shares of our redeemable convertible preferred stock will be converted into shares of our common stock. See Note 10 to our audited financial statements included elsewhere in this prospectus for a description of our currently outstanding redeemable convertible preferred stock. Immediately prior to the consummation of this offering, our amended and restated certificate of incorporation will be amended and restated to delete all references to such shares of redeemable convertible preferred stock. From and after the consummation of this offering, our board of directors will have the authority, without further action by our stockholders, to issue up to shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting, or the designation of, such series, any or all of which may be greater than the rights of our common stock. The issuance of our preferred stock could adversely affect the voting power of holders of our common stock and the likelihood that such holders will receive dividend payments and payments upon our liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing a change in control or other corporate action. Immediately after consummation of this offering, no shares of preferred stock will be outstanding, and we have no present plan to issue any shares of preferred stock.

## **Options**

As of June 30, 2019, we had outstanding options to purchase 2,192,230 shares of our common stock, with a per share weighted-average exercise price of \$6.10, under our 2015 Equity Incentive Plan.

#### **Warrants**

As of June 30, 2019, we had warrants outstanding with the option to purchase 68,669 shares of our common stock, with a weighted-average exercise price of \$1.85 per share.

# **Registration Rights**

Under our amended and restated investors' rights agreement, based on the number of shares outstanding as of December 31, 2018, following the consummation of this offering, the holders of approximately 7.4 million shares of our common stock, or their transferees, have the right to require us to register their shares under the Securities Act so that those shares may be publicly resold, and the holders of approximately 7.4 million shares of our common stock, or their transferees, have the right to include their shares in any registration statement we file, in each case as described below.

## **Demand Registration Rights**

Based on the number of shares outstanding as of December 31, 2018, after the consummation of this offering, the holders of approximately 7.4 million shares of our common stock (on an as-converted basis), or their transferees, will be entitled to certain demand registration rights. Beginning 180 days following the effectiveness of the registration statement of which this prospectus is a part, the holders of at least 50% of these shares can, on not more than two occasions, request that we register all or a portion of their shares if the aggregate price to the public of the shares offered is at least \$30.0 million (before deductions of underwriters' commissions and expenses). Additionally, we will not be required to effect a demand registration during the period beginning 60 days prior to the filing and ending 180 days following the effectiveness of a company-initiated registration statement relating to an initial public offering of our securities.

## Piggyback Registration Rights

Based on the number of shares outstanding as of December 31, 2018, after the consummation of this offering, in the event that we determine to register any of our securities under the Securities Act (subject to certain exceptions), either for our own account or for the account of other security holders, the holders of approximately 7.4 million shares of our common stock (on an as-converted basis), or their transferees, will be entitled to certain "piggyback" registration rights allowing the holders to include their shares in such registration, subject to certain marketing and other limitations. As a result, whenever we propose to file a registration statement under the Securities Act, other than with respect to a registration related to employee benefit plans, the offer and sale of debt securities, or corporate reorganizations or certain other transactions, the holders of these shares are entitled to notice of the registration and have the right, subject to limitations that the underwriters may impose on the number of shares included in the registration, to include their shares in the registration. In an underwritten offering, the managing underwriter, if any, has the right, subject to specified conditions, to exclude or limit the number of shares such holders may include.

# Form S-3 Registration Rights

Based on the number of shares outstanding as of December 31, 2018, after the consummation of this offering, the holders of approximately 7.4 million shares of our common stock (on an as-converted basis), or their transferees, will be entitled to certain Form S-3 registration rights. The holders of at least 30% of the registrable securities then outstanding of these shares can make a written request that we register their shares on Form S-3 if we are eligible to file a registration statement on Form S-3 and if the aggregate price to the public of the shares offered is at least \$5.0 million (before deductions of underwriters' commissions and expenses). These stockholders may make an unlimited number of requests for registration on Form S-3, but in no event shall we be required to file more than two registrations on Form S-3 in any given twelve-month period.

## **Expenses of Registration**

We will pay the registration expenses of the holders of the shares registered pursuant to the demand, piggyback and Form S-3 registration rights described above, including the expenses in an amount not to exceed \$25,000 of one special counsel for the selling holders.

## **Expiration of Registration Rights**

The demand, piggyback and Form S-3 registration rights described above will expire, with respect to any particular stockholder, upon the earlier of five years after the consummation of this offering or when that stockholder can sell all of its shares under Rule 144 of the Securities Act during any three-month period (and without the requirement for us to be in compliance with the current public information required under Section c(1) of Rule 144 of the Securities Act).

# Anti-Takeover Effects of Provisions of Our Amended and Restated Certificate of Incorporation, Our Amended and Restated Bylaws and Delaware Law

Certain provisions of Delaware law and our amended and restated certificate of incorporation and our amended and restated bylaws that will become effective immediately prior to the consummation of this offering contain provisions that could make the following transactions more difficult: acquisition of us by means of a tender offer; acquisition of us by means of a proxy contest or otherwise; or removal of our incumbent officers and directors. It is possible that these provisions could make it more difficult to accomplish or could deter transactions that stockholders may otherwise consider to be in their best interest or in our best interests, including transactions that might result in a premium over the market price for our shares.

These provisions, summarized below, are expected to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to encourage persons seeking to acquire control of us to first negotiate with our board of directors. We believe that the benefits of increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure us outweigh the disadvantages of discouraging these proposals because negotiation of these proposals could result in an improvement of their terms.

## Delaware Anti-Takeover Statute

We are subject to Section 203 of the Delaware General Corporation Law, which prohibits persons deemed "interested stockholders" from engaging in a "business combination" with a publicly-held Delaware corporation for three years following the date these persons become interested stockholders unless the business combination is, or the transaction in which the person became an interested stockholder was, approved in a prescribed manner or another prescribed exception applies. Generally, an "interested stockholder" is a person who, together with affiliates and associates, owns, or within three years prior to the determination of interested stockholder status did own, 15% or more of a corporation's voting stock. Generally, a "business combination" includes a merger, asset or stock sale, or other transaction resulting in a financial benefit to the interested stockholder. The existence of this provision may have an anti-takeover effect with respect to transactions not approved in advance by our board of directors, such as discouraging takeover attempts that might result in a premium over the market price of our common stock.

## **Undesignated Preferred Stock**

The ability to authorize undesignated preferred stock will make it possible for our board of directors to issue preferred stock with voting or other rights or preferences that could impede the success of any attempt to change control of us. These and other provisions may have the effect of deterring hostile takeovers or delaying changes in control or management.

# Special Stockholder Meetings

Our amended and restated bylaws will provide that a special meeting of stockholders may be called at any time by our board of directors, or our President or Chief Executive Officer, but such special meetings may not be called by our stockholders or any other person or persons.

## Requirements for Advance Notification of Stockholder Nominations and Proposals

Our amended and restated bylaws will establish advance notice procedures with respect to stockholder proposals and the nomination of candidates for election as directors, other than nominations made by or at the direction of our board of directors or a committee of our board of directors.

## Elimination of Stockholder Action by Written Consent

Our amended and restated certificate of incorporation and our amended and restated bylaws will eliminate the right of stockholders to act by written consent without a meeting.

## Classified Board; Election and Removal of Directors; Filling Vacancies

Effective upon the consummation of this offering, our board of directors will be divided into three classes. The directors in each class will serve for a three-year term, one class being elected each year by our stockholders, with staggered three-year terms. Only one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms. Because our stockholders do not have cumulative voting rights, our stockholders holding a majority of the shares of our common stock outstanding will be able to elect all of our directors. Our amended and restated certificate of incorporation will provide for the removal of any of our directors only for cause and requires a stockholder vote by the holders of at least a 66 2/3% of the voting power of the then outstanding voting stock. For more information on the classified board, see the section titled "Management—Board Composition." Furthermore, any vacancy on our board of directors, however occurring, including a vacancy resulting from an increase in the size of our board of directors, may only be filled by a resolution of our board of directors unless our board of directors determines that such vacancies shall be filled by our stockholders. This system of electing and removing directors and filling vacancies may tend to discourage a third party from making a tender offer or otherwise attempting to obtain control of us, because it generally makes it more difficult for stockholders to replace a majority of the directors.

## **Choice of Forum**

Our amended and restated certificate of incorporation and our amended and restated bylaws will provide that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the exclusive forum for the following types of actions or proceedings and Delaware statutory or common law: (i) any derivative action or proceeding brought on our behalf; (ii) any action asserting a claim of breach of fiduciary duty; (iii) any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws; (iv) any action to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws; or (v) any action asserting a claim against us that is governed by the internal affairs doctrine. Although our amended and restated certificate of incorporation and amended and restated bylaws will contain the choice of forum provision described above, it is possible that a court could find that such a provision is inapplicable for a particular claim or action or that such provision is unenforceable. The choice of forum provision does not apply to any actions arising under the Securities Act or the Exchange Act.

#### Amendment of Charter Provisions

The amendment of any of the above provisions in our amended and restated certificate of incorporation, except for the provision making it possible for our board of directors to issue undesignated preferred stock, would require approval by a stockholder vote by the holders of at least a 66 2/3% of the voting power of the then outstanding voting stock.

The provisions of the Delaware General Corporation Law, our amended and restated certificate of incorporation and our amended and restated bylaws could have the effect of discouraging others from attempting hostile takeovers and, as a consequence, they may also inhibit temporary fluctuations in the market price of our common stock that often result from actual or rumored hostile takeover attempts.

These provisions may also have the effect of preventing changes in our management. It is possible that these provisions could make it more difficult to accomplish transactions that stockholders may otherwise deem to be in their best interests.

# **Limitations of Liability and Indemnification Matters**

For a discussion of liability and indemnification, see the section titled "Management—Limitation on Liability and Indemnification Matters."

# Listing

We have applied to have our common stock listed on The Nasdaq Global Market under the symbol "DDDD."

# **Transfer Agent and Registrar**

The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, LLC. The transfer agent and registrar's address is 6201 15th Avenue, Brooklyn, New York 11219.

#### SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our common stock. Future sales of our common stock, including shares issued upon the exercise of outstanding options or warrants, in the public market after this offering, or the perception that those sales may occur, could cause the prevailing market price for our common stock to fall or impair our ability to raise equity capital in the future. As described below, only a limited number of shares of our common stock will be available for sale in the public market for a period of several months after consummation of this offering due to contractual and legal restrictions on resale described below. Future sales of our common stock in the public market either before (to the extent permitted) or after restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price of our common stock at such time and our ability to raise equity capital at a time and price we deem appropriate.

## Sale of Restricted Shares

Based on the number of shares of our common stock outstanding as of June 30, 2019 and assuming an initial public offering price of \$ per share, the midpoint of the estimated price range set forth on the cover of this prospectus, upon the consummation of this offering and assuming (i) the conversion of all shares of our outstanding Series A, Series A-1 and Series B redeemable convertible preferred stock as of June 30, 2019, (ii) no exercise of the underwriters' over-allotment option and (iii) no exercise of any of our outstanding options or warrants, we will have outstanding an aggregate of approximately shares of our common stock. Of these shares, all of the shares of our common stock to be sold in this offering, and any shares sold upon exercise of the underwriters' over-allotment option, will be freely tradable in the public market without restriction or further registration under the Securities Act, unless the shares are held by any of our "affiliates" as such term is defined in Rule 144 of the Securities Act. All remaining shares of our common stock held by existing stockholders immediately prior to the consummation of this offering will be "restricted securities" as such term is defined in Rule 144. These restricted securities were issued and sold by us, or will be issued and sold by us, in private transactions and are eligible for public sale only if registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including the exemptions provided by Rule 144 or Rule 701, which rules are summarized below.

As a result of the lock-up agreements referred to below and the provisions of Rule 144 and Rule 701 under the Securities Act, based on the number of shares of our common stock outstanding as of June 30, 2019 and assumptions (i)-(iii) described above, the shares of our common stock (excluding the shares sold in this offering) that will be available for sale in the public market, subject (A) to any waivers by the underwriters and/or our board of directors under the respective lock-up agreements and (B) with respect to shares held by directors, executive officers and other affiliates, the volume limitations under Rule 144 under the Securities Act, are as follows:

Approximate Number of	
Shares	First Date Available for Sale into Public Market
shares	180 days after the date of this prospectus upon expiration of the lock-up agreements referred to below,
	subject in some cases to applicable volume limitations under Rule 144

# **Lock-Up Agreements**

In connection with this offering, we, our executive officers, our directors and substantially all of our other security holders have agreed, subject to certain exceptions, with the underwriters not to

dispose of or hedge any shares of our common stock or securities convertible into or exchangeable for shares of our common stock during the period from the date of the lock-up agreement continuing through the date 180 days after the date of this prospectus, except with the prior written consent of Goldman Sachs & Co. LLC and Evercore Group L.L.C on behalf of the underwriters.

Prior to the completion of the offering, certain of our employees, including our executive officers, and/or directors may enter into written trading plans that are intended to comply with Rule 10b5-1 under the Exchange Act. Sales under these trading plans would not be permitted until the expiration of the lock-up agreements relating to the offering described above.

Following the lock-up periods set forth in the agreements described above, and assuming that the representatives of the underwriters do not release any parties from these agreements, all of the shares of our common stock that are restricted securities or are held by our affiliates as of the date of this prospectus will be eligible for sale in the public market in compliance with Rule 144 under the Securities Act.

#### **Rule 144**

In general, under Rule 144, as currently in effect, once we have been subject to the public company reporting requirements of the Exchange Act for at least 90 days, a person (or persons whose shares are required to be aggregated) who is not deemed to have been one of our "affiliates" for purposes of Rule 144 at any time during the three months preceding a sale, and who has beneficially owned restricted securities within the meaning of Rule 144 for at least six months, including the holding period of any prior owner other than one of our "affiliates," is entitled to sell those shares in the public market (subject to the lock-up agreement referred to above, if applicable) without complying with the manner of sale, volume limitations or notice provisions of Rule 144, but subject to compliance with the public information requirements of Rule 144. If such a person has beneficially owned the shares proposed to be sold for at least one year, including the holding period of any prior owner other than "affiliates," then such person is entitled to sell such shares in the public market without complying with any of the requirements of Rule 144 (subject to the lock-up agreement referred to above, if applicable). In general, under Rule 144, as currently in effect, once we have been subject to the public company reporting requirements of the Exchange Act for at least 90 days, our "affiliates," as defined in Rule 144, who have beneficially owned the shares proposed to be sold for at least six months are entitled to sell in the public market, upon expiration of any applicable lock-up agreements and within any three-month period, a number of those shares of our common stock that does not exceed the greater of:

- 1% of the number of common shares then outstanding, which will equal approximately shares of our common stock immediately after this offering (calculated as of June 30, 2019 on the basis of the assumptions (i)-(iii) described above); or
- the average weekly trading volume of our common stock on The Nasdaq Global Market during the four calendar weeks
  preceding the filing of a notice on Form 144 with respect to such sale.

Such sales under Rule 144 by our "affiliates" or persons selling shares on behalf of our "affiliates" are also subject to certain manner of sale provisions, notice requirements and to the availability of current public information about us. Notwithstanding the availability of Rule 144, the holders of substantially all of our restricted securities have entered into lock-up agreements as referenced above and their restricted securities will become eligible for sale (subject to the above limitations under Rule 144) upon the expiration of the restrictions set forth in those agreements.

# **Rule 701**

In general, under Rule 701 as currently in effect, any of our employees, directors, officers, consultants or advisors who acquired common stock from us in connection with a written

compensatory stock or option plan or other written agreement in compliance with Rule 701 under the Securities Act before the effective date of the registration statement of which this prospectus is a part (to the extent such common stock is not subject to a lock-up agreement) is entitled to rely on Rule 701 to resell such shares beginning 90 days after we become subject to the public company reporting requirements of the Exchange Act in reliance on Rule 144, but without compliance with the holding period requirements contained in Rule 144. Accordingly, subject to any applicable lock-up agreements, beginning 90 days after we become subject to the public company reporting requirements of the Exchange Act, under Rule 701 persons who are not our "affiliates," as defined in Rule 144, may resell those shares without complying with the minimum holding period or public information requirements of Rule 144, and persons who are our "affiliates" may resell those shares without compliance with Rule 144's minimum holding period requirements (subject to the terms of the lock-up agreement referred to above).

## **Registration Rights**

Based on the number of shares outstanding as of June 30, 2019, after the consummation of this offering, the holders of approximately 7.4 million shares of our common stock, or their transferees, will, subject to the lock-up agreements referred to above, be entitled to certain rights with respect to the registration of the offer and sale of those shares under the Securities Act. For a description of these registration rights, see the section titled "Description of Capital Stock—Registration Rights." If the offer and sale of these shares are registered, they will be freely tradable without restriction under the Securities Act.

#### Stock Plans

We intend to file with the SEC a registration statement under the Securities Act covering the shares of our common stock that we may issue upon exercise of outstanding options reserved for issuance under our 2015 Equity Incentive Plan and our 2019 Plan. Such registration statement is expected to be filed and become effective as soon as practicable after the consummation of this offering. Accordingly, shares registered under such registration statement will be available for sale in the open market following its effective date, subject to Rule 144 volume limitations and the lock-up agreements described above, if applicable.

## MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES TO NON-U.S. HOLDERS

The following discussion is a summary of the material U.S. federal income tax consequences to Non-U.S. Holders (as defined below) of the purchase, ownership and disposition of our common stock issued pursuant to this offering, but does not purport to be a complete analysis of all potential tax effects. The effects of other U.S. federal tax laws, such as estate and gift tax laws, and any applicable state, local or non-U.S. tax laws are not discussed. This discussion is based on the Code, Treasury Regulations promulgated thereunder, judicial decisions, and published rulings and administrative pronouncements of the IRS in each case in effect as of the date hereof. These authorities may change or be subject to differing interpretations. Any such change or differing interpretation may be applied retroactively in a manner that could adversely affect a Non-U.S. Holder. We have not sought and will not seek any rulings from the IRS regarding the matters discussed below. There can be no assurance the IRS or a court will not take a contrary position to that discussed below regarding the tax consequences of the purchase, ownership and disposition of our common stock.

This discussion is limited to Non-U.S. Holders that hold our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment). This discussion does not address all U.S. federal income tax consequences relevant to a Non-U.S. Holder's particular circumstances, including the impact of the Medicare contribution tax on net investment income. In addition, it does not address consequences relevant to Non-U.S. Holders subject to special rules, including, without limitation:

- · U.S. expatriates and former citizens or long-term residents of the United States;
- persons subject to the alternative minimum tax;
- persons holding our common stock as part of a hedge, straddle or other risk reduction strategy or as part of a conversion transaction or other integrated investment;
- banks, insurance companies, and other financial institutions;
- brokers, dealers or traders in securities;
- "controlled foreign corporations," "passive foreign investment companies" and corporations that accumulate earnings to avoid U.S. federal income tax:
- partnerships or other entities or arrangements treated as partnerships for U.S. federal income tax purposes (and investors therein);
- tax-exempt organizations or governmental organizations;
- persons deemed to sell our common stock under the constructive sale provisions of the Code;
- · tax-qualified retirement plans; and
- "qualified foreign pension funds" as defined in Section 897(I)(2) of the Code and entities all of the interests of which are held by qualified foreign pension funds.

If an entity treated as a partnership for U.S. federal income tax purposes holds our common stock, the tax treatment of a partner in the partnership will depend on the status of the partner, the activities of the partnership and certain determinations made at the partner level. Accordingly, partnerships holding our common stock and the partners in such partnerships should consult their tax advisors regarding the U.S. federal income tax consequences to them.

THIS DISCUSSION IS NOT TAX ADVICE. INVESTORS SHOULD CONSULT THEIR TAX ADVISORS WITH RESPECT TO THE APPLICATION OF THE U.S. FEDERAL INCOME TAX LAWS TO THEIR PARTICULAR SITUATIONS AS WELL AS ANY TAX CONSEQUENCES OF THE

PURCHASE, OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK ARISING UNDER THE U.S. FEDERAL ESTATE OR GIFT TAX LAWS OR UNDER THE LAWS OF ANY STATE, LOCAL OR NON-U.S. TAXING JURISDICTION OR UNDER ANY APPLICABLE INCOME TAX TREATY.

#### **Definition of Non-U.S. Holder**

For purposes of this discussion, a "Non-U.S. Holder" is any beneficial owner of our common stock that is neither a "U.S. person" nor an entity treated as a partnership for U.S. federal income tax purposes. A U.S. person is any person that, for U.S. federal income tax purposes, is or is treated as any of the following:

an individual who is a citizen or resident of the United States:

a corporation created or organized under the laws of the United States, any state thereof, or the District of Columbia;

an estate, the income of which is subject to U.S. federal income tax regardless of its source; or

a trust that (i) is subject to the primary supervision of a U.S. court and all substantial decisions of which are subject to the control of one or more "United States persons" (within the meaning of Section 7701(a)(30) of the Code), or (ii) has a valid election in effect to be treated as a United States person for U.S. federal income tax purposes.

#### **Distributions**

As described in the section titled "Dividend Policy," we do not anticipate paying any cash dividends in the foreseeable future. However, if we do make distributions of cash or property on our common stock, such distributions will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. Amounts not treated as dividends for U.S. federal income tax purposes will constitute a return of capital and first be applied against and reduce a Non-U.S. Holder's adjusted tax basis in its common stock, but not below zero. Any excess will be treated as capital gain and will be treated as described below under "—Sale or Other Taxable Disposition."

Subject to the discussion below on effectively connected income, dividends paid to a Non-U.S. Holder will be subject to U.S. federal withholding tax at a rate of 30% of the gross amount of the dividends (or such lower rate specified by an applicable income tax treaty, provided the Non-U.S. Holder furnishes a valid IRS Form W-8BEN or W-8BEN-E (or other applicable documentation) certifying qualification for the lower treaty rate). A Non-U.S. Holder that does not timely furnish the required documentation, but that qualifies for a reduced treaty rate, may obtain a refund of any excess amounts withheld by timely filing an appropriate claim for refund with the IRS. Non-U.S. Holders should consult their tax advisors regarding their entitlement to benefits under any applicable tax treaties.

If dividends paid to a Non-U.S. Holder are effectively connected with the Non-U.S. Holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, the Non-U.S. Holder maintains a permanent establishment in the United States to which such dividends are attributable), the Non-U.S. Holder will be exempt from the U.S. federal withholding tax described above. To claim the exemption, the Non-U.S. Holder must furnish to the applicable withholding agent a valid IRS Form W-8ECI, certifying that the dividends are effectively connected with the Non-U.S. Holder's conduct of a trade or business within the United States.

Any such effectively connected dividends will be subject to U.S. federal income tax on a net income basis at the regular graduated rates. A Non-U.S. Holder that is a corporation also may be

subject to a branch profits tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty) on such effectively connected dividends, as adjusted for certain items. Non-U.S. Holders should consult their tax advisors regarding any applicable tax treaties that may provide for different rules.

## Sale or Other Taxable Disposition

Subject to the discussions below regarding backup withholding, a Non-U.S. Holder will not be subject to U.S. federal income tax on any gain realized upon the sale or other taxable disposition of our common stock unless:

- the gain is effectively connected with the Non-U.S. Holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, the Non-U.S. Holder maintains a permanent establishment in the United States to which such gain is attributable);
- the Non-U.S. Holder is a nonresident alien individual present in the United States for 183 days or more during the taxable year of the disposition and certain other requirements are met; or
- our common stock constitutes a U.S. real property interest (USRPI) by reason of our status as a U.S. real property holding corporation (USRPHC) for U.S. federal income tax purposes.

Gain described in the first bullet point above generally will be subject to U.S. federal income tax on a net income basis at the regular graduated rates. A Non-U.S. Holder that is a corporation also may be subject to a branch profits tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty) on such effectively connected gain, as adjusted for certain items.

Gain described in the second bullet point above will be subject to U.S. federal income tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty), which may be offset by certain U.S. source capital losses of the Non-U.S. Holder (even though the individual is not considered a resident of the United States), provided the Non-U.S. Holder has timely filed U.S. federal income tax returns with respect to such losses.

With respect to the third bullet point above, we believe we currently are not, and do not anticipate becoming, a USRPHC. Because the determination of whether we are a USRPHC depends, however, on the fair market value of our USRPIs relative to the fair market value of our non-U.S. real property interests and our other business assets, there can be no assurance we currently are not a USRPHC or will not become one in the future. Even if we are or were to become a USRPHC, gain arising from the sale or other taxable disposition by a Non-U.S. Holder will not be subject to U.S. federal income tax if our common stock is "regularly traded," as defined by applicable Treasury Regulations, on an established securities market, and such Non-U.S. Holder owned, actually and constructively, 5% or less of our common stock throughout the shorter of the five-year period ending on the date of the sale or other taxable disposition or the Non-U.S. Holder's holding period.

Non-U.S. Holders should consult their tax advisors regarding any applicable tax treaties that may provide for different rules.

# Information Reporting and Backup Withholding

Payments of dividends on our common stock will not be subject to backup withholding, provided the Non-U.S. Holder certifies its non-U.S. status, such as by furnishing a valid IRS Form W-8BEN, W-8BEN-E or W-8ECI, or otherwise establishes an exemption. However, information returns are

required to be filed with the IRS in connection with any dividends on our common stock paid to the Non-U.S. Holder, regardless of whether any tax was actually withheld. In addition, proceeds of the sale or other taxable disposition of our common stock within the United States or conducted through certain U.S.-related brokers generally will not be subject to backup withholding or information reporting if the applicable withholding agent receives the certification described above or the Non-U.S. Holder otherwise establishes an exemption. Proceeds of a disposition of our common stock conducted through a non-U.S. office of a non-U.S. broker that does not have certain enumerated relationships with the United States generally will not be subject to backup withholding or information reporting.

Copies of information returns that are filed with the IRS may also be made available under the provisions of an applicable treaty or agreement to the tax authorities of the country in which the Non-U.S. Holder resides or is established.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules may be allowed as a refund or a credit against a Non-U.S. Holder's U.S. federal income tax liability, provided the required information is timely furnished to the IRS.

## Additional Withholding Tax on Payments Made to Foreign Accounts

Withholding taxes may be imposed under Sections 1471 to 1474 of the Code (such Sections commonly referred to as the Foreign Account Tax Compliance Act (FATCA)) on certain types of payments made to non-U.S. financial institutions and certain other non-U.S. entities. Specifically, a 30% withholding tax may be imposed on dividends on, or (subject to the proposed Treasury Regulations discussed below) gross proceeds from the sale or other disposition of, our common stock paid to a "foreign financial institution" or a "non-financial foreign entity" (each as defined in the Code), unless (i) the foreign financial institution undertakes certain diligence and reporting obligations, (ii) the non-financial foreign entity either certifies it does not have any "substantial United States owners" (as defined in the Code) or furnishes identifying information regarding each substantial United States owner, or (iii) the foreign financial institution or non-financial foreign entity otherwise qualifies for an exemption from these rules. If the payee is a foreign financial institution and is subject to the diligence and reporting requirements in (i) above, it must enter into an agreement with the U.S. Department of the Treasury requiring, among other things, that it undertake to identify accounts held by certain "specified United States persons" or "United States owned foreign entities" (each as defined in the Code), annually report certain information about such accounts, and withhold 30% on certain payments to non-compliant foreign financial institutions and certain other account holders. Foreign financial institutions located in jurisdictions that have an intergovernmental agreement with the United States governing FATCA may be subject to different rules.

Under the applicable Treasury Regulations and administrative guidance, withholding under FATCA generally applies to payments of dividends on our common stock. While withholding under FATCA would have applied also to payments of gross proceeds from the sale or other disposition of stock on or after January 1, 2019, recently proposed Treasury Regulations eliminate FATCA withholding on payments of gross proceeds entirely. Taxpayers generally may rely on these proposed Treasury Regulations until final Treasury Regulations are issued.

Prospective investors should consult their tax advisors regarding the potential application of withholding under FATCA to their investment in our common stock.

## **UNDERWRITING**

We and the underwriters named below have entered into an underwriting agreement with respect to the shares being offered. Subject to certain conditions, each underwriter has severally agreed to purchase the number of shares indicated in the following table. Goldman Sachs & Co. LLC and Evercore Group L.L.C. are the representatives of the underwriters.

Underwriters	Number of Shares
Goldman Sachs & Co. LLC	
Evercore Group L.L.C.	
William Blair & Company, L.L.C.	
Chardan Capital Markets LLC	
Total	

The underwriters are committed to take and pay for all of the shares being offered, if any are taken, other than the shares covered by the option described below unless and until this option is exercised.

The underwriters have an over-allotment option to buy up to an additional shares from us to cover sales by the underwriters of a greater number of shares than the total number set forth in the table above. They may exercise that option for 30 days. If any shares are purchased pursuant to this option, the underwriters will severally purchase shares in approximately the same proportion as set forth in the table above. The option may be exercised only to cover any over-allotments of common stock.

The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters by us. Such amounts are shown assuming both no exercise and full exercise of the underwriters' over-allotment option.

	No Exercise	Full Exercise
Per Share	\$	\$
Total	\$	\$

Shares sold by the underwriters to the public will initially be offered at the initial public offering price set forth on the cover of this prospectus. Any shares sold by the underwriters to securities dealers may be sold at a discount of up to \$ per share from the initial public offering price. After the initial offering of the shares, the representatives may change the offering price and the other selling terms. The offering of the shares by the underwriters is subject to receipt and acceptance and subject to the underwriters' right to reject any order in whole or in part.

We and our executive officers, directors, and holders of substantially all of our common stock and securities convertible into or exchangeable for our common stock have agreed or will agree with the underwriters, subject to certain exceptions, not to dispose of or hedge any of our or their common stock or securities convertible into or exchangeable for shares of our common stock during the period from the date of this prospectus continuing through the date 180 days after the date of this prospectus, except with the prior written consent of Goldman Sachs & Co. LLC and Evercore Group L.L.C. This agreement does not apply to any existing employee benefit plans. See the section titled "Shares Eligible for Future Sale" for a discussion of certain transfer restrictions.

Prior to the offering, there has been no public market for the shares. The initial public offering price has been negotiated among us and the representatives. Among the factors to be considered in

determining the initial public offering price of the shares, in addition to prevailing market conditions, will be the our historical performance, estimates of the business potential and earnings prospects, an assessment of our management and the consideration of the above factors in relation to market valuation of companies in related businesses.

We have applied to list our common stock on The Nasdaq Global Market under the symbol "DDDD."

In connection with the offering, the underwriters may purchase and sell shares of our common stock in the open market. These transactions may include short sales, stabilizing transactions and purchases to cover positions created by short sales. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in the offering, and a short position represents the amount of such sales that have not been covered by subsequent purchases. A "covered short position" is a short position that is not greater than the amount of additional shares for which the underwriters' over-allotment option described above may be exercised. The underwriters may cover any covered short position by either exercising their over-allotment option or purchasing shares in the open market. In determining the source of shares to cover the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase additional shares pursuant to the over-allotment option described above. "Naked" short sales are any short sales that create a short position greater than the amount of additional shares for which the over-allotment option described above may be exercised. The underwriters must cover any such naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in the offering. Stabilizing transactions consist of various bids for or purchases of common stock made by the underwriters in the open market prior to the completion of the offering.

The underwriters may also impose a penalty bid. This occurs when a particular underwriter repays to the underwriters a portion of the underwriting discount received by it because the representatives have repurchased shares sold by or for the account of such underwriter in stabilizing or short covering transactions.

Purchases to cover a short position and stabilizing transactions, as well as other purchases by the underwriters for their own accounts, may have the effect of preventing or retarding a decline in the market price of our common stock, and together with the imposition of the penalty bid, may stabilize, maintain or otherwise affect the market price of our common stock. As a result, the price of our common stock may be higher than the price that otherwise might exist in the open market. The underwriters are not required to engage in these activities and may end any of these activities at any time. These transactions may be effected on The Nasdaq Global Market, in the over-the-counter market or otherwise.

We estimate that our share of the total expenses of the offering, excluding underwriting discounts and commissions, will be approximately \$\\$. We have agreed to reimburse the underwriters for certain of their expenses in an amount up to \$\\$.

We have agreed to indemnify the several underwriters against certain liabilities, including liabilities under the Securities Act.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include sales and trading, commercial and investment banking, advisory, investment management, investment research, principal investment, hedging, market making, brokerage and other financial and non-financial activities and services. Certain of the underwriters and

their respective affiliates may in the future provide, a variety of these services to the issuer and to persons and entities with relationships with the issuer, for which they will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and their respective affiliates, officers, directors and employees may purchase, sell or hold a broad array of investments and actively trade securities, derivatives, loans, commodities, currencies, credit default swaps and other financial instruments for their own account and for the accounts of their customers, and such investment and trading activities may involve or relate to assets, securities or instruments of the issuer (directly, as collateral securing other obligations or otherwise) or persons and entities with relationships with the issuer. The underwriters and their respective affiliates may also communicate independent investment recommendations, market color or trading ideas or publish or express independent research views in respect of such assets, securities or instruments and may at any time hold, or recommend to clients that they should acquire, long and/or short positions in such assets, securities and instruments.

## **European Economic Area**

In relation to each member state of the European Economic Area (each, a Member State), no securities have been offered or will be offered pursuant to the offering to the public in that Member State prior to the publication of a prospectus in relation to the securities which has been approved by the competent authority in that Member State or, where appropriate, approved in another Member State and notified to the competent authority in that Member State, all in accordance with the Prospectus Regulation, except that offers of securities may be made to the public in that Member State at any time under the following exemptions under the Prospectus Regulation:

- to any legal entity which is a qualified investor as defined in the Prospectus Regulation;
- to fewer than 150 natural or legal persons (other than qualified investors as defined in the Prospectus Regulation), subject to
  obtaining the prior consent of the representatives for any such offer; or
- in any other circumstances falling within Article 1(4) of the Prospectus Regulation,

*provided* that no such offer of securities shall require us or any underwriter to publish a prospectus pursuant to Article 3 of the Prospectus Regulation or supplement a prospectus pursuant to Article 23 of the Prospectus Regulation.

For the purposes of this provision, the expression an "offer to the public" in relation to any securities in any Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any securities to be offered so as to enable an investor to decide to purchase or subscribe for any securities, and the expression "Prospectus Regulation" means Regulation (EU) 2017/1129.

This European Economic Area selling restriction is in addition to any other selling restrictions set out below.

## **United Kingdom**

In the United Kingdom, this prospectus is only addressed to and directed at qualified investors who are (i) investment professionals falling within Article 19(5) of the Financial Services and Markets

Act 2000, as amended (Financial Promotion), Order 2005 (the Order); or (ii) high net worth entities and other persons to whom it may lawfully be communicated, falling within Article 49(2)(a) to (d) of the Order (all such persons together being referred to as "relevant persons"). Any investment or investment activity to which this prospectus relates is available only to relevant persons and will only be engaged in with relevant persons. Any person who is not a relevant person should not act or rely on this prospectus or any of its contents.

## Canada

The securities may be sold in Canada only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions, and Ongoing Registrant Obligations. Any resale of the securities must be made in accordance with an exemption form, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

## **Hong Kong**

The securities may not be offered or sold in Hong Kong by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32 of the Laws of Hong Kong) (Companies (Winding Up and Miscellaneous Provisions) Ordinance) or which do not constitute an invitation to the public within the meaning of the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong) (Securities and Futures Ordinance), or (ii) to "professional investors" as defined in the Securities and Futures Ordinance and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a "prospectus" as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance, and no advertisement, invitation or document relating to the securities may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" in Hong Kong as defined in the Securities and Futures Ordinance and any rules made thereunder.

## **Singapore**

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or

distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor (as defined under Section 4A of the Securities and Futures Act, Chapter 289 of Singapore (the SFA)) under Section 274 of the SFA, (ii) to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA, in each case subject to conditions set forth in the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor, the securities (as defined in Section 239(1) of the SFA) of that corporation shall not be transferable for 6 months after that corporation has acquired the shares under Section 275 of the SFA except: (i) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (ii) where such transfer arises from an offer in that corporation's securities pursuant to Section 275(1A) of the SFA, (iii) where no consideration is or will be given for the transfer, (iv) where the transfer is by operation of law, (v) as specified in Section 276(7) of the SFA, or (vi) as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore (Regulation 32).

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a trust (where the trustee is not an accredited investor (as defined in Section 4A of the SFA)) whose sole purpose is to hold investments and each beneficiary of the trust is an accredited investor, the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferable for 6 months after that trust has acquired the shares under Section 275 of the SFA except: (i) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (ii) where such transfer arises from an offer that is made on terms that such rights or interest are acquired at a consideration of not less than S\$200,000 (or its equivalent in a foreign currency) for each transaction (whether such amount is to be paid for in cash or by exchange of securities or other assets), (iii) where no consideration is or will be given for the transfer, (iv) where the transfer is by operation of law, (v) as specified in Section 276(7) of the SFA, or (vi) as specified in Regulation 32.

Solely for the purposes of its obligations pursuant to Section 309B of the SFA, we have determined, and hereby notify all relevant persons (as defined in the CMP Regulations 2018), that the shares are "prescribed capital markets products" (as defined in the CMP Regulations 2018) and Excluded Investment Products (as defined in MAS Notice SFA 04-N12: Notice on the Sale of Investment Products and MAS Notice FAA-N16: Notice on Recommendations on Investment Products).

## Japan

The securities have not been and will not be registered under the Financial Instruments and Exchange Act of Japan (Act No. 25 of 1948, as amended) (the FIEA). The securities may not be offered or sold, directly or indirectly, in Japan or to or for the benefit of any resident of Japan (including any person resident in Japan or any corporation or other entity organized under the laws of Japan) or to others for reoffering or resale, directly or indirectly, in Japan or to or for the benefit of any resident of Japan, except pursuant to an exemption from the registration requirements of the FIEA and otherwise in compliance with any relevant laws and regulations of Japan.

#### Australia

No placement document, prospectus, product disclosure statement or other disclosure document has been lodged with the Australian Securities and Investments Commission (ASIC), in relation to the offering. This offering document does not constitute a prospectus, product disclosure statement or other disclosure document under the Corporations Act 2001 (the Corporations Act), and does not purport to include the information required for a prospectus, product disclosure statement or other disclosure document under the Corporations Act.

Any offer in Australia of the shares may only be made to persons (the Exempt Investors) who are "sophisticated investors" (within the meaning of section 708(8) of the Corporations Act), "professional investors" (within the meaning of section 708(11) of the Corporations Act) or otherwise pursuant to one or more exemptions contained in section 708 of the Corporations Act so that it is lawful to offer the shares without disclosure to investors under Chapter 6D of the Corporations Act.

The shares applied for by Exempt Investors in Australia must not be offered for sale in Australia in the period of 12 months after the date of allotment under the offering, except in circumstances where disclosure to investors under Chapter 6D of the Corporations Act would not be required pursuant to an exemption under section 708 of the Corporations Act or otherwise or where the offer is pursuant to a disclosure document which complies with Chapter 6D of the Corporations Act. Any person acquiring shares must observe such Australian on-sale restrictions.

This offering document contains general information only and does not take account of the investment objectives, financial situation or particular needs of any particular person. It does not contain any securities recommendations or financial product advice. Before making an investment decision, investors need to consider whether the information in this offering document is appropriate to their needs, objectives and circumstances, and, if necessary, seek expert advice on those matters.

## **Dubai International Financial Centre**

This offering document relates to an Exempt Offer in accordance with the Offered Securities Rules of the Dubai Financial Services Authority (DFSA). This offering document is intended for distribution only to persons of a type specified in the Offered Securities Rules of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus nor taken steps to verify the information set forth in this prospectus and has no responsibility for the offering document. The securities to which this offering document relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the securities offered should conduct their own due diligence on the securities. If you do not understand the contents of this offering document you should consult an authorized financial advisor.

## **Switzerland**

We have not and will not register with the Swiss Financial Market Supervisory Authority (FINMA) as a foreign collective investment scheme pursuant to Article 119 of the Federal Act on Collective Investment Scheme of 23 June 2006, as amended (CISA), and accordingly the securities being offered pursuant to this prospectus have not and will not be approved, and may not be licenseable, with FINMA. Therefore, the securities have not been authorized for distribution by FINMA as a foreign collective investment scheme pursuant to Article 119 CISA and the securities offered hereby may not be offered to the public (as this term is defined in Article 3 CISA) in or from Switzerland. The securities may solely be offered to "qualified investors," as this term is defined in Article 10 CISA, and in the

circumstances set out in Article 3 of the Ordinance on Collective Investment Scheme of 22 November 2006, as amended (CISO), such that there is no public offer. Investors, however, do not benefit from protection under CISA or CISO or supervision by FINMA. This prospectus and any other materials relating to the securities are strictly personal and confidential to each offeree and do not constitute an offer to any other person. This prospectus may only be used by those qualified investors to whom it has been handed out in connection with the offer described in this prospectus and may neither directly or indirectly be distributed or made available to any person or entity other than its recipients. It may not be used in connection with any other offer and shall in particular not be copied and/or distributed to the public in Switzerland or from Switzerland. This prospectus does not constitute an issue prospectus as that term is understood pursuant to Article 652a and/or 1156 of the Swiss Federal Code of Obligations. We have not applied for a listing of the securities on the SIX Swiss Exchange or any other regulated securities market in Switzerland, and consequently, the information presented in this prospectus does not necessarily comply with the information standards set out in the listing rules of the SIX Swiss Exchange and corresponding prospectus schemes annexed to the listing rules of the SIX Swiss Exchange.

## **LEGAL MATTERS**

The validity of the issuance of our common stock offered in this prospectus will be passed upon for us by Latham & Watkins LLP, Menlo Park, California. Cooley LLP, San Francisco, California, is acting as counsel for the underwriters in connection with this offering.

## **EXPERTS**

The financial statements as of December 31, 2017 and December 31, 2018 and for the years then ended included in this Prospectus have been so included in reliance on the report of PricewaterhouseCoopers LLP, an independent registered public accounting firm, given on the authority of said firm as experts in auditing and accounting.

## WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of our common stock offered hereby. This prospectus, which constitutes a part of the registration statement, does not contain all of the information set forth in the registration statement or the exhibits and schedules filed therewith. For further information with respect to 4D Molecular Therapeutics, Inc. and the common stock offered hereby, reference is made to the registration statement and the exhibits and schedules filed therewith. Statements contained in this prospectus regarding the contents of any contract or any other document that is filed as an exhibit to the registration statement are not necessarily complete, and each such statement is qualified in all respects by reference to the full text of such contract or other document filed as an exhibit to the registration statement. The SEC maintains a website that contains reports, proxy and information statements and other information regarding registrants that file electronically with the SEC. The address is www.sec.gov.

Upon completion of this offering, we will become subject to the information and periodic reporting requirements of the Exchange Act and, in accordance therewith, will file periodic reports, proxy statements and other information with the SEC. Such periodic reports, proxy statements and other information will be available for inspection and copying at the public reference room and website of the SEC referred to above. We maintain a website at www.4dmoleculartherapeutics.com. Upon completion of this offering, you may access our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act with the SEC free of charge at our website as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC. The reference to our website address does not constitute incorporation by reference of the information contained on our website, and you should not consider the contents of our website in making an investment decision with respect to our common stock.

# 4D Molecular Therapeutics, Inc.

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#### REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

## To the Board of Directors and Stockholders of 4D Molecular Therapeutics, Inc.

## Opinion on the Financial Statements

We have audited the accompanying balance sheets of 4D Molecular Therapeutics, Inc. (the "Company") as of December 31, 2018 and 2017, and the related statements of operations and comprehensive loss, redeemable convertible preferred stock and stockholders' deficit and cash flows for the years then ended, including the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2018 and 2017, and the results of its operations and its cash flows for the years then ended in conformity with accounting principles generally accepted in the United States of America.

## **Basis for Opinion**

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) ("PCAOB") and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits of these financial statements in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ PricewaterhouseCoopers LLP

San Jose, California August 2, 2019

We have served as the Company's auditor since 2016.

# 4D Molecular Therapeutics, Inc.

# Balance Sheets (In thousands, except share and per share amounts)

		As of December 31,		Pro Forma As of June 30,
	2017	2018	June 30, 2019	2019
Accepte			(Un	audited)
Assets Current assets				
Cash and cash equivalents	\$ 23.850	\$ 91.761	\$ 72.869	
Accounts receivable	321	1,124	1,161	
Prepaid expenses and other current assets (includes \$0, \$0 and \$373 (unaudited), at December 31, 2017,	0	_,	-,	
December 31, 2018 and June 30, 2019, respectively, attributable to related parties)	328	1,183	1,415	
Total current assets	24,499	94,068	75,445	
Property and equipment, net	2,716	2,472	4,026	
Other assets	105	429	1,029	
Total assets	\$ 27,320	\$ 96,969	\$ 80,500	
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' (Deficit) Equity				<del></del>
Current liabilities				
Accounts payable	\$ 460	\$ 954	\$ 1,115	
Accrued and other current liabilities	1,066	2,193	3,242	
Deferred revenue (includes \$28, \$0 and \$0 (unaudited), at December 31, 2017, December 31, 2018 and				
June 30, 2019, respectively, attributable to related parties)	4,382	4,907	4,246	
Total current liabilities	5,908	8,054	8,603	
Deferred revenue, net of current portion (includes \$5,000, \$0 and \$0 (unaudited), at December 31, 2017, December 31,				
2018 and June 30, 2019, respectively, attributable to related parties)	22,188	13,076	10,777	
Derivative liability	78	64	70	
Other liabilities	228	382	374	
Total liabilities	28,402	21,576	19,824	
Commitments and contingencies (Note 8)				
Redeemable convertible preferred stock, \$0.0001 par value; 2,309,312 shares authorized; 2,220,999 shares issued and outstanding at December 31, 2017. 7,375,638 shares authorized; 7,375,631 shares issued and outstanding at December 31, 2018 and June 30, 2019 (unaudited). Liquidation value of \$18,596 at December 31, 2017 and \$108,596 at December 31, 2018 and June 30, 2019 (unaudited). No shares authorized, issued or outstanding, pro				
forma (unaudited).	18,508	102,980	102,980	
Stockholders' (deficit) equity				
Common stock, \$0.0001 par value; 50,000,000 shares authorized as of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited); 5,063,303, 5,126,344 and 5,141,344 shares issued and outstanding at December 31, 2017, December 31, 2018, and June 30, 2019 (unaudited), respectively; shares				
authorized and shares issued and outstanding, pro forma (unaudited)	1	1	1	
Additional paid-in-capital	884	2,438	3,930	
Accumulated deficit	(20,475)	(30,026)	(46,235)	
Total stockholders' (deficit) equity	(19,590)	(27,587)	(42,304)	
Total liabilities, redeemable convertible preferred stock and stockholders' (deficit) equity	\$ 27,320	\$ 96,969	\$ 80,500	

# 4D Molecular Therapeutics, Inc.

# Statements of Operations and Comprehensive Loss (In thousands, except share and per share amounts)

	Year Ended December 31,			Six Months Ended June 30,			ed	
		2017	2018		_	2018		2019
						(Una	udited)	
Revenue:								
Collaboration and research revenue, related parties	\$	3,176	\$	5,143	\$	143	\$	26
Collaboration and research revenue		2,614		8,987		4,926		4,390
Total revenue		5,790		14,130		5,069		4,416
Operating expenses:								
Research and development (includes \$2,881 and \$160 for the years ended December 31, 2017 and 2018 and \$135 (unaudited) and \$99 (unaudited) for the six months ended June 30, 2018 and 2019, respectively, attributable to related parties)		13.573		18,362		8,208		16.985
General and administrative (includes \$15 and \$220 for the years ended December 31, 2017 and 2018 and \$61 (unaudited) and \$0 (unaudited) for the six months ended June 30, 2018 and 2019, respectively, attributable to related		2.400		C 107		2 100		4.004
parties)		3,489		6,167	_	2,198		4,884
Total operating expenses		17,062	_	24,529	_	10,406	_	21,869
Loss from operations		(11,272)		(10,399)		(5,337)		(17,453)
Other income (expense):								
Interest income		89		850		147		945
Other income (expense), net		(40)		(2)	_	4		(8)
Total other income (expense)		49		848		151		937
Net loss and comprehensive loss	\$ (	(11,223)	\$	(9,551)	\$	(5,186)	\$	(16,516)
Net loss per share attributable to common stockholders, basic and diluted		(2.27)		(1.89)		(1.03)		(3.22)
Weighted-average shares outstanding used in computing net loss per share attributable to common stockholders, basic and diluted	4,9	53,419	5	5,049,203		5,021,003		5,132,714
Pro forma net loss per share, basic and diluted (unaudited)								
Weighted-average shares outstanding used in computing pro forma net loss per share, basic and diluted (unaudited)			_				<u> </u>	

# 4D Molecular Therapeutics, Inc.

# Statements of Redeemable Convertible Preferred Stock and Stockholders' Deficit (In thousands, except share amounts)

	Redeemable Convertible Preferred Stock		Convertible		Stock	Additional Paid-In	Accumulated	Total Stockholders'
	Shares	Amount	Shares	Amount	Capital	Deficit	Deficit	
Balances at December 31, 2016	2,220,999	\$ 18,508	5,048,932	\$ 1	\$ 612	\$ (9,252)	\$ (8,639)	
Exercise of common stock options	_	_	14,371	_	16	_	16	
Stock-based compensation	_	_	_	_	256	_	256	
Net loss						(11,223)	(11,223)	
Balances at December 31, 2017	2,220,999	18,508	5,063,303	1	884	(20,475)	(19,590)	
Issuance of redeemable convertible preferred stock, net of								
\$5,528 of issuance cost	5,154,632	84,472	_	_		_	_	
Exercise of common stock options	_	_	63,041	_	105	_	105	
Issuance of common stock warrants	_	_	_	_	72	_	72	
Stock-based compensation	_	_	_	_	1,377	_	1,377	
Net loss						(9,551)	(9,551)	
Balances at December 31, 2018	7,375,631	\$102,980	5,126,344	\$ 1	\$ 2,438	\$ (30,026)	\$ (27,587)	

# 4D Molecular Therapeutics, Inc.

# Statements of Redeemable Convertible Preferred Stock and Stockholders' Deficit – (Continued) (In thousands, except share amounts)

	Redeer Conve <u>Preferred</u> Shares	rtible	Common S	Stock Amount	Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Deficit
Balances at December 31, 2017	2,220,999	\$ 18.508	5,063,303	\$ 1	\$ 884	\$ (20,475)	\$ (19,590)
Exercise of common stock options (unaudited)	· · · —	· · ·	19,753	_	20		20
Issuance of common stock warrants (unaudited)	_	_	_	_	59	_	59
Stock-based compensation	_	_	_	_	403	_	403
Net loss (unaudited)	_	_		_	_	(5,186)	(5,186)
Balances at June 30, 2018 (unaudited)	2,220,999	\$ 18,508	5,083,056	\$ 1	\$ 1,366	\$ (25,661)	\$ (24,294)
Balances at December 31, 2018	7,375,631	\$102,980	5,126,344	\$ 1	\$ 2,438	\$ (30,026)	\$ (27,587)
Cumulative effect of adoption of ASC 606 (unaudited)	_	_	_	_	_	307	307
Exercise of common stock options (unaudited)	_	_	15,000	_	20	_	20
Stock-based compensation (unaudited)	_	_	_	_	1,472	_	1,472
Net loss (unaudited)						(16,516)	(16,516)
Balances at June 30, 2019 (unaudited)	7,375,631	\$102,980	5,141,344	\$ 1	\$ 3,930	\$ (46,235)	\$ (42,304)

# 4D Molecular Therapeutics, Inc.

# Statements of Cash Flows (In thousands)

		ber 31,	Six Months Ended June 30,		
	2017	2018	2018	2019	
Oarly flavor frame accounting a sticities			(Unai	udited)	
Cash flows from operating activities  Net loss	<b>ተ/11 ጋጋጋ</b> \	¢ (0 EE1)	¢ (E 10€)	Φ (1 C E1 C)	
110111000	\$(11,223)	\$ (9,551)	\$ (5,186)	\$(16,516)	
Adjustments to reconcile net loss to net cash provided by (used in) operating activities  Stock-based compensation expense	256	1,377	403	1,472	
Change in fair value of warrant obligation	31	1,377	403	1,472	
Change in fair value of derivative liability	31	(14)	(20)	6	
Depreciation and amortization	626	697	341	406	
Loss on disposition of property and equipment	8	16	16	400	
Changes in operating assets and liabilities	O	10	10	_	
Accounts receivable	(320)	(803)	(1,415)	(37)	
Prepaid expenses and other current assets	(320)	(855)	(1,413)	(232)	
Other assets	0	(324)	(40)	(300)	
Accounts payable	(114)	438	559	62	
Accrued and other liabilities	(59)	1,354	261	624	
Deferred revenue	19,026	(8,587)	(1,401)	(2,653)	
Net cash provided by (used in) operating activities	8,237	(16,252)	(6,422)	(17,168)	
, , , , , ,	0,237	(10,232)	(0,422)	(17,100)	
Cash flows from investing activities	24				
Proceeds on sale of property and equipment	24 (572)	(41.4)	(22)	(1,000)	
Acquisition of property and equipment	(573)	(414)	(33)	(1,662)	
Proceeds from maturities of short-term investments	8,224				
Net cash provided by (used in) investing activities	7,675	(414)	(33)	(1,662)	
Cash flows from financing activities					
Issuance of redeemable convertible preferred stock, net of issuance costs	_	84,472	_		
Issuance of common stock	16	105	20	20	
Deferred offering costs				(82)	
Net cash provided by (used in) financing activities	16	84,577	20	(62)	
Net increase (decrease) in cash and cash equivalents	15,928	67,911	(6,435)	(18,892)	
Cash and cash equivalents, beginning of period	7,922	23,850	23,850	91,761	
Cash and cash equivalents, end of period	\$ 23,850	\$ 91,761	\$17,415	\$ 72,869	
Supplemental disclosures of non-cash investing and financing information					
Purchases of property and equipment in accounts payable and accrued and other liabilities	\$ —	\$ 56	\$ —	\$ 298	
Unpaid deferred offering costs	\$ —	\$ —	\$ —	\$ 308	
Issuance of common stock warrants in return for services	\$ —	\$ 72	\$ 59	\$ —	

# 4D Molecular Therapeutics, Inc. Notes to Financial Statements

## 1. Organization and Nature of the Business

## **Organization and Business**

4D Molecular Therapeutics, Inc. (the "Company") was formed as a limited liability company in September 2013 under the name 4D Molecular Therapeutics, LLC. The Company changed its name and converted into a corporation which was incorporated in the state of Delaware in March 2015. The Company is a development stage precision gene therapy company dedicated to the development of targeted therapies based on its adeno-associated viruses ("AAV") vectors discovered through its proprietary Therapeutic Vector Evolution platform.

## Liquidity

The Company experienced negative operating cash flows of \$16.3 million for the year ended December 31, 2018 and \$17.2 million (unaudited) for the six months ended June 30, 2019. The Company had an accumulated deficit of \$30.0 million as of December 31, 2018 and \$46.2 million (unaudited) as of June 30, 2019. Since its inception, the Company has funded its operations primarily with proceeds from sales of redeemable convertible preferred stock and to a lesser extent collaboration and licensing arrangements. In August 2018, the Company received \$84.5 million net proceeds from issuance of Series B redeemable convertible preferred stock.

The Company expects to increase operating losses and negative operating cash flows for the foreseeable future as the Company advances its product candidates into clinical trials, conducts additional preclinical studies, and expands internal manufacturing capabilities. The Company is seeking to complete an initial public offering ("IPO") of its common stock. In the event the Company does not complete an IPO, the Company expects to seek additional funding through private equity financings, debt financings, or other capital sources, including collaborations with other companies, or other strategic transactions.

The Company may not be able to obtain funding on acceptable terms, or at all. If the expected inflows are delayed or not received, or available capital resources are consumed more rapidly than currently expected, the Company expects to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion or investment in internal manufacturing capabilities, which could adversely affect its business prospects. The Company expects that its existing cash and cash equivalents will be sufficient to fund its operations through at least 12 months from the date these financial statements are available for issuance.

## 2. Summary of Significant Accounting Policies

## **Basis of Presentation**

The accompanying financial statements have been prepared in accordance with United States Generally Accepted Accounting Principles ("U.S. GAAP").

## **Unaudited Interim Financial Information**

The accompanying balance sheet as of June 30, 2019, the statements of operations and comprehensive loss, of redeemable convertible preferred stock and stockholders' deficit and of cash flows for the six months ended June 30, 2018 and June 30, 2019 are unaudited. In the opinion of management, the unaudited data reflects all adjustments, which include only normal recurring

adjustments, necessary for the fair statement of the Company's financial position as of June 30, 2019 and the results of its operations and comprehensive loss and its cash flows for the six months ended June 30, 2018 and June 30, 2019. The financial data and other information disclosed in these notes related to the six months ended June 30, 2018 and June 30, 2019 are also unaudited. The results for the six months ended June 30, 2019 are not necessarily indicative of results to be expected for the year ending December 31, 2019, any other interim periods, or any future year or period.

## **Unaudited Pro Forma Information**

The unaudited pro forma basic and diluted net loss per share were computed to give effect to the conversion of all outstanding shares of redeemable convertible preferred stock on a one-for-one basis into shares of common stock immediately prior to the completion of the planned IPO as though the conversion had occurred as of the beginning of the period or the date of issuance, if later.

The unaudited pro forma redeemable convertible preferred stock and stockholders' deficit were computed to give effect to the conversion of all outstanding shares of redeemable convertible preferred stock on a one-for-one basis into shares of common stock immediately prior to the completion of the planned IPO. The unaudited pro forma information does not assume any proceeds from the planned IPO.

## **Use of Estimates**

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenue and expenses; and disclosure of contingent assets and liabilities as of the date of the financial statements. Such estimates include the determination of useful lives for property and equipment, the research periods of collaboration agreements, as well as estimates of the fair value of common stock, stock options and derivative instruments and income tax uncertainties. Actual results could differ from those estimates.

## **Segments**

The Company operates and manages its business as one reportable and operating segment. The Company's chief executive officer, who is the chief operating decision maker, reviews financial information on a consolidated basis for purposes of allocating resources and assessing financial performance.

## Concentration of Credit Risk

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash and cash equivalents and accounts receivable. The Company's cash is held at two financial institutions in the United States of America. The Company's cash equivalents are invested in money market funds. The Company has not experienced any losses on its deposits of cash and cash equivalents. Such deposits may, at times, exceed federally insured limits.

The Company's partners in collaboration and license agreements who represent 10% or more of the Company's total revenue are as follows:

	Year Ended December 31, 2017	Year Ended December 31, 2018	Six Months Ended June 30, 2018 (Unaudited)	Six Months Ended June 30, 2019 (Unaudited)
Partner A	55%	*	*	*
Partner B	19%	*	*	*
Partner C	14%	53%	79%	86%
Partner D	*	35%	*	*
Partner E	*	*	10%	12%
Total	<u>88</u> %	88%	89%	98%

<sup>\*</sup> Less than 10%

The Company's accounts receivable as of December 31, 2017 and 2018 and June 30, 2019 (unaudited) was comprised of one partner.

The Company's total revenues by geographic region, based on the location of the customer, are as follows (in thousands):

	Dece	Year Ended December 31, 2017  Year Ended December 31, 2018		mber 31,	Ju	Months inded ine 30, 2018 audited)	Ju 2	Months inded ine 30, 2019 audited)
Australia	\$	408	\$	200	\$	100	\$	7
Netherlands		3,176		143		143		26
Switzerland		839		7,460		4,027		3,816
United States		1,367		6,327		799		567
	\$	5,790	\$	14,130	\$	5,069	\$	4,416

## Cash and Cash Equivalents

The Company considers all highly liquid investments with original maturities of three months or less at the date of purchase to be cash equivalents. Cash equivalents consist of money market funds.

## Other Risks and Uncertainties

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, protection of proprietary technology, dependence on key personnel, suppliers for key raw materials, contract manufacturing organizations ("CMOs") and contract research organizations ("CROs"), compliance with government regulations and the need to obtain additional financing to fund operations. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical studies and clinical trials and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance and reporting.

There can be no assurance that the Company's research and development will be successfully completed, that adequate protection for the Company's intellectual property will be obtained or maintained, that any products developed will obtain necessary government regulatory approval or that

any approved products will be commercially viable. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from other pharmaceutical and biotechnology companies. In addition, the Company is dependent upon the services of its employees, consultants and other third parties (including for clinical trials and some aspects of research and preclinical testing).

## **Fair Value Measurements**

The Company applies fair value accounting for all financial assets and liabilities and non-financial assets and liabilities that are recognized or disclosed at fair value in the financial statements on a recurring basis. Fair value is an exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability.

As a basis for considering such assumptions, a three-level fair value hierarchy has been established, which prioritizes the inputs used in measuring fair value as follows:

- · Level 1—Observable inputs, such as quoted prices in active markets for identical assets and liabilities.
- Level 2—Observable inputs other than Level 1 that are observable, either directly or indirectly, such as quoted prices for similar
  assets or liabilities, quoted prices in markets that are not active, or other inputs that are observable or can be corroborated by
  observable market data for substantially the full term of the assets or liabilities.
- Level 3— Unobservable inputs that are significant to the measurement of the fair value of the assets or liabilities that are supported by little or no market data.

The Company accounts for transfers of financial instruments between levels of the fair value hierarchy on the date of the event or change in circumstance that caused the transfer.

#### Accounts Receivable—Allowance for Doubtful Accounts

The Company regularly reviews accounts receivable for collectability and establishes an allowance for probable credit losses and writes off uncollectible accounts as necessary. The Company has determined that no allowance was required at December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited). The Company did not have any write-offs relating to uncollectible accounts receivable during the years ended December 31, 2017 and 2018 and six months ended June 30, 2018 and 2019 (unaudited).

## Property and Equipment, net

Property and equipment are stated at cost less accumulated depreciation for acquired assets. Depreciation is computed using the straight-line method over the estimated useful lives of assets, ranging from three to five years. Leasehold improvements are amortized over the shorter of the useful life of the assets or the length of the lease. Upon sale or retirement of assets, the costs and related accumulated depreciation are removed from the balance sheet and the resulting gain or loss is reflected within operating expenses in the statement of operations and comprehensive loss. Maintenance and repairs are charged to expense as incurred.

## Impairment of Long-Lived Assets

The Company reviews long-lived assets for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Recoverability is

measured by comparison of the carrying amount to the future undiscounted net cash flows, which the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is typically measured by the amount by which the carrying amount of the assets exceeds the projected discounted future net cash flows arising from the asset. There have been no such impairments of long-lived assets in the years ended December 31, 2017 and 2018 and six months ended June 30, 2018 and 2019 (unaudited).

## Redeemable Convertible Preferred Stock

The Company records all shares of redeemable convertible preferred stock at their respective fair values on the dates of issuance, net of issuance costs. The redeemable convertible preferred stock is recorded outside of permanent equity because while it is not mandatorily redeemable, in certain events considered not solely within the Company's control, such as a merger or consolidation, sale, lease, or license of substantially all of the Company's assets (each, a "deemed liquidation event"), the convertible preferred stock will become redeemable at the option of the majority of outstanding shareholders of such series of redeemable convertible preferred stock. The Company has not adjusted the carrying values of the redeemable convertible preferred stock to the liquidation preference of such shares because a deemed liquidation event obligating the Company to pay the liquidation is not considered probable. Subsequent adjustments to the carrying values to the liquidation preference will be made only when it becomes probable that such a deemed liquidation event will occur.

## **Common Stock Warrants**

The Company accounts for common stock warrants which meet the definition of a derivative as liabilities if the warrant requires net cash settlement or gives the holder the option of net cash settlement. The Company accounts for common stock warrants as equity if the contract requires physical settlement or net physical settlement or if the Company has the option of physical settlement or net physical settlement. Common stock warrants classified as liabilities are initially recorded at fair value and remeasured at fair value each balance sheet date with the offset adjustments recorded in other income (expense), net within the statements of operations and comprehensive loss. Common stock warrants classified as equity are initially measured at fair value on the grant date and are not subsequently remeasured.

## **Revenue Recognition**

Effective January 1, 2019, the Company adopted ASU 2014-09, *Revenue from Contracts with Customers (Topic 606)* ("ASC 606"), using the modified retrospective transition method. The Company determines revenue recognition for arrangements within the scope of ASC 606 by performing the following five steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations based on estimated selling prices; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation.

The Company's revenues are primarily derived through its license, research, development and commercialization agreements. The terms of these types of agreements may include (i) licenses to the Company's technology, (ii) research and development services, and (iii) services or obligations in connection with participation in research or steering committees. Payments to the Company under these arrangements typically include one or more of the following: nonrefundable upfront and license fees, research funding, milestone and other contingent payments to the Company for the achievement of defined collaboration objectives and certain preclinical, clinical, regulatory and sales-based events,

as well as royalties on sales of any commercialized products. Arrangements that include upfront payments are recorded as deferred revenue upon receipt or when due and may require deferral of revenue recognition to a future period until performance obligations are met. The event-based milestone payments, royalties and cost reimbursements represent variable consideration, and the Company uses the most likely amount method to estimate this variable consideration. Royalty payments are recognized when earned or as the sales occur. The Company records cost reimbursements as accounts receivable when right to consideration is unconditional.

A performance obligation is a promise in a contract to transfer a distinct good or service to the customer and is the unit of account in ASC 606. The Company allocates the total transaction price to each performance obligation based on the estimated relative to standalone selling price and recognizes revenue when, or as, the performance obligation is satisfied. The Company includes the unconstrained amount of estimated variable consideration in the transaction price. At the end of each reporting period, the Company reevaluates the estimated variable consideration included in the transaction price and any related constraint, and if necessary, adjusts its estimate of the overall transaction price.

Prior to the adoption of ASC 606 on January 1, 2019, the Company recognized revenue when all of the following criteria were met: persuasive evidence of an arrangement exists; transfer of technology has been completed or services have been rendered; the price to the customer is fixed or determinable and collectability is reasonably assured.

In arrangements involving the delivery of more than one element, each required deliverable was evaluated to determine whether it qualified as a separate unit of accounting. The determination was based on whether the deliverable had "standalone value" to the customer. If a deliverable did not qualify as a separate unit of accounting, it was combined with the other applicable undelivered item(s) within the arrangement and these combined deliverables were treated as a single unit of accounting.

The arrangement's consideration that was fixed or determinable was allocated to each separate unit of accounting based on the relative selling price methodology in accordance with the selling price hierarchy, which included vendor-specific objective evidence (VSOE) of selling price, if available, or third-party evidence of selling price if VSOE was not available, or the best estimate of selling price, if neither VSOE nor third-party evidence was available.

Payments or reimbursements for our research and development efforts for the arrangements where such efforts were considered as deliverables were recognized as the services were performed and were presented on a gross basis. When upfront payments were received and if there was no discernible pattern of performance, the Company recognized revenue ratably over the associated period of performance.

## **Research and Development Expenses**

Costs related to research, design and development of programs are charged to research and development expense as incurred. Research and development costs include, but are not limited to, payroll and personnel expenses including stock-based compensation, materials, laboratory supplies, outside services and allocated overhead, including rent, insurance, repairs and maintenance, depreciation and utilities. The Company expenses all research and development costs in the period in which they are incurred.

Costs incurred in obtaining technology licenses are charged to research and development expense as acquired in-process research and development if the technology licensed has not reached technological feasibility and has no alternative future use.

## **Accrued Research and Development**

The Company has entered into various agreements with CROs and CMOs. The Company's research and development accruals are estimated based on the level of services performed, progress of the studies, including the phase or completion of events, and contracted costs. The estimated costs of research and development provided, but not yet invoiced, are included in accrued and other current liabilities on the balance sheet. If the actual timing of the performance of services or the level of effort varies from the original estimates, the Company will adjust the accrual accordingly. Payments made to CROs or CMOs under these arrangements in advance of the performance of the related services are recorded as prepaid expenses and other current assets until the services are rendered.

## **Stock-Based Compensation**

The Company accounts for stock-based compensation as measured at grant date, based on the fair value of the award. The Company measures the fair value of awards granted using the Black-Scholes option pricing model and recognizes the expense in the Company's Statements of Operations and Comprehensive Loss over the requisite service period using the straight-line method. Forfeitures are accounted for as they occur. The Company's policy for issuing stock upon stock option exercise is to issue new common stock.

The Company recognizes the fair value of stock options granted to nonemployees as stock-based compensation expense over the period in which the related services are received. Stock-based compensation expense related to stock options granted to nonemployees is recognized based on the vesting date fair value of awards as the stock options are earned. The Company believes that the estimated fair value of stock options is more readily measurable than the fair value of the services rendered. In addition, the Company estimates the service period for the awards based on the time that would be required to satisfy the service condition, assuming the service condition will be satisfied. Stock-based compensation expense is recognized over the estimated service period but is accelerated if the performance condition is achieved earlier than estimated.

## **Income Taxes**

The Company accounts for income taxes under the asset and liability method, which requires, among other things, that deferred income taxes be provided for temporary differences between the tax basis of the Company's assets and liabilities and their financial statement reported amounts. In addition, deferred tax assets are recorded for the future benefit of utilizing net operating losses and research and development credit carryforwards and are measured using the enacted tax rates and laws that will be in effect when such items are expected to reverse. A valuation allowance is provided against deferred tax assets unless it is more likely than not that they will be realized.

The Company accounts for uncertain tax positions by assessing all material positions taken in any assessment or challenge by relevant taxing authorities. Assessing an uncertain tax position begins with the initial determination of the position's sustainability and is measured at the largest amount of benefit that is greater than fifty percent likely of being realized upon ultimate settlement. The Company's policy is to recognize interest and penalties related to the underpayment of income taxes as a component of income tax expense or benefit. To date, there have been no interest or penalties charged in relation to the unrecognized tax benefits.

## **Embedded Derivative**

Embedded derivatives that are required to be bifurcated from the underlying host instrument are accounted for and valued as a separate financial instrument. An embedded derivative exists in the

award agreement with the Cystic Fibrosis Foundation Therapeutics, Inc. ("CFFT"). As described in Note 15, the embedded derivative has been bifurcated and is classified as a liability on the balance sheet and separately accounted for at its fair value. The derivative liability is subject to remeasurement to fair value each reporting period. Changes in the fair value of the derivative liability are recognized as a component of other income (expense), net within the statements of operations and comprehensive loss.

## **Net Loss Per Share Attributable to Common Stockholders**

The Company calculates basic and diluted net loss per share to common stockholders in conformity with the two-class method required for companies with participating securities. The Company considers all series of redeemable convertible preferred stock and early exercised stock options to be participating securities as the holders are entitled to receive non-cumulative dividends on a pari passu basis in the event the dividend is paid on common shares. Under the two-class method, the net loss attributable to common stockholders is not allocated to the redeemable convertible preferred stock as the holders of redeemable convertible preferred stock do not have a contractual obligation to share in losses.

Under the two-class method, basic net loss per share attributable to common stockholders is calculated by dividing the net loss by the weighted-average number of common shares outstanding during the period, less shares subject to repurchase. Diluted net loss per share attributable to common stockholders is computed by giving effect to all potentially dilutive common shares outstanding for the period. For purposes of this calculation, redeemable convertible preferred shares, stock options to acquire common shares and contingently issuable shares, and are considered potentially dilutive common shares, but have been excluded from the calculation of diluted net loss per share attributable to common stockholders as their effect is antidilutive.

## **Deferred Offering Costs**

The Company capitalizes certain legal, accounting and other third-party fees that are directly related to the Company's in-process equity financings, including the planned IPO, until such financings are consummated. After consummation of the equity financing, these costs are recorded as a reduction of the proceeds received as a result of the offering. Should the planned equity financing be abandoned, the deferred offering costs will be expensed immediately within the Company's statements of operations and comprehensive loss. As of June 30, 2019, \$0.4 million (unaudited) of costs were recorded as deferred offering costs on the balance sheet. There were no material deferred offering costs capitalized as of December 31, 2017 and 2018.

## **Recent Accounting Pronouncements**

Recently Adopted Accounting Pronouncements

In May 2014, the FASB issued ASC 606. This standard outlines a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. The main principle of ASC 606 is to recognize revenue when promised goods or services are transferred to customers in an amount that reflects the consideration that is expected to be received for those goods or services. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services.

The Company adopted ASC 606 effective January 1, 2019 using the modified retrospective method only to contracts not completed as of this date. The Company recognized the cumulative effect

of initially applying ASC 606 as an adjustment to the balance of accumulated deficit at January 1, 2019. The new guidance has been applied to the most current period presented with a cumulative effect adjustment of \$0.3 million (unaudited) reflected as a decrease to the opening balance of accumulated deficit and a decrease to deferred revenue. See Note 6 for further discussion on research and collaboration arrangements.

The following tables summarize the amount by which each financial statement line item was affected by the impact of the cumulative adjustment and as compared with the guidance that was in effect prior to the adoption (in thousands):

# Impact of ASC 606 Adoption on Balance Sheet as of January 1, 2019 (Unaudited)

	As reported under ASC 606	Adjustments	Balances without adoption of ASC 606
Deferred revenue, current portion	\$ (6,202)	\$ 1,295	\$ (4,907)
Deferred revenue, noncurrent portion	\$ (11,474)	\$ (1,602)	\$(13,076)
Accumulated deficit	\$ (29,719)	\$ (307)	\$(30,026)

# Impact of ASC 606 Adoption on Balance Sheet as of June 30, 2019 (Unaudited)

	As reported under ASC			Balances without adoption of ASC
	606	Adju	stments	606
Deferred revenue, current portion	\$ (4,246)	\$	306	\$ (3,940)
Deferred revenue, noncurrent portion	\$ (10,777)	\$	(382)	\$(11,159)
Accumulated deficit	\$ (46,235)	\$	(76)	\$(46,311)

## Impact of ASC 606 Adoption on Statements of Operations and Comprehensive Loss Six Month Ended June 30, 2019 (Unaudited)

	As reported under ASC 606	Adjus	stments	witho	alances out adoption ASC 606
Collaboration and research revenue	\$ 4,416	\$	231	\$	4,647
Net loss	\$ (16,516)	\$	231	\$	(16,285)
Net loss per share—basic and diluted	\$ (3.22)	\$	0.05	\$	(3.17)

# Impact of ASC 606 Adoption on Statements of Cash Flows for the Six Month Ended June 30, 2019 (Unaudited)

	As reported under ASC 606	Adju	stments	witho	Salances out adoption ASC 606
Cash flows from operating activities:					
Net loss	\$ (16,516)	\$	231	\$	(16,285)
Changes in operating assets and liabilities:					
Deferred revenue	\$ (2,653)	\$	(231)	\$	(2,884)

In May 2017, the FASB issued Accounting Standards Update ("ASU") 2017-09, Compensation-Stock Compensation (Topic 718): Scope of Modification Accounting. This ASU provides guidance about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting. This ASU is effective for fiscal years beginning after December 15, 2017, and interim periods within those years. The Company adopted this standard on January 1, 2018 with no impact to the financial statements and related disclosures.

In November 2015, the FASB issued ASU 2015-17, *Balance Sheet Classification of Deferred Taxes*. This ASU amends existing guidance on income taxes to require the classification of all deferred tax assets and liabilities as non-current on the balance sheets. This ASU is effective for fiscal years beginning after December 15, 2016. Early adoption is permitted and the guidance may be applied either prospectively or retrospectively. The Company adopted this standard on January 1, 2017 with no impact to the financial statements and related disclosures.

## New Accounting Pronouncements Not Yet Adopted

In November 2018, the FASB issued ASU 2018-18, *Collaborative Arrangements (Topic 808): Clarifying the Interaction Between Topic 808 and Topic 606.* This ASU clarifies that certain transactions between participants in a collaborative arrangement should be accounted for under ASC 606, *Revenue from Contracts with Customers*, when the counterparty is a customer. This ASU also precludes an entity from presenting consideration received from a transaction as revenue from contracts with customers if the counterparty is not a customer for that transaction. This ASU is effective for fiscal years beginning after December 15, 2020, and interim periods within fiscal years beginning after December 15, 2021. Early adoption is permitted for entities that have adopted ASC 606, *Revenue from Contracts with Customers*. The Company is currently evaluating the impact the adoption of this standard will have on its financial statements and related disclosures.

In August 2018, FASB issued ASU 2018-13, *Fair Value Measurement (Topic 820), Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement.* This ASU amends the disclosure requirement in ASC 820, Fair Value Measurement, by adding, changing, or removing certain disclosures. This ASU applies to all entities that are required under this guidance to provide disclosure about recurring or nonrecurring fair value measurements. The amendments require new disclosures related to: changes in unrealized gains and losses for the period included in other comprehensive income for recurring Level 3 fair value measurements held at the end of the reporting period; range and weighted-average of significant unobservable inputs used to develop Level 3 fair value measurements. In addition, there are certain changes in disclosure requirements in the existing guidance. For all entities, this ASU is effective for fiscal years beginning after December 15, 2019, and interim periods within those fiscal years. Early adoption is permitted. The Company is currently evaluating the impact the adoption of this standard will have on its financial statements and related disclosures.

In June 2018, FASB issued ASU 2018-07, Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting. This ASU aligns the measurement and classification guidance for share-based payments to nonemployees with the guidance for share-based payment to employees. Under this ASU, the measurement of equity-classified nonemployee awards will be fixed at the grant date, which may lower their cost and reduce volatility in the statement of operations and comprehensive loss. The transition method provided by this ASU is on a modified retrospective basis, which recognizes a cumulative-effect adjustment to the opening balance of retained earnings in the period of adoption. This ASU is effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. Early adoption is permitted, but may take place no earlier than a company's adoption date of ASC 606, Revenue from Contracts with Customers. The Company is currently evaluating the impact the adoption of this standard will have on its financial statements and related disclosures.

In July 2017, FASB issued ASU 2017-11, Earnings Per Share (Topic 260) Distinguishing Liabilities from Equity (Topic 480) Derivatives and Hedging (Topic 815) (Part I) Accounting for Certain Financial Instruments with Down Round Features, (Part II) Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception. This ASU simplifies the accounting for certain financial instruments with down round features, a provision in an equity-linked financial instrument (or embedded feature) that provides a downward adjustment of the current exercise price based on the price of future equity offerings. Down round features are common in warrants, preferred shares, and convertible debt instruments issued by private companies and early-stage public companies. This ASU requires companies to disregard the down round feature when assessing whether the instrument is indexed to its own stock, for purposes of determining liability or equity classification. The amendments in Part I of this ASU are effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. Early adoption is permitted. The amendments in Part I should be applied (1) retrospectively to outstanding financial instruments with a down round feature by means of a cumulative-effect adjustment to the balance sheet as of the beginning of the first fiscal year and interim periods; (2) retrospectively to outstanding financial instruments with a down round feature for each prior reporting period presented. The amendments in Part II of this ASU do not require any transition guidance because those amendments do not have an accounting effect. The Company is currently evaluating the impact the adoption of this standard will have on its financial statements and related disclosures.

In August 2016, the FASB issued ASU 2016-15, Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments. This ASU requires changes to how cash receipts and cash payments are presented and classified in the statement of cash flows. This ASU is effective for fiscal years beginning after December 15, 2018, and interim periods within fiscal years beginning after December 15, 2019. Early adoption is permitted. This ASU requires adoption on a retrospective basis. The Company is currently evaluating the impact the adoption of this standard will have on its financial statements and related disclosures.

In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842) ("ASC 842")*, which sets out the principles for the recognition, measurement, presentation and disclosure of leases for both parties to a contract (i.e. lessees and lessors). In July 2018, the FASB issued ASU 2018-10, *Codification Improvements to Topic 842*, *Leases*, which provides clarification to ASU 2016-02. These ASUs (collectively the "new leasing standard") requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease, respectively. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. Leases with a term of 12 months or less will be accounted for similar to existing guidance for operating leases today. ASC 842 supersedes the previous leases standard, ASC 840 Leases. The new leasing standard is effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. Early adoption is permitted. In July 2018, the FASB issued ASU 2018-11, *Leases (Topic 842): Targeted Improvements*, which allows entities to elect an optional transition method where entities may continue to apply the existing lease guidance during the comparative periods and apply the new lease requirements through a cumulative effect adjustment in the period of adoption rather than in the earliest period presented. In March 2019, the FASB issued ASU 2019-01, which provides clarification on implementation issues associated with adopting ASU 2016-02. The Company is currently evaluating the impact the adoption of this standard will have on its financial statements and related disclosures.

In January 2016, the FASB issued ASU 2016-01, *Financial Instruments—Overall: Recognition and Measurement of Financial Assets and Financial Liabilities*. This ASU enhances the reporting model for financial instruments, which includes amendments to address aspects of recognition, measurement, presentation and disclosure. This ASU is effective for fiscal years beginning after December 15, 2018, and interim periods within fiscal years beginning after December 15, 2019. In February 2018, the FASB issued ASU 2018-03, *Technical Corrections and Improvements to Financial Instruments—Overall (Subtopic 825-10)*. This ASU clarified certain aspects of the previously issued standard. This ASU is effective on the same effective date as ASU 2016-01. The Company is currently evaluating the impact the adoption of this standard will have on its financial statements and related disclosures.

## 3. Fair Value Measurements

As of December 31, 2017, the Company's fair value hierarchy for financial assets and financial liabilities measured at fair value are as follows (in thousands):

		Basis for Fair Value Measurements				
	(Level 1)	(Level 1) (Level 2) (Level 3)		December 31, 2017		
Assets	<u></u>	<del></del>				
Money market funds	\$23,150	<u> </u>	\$ <u>—</u>	\$ 23,150		
Total	\$23,150	\$ —	\$ —	\$ 23,150		
Liabilities			·			
Common stock warrant obligation	\$ —	\$ —	\$ 60	\$ 60		
Derivative liability	_	_	78	78		
Total	<u> </u>	\$ <u></u>	\$ 138	\$ 138		

As of December 31, 2018, the Company's fair value hierarchy for financial assets and financial liabilities measured at fair value are as follows (in thousands):

		Basis for Fair Value Measurements				
	(Level 1)	(Level 2)	(Level 3)	December 31, 2018		
Assets						
Money market funds	\$91,761	<u> </u>	<u> </u>	\$ 91,761		
Total	\$91,761	\$ —	\$ —	\$ 91,761		
Liabilities		<del></del>				
Derivative liability	\$ —	\$ —	\$ 64	\$ 64		
Total	\$ —	\$ —	\$ 64	\$ 64		

As of June 30, 2019 (unaudited), the Company's fair value hierarchy for financial assets and financial liabilities measured at fair value are as follows (in thousands):

		Basis for Fair Value Measurements					
	(Level 1)	(Level 2)	(Level 3)	) June 30, 2019			
Assets							
Money market funds	\$72,869	\$ —	\$ —	\$ 72,869			
Total	\$72,869	\$ —	\$ —	\$ 72,869			
Liabilities	<del></del>		·				
Derivative liability	\$ —	\$ —	\$ 70	\$ 70			
Total	<del>\$</del> —	\$ —	\$ 70	\$ 70			

## **Level 3 Inputs**

The fair value of the warrant obligation is based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The fair value of the common stock warrant obligation was determined using the Black-Scholes option pricing model. In determining the fair value of the warrant obligation, the inputs impacting fair value include the expected term, expected volatility, risk-free interest rate and dividend yield. See Note 13 for further discussion on common stock warrant obligation.

The fair value of the derivative liability is based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The fair value of the derivative liability was determined using a present value analysis with multiple scenarios. In determining the fair value of the derivative liability, the inputs impacting fair value include the change of control payment to CFFT, the probability of a change of control event, the product status at time of a change of control event and the discount rate. See Note 15 for further discussion on embedded derivative.

There were no transfers between Level 1, 2 and 3 during the periods presented.

The following table sets forth a summary of the changes in the fair value of the Company's Level 3 financial instruments (in thousands):

	Common Stock Warrant Obligation		Derivative Liability	
Balance as of December 31, 2016	\$ 29	\$		
Issuance of derivative liability	_		78	
Change in fair value included in other income (expense), net	31		_	
Balance as of December 31, 2017	\$ 60	\$	78	
Issuance of common stock warrants	(59)		_	
Change in fair value included in other income (expense), net	 (1)		(14)	
Balance as of December 31, 2018	\$ _	\$	64	
Change in fair value included in other income (expense), net (unaudited)	 _		6	
Balance as of June 30, 2019 (unaudited)	\$ _	\$	70	

## 4. Property and Equipment, Net

Property and equipment, net, consisted of the following (in thousands):

	December 31, 2017		December 31, 2018		_	une 30, 2019 naudited)
Machinery and equipment	\$	2,058	\$	2,433	\$	3,035
Leasehold improvements		1,349		1,349		1,362
Furniture and fixtures		182		182		188
Office equipment		63		74		74
Computer equipment and software		28		52		190
Construction in progress		_		34		1,235
Total Property and Equipment		3,680		4,124		6,084
Less: Accumulated depreciation and amortization		(964)		(1,652)		(2,058)
Property and equipment, net	\$	2,716	\$	2,472	\$	4,026

All property and equipment are maintained in the United States. Depreciation expense was \$0.6 million and \$0.7 million for the years ended December 31, 2017 and 2018 and \$0.3 million (unaudited) and \$0.4 million (unaudited) for the six months ended June 30, 2018 and 2019, respectively.

## 5. Accrued and Other Current Liabilities

Accrued and other current liabilities consisted of the following (in thousands):

	De	December 31, 2017		•		June 30, 2019 (Unaudited)	
Payroll and related	\$	787	\$	1,313	\$ 1,069		
Accrued clinical and preclinical study costs		98		75	485		
Consulting and professional		79		549	1,284		
Accrued state taxes		51		93	181		
Other accrued expenses		51		163	223		
	\$	1,066	\$	2,193	\$ 3,242		

## 6. Research and Collaboration Arrangements

Collaboration and license revenue for each period was as follows (in thousands):

	nths Ended ine 30,	
2018	2010	
	2019	
(Unaudited)	(Unaudited)	
\$ 143	\$ 26	
100	7	
272	_	
_	_	
_	_	
4,027	3,816	
27	19	
500	548	
\$ 5,069	\$ 4,416	
	(Unaudited) \$ 143 100 272 — 4,027 27 500	

Deferred revenue for each period was as follows (in thousands):

	Deferred Revenue						
	December 31, 2017		December 31, 2018				ne 30, 019 udited)
uniQure	\$ 28	\$	_	\$	_		
Benitec	383		183		_		
AGTC	337		_		_		
CRF	_		_		_		
Pfizer	5,000		_		_		
Roche	20,522		17,055	1	L4,587		
CFFT	300		245		436		
AstraZeneca	_		500		_		
	\$ 26,570	\$	17,983	\$ 1	L5,023		

The total amount of revenue in the six months ended June 30, 2019, which was included in deferred revenue at January 1, 2019, was \$4.2 million (unaudited).

## uniQure

In January 2014, the Company and uniQure biopharma B.V. ("uniQure") entered into a Collaboration and License Agreement to collaborate on the discovery and non-clinical research activities related to the Company's Therapeutic Vector Evolution platform in order to generate and validate vectors for gene delivery to treat diseases within the central nervous system and liver (the "uniQure Agreement").

The uniQure Agreement provides uniQure with a research license as well as an exclusive development and commercialization license for each project variant selected for further development. The initial research term is three years with an option for uniQure to extend the research term one time for an additional year. Once the Company's research plan has concluded, uniQure is solely responsible for the continued development, manufacturing and commercialization of the project variants as potential product candidates. In October 2016, uniQure exercised its option to extend the research term for an additional year to January 2018.

Pursuant to the uniQure Agreement, the Company received upfront payments of \$0.2 million, and is entitled to receive (i) contingent payments for the achievement of research and development milestones of up to \$5.0 million for each licensed product selected under the arrangement, and (ii) royalties in the single digit range on future sales of the potential product candidates and sublicense consideration in the low teens to low thirties range on any future sublicensing arrangements. The Company will also receive capped research and development service fees based on contractual full-time employee rates per year. In connection with the performance obligations under the uniQure Agreement, the founders of 4D Molecular Therapeutics, LLC received equity options to purchase an aggregate of 609,744 of uniQure ordinary shares that vest over the initial three-year term of the agreement.

The upfront payment of \$0.2 million was recorded as deferred revenue and was recognized on a ratable basis over the estimated performance period of four years. Payments and reimbursements for research costs are recognized on an as-incurred basis. The options to purchase uniQure shares were deemed to be a noncash component of the arrangement consideration, as the vesting of options is linked to the uniQure Agreement and there is a requirement for the holders of the options to provide services under the agreement. The fair value of the uniQure options, which was estimated to be

\$10.6 million, was recognized ratably as revenue over the estimated performance period of four years and the associated compensation expense related to the stock options were recorded as research and development expense.

During the years ended December 31, 2017 and 2018, the Company recognized revenue of \$3.2 million and \$0.1 million under the uniQure Agreement, respectively. The revenue recognized in 2017 consisted of \$2.7 million from the ratable recognition of the value of the options, \$0.4 million for the reimbursement of research services and \$0.1 million for the ratable recognition of the upfront payment. During the six months ended June 30, 2018 and 2019, the Company recognized revenue of \$0.1 million (unaudited) and less than \$0.1 million (unaudited), respectively. No adjustment was necessary upon adoption of ASC 606. As of December 31, 2017, December 31, 2018 and June 30, 2019, deferred revenue relating to the uniQure Agreement was less than \$0.1 million, \$0 and \$0 (unaudited), respectively. There were no amounts due from uniQure under the uniQure Agreement as of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited).

## **Benitec**

In November 2014, the Company and Benitec Biopharma Limited ("Benitec") entered into a collaboration and license agreement to collaborate on the discovery and non-clinical research activities related to the Company's Therapeutic Vector Evolution platform in order to generate and validate vectors for gene delivery to treat certain ophthalmic diseases (the "Benitec Agreement"). Benitec has the option of nominating up to three project variants as part of the Benitec Agreement.

The Benitec Agreement provides Benitec with a temporary research license as well as an exclusive development and commercialization license for each project variant selected to further develop. The initial research term is two years and is automatically extended in six-month increments, if necessary, in order to complete additional required studies, for a maximum of five years. Once the Company's research plan has concluded, Benitec is solely responsible for the continued development, manufacturing and commercialization of the project variants as potential product candidates.

Pursuant to the Benitec Agreement, the Company received as consideration (i) an upfront payment of \$0.5 million, (ii) capped research and development service fees based in part on prescribed full-time equivalent labor rates and (iii) reimbursements of pass-through and overhead costs incurred on behalf of Benitec.

On January 24, 2017, the Benitec Agreement was amended to give Benitec sole responsibility for the performance of certain research work which would have generated research services revenue for the Company under the original agreement. Pursuant to the amendment, the Company received \$0.5 million as consideration. This \$0.5 million was recorded as deferred revenue and is being recognized over the same period as the upfront payment.

In March 2019, the Benitec Agreement was terminated based on mutual agreement between the Company and Benitec.

Under ASC 605, the payments of \$1.0 million were recorded as deferred revenue and were being recognized on a ratable basis over the estimated performance period of five years. Payments and reimbursements for research costs, including pass-through and other out-of-pocket costs, were recognized on an as-incurred basis under ASC 605. Under ASC 606, the Company uses the input method to measure progress toward completion of the performance obligation and concluded that revenue will be recognized based on actual costs incurred as a percentage of total budgeted costs as the Company completes its performance obligation. Upon adoption of ASC 606 on January 1, 2019, the Company recorded an additional \$0.2 million of revenue through a decrease in deferred revenue

and decrease in beginning accumulated deficit, based on the difference between the input method used under ASC 606 and the ratable recognition previously used under ASC 605.

The Company identified one combined performance obligation to provide the research license, exclusive development and commercialization licenses for each project variance selected to further develop, research services and participation in the joint steering committee ("JSC"). The transaction price included the \$1.0 million non-refundable upfront fees and \$2.4 million reimbursement for costs incurred and the value of labor hours expended. The Company excluded any consideration related to sales-based milestones, including royalties, which are recognized when the related sales occur. For the six months ended June 30, 2019, there was no change in the transaction price.

During the years ended December 31, 2017 and 2018, the Company recognized revenue of \$0.4 million and \$0.2 million under the Benitec Agreement, respectively. During the six months ended June 30, 2018 and 2019, the Company recognized revenue of \$0.1 million (unaudited) and less than \$0.1 million (unaudited), respectively. As of December 31, 2017, December 31, 2018 and June 30, 2019, deferred revenue relating to the Benitec Agreement was \$0.4 million, \$0.2 million and \$0 (unaudited), respectively. There were no amounts due from Benitec under the Benitec Agreement as of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited). Upon termination of the Benitec Agreement in March 2019, the Company had no further obligations impacting revenue recognition.

## **AGTC**

In April 2015, the Company entered into a collaboration and option agreement with Applied Genetic Technologies Corporation ("AGTC") to discover and develop optimized AAV vectors to treat specific ophthalmic disease indications with high unmet medical need (the "AGTC Agreement"). The AGTC Agreement included both a research funding component as well as a licensing component, wherein AGTC was granted the option to license up to three resulting project variants for up to six products for further development and commercialization. The AGTC Agreement expired in October 2018 when AGTC did not exercise their option to license during the option period.

In accordance with ASC 605 the Company identified the following deliverables at the inception of the AGTC Agreement: (i) the research license, (ii) research services, and (iii) participation in a joint research steering committee. The Company determined that neither the research license nor participation in the joint research steering committee has stand-alone value to AGTC due to the specialized nature of the research services to be provided by the Company, and accordingly, this deliverable was combined with the research services as a single unit of accounting. Further, at the inception of the AGTC Agreement, AGTC's options to obtain an exclusive development and commercialization license for each research project target did not represent deliverables because they are substantive options and do not contain a significant or incremental discount. No adjustment was necessary upon adoption of ASC 606. The Company elected to use the practical expedients permitted related to adoption, which do not require the Company to apply the revenue standard to contracts that are completed as of the date of initial application.

Pursuant to the AGTC Agreement, the Company received two upfront payments totaling \$3.0 million as consideration. The upfront payments of \$3.0 million were recorded as deferred revenue and were recognized on a ratable basis over the estimated performance period of three years.

Revenue was fully recognized on the AGTC Agreement in the year ended December 31, 2018. During the years ended December 31, 2017 and 2018, the Company recognized revenue of \$1.1 million and \$0.3 million, respectively. During the six months ended June 30, 2018, the Company recognized revenue of \$0.3 million (unaudited). As of December 31, 2017, December 31, 2018 and

June 30, 2019, deferred revenue relating to the AGTC Agreement was \$0.3 million, \$0 and \$0 (unaudited), respectively. No amount was due from AGTC under the AGTC Agreement as of December 31, 2017, December 31, 2018, and June 30, 2019 (unaudited).

## CRF

In November 2015, the Company entered into a research funding and collaboration agreement (the "CRF Agreement") with the Choroideremia Research Foundation ("CRF"), a non-profit organization dedicated to finding a cure for Choroideremia, a rare inherited disorder that causes progressive vision loss, ultimately leading to complete blindness. The goal of the CRF Agreement is for CRF to contribute funding to help with the advancement of the Company's Choroideremia research program. The Company is responsible for all decision making and execution of any and all of the related activities to be completed in its sole discretion. The initial term of the CRF Research Plan is two years. The agreement includes contribution to CRF of up to \$2.5 million upon certain development or approval milestones. The overall arrangement has automatic extensions of up to three additional years.

Pursuant to the CRF Agreement, the Company received an initial upfront grant of \$50,000 in 2015 followed by a second upfront grant of \$0.5 million in 2016 as consideration for the services to be provided.

Revenue was fully recognized for this agreement in the year ended December 31, 2017. During the year ended December 31, 2017, the Company recognized revenue of \$0.2 million. There was no deferred revenue relating to the CRF Agreement as of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited). No amount was due from CRF under the CRF Agreement as of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited).

No adjustment was necessary upon adoption of ASC 606. The Company elected to use the practical expedient permitted related to adoption, which does not require the Company to apply ASC 606 to contracts that are completed as of the date of initial application.

## Pfizer

In December 2015, the Company signed a collaboration and license agreement with Pfizer, Inc. ("Pfizer"). Under the terms of the Agreement, the Company agreed to deploy its Therapeutic Vector Evolution platform to generate and validate up to three project variants for gene delivery to treat diseases in cardiac tissue (the "Pfizer Agreement"). Once the Company's research activities concluded, Pfizer would be solely responsible for the continued development, manufacturing and commercialization of the project variants as potential product candidates.

Pursuant to the Pfizer Agreement, the Company received a non-refundable upfront payment of \$5.0 million as consideration. No revenue was recognized under the Pfizer Agreement until Pfizer terminated this agreement for convenience in December 2018. The entire upfront payment of \$5.0 million was recognized as revenue in December 2018 as the Company had no further obligations impacting revenue recognition. As of December 31, 2017, December 31, 2018 and June 30, 2019, deferred revenue relating to the Pfizer Agreement was \$5.0 million, \$0 and \$0 (unaudited), respectively. No amount was due from Pfizer under the Pfizer Agreement as of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited).

No adjustment was necessary upon adoption of ASC 606. The Company elected to use the practical expedient permitted related to adoption, which does not require the Company to apply ASC 606 to contracts that are completed as of the date of initial application.

## Roche

In February 2015, the Company entered into a collaboration and license agreement with F. Hoffmann-La Roche LTD and Hoffmann-La Roche Inc. (together "Roche") to discover and develop optimized next-generation AAV vectors for indications focusing on various rare retinal diseases caused by gene defects (the "2015 Roche Agreement"). As part of the 2015 Roche Agreement, two agreements were executed: (i) a Research Funding and Option Agreement requiring the Company to provide certain early-stage research services to Roche which are dependent on the licensed technology and gives Roche the right to choose to license the resulting project variants for further development and commercialization and (ii) a License Agreement which goes into effect if Roche elects to license the project variants.

The initial research term was two years with the option of three six-month extensions. The research program was completed on schedule and Roche chose not to extend the term. Roche also did not exercise its option to obtain the exclusive development and commercialization license for any of the research project targets.

The upfront payments of \$1.0 million were recorded as deferred revenue and initially recognized on a ratable basis over the estimated performance period of three and a half years. Once the extension notification date passed, the balance of the unamortized deferred revenue was recognized in Q1 2017.

During the year ended December 31, 2017, the Company recognized revenue of \$0.1 million under the 2015 Roche Agreement. There was no deferred revenue balance related to the 2015 Roche Agreement as of December 31, 2017, December 31, 2018 and June 30, 2019. No amount was due from Roche under the 2015 Roche Agreement as of December 31, 2017, December 31, 2018 and June 30, 2019.

No adjustment was necessary upon adoption of ASC 606 as it relates to the 2015 Roche Agreement. The Company elected to use the practical expedient permitted related to adoption, which does not require the Company to apply ASC 606 to contracts that are completed as of the date of initial application.

In November 2017, the Company entered into a new collaboration and license agreement (the "2017 Roche Agreement") with Roche to discover and develop products containing optimized next generation AAV Vectors focused on ophthalmological diseases and disorders excluding select criteria. The Company and Roche both have the ability to nominate products to discover, develop and commercialize.

At the effective date, Choroideremia was designated a Roche product. The Company is responsible for conducting research and development services prior to pivotal clinical studies, and Roche is responsible for conducting subsequent development and commercialization activities. In addition, Roche agreed to pay for research and development services at the agreed upon full-time employee rate for work performed for Choroideremia under the 2017 Roche Agreement, except for the costs associated with the manufacturing work for Choroideremia.

For any product that the Company nominates and conducts research and development services prior to pivotal clinical studies, Roche has an option to convert the status of the product to a Roche product during the 90-day option period. If Roche chooses to not exercise its option, the Company can continue subsequent development and commercialization activities and Roche will have no further rights with respect to such product.

Pursuant to the 2017 Roche Agreement, the Company received an upfront payment of \$21.0 million as consideration. In addition, the Company is entitled to contingent payments including

(i) \$1.0 million for each Roche nominated product beyond the first three, (ii) up to \$30.0 million upon exercise of the option to convert a product the Company nominated and developed prior to pivotal clinical studies (iii) development milestone payments of up to \$223.0 million, \$86.0 million of which relate to Choroideremia and the rest relate to other licensed products; and (iv) sales-based milestones of up to \$123.0 million in connection with licensed products. The 2017 Roche Agreement also includes provisions that entitle the Company to receive royalty payments ranging from the mid-single digits to the mid-teens for the net sales of the licensed products, in each case subject to the reductions in accordance with the terms of the agreement.

Under ASC 605, the upfront payment of \$21.0 million was recorded as deferred revenue and was being recognized on a ratable basis over the estimated performance period of five and a half years. Under ASC 606, the Company uses the input method to measure progress toward completion of the performance obligation and concluded that revenue will be recognized based on actual resources consumed, labor hours expended and costs incurred as a percentage of total budgeted costs. Upon adoption of ASC 606, the Company recognized an additional \$0.4 million of revenue through a decrease to deferred revenue and a decrease in beginning accumulated deficit, based on the difference between the input method used under ASC 606 and the ratable recognition previously used under ASC 605.

Under ASC 606, the Company identified one single combined performance obligation for the license, research services and participation in the JSC. Furthermore, the Company concluded that at the inception of the agreement, Roche's option, exercisable prior to pivotal clinical study initiation, does not represent a material right and should be allocated to the single performance obligation and recognized as revenue upon Roche's exercise of the option. The transaction price related to the agreement includes the \$21.0 million non-refundable upfront fee and \$15.0 million for estimated reimbursements for research and development services at the agreed upon full-time employee rate and third party costs. The Company's contract with Roche does not include a significant financing component. The Company concluded that the transaction price should not include the variable consideration related to development milestones as they were considered to be constrained as it is probable that the inclusion of such variable consideration could result in a significant reversal of cumulative revenue in the future. The Company excluded any consideration related to sales-based milestones, including royalties, which are recognized when the related sales occur. The transaction price and estimated period of performance will be re-evaluated at each reporting period. For the six months ended June 30, 2019, an adjustment of \$4.4 million was made to the transaction price to reflect an increase in the scope of the project and expected reimbursable costs. The increase in the transaction price and total budgeted costs resulted in a \$0.1 million decrease in revenue recognized in the six months ended June 30, 2019 related to performance obligations partially satisfied in periods prior to January 1, 2019.

During the years ended December 31, 2017 and 2018, the Company recognized revenue of \$0.7 million and \$7.5 million, respectively. During the six months ended June 30, 2018 and 2019, the Company recognized revenue of \$4.0 million (unaudited) and \$3.8 million (unaudited), respectively. As of December 31, 2017, December 31, 2018 and June 30, 2019, deferred revenue relating to the Roche Agreement was \$20.5 million, \$17.1 million, and \$14.6 million (unaudited), respectively. Accounts receivable from Roche under this agreement as of December 31, 2017, December 31, 2018 and June 30, 2019 was \$0.3 million, \$1.1 million and \$1.2 million (unaudited), respectively. As of June 30, 2019, the aggregate amount of the transaction price allocated to the remaining performance obligation was \$23.7 million (unaudited). Based on current timelines, the revenue is expected to be recognized over the next five to seven years as the Company continues to develop nominated products until the initiation of pivotal studies.

## **CFFT**

In September 2016, the Company entered into an award agreement for the Optimized Adeno- Associated Virus for Lung Epithelia Gene Delivery Development Program with CFFT, a non-profit organization dedicated to finding a cure for Cystic Fibrosis ("CF"), an inherited disorder that causes disease in the pulmonary airways leading to morbidity and mortality. Under this agreement, CFFT contributes funding to help advance the Company's CF research program. The agreement was subsequently amended in September 2017 and August 2018 (all three agreements are collectively referred to as the "CFFT Agreements"). The total amount of the award under the CFFT Agreements is \$3.5 million. As of December 31, 2017, December 31, 2018 and June 30, 2019, the Company received \$0.6 million under the CFFT Agreements. The remaining award amount will be paid by CFFT based on achievement of certain development milestones by the Company.

The Company expects to make payments to CFFT equal to six times the actual award received by the Company in three installments within the first four years of the first commercial sale of a product developed under this agreement. The Company also has agreed to make future sales-based milestone payments to CFFT of up to three times the actual award received upon achieving specified commercialization milestones with respect to the first of any product developed utilizing any compound covered under the collaboration agreement. The CFFT Agreements also require the Company to pay to CFFT royalties of a mid-single digit percentage, up to six times the actual award received, on any amounts received by the Company from the sale, license or transfer to a third-party of rights in the technology developed as a result of this collaboration. Any such royalty payments shall be credited against the payments owed by the Company upon first commercial sale. In the event of a change of control of the Company, CFFT will receive certain payments, depending on the timing of the change of control and the size of the transaction.

To date, the Company had not developed a commercial product in connection with this award agreement, and it had not licensed, sold or otherwise transferred to another party the product developed under the agreement or the underlying technology.

If at any time prior to the first commercial sale of a product developed as a result of the agreement, the Company ceases to use commercially reasonable efforts to develop or commercialize any product under this agreement for a continuous period of 180 consecutive days and fails to present a reasonable plan to resume commercially reasonable efforts, the Company will grant to CFFT an irrevocable, exclusive worldwide interruption license under all of the Company's interest in the research plan technology to exploit such product. Any third-party license granted by the Company shall be subject to such interruption license.

Under ASC 605, the Company recognizes revenue under this agreement on a ratable basis over the estimated performance period of all milestones. Under ASC 606, the Company uses the input method to measure progress toward completion of the performance obligation and concluded that revenue will be recognized based on actual resources consumed, labor hours expended and costs incurred as a percentage of total budgeted costs. Upon adoption of ASC 606, the Company decreased revenue by \$0.2 million through an increase to deferred revenue and an increase to beginning accumulated deficit, based on the difference between the input method under ASC 606 and the ratable recognition previously used under ASC 605.

Under ASC 606, the Company identified one performance obligation within the CFFT grant agreement for research activities. The transaction price related to the agreement includes the \$0.6 million non-refundable upfront fees and milestones previously met under the initial grant agreement. The Company's contract with CFFT does not include a significant financing component. The Company concluded that the transaction price should not include the variable consideration related to future

research milestones as they were considered to be constrained as it is probable that the inclusion of such variable consideration could result in a significant reversal of cumulative revenue in the future. The Company will re-evaluate the transaction price and estimated period of performance at each reporting period. For the six months ended June 30, 2019 there were no adjustments made to the transaction price.

During each of the years ended December 31, 2017 and 2018, the Company recognized revenue of \$0.1 million. During each of the six months ended June 30, 2018 and 2019, the Company recognized revenue of less than \$0.1 million (unaudited). As of December 31, 2017, December 31, 2018 and June 30, 2019, deferred revenue relating to the CFFT Agreement was \$0.3 million, \$0.2 million and \$0.4 million (unaudited), respectively. No amount was due from CFFT under this agreement as of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited). As of June 30, 2019, the aggregate amount of the transaction price allocated to the remaining performance obligation was \$0.4 million (unaudited). Based on current timelines, the revenue is expected to be recognized over the next five years as the Company performs research services through the completion of IND-enabling studies.

The obligation to make payments to CFFT upon a change of control meets the definition of an embedded derivative that is required to be bifurcated and separately accounted for as a derivative liability. The Company determined the estimated fair value of this derivative liability to be \$0.1 million as of December 31, 2017, December 31, 2018 and June 30, 2019. See Note 15 for further discussion of the embedded derivative.

## AstraZeneca

In December 2017, the Company entered into a collaboration and option agreement with MedImmune, Inc., the global biologics research and development arm of AstraZeneca, ("AstraZeneca") to discover and develop optimized AAV vectors to treat specific lung disease indications ("the AstraZeneca Agreement"). The AstraZeneca agreement included both a research funding component as well as a licensing component, wherein AstraZeneca was granted the option to license up to three resulting project vector variants for further development and commercialization.

The initial research term was approximately twelve months with AstraZeneca's option to extend the term for an additional six months. AstraZeneca requested the six-month extension in October 2018. AstraZeneca's option to license the resulting project variants expires twelve months after the conclusion of the research phase. Once the Company's research activities have concluded, AstraZeneca is solely responsible for the continued development, manufacturing and eventual commercialization of the project variants as potential product candidates.

Pursuant to the AstraZeneca Agreement, the Company received an upfront payment of \$1.5 million as consideration. In addition, the Company is entitled to contingent payments including (i) a non-refundable license option exercise fee of \$2.0 million and (ii) milestones up to \$45.0 million for each product. The AstraZeneca Agreement also includes provisions that entitle the Company to receive royalties in the single digit range on future sales of the potential product candidates.

The Company has identified one single combined performance obligations within the AstraZeneca agreement for the research program license, research and development activities and participation in the joint project team and JSC. The Company concluded that the performance obligations are not distinct and, therefore, should be combined into a single combined performance obligation. Furthermore, the Company concluded that at the inception of the agreement, AstraZeneca's license option, does not represent a material right and should be allocated to the single performance obligation and recognized as revenue upon AstraZeneca's exercise of the Option.

The transaction price related to the agreement consists of the \$1.5 million non-refundable upfront fee. The Company concluded that the transaction price should not include the variable consideration related to developmental milestones as they were considered to be constrained as it is probable that the inclusion of such variable consideration could result in a significant reversal of cumulative revenue in the future. The Company excluded any consideration related to sales-based milestones, including royalties, which are recognized when the related sales occur. The Company will re-evaluate the transaction price and estimated period of performance at each reporting period. The Company's contract with AstraZeneca does not include a significant financing component.

Under ASC 605, the \$1.5 million upfront payment was recorded as deferred revenue and was being recognized as revenue on a ratable basis over the estimated performance period of one and a half years. Under ASC 606, the Company used the input method to measure progress toward completion of the performance obligation and concluded that revenue will be recognized based on actual resources consumed, labor hours expended and costs incurred as a percentage of total budgeted costs. Upon adoption of ASC 606, the Company reduced revenue by \$48,000 through an increase to deferred revenue and an increase to beginning accumulated deficit, based on the difference between the input method used under ASC 606 and the ratable recognition previously used under ASC 605.

In June 2019, the research phase concluded and the Company delivered their final report to AstraZeneca. The option term continues for twelve months after AstraZeneca's receipt of the final report where they may exercise the option to obtain the license of up to three project vector variants.

During the years ended December 31, 2017 and 2018, the Company recognized revenue of \$0 and \$1.0 million, respectively. During each of the six months ended June 30, 2018 and 2019, the Company recognized revenue of \$0.5 million (unaudited). As of December 31, 2017, December 31, 2018 and June 30, 2019, deferred revenue relating to the AstraZeneca Agreement was \$0, \$0.5 million and \$0 (unaudited), respectively. No amount was due from AstraZeneca under this agreement as of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited).

## 7. License Arrangements

The Company has exclusive, worldwide license agreements (the "UC Agreements") with the Regents of the University of California (the "UC Regents") relating to the use of certain patents and intellectual property surrounding its core technologies, including Therapeutic Vector Evolution. Pursuant to each of the UC Agreements executed prior to January 2019, the Company was obligated to pay a (i) non-refundable license fee of \$5,000 upon execution, (ii) a non-refundable license fee of \$5,000 each year thereafter, until sales of a licensed product are made and royalties are paid to the UC Regents, (iii) reimbursement of domestic and foreign patent filing, prosecution and maintenance fees, and (iv) either \$50,000 or issuance of a 3% equity interest in the Company upon the closing of the first qualified financing at the option of the UC Regents. The Company's first qualified financing occurred in 2015 and at the election of the UC Regents, the Company issued the UC Regents in January 2016 an amount of common stock equal to 6% of the equity interests in the Company pursuant to the applicable clause in each of the UC Agreements.

Pursuant to an agreement with the UC Regents executed on January 2019 the Company paid a non-refundable license fee of \$50,000 to the UC Regents upon execution of the agreement. The Company is obligated pay a non-refundable license fee of \$5,000 on the one-year anniversary of the contract effective date and each year thereafter, until sales of a licensed product are made and royalties are paid to the UC Regents.

In addition, the Company is obligated to make certain contingent payments including (i) development milestones up to \$3.1 million, (ii) low single digit royalties on the net sales of its

developed products that consists of a minimum annual royalty of up to \$0.1 million per year for the term of the Agreement beginning in the first calendar year after the year in which net sales first occurred, and (iii) sublicense consideration in the mid-teens to the mid-twenties-range on any future sublicensing arrangements the Company may enter into with third-party licensees.

During each of the years ended December 31, 2017 and 2018, the Company incurred expenses of \$0.1 million under the provisions of the UC Agreements. During the six months ended June 30, 2018 and 2019, the Company incurred expenses of \$0.1 million (unaudited) and \$0.2 million (unaudited), respectively, under the provisions of the UC Agreements.

## 8. Commitments and Contingencies

## **Operating Lease Commitments**

In May 2015, the Company executed a lease agreement for office and laboratory space in Emeryville, California. In January 2016, the Company executed the first amendment to the lease agreement for additional rentable office and laboratory space which extends the lease to March 31, 2023. In October 2018, the Company executed a second amendment to extend the lease to end at the same time as the new lease discussed below. Additionally, the second amendment provided a tenant improvement allowance of \$0.2 million, which was paid to the Company in November 2018. The Company amortizes the tenant improvement allowance on a straight-line basis over the remaining term of the lease as a reduction of rent expense.

In October 2018, the Company executed a second lease agreement for additional office and laboratory space in Emeryville, California. The new lease has an initial term of 87 months beginning on the rent commencement date with the option to renew the lease for one additional term of five years. The Company did not have to pay rent until March 2019. This lease agreement also provides for a tenant improvement allowance of \$0.4 million.

In May 2019, the Company amended the second lease agreement executed in October 2018 to add additional office and laboratory space. The amendment extended the term of the lease to December 31, 2029. The Company does not have to pay rent until December 2019. The annual rent for the additional space is \$1.0 million per annum and escalates at 3% annually. This lease agreement also provides for a tenant improvement allowance of at least \$1.6 million.

The Company recognizes rent expense on a straight-line basis over the lease term with the difference between the rent payments and the straight-line rent expense recorded as deferred rent. Rent expense for the years ended December 31, 2017 and 2018 and six months ended June 30, 2018 and 2019 for these facilities was \$0.5 million, \$0.5 million, \$0.2 million (unaudited) and \$0.3 million (unaudited), respectively. Deferred rent as of December 31, 2017, December 31, 2018 and June 30, 2019 was \$0.1 million, \$0.4 million and \$0.2 million (unaudited), respectively. In conjunction with the lease agreements and amendments, the Company paid total security deposits of \$0.1 million, \$0.3 million and \$0.6 million (unaudited), which are included in other assets within the balance sheets as of December 31, 2017, December 31, 2018 and June 30, 2019, respectively. The following table summarizes the Company's future minimum commitments under lease contracts (in thousands):

December 31, 2018	
2019	\$ 850
2020	1,728
2021	1,804
2022	1,855
2023	1,911
2024 and beyond	5,379
Total	5,379 \$13,527

June 30, 2019 (unaudited)	
Remainder of 2019	\$ 567
2020	2,750
2021	2,885
2022	2,968
2023	3,058
2024 and beyond	18,213
Total	\$ 30,441

## **Common Stock Warrant Obligation**

As of December 31, 2017, the Company had an obligation to issue a warrant for 23,669 shares of the Company's common stock to a service provider. The Company issued the warrant in May 2018. See Note 13 for further discussion on common stock warrant obligation.

## **Indemnification Agreements**

In the ordinary course of business, the Company enters into agreements that may include indemnification provisions. Pursuant to such agreements, the Company may indemnify, hold harmless and defend an indemnified party for losses suffered or incurred by the indemnified party. Some of the provisions will limit losses to those arising from third-party actions. In some cases, the indemnification will continue after the termination of the agreement. The maximum potential amount of future payments the Company could be required to make under these provisions is not determinable. The Company has never incurred material costs to defend lawsuits or settle claims related to these indemnification provisions. The Company has also entered into indemnification agreements with its directors and officers that require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers to the fullest extent permitted by Delaware corporate law. The Company currently maintains directors' and officers' liability insurance that would generally enable it to recover a portion of any future amounts paid. The Company believes the estimated fair value of its indemnification agreements in excess of applicable insurance coverage is not material.

## **Legal Proceedings**

From time to time, the Company may become involved in legal proceedings arising from the ordinary course of its business. If applicable, the Company records a legal liability when it believes that it is both probable that a liability may be imputed, and the amount of the liability can be reasonably estimated. Significant judgment by the Company is required to determine both probability and the estimated amount. Management is currently not aware of any matters that could have a material adverse effect on the Company's financial position, results of operations or cash flows.

## 9. Income Taxes

The Company did not record any income tax expense during the years ended December 31, 2017 and 2018. The Company has a net operating loss and has provided a valuation allowance against net deferred tax assets due to uncertainties regarding the Company's ability to realize these assets. All losses before income taxes arose in the United States.

The effective tax rate of the Company's income tax expense (benefit) differs from the federal statutory rate as follows:

	2017	2018
Federal statutory income tax rate	34.0%	21.0%
Research tax credit	4.4%	10.0%
Permanent differences	(0.5%)	(1.1%)
Valuation allowance	(15.6%)	(27.4%)
2017 Tax Act—Tax rate change	(22.3%)	0.0%
Section 382 limitation	0.0%	(2.5%)
Provision for income taxes	0.0%	0.0%

The tax effects of temporary differences that give rise to significant components of the deferred taxes are as follows (in thousands):

	2017	2018
Deferred Tax Assets		
Net operating loss carryforwards	\$ 2,853	\$ 1,990
Other accrued liabilities	171	103
Deferred revenue	1,152	3,598
Research tax credits	951	1,872
Stock compensation	31	212
Total deferred tax assets	\$ 5,158	\$ 7,775
Less: valuation allowance	(5,158)	(7,775)
Net deferred tax assets	<u>\$</u>	<u> </u>

ASC 740 requires that the tax benefit of net operating losses, temporary differences and credit carryforwards be recorded as an asset to the extent that management assesses that realization is "more likely than not." Realization of the future tax benefits is dependent on the Company's ability to generate sufficient taxable income within the carryforward period. Because of the Company's recent history of operating losses, management believes that recognition of the deferred tax assets arising from future tax benefits is currently not likely to be realized and, accordingly, has provided a full valuation allowance. The valuation allowance increased by \$1.7 million and \$2.6 million during the years ended December 31, 2017 and 2018, respectively.

On December 22, 2017, the U.S. government enacted comprehensive tax legislation commonly referred to as the Tax Cuts and Jobs Act (the "Tax Act"). The Tax Act makes broad and complex changes to the U.S. tax code, including, but not limited to: (i) reducing the U.S. federal corporate tax rate from 35% to 21%; (ii) requiring companies to pay a one-time transition tax on certain unrepatriated earnings of foreign subsidiaries; (iii) generally eliminating U.S. federal income taxes on dividends from foreign subsidiaries; (iv) requiring a current inclusion in U.S. federal taxable income of certain earnings of controlled foreign corporations; (v) eliminating the corporate alternative minimum tax ("AMT") and changing how existing AMT credits can be realized; (vi) creating the base erosion anti-abuse tax ("BEAT"), a new minimum tax; (vii) creating a new limitation on deductible interest expense; and (viii) changing rules related to uses and limitations of net operating loss carryforwards created in tax years beginning after December 31, 2017.

As of December 31, 2018, the Company has completed its analysis of the income tax effects of the Tax Act and there was no material impact to the Company's financial statements when the analysis was complete. As of December 31, 2017 and 2018, the Company had federal net operating loss

carryforwards of \$13.6 million and \$9.5 million, respectively, available to reduce future taxable income, if any, for federal income tax purposes, respectively. The federal net operating loss carryforwards expire in 2037.

As of December 31, 2017 and 2018, the Company had federal research and development credit carryforwards of \$0.7 million and \$1.3 million and state research and development credit carryforwards of \$0.8 million and \$1.4 million available to reduce future taxable income, if any, for federal and California state income tax purposes, respectively. The federal credit carryforwards begin expiring in 2035 and the state credits carryforward indefinitely.

Utilization of the net operating loss carryforwards and research credit carryforwards may be subject to an annual limitation due to the ownership percentage change limitations provided by the Internal Revenue Code and similar state provisions. Annual limitations may result in the expiration of the net operating losses and tax credit carryforwards before they are utilized. The Company has experienced ownership changes in the past as a result of its Series B redeemable convertible preferred stock financing. As a result of the ownership changes, the Company has determined that \$0.9 million of its NOLs will expire unutilized for federal income tax purposes and such amounts are excluded from its NOLs as of December 31, 2018. Subsequent ownership changes may affect the limitation in future years.

The reconciliation of the beginning and ending unrecognized tax benefits amounts is as follows (in thousands):

	Inco	cognized ome Tax enefits
Balance as of December 31, 2016	\$	143
Additions for current year tax positions		209
Balance as of December 31, 2017		352
Additions for current year tax positions		333
Reductions of prior year positions		(26)
Balance as of December 31, 2018	\$	659

The entire amount of the unrecognized tax benefits would not impact the Company's effective tax rate if recognized. During the years ended December 31, 2017 and 2018, the Company did not recognize accrued interest and penalties related to unrecognized tax benefits. The Company does not anticipate that the amount of existing unrecognized tax benefits will materially increase or decrease during the next 12 months.

The Company files income tax returns in the U.S. federal and California tax jurisdictions. All tax returns from inception to December 31, 2018 remain subject to examination. The Company has no ongoing income tax examinations by tax authorities at this time.

## 10. Redeemable Convertible Preferred Stock

In August 2018, the Company issued 5,154,632 shares of Series B redeemable convertible preferred stock at \$17.46 per share for gross proceeds of \$90.0 million.

As of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited), the Company's certificate of incorporation authorized the Company to issue up to 2,309,312 shares and 7,375,638 and 7,375,638 shares of redeemable convertible preferred stock, respectively, at a par value of \$0.0001 per share.

Redeemable convertible preferred stock consists of the following as of December 31, 2017 (in thousands, except per share and share amounts):

	Shares Authorized	Original Issuance Price	Shares Issued and Outstanding	Liquidation Value	Proceeds Net of Issuance Cost
Series A	909,312	\$ 7.70	909,312	\$ 7,001	\$ 6,960
Series A-1	1,400,000	\$ 8.84	1,311,687	11,595	11,548
Total	2,309,312		2,220,999	\$ 18,596	\$ 18,508

Redeemable convertible preferred stock consists of the following as of December 31, 2018 (in thousands, except per share and share amounts):

	Shares Authorized	Original Issuance Price	Shares Issued and Outstanding	Liquidation Value	Proceeds Net of Issuance Cost
Series A	909,312	\$ 7.70	909,312	\$ 7,001	\$ 6,960
Series A-1	1,311,687	\$ 8.84	1,311,687	11,595	11,548
Series B	5,154,639	\$ 17.46	5,154,632	90,000	84,472
Total	7,375,638		7,375,631	\$ 108,596	\$ 102,980

Redeemable convertible preferred stock consists of the following as of June 30, 2019 (unaudited) (in thousands, except per share and share amounts):

	Shares Authorized	Original Issuance Price	Shares Issued and Outstanding	Liquidation Value	Proceeds Net of Issuance Cost
Series A	909,312	\$ 7.70	909,312	\$ 7,001	\$ 6,960
Series A-1	1,311,687	\$ 8.84	1,311,687	11,595	11,548
Series B	5,154,639	\$ 17.46	5,154,632	90,000	84,472
Total	7,375,638		7,375,631	\$ 108,596	\$ 102,980

The holders of redeemable convertible preferred stock have various rights and preferences including the following:

Liquidation Preference—In the event of a liquidation event, the holders of the Series B redeemable convertible preferred stock are entitled to receive any distribution of any of the assets of the Company in preference to the holders of the Series A-1 redeemable convertible preferred stock, Series A redeemable convertible preferred stock or common stock, an amount per share equal to the greater of (i) the Original Series B issue price plus all declared but unpaid dividends and (ii) such amount per share payable had all shares of Series B redeemable convertible preferred stock been converted into common stock. After full payment to holders of the Series B redeemable convertible preferred stock, in preference to the holders of the Series A redeemable convertible preferred stock or common stock, in an amount equal to the greater of (i) the Original Series A-1 issue price plus all declared but unpaid dividends and (ii) such amount per share payable had all shares of Series A-1 redeemable convertible preferred stock been converted into common stock. After full payment to holders of the Series A-1 redeemable convertible preferred stock, payment should be made to the holders of Series A redeemable convertible preferred stock, in preference to the holders of the common stock, in an amount equal to the greater of (i) the Original Series A issue price plus all declared but unpaid dividends and (ii) such amount per share payable had all shares of Series A

redeemable convertible preferred stock been converted into common stock. If upon the occurrence of a liquidation event, the available assets are insufficient to pay the holders of a given series of redeemable convertible preferred stock the full amount to which they are entitled, then the available assets shall be distributed to the holders of such preferred stock on pro rata, on an equal priority, pari passu basis, in proportion to the full preferential amount such holder is otherwise entitled to receive.

Notwithstanding the above, for purposes of determining the amount each holder of shares of redeemable convertible preferred stock is entitled to receive with respect to a Liquidation Event, each such holder of shares of a series of redeemable convertible preferred stock shall be deemed to have converted such holder's shares of such series into shares of common stock immediately prior to the Liquidation Event if, as a result of an actual conversion, such holder would receive, in the aggregate, an amount greater than the amount that would be distributed to such holder if such holder did not convert such series of redeemable convertible preferred stock into shares of common stock. If any such holder shall be deemed to have converted shares of redeemable convertible preferred stock into common stock pursuant to this paragraph, then such holder shall not be entitled to receive any distribution that would otherwise be made to holders of redeemable convertible preferred stock that have not converted into shares of common stock.

Conversion—Shares of any series of redeemable convertible preferred stock can be converted, at the option of the holder, into such number of fully paid and non-assessable shares of common stock using a conversion rate determined by dividing the applicable original issue price by the applicable conversion price, as adjusted for any anti-dilution adjustments. If, after the issuance date of the redeemable convertible preferred stock, the Company issues or sells, or is deemed to have sold, additional shares of common stock at a price lower than the original issuance price, except for certain exceptions allowed, the conversion price of the redeemable convertible preferred stock would be adjusted. As of December 31, 2018 and June 30, 2019 (unaudited), the conversion price for the Series A, A-1 and B redeemable convertible preferred stock is \$7.700 per share, \$8.839 per share and \$17.460 per share, respectively, and the conversion ratio is one-for-one.

As of December 31, 2018 and June 30, 2019, shares of redeemable convertible preferred stock shall automatically be converted into shares of common stock at the then effective conversion price for such share, immediately prior to either: (i) the completion of an underwritten public offering of the Company's common stock at a price of at least 1.5 times the original Series B issuance price for any initial public offering consummated at any time prior to the first anniversary of the Series B original issuance date, or 1.25 times the original Series B issuance price for any such IPO thereafter and that provides at least \$30.0 million of gross proceeds to the Company (a "Qualified IPO") or (ii) the conversion by the holders of redeemable convertible preferred stock, which requires the vote of the holders of a majority of the then outstanding shares of redeemable convertible preferred stock, voting together as a single class on an as-converted to common stock basis.

Dividends—Holders of shares of redeemable convertible preferred stock shall be entitled to non-cumulative dividends prior to, and in preference to any declaration or payment of any dividend on common stock. The amount of such dividends payable per share of preferred stock is at least equal to the dividend payable per share of common stock. Through December 31, 2018 and June 30, 2019 (unaudited), no dividends had been declared. Given this dividend preference, the Company considers all series of redeemable convertible preferred stock to be participating securities.

Voting Rights—Each holder of redeemable convertible preferred stock shall be entitled to the number of votes equal to the number of shares of common stock into which the shares of redeemable convertible preferred stock held by such holder could be converted as of the record date. Holders of redeemable convertible preferred stock and common stock generally vote as a single class. So long as at least 916,380 of the Series B redeemable convertible preferred stock issued remain outstanding, the

holders of a majority of the Series B redeemable convertible preferred stock will be entitled to designate one director. So long as Pfizer and its Affiliates continue to own at least 25% of the Series A-1 redeemable convertible preferred stock, Pfizer will be entitled to designate one director.

Redemption and Balance Sheet Classification—The redeemable convertible preferred stock is recorded in mezzanine equity because while it is not mandatorily redeemable, it will become redeemable at the option of the stockholders upon the occurrence of a deemed liquidation event that is considered not solely within the Company's control.

## 11. Common Stock

As of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited), the Company's certificate of incorporation authorized the Company to issue 50,000,000 shares of common stock at the par value of \$0.0001 per share. The holder of each share of common stock is entitled to one vote per share.

Common stockholders are entitled to dividends if and when declared by the board of directors, subject to the prior rights of the redeemable preferred stockholders. As of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited), no dividends on common stock had been declared by the board of directors.

The number of authorized shares of common stock may be increased or decreased (but not below the number of shares thereof then outstanding or reserved for issuance) by the affirmative vote of the holders of a majority (assuming the conversion of all redeemable convertible preferred stock) of the capital stock of the Company entitled to vote and without a separate class vote of the common stock.

As of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited), the Company has reserved common stock, on an as-converted basis, for future issuance as follows:

	December 31, 2017	December 31, 2018	June 30, 2019 (Unaudited)
Conversion of redeemable convertible preferred stock	2,220,999	7,375,631	7,375,631
Stock options available for future stock option grant	1,004,239	578,842	399,886
Options issued and outstanding	792,290	2,028,274	2,192,230
Common stock warrants	68,669	68,669	68,669
Total common stock reserved	4,086,197	10,051,416	10,036,416

## **Restricted Common Stock**

During 2015, the Company issued common stock to the Company founders of 4,710,060 shares, of which 4,473,374 were fully vested upon issuance. The remainder were deemed to be restricted based on their vesting conditions. The stock agreement contains certain provisions that allow the Company to repurchase unvested portions of stock from such founders in the event they depart from the Company. The repurchase rights on the restricted common stock lapse over time and fully expired in March 2019. As of December 31, 2017, December 31, 2018, and June 30, 2019, 73,964, 14,793 and 0 (unaudited) shares of restricted common stock remained subject to repurchase, respectively.

## 12. Stock-based Compensation

## 2015 Equity Incentive Plan

In March 2015, the Company adopted the 2015 Equity Incentive Plan (the "2015 Plan") under which the board of directors is authorized to issue grants of stock options, stock appreciation rights,

restricted stock and restricted stock unit awards to employees, directors and consultants of the Company. As of December 31, 2018 and June 30, 2019 (unaudited), there were 2,694,528 shares authorized and reserved for issuance. Under the 2015 Plan, as of December 31, 2018 and June 30, 2019, 578,842 and 399,886 (unaudited) of these shares respectively were available for grant. All options are exercisable over a period not to exceed the contractual term of ten years from the date the stock options were issued and are granted at prices not less than the estimated fair market value of the Company's common stock on the grant date as determined by the board of directors. If an individual owns stock representing more than 10% of the Company's outstanding shares, the exercise price of each share shall be at least 110% of the fair market value on the date of grant.

## **Stock Options**

The following table summarizes the stock options activity:

		Options Ou	g				
	Number of Shares Available for Grant	Number of Shares Underlying Outstanding Options	Ave Exe	ghted- erage ercise rice	Weighted- Average Remaining Contractual Term (in years)	ı	ggregate ntrinsic Value :housands)
Balances at December 31, 2016	975,785	835,115	\$	1.09	9.10	\$	421
Options granted	(163,000)	163,000		1.59			
Options exercised	_	(14,371)		1.10			30
Options expired	10,833	(10,833)		1.14			
Options forfeited	180,621	(180,621)		1.22			
Balances at December 31, 2017	1,004,239	792,290	\$	1.16	8.27	\$	1,609
Options authorized	873,628						
Options granted	(1,454,173)	1,454,173		6.56			
Options exercised	_	(63,041)		1.66			488
Options expired	111,401	(111,401)		1.13			
Options forfeited	43,747	(43,747)		1.95			
Balances at December 31, 2018	578,842	2,028,274	\$	5.00	8.97	\$	8,939
Options granted (unaudited)	(688,740)	688,740		9.41			
Options exercised (unaudited)	_	(15,000)		1.34			147
Options expired (unaudited)	72,833	(72,833)		1.80			
Options forfeited (unaudited)	436,951	(436,951)		7.09			
Balances at June 30, 2019 (unaudited)	399,886	2,192,230	\$	6.10	8.78	\$	15,319
Exercisable, December 31, 2018		603,141	\$	1.56	7.64	\$	4,733
Vested and expected to vest, December 31, 2018		2,028,274	\$	5.00	8.97	\$	8,939
Exercisable, June 30, 2019 (unaudited)		696,012	\$	3.78	7.45	\$	7,540
Vested and expected to vest, June 30, 2019 (unaudited)		2,192,230	\$	6.10	8.78	\$	15,319
Options granted (unaudited) Options exercised (unaudited) Options expired (unaudited) Options forfeited (unaudited) Balances at June 30, 2019 (unaudited) Exercisable, December 31, 2018 Vested and expected to vest, December 31, 2018 Exercisable, June 30, 2019 (unaudited) Vested and expected to vest, June 30, 2019	(688,740) 	688,740 (15,000) (72,833) (436,951) 2,192,230 603,141 2,028,274 696,012	\$ \$ \$	9.41 1.34 1.80 7.09 6.10 1.56 5.00 3.78	8.78 7.64 8.97 7.45	\$ \$ \$	15,3 4,7 8,9 7,5

The following table is a summary of stock compensation expense for employees and nonemployees by function (in thousands):

	Year Ended December 31, 2017		Year Ended December 31, 2018		Six Months Ended June 30, 2018 (Unaudited)		Six Months Ended June 30, 2019 (Unaudited)	
Research and development	\$	188	\$	695	\$	184	\$	950
General and administrative		68		682		219		522
Total stock-based compensation	\$	256	\$	1,377	\$	403	\$	1,472

During the years ended December 31, 2017 and 2018, the Company granted 153,000 and 1,234,173 stock options to employees with a weighted-average grant date fair value of \$1.12 and \$5.50 per share, respectively, and 10,000 and 220,000 stock options to nonemployees with a weighted-average grant date fair value of \$1.32 and \$3.38, respectively. During the six month ended June 30, 2018 and 2019, the Company granted 298,436 (unaudited) and 653,740 (unaudited) stock options to employees with a weighted-average grant date fair value of \$2.30 (unaudited) and \$7.32 (unaudited) per share, respectively, and 190,000 and 35,000 stock options to nonemployees with a weighted-average grant date fair value of \$2.67 and \$8.43 and respectively. The total fair value of options vested during the years ended December 31, 2017 and 2018 was \$0.3 million and \$0.8 million, respectively, and \$0.3 million (unaudited) and \$0.6 million (unaudited) during the six months ended June 30, 2018 and 2019, respectively. As of December 31, 2018 and June 30, 2019, the unrecognized stock-based compensation of unvested options was \$8.0 million and \$9.4 million (unaudited), respectively, and is expected to be recognized over a weighted-average period of 3.4 years and 3.2 years (unaudited), respectively. Stock-based compensation expense recorded for employee options was \$0.2 million and \$0.6 million for the years end December 31, 2017 and 2018 and \$0.2 million (unaudited) and \$1.1 million (unaudited) for the six months ended June 30, 2018 and June 30, 2019, respectively. Stock-based compensation expense recorded for nonemployee consultants was \$0.1 million and \$0.8 million for years ended December 31, 2017 and 2018, respectively, and \$0.2 million (unaudited) and \$0.4 million (unaudited) for the six months ended June 30, 2018 and 2019, respectively, and \$0.2 million (unaudited) and \$0.4 million (unaudited) for the six months ended June 30, 2018 and 2019, respectively.

The Company is a privately held company with no active public market for the Company's common stock. The fair value of the shares of common stock underlying the stock options was estimated by the board of directors at various dates considering the Company's most recently available third-party valuations of common stock as well as a number of objective and subjective factors including valuation of comparable companies, sales of redeemable convertible preferred stock, operating and financial performance and general and industry specific economic outlook, amongst other factors. The fair value was determined in accordance with the guidance provided by the American Institute of Certified Public Accountants' Practice Guide, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*.

The Company estimates the fair value of employee and nonemployee stock options using the Black-Scholes valuation model. The fair value of employee and nonemployee stock options is recognized on a straight-line basis over the requisite service period of the awards. The fair value of the Company's stock options was estimated using the following assumptions for the years ended December 31, 2017 and 2018 and six months ended June 30, 2018 and 2019 (unaudited), respectively:

	Decem	Year Ended December 31, 2017		Year Ended December 31, 2018		ns Ended 30, L8	Six Months Ended June 30, 2019	
	Employee	Nonemployee	Employee	Nonemployee	Employee Nonemployee		Employee	Nonemployee
						(Unaudited)		dited)
Expected term	5.6 –5.9 years	7.7 –10.0 years	5.5 –6.3 years	6.7 –9.9 years	5.5 –6.3 years	7.2 –9.9 years	5.3 –6.3 years	6.2 –9.7 years
Expected volatility	84.7% -84.9%	81.9% -85.6%	81.4% -84.1%	80.8% -83.9%	83.4% -84.1%	81.8% -83.9%	82.8% -83.0%	81.5% -83.7%
Risk-free interest rate	1.9% - 1.9%	2.2% - 2.4%	2.7% -3.0%	2.6% -3.2%	2.8% - 2.9%	2.7% -2.9%	2.3% -2.3%	1.8% -2.4%
Dividend yield	0%	0%	0%	0%	0%	0%	0%	0%

Expected Term. The expected term for employee options is calculated using the simplified method as the Company does not have sufficient historical information to provide a basis for estimate. The simplified method is based on the average of the vesting tranches and the contractual life of each grant. The expected term for nonemployee options is the contractual term of the options.

Expected Volatility. The expected volatility was estimated based on a study of publicly traded peer companies as the Company did not have any trading history for its common stock. The Company selected the peer group based on similarities in industry, stage of development, size and financial leverage with the Company's principal business operations. For each grant, the Company measured historical volatility over a period equivalent to the expected term.

*Risk-free Interest Rate.* The risk-free interest rate is based on the yield available on U.S. Treasury zero-coupon issues whose term is similar in duration to the expected term of the respective stock option.

Expected Dividend Yield. The Company has not paid and does not anticipate paying any dividends on its common stock in the future. Accordingly, the Company has estimated the dividend yield to be zero.

## 13. Common Stock Warrants

In 2016, the Company issued a warrant for 45,000 shares of the Company's common stock to a service provider with an exercise price of \$1.14 per share, of which 15,000 warrant shares become exercisable upon completion of an offering of securities in a private placement by the Company with net proceeds in excess of \$25.0 million and 30,000 warrant shares become exercisable upon completion of an IPO by the Company. As the services had been completed at the date the warrant had been issued, the fair value of the warrant was determined at the issuance date. In 2018, 15,000 of these warrant shares became exercisable upon the completion of the Series B financing and the \$13,000 fair value of these warrant shares, as determined under the Black-Scholes Model, was recorded within operating expenses in the statements of operations and comprehensive loss and within additional paid-in-capital in the balance sheets. The Company has not recognized expense for the remaining 30,000 warrant shares that become exercisable upon completion of an IPO as the vesting condition was not considered probable as of December 31, 2018 and June 30, 2019 (unaudited). If an IPO had occurred on June 30, 2019 (unaudited), the Company would have recorded less than \$0.1 million within operating expenses in the statements of operations and comprehensive loss and within additional paid-in-capital in the balance sheets for the 30,000 warrant shares becoming exercisable. The warrant expires in 2023.

The Company also agreed to issue a warrant for 23,669 common stock shares with an exercise price of \$3.19 per share to a third-party. As the Company had not issued the warrant as of December 31, 2017, the obligation to issue this common stock warrant was remeasured to its fair value of \$60,000 as of December 31, 2017 using the Black-Scholes option pricing model. The warrant was issued in May 2018 and

the obligation to issue the common stock warrant was remeasured at the fair value of \$59,000 and recorded within additional paid-incapital in the balance sheets upon issuance of the warrants. The warrant expires in 2025.

## 14. Net Loss Per Share Attributable to Common Stockholders

The following table sets forth the computation of basic and diluted net loss per share attributable to common stockholders (in thousands, except share and per share data):

	Year Ended December 31, 2017	Year Ended December 31, 2018	Six Months Ended June 30, 2018 (Unaudited)	Six Months Ended June 30, 2019 (Unaudited)
Numerator			•	,
Net loss attributable to common stockholders	\$ (11,223)	\$ (9,551)	\$ (5,186)	\$ (16,516)
Denominator				
Weighted average shares outstanding	5,054,375	5,090,988	5,077,668	5,135,166
Less: weighted average shares subject to repurchase used in computing net loss per share attributable to common stockholders, basic and diluted	(100,956)	(41,785)	(56,665)	(2,452)
Weighted average shares outstanding used in computing net loss per share attributable to common stockholders, basic and diluted	4,953,419	5,049,203	5,021,003	5,132,714
Net loss per share attributable to common stockholders – basic and diluted	<u>\$ (2.27)</u>	<u>\$ (1.89)</u>	\$ (1.03)	\$ (3.22)

The following outstanding shares of potentially dilutive securities were excluded from the computation of diluted net loss per share attributable to common stockholders for the period presented because including them would have been anti-dilutive:

	Year Ended December 31, 2017	Year Ended December 31, 2018	Six Months Ended June 30, 2018 (Unaudited)	Six Months Ended June 30, 2019 (Unaudited)
Redeemable convertible preferred stock	2,220,999	7,375,631	2,220,999	7,375,631
Options to purchase common stock	792,290	2,028,274	1,154,988	2,192,230
Unvested restricted stock awards	73,964	14,793	44,379	_
Common stock warrant	68,669	68,669	68,669	68,669
Total	3,155,922	9,487,367	3,489,035	9,636,530

## **Unaudited Pro Forma Net Loss Per Share**

Unaudited pro forma basic and diluted net loss per share were computed to give effect to the automatic one-for-one conversion of all outstanding shares of redeemable convertible preferred stock

into shares of common stock in connection with the closing of the planned IPO, using the as-converted method as though the conversion had occurred as of the beginning of the period presented or the date of issuance, if later.

Unaudited pro forma basic and diluted loss per share is computed as follows (in thousands, except share and per share data):

	Year Ended December 31, 2018	Six Months Ended June 30, 2019
Numerator		
Net loss attributable to common stockholders		
Denominator		
Weighted-average shares used in computing net loss per share, basic and diluted		
Adjust: Conversion of redeemable convertible preferred stock		
Weighted-average shares used in computing pro forma net loss per share, basic and diluted		
Pro forma net loss per share, basic and diluted		

## 15. Derivative Liability

The Company identified an embedded derivative resulting from the change of control provision in the CFFT collaboration agreement. Embedded derivatives that are required to be bifurcated from the underlying host instrument are accounted for and valued as separate financial instruments. At the inception of the derivative in 2017, the Company recognized this derivative as a liability and revenue was reduced by the initial fair value of the derivative liability. The Company remeasures the derivative liability to fair value at each reporting period and records the change in fair value of the derivative liability as other income (expense), net. The Company uses a present value analysis with multiple scenarios, which incorporates assumptions and estimates to value the derivative instrument. The Company assesses these assumptions and estimates on a periodic basis as additional information impacting the assumptions is obtained. Estimates and assumptions impacting the fair value measurement include the change of control payment to CFFT, the probability of a change of control event, the probability of the product achieving development or commercial status at time of change of control and the discount rate. The Company determined the estimated fair value of this liability as of the inception date of the CFFT Agreement and concluded that the amount was immaterial. The Company determined the fair value of this derivative liability was \$0.1 million as of December 31, 2017, December 31, 2018 and June 30, 2019 (unaudited).

## 16. Related Party Transactions

In January 2014, in connection with the performance obligations under the uniQure Agreement, the founders of the Company received equity options to purchase an aggregate of 609,744 of uniQure ordinary shares that vest over the initial three-year term of the agreement and one of the founders of the Company agreed to serve as a director of uniQure. The accounting for this arrangement is discussed in Note 6. See Note 17 for subsequent events related to the Company's agreements with uniQure.

In 2015, the Company signed a collaboration and license agreement with Pfizer and recorded deferred revenue of \$5.0 million related to the upfront payment received from Pfizer under the arrangement. In 2018, Pfizer terminated this agreement for convenience. Upon the termination of the

agreement, the Company recognized revenue on the \$5.0 million upfront payment. As of December 31, 2018 and June 30, 2019 (unaudited), Pfizer owns 1,474,992 shares of the Company's redeemable convertible preferred stock and has a representative director on the Company's board of directors.

The Company's former Chief Operating Officer, who was employed at the Company through November 2017, was the Executive Chairman of Dark Horse Consulting. During the year ended December 31, 2017, the Company paid \$0.2 million to Dark Horse Consulting under a consulting agreement to design and implement pharmaceutical quality manufacturing of and controls for drug products. The consulting agreement terminated in December 2017.

In each of the years ended December 31, 2017 and 2018, the Company paid \$56,500 and \$50,000, respectively, and \$25,000 (unaudited) in each of the six months ended June 30, 2018 and 2019 to the co-founder and Chief Scientific Advisor of the Company for consulting services. In April 2019, the Company entered into two sponsored research agreements with the UC Regents. See Note 17 for further details of this transaction involving the co-founder and Chief Scientific Advisor of the Company.

In 2016, the Company entered into a consulting agreement with one its former directors, who resigned as a director in December 2018, to provide business development strategy services. In connection with this agreement, the former director was granted stock options. In the years ended December 31, 2017 and 2018, the Company recorded \$14,700 and \$219,500 of stock-based compensation expense related to such stock options. In the six months ended June 30, 2018, the Company recorded \$61,400 (unaudited) of stock-based compensation expense related to such stock options.

In 2016, the Chief Executive Officer and several other employees of the Company founded Ignite Immunotherapy ("Ignite"). The Company's Chief Executive Officer also serves as the Chief Executive Officer and Executive Chairman of Ignite. Certain executives of the Company hold ownership interests in Ignite and are members of the board of directors of Ignite. Additionally, Pfizer, who is a related party of the Company holds a significant equity stake in Ignite. There have been no transactions between the Company and Ignite in the years ended December 31, 2017 and 2018.

## 17. Subsequent Events

The Company evaluated subsequent events through August 2, 2019, the date on which the financial statements were available for issuance.

In January 2019, the Company entered into a license agreement with the UC Regents relating to the use of certain patents and intellectual property surrounding its core technologies. The Company paid a non-refundable license fee of \$50,000 to the UC Regents upon execution of the agreement.

In March 2019, the Benitec Agreement was terminated based on mutual agreement between the Company and Benitec.

In April 2019, the Company entered into two sponsored research agreements ("SRAs") with the UC Regents to conduct research in a research facility on the Berkeley campus, under the direction of Professor David Schaffer, the Company's co-founder and Chief Scientific Advisor. The SRAs have a three year term ending in May 2022. Under the SRAs, the Company has an option to license (on a royalty-bearing basis) all intellectual property generated under the SRAs. The total amount the Company is committed to pay to the UC Regents under the SRAs is \$1.5 million, of which \$0.4 million was paid upon the execution of the SRAs. Any patent prosecution costs incurred under the SRAs will

also be borne by the Company. The Company can terminate the SRAs for convenience and without cause with 60 days' notice.

In May 2019, the Company amended the lease agreement executed in October 2018 to add 17,497 square feet to the space being leased in Emeryville, California. The amendment extended the term of the lease to December 31, 2029. The Company does not have to pay rent until December 2019. The annual rent for the additional space is \$1.0 million per annum and escalates at 3% annually. This lease agreement also provides for a tenant improvement allowance of \$1.6 million.

From January 1, 2019 through August 2, 2019, the Company has granted options for the purchase of 1.0 million shares of common stock at an exercise price ranging from \$9.41 to \$10.10 per share. These options vest over a period of one to four years.

## 18. Subsequent Events (unaudited)

In connection with the issuance of the unaudited interim financial statements, the Company has evaluated subsequent events through August 30, 2019, the date the financial statements were available to be issued.

In August 2019, the Company entered into an Amended and Restated Collaboration and License Agreement ("amended uniQure Agreement") with uniQure which amended and restated the uniQure Agreement. Under the amended uniQure Agreement, the Company granted uniQure a license for select AAV capsid variants (Selected Variants) in the central nervous system and the liver (together the uniQure Field). uniQure is solely responsible, at its cost and expense, to develop and commercialize the compounds and products containing the Selected Variants.

Also in August 2019, the Company entered into a separate Collaboration and License Agreement with uniQure ("Second uniQure Agreement"). Under the Second uniQure Agreement, the parties agreed to research and develop new AAV capsid variants that are not Selected Variants (New Variants) in the uniQure Field and that affect certain targets (uniQure Targets). The Company is responsible for the research of the New Variants. The Company granted uniQure a license for a certain number of the New Variants (uniQure New Variants) that affect the uniQure Targets. uniQure is solely responsible, at its cost and expense, to develop and commercialize the compounds and products containing the uniQure New Variants that affect the uniQure Targets.

Under both the Amended and Restated uniQure Agreement and the Second uniQure Agreement, uniQure will be required to pay the Company royalties on worldwide annual net sales of licensed products at a mid-single digit percentage rate, subject to certain specified reductions. uniQure will also be required to pay the Company a portion of the amounts it receives for sublicensing to third parties the Company's intellectual property rights licensed under the agreement at a rate between mid-single digit to mid-twenties percentages.

## **Shares**

## 4D Molecular Therapeutics, Inc.

Common Stock



Goldman Sachs & Co. LLC

Evercore ISI Chardan William Blair

Through and including , 2019 (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.

## PART II INFORMATION NOT REQUIRED IN PROSPECTUS

## Item 13. Other Expenses of Issuance and Distribution.

The following table sets forth the costs and expenses, other than the underwriting discounts and commissions, payable by the registrant in connection with the sale of common stock being registered. All amounts are estimates except for the SEC registration fee, the Financial Industry Regulatory Authority, Inc. (FINRA) filing fee and The Nasdaq Global Market listing fee.

<u>Item</u>	ount paid or to be paid
SEC registration fee	\$ 12,120
FINRA filing fee	*
The Nasdaq Global Market listing fee	*
Printing and engraving expenses	*
Legal fees and expenses	*
Accounting fees and expenses	*
Blue Sky, qualification fees and expenses	*
Transfer Agent fees and expenses	*
Miscellaneous fees and expenses	*
Total	\$ *

<sup>\*</sup> To be completed by amendment.

## Item 14. Indemnification of Directors and Officers.

As permitted by Section 102 of the Delaware General Corporation Law, we have adopted provisions in our amended and restated certificate of incorporation and amended and restated bylaws, to be in effect immediately prior to the consummation of this offering, that will limit or eliminate the personal liability of our directors for a breach of their fiduciary duty of care as a director. The duty of care generally requires that, when acting on behalf of the corporation, directors exercise an informed business judgment based on all material information reasonably available to them. Consequently, a director will not be personally liable to us or our stockholders for monetary damages for breach of fiduciary duty as a director, except for liability for:

- · any breach of the director's duty of loyalty to us or our stockholders;
- · any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- · any act related to unlawful stock repurchases, redemptions or other distributions or payment of dividends; or
- · any transaction from which the director derived an improper personal benefit.

These limitations of liability do not affect the availability of equitable remedies such as injunctive relief or rescission. Our amended and restated certificate of incorporation will also authorize us to indemnify our officers, directors and other agents to the fullest extent permitted under Delaware law.

As permitted by Section 145 of the Delaware General Corporation Law, our amended and restated bylaws will provide that:

• we may indemnify our directors, officers, and employees to the fullest extent permitted by the Delaware General Corporation Law, subject to limited exceptions;

- we may advance expenses to our directors, officers and employees in connection with a legal proceeding to the fullest extent permitted by the Delaware General Corporation Law, subject to limited exceptions; and
- the rights provided in our amended and restated bylaws are not exclusive.

Our amended and restated certificate of incorporation, to be attached as Exhibit 3.2 hereto, and our amended and restated bylaws, to be attached as Exhibit 3.4 hereto, will provide for the indemnification provisions described above and elsewhere herein. We have entered into separate indemnification agreements with our directors and officers which may be broader than the specific indemnification provisions contained in the Delaware General Corporation Law. These indemnification agreements generally require us, among other things, to indemnify our officers and directors against liabilities that may arise by reason of their status or service as directors or officers, other than liabilities arising from willful misconduct. These indemnification agreements also generally require us to advance any expenses incurred by the directors or officers as a result of any proceeding against them as to which they could be indemnified. In addition, we have purchased a policy of directors' and officers' liability insurance that insures our directors and officers against the cost of defense, settlement or payment of a judgment in some circumstances. These indemnification provisions and the indemnification agreements may be sufficiently broad to permit indemnification of our officers and directors for liabilities, including reimbursement of expenses incurred, arising under the Securities Act.

The form of Underwriting Agreement, to be attached as Exhibit 1.1 hereto, provides for indemnification by the underwriters of us and our officers who sign this Registration Statement and directors for specified liabilities, including matters arising under the Securities Act.

## Item 15. Recent Sales of Unregistered Securities.

The following list sets forth information as to all securities we have sold since January 1, 2016, which were not registered under the Securities Act.

- 1. On August 27, 2018 and August 28, 2018, we issued an aggregate of 5,154,632 shares of our Series B redeemable convertible preferred stock at a price per share of \$17.46 for aggregate proceeds to us of \$89,999,874.72.
- 2. On June 30, 2016, we issued a warrant to purchase 45,000 shares of our common stock at a per share exercise price of \$1.14.
- 3. On May 18, 2018, we issued a warrant to purchase 23,669 shares of our common stock at a per share exercise price of \$3.19.
- 4. We granted stock options and stock awards to employees, directors and consultants under our 2015 Equity Incentive Plan, covering 3,239,613 shares of common stock, at a weighted-average exercise price of \$6.32 per share. Of these, options covering an aggregate of 848,397 shares of common stock were cancelled without being exercised.

We claimed exemption from registration under the Securities Act for the sale and issuance of securities in the transactions described in paragraphs (1) through (3) by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering. All of the purchasers of unregistered securities for which we relied on Section 4(a)(2) and/or Regulation D represented that they were accredited investors as defined under the Securities Act. We claimed such exemption on the basis that (a) the purchasers in each case represented that they intended to acquire the securities for investment only and not with a view to the distribution thereof and that they either received adequate information about the registrant or had access, through employment or other relationships, to such information and (b) appropriate legends were affixed to the stock certificates issued in such transactions.

We claimed exemption from registration under the Securities Act for the sales and issuances of securities in the transactions described in paragraph (4) above under Section 4(a)(2) of the Securities Act in that such sales and issuances did not involve a public offering or under Rule 701 promulgated under the Securities Act, in that they were offered and sold either pursuant to written compensatory plans or pursuant to a written contract relating to compensation, as provided by Rule 701.

## Item 16. Exhibits and Financial Statement Schedules.

## (a) Exhibits.

Exhibit <u>Number</u>	Exhibit Description
1.1*	Form of Underwriting Agreement.
3.1	Amended and Restated Certificate of Incorporation, currently in effect.
3.2*	Form of Amended and Restated Certificate of Incorporation, to be in effect immediately prior to the consummation of this offering.
3.3	Bylaws, currently in effect.
3.4*	Form of Amended and Restated Bylaws, to be in effect immediately prior to the consummation of this offering.
4.1	Reference is made to exhibits 3.1 through 3.4.
4.2*	Form of Common Stock Certificate.
4.3	Amended and Restated Investors' Rights Agreement, dated as of August 27, 2018, among the Registrant and the investors party thereto.
5.1*	Opinion of Latham & Watkins LLP.
10.1(a)#	2015 Equity Incentive Plan.
10.1(b)#	Form of Stock Option Agreement under 2015 Equity Incentive Plan.
10.2(a)#*	2019 Incentive Award Plan.
10.2(b)#*	Form of Stock Option Grant Notice and Stock Option Agreement under the 2019 Incentive Award Plan.
10.2(c)#*	Form of Restricted Stock Award Grant Notice and Restricted Stock Award Agreement under the 2019 Incentive Award Plan.
10.2(d)#*	Form of Restricted Stock Unit Award Grant Notice and Restricted Stock Unit Award Agreement under the 2019 Incentive Award Plan.
10.3#*	2019 Employee Stock Purchase Plan.
10.4*	Form of Indemnification Agreement for directors and officers.
10.5*†	Collaboration and License Agreement, dated November 16, 2017, among the Registrant, F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc.
10.6*†	Collaboration and Option Agreement, dated December 22, 2017, between the Registrant and MedImmune, LLC.
10.7*†	Amended and Restated Collaboration and License Agreement, dated August 6, 2019, between the Registrant and uniQure biopharma B.V.
10.8*†	Collaboration and License Agreement, dated August 6, 2019, by and between the Registrant and uniQure biopharma B.V.

Exhibit <u>Number</u>	Exhibit Description
10.9*†	Exclusive License and Bailment Agreement, dated December 13, 2013, between the Registrant and The Regents of the University of California.
10.10*†	Exclusive License and Bailment Agreement, dated December 19, 2013, between the Registrant and The Regents of the University of California.
10.11#*	Offer Letter, dated March 20, 2015 between David Kirn, M.D. and the Registrant.
10.12#*	Employment Agreement, dated January 15, 2019 between Peter Francis, M.D., Ph.D. and the Registrant.
10.13#*	Offer Letter, dated July 31, 2018 between Fred Kamal, Ph.D. and the Registrant.
23.1	Consent of independent registered public accounting firm.
23.2*	Consent of Latham & Watkins LLP (included in Exhibit 5.1).
24.1	Power of Attorney. Reference is made to the signature page to the Registration Statement.
	<u></u>

To be filed by amendment

## Item 17. Undertakings.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the Registrant pursuant to the foregoing provisions, or otherwise, the Registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act, and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the Registrant of expenses incurred or paid by a director, officer, or controlling person of the Registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question of whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes that:

- 1. For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this Registration Statement in reliance upon Rule 430A and contained in a form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this Registration Statement as of the time it was declared effective.
- 2. For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

The undersigned Registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

<sup>†</sup> Certain confidential portions (indicated by brackets and asterisks) have been omitted from this exhibit.

<sup>#</sup> Indicates management contract or compensatory plan.

<sup>(</sup>b) Financial Statement Schedules. Schedules not listed above have been omitted because the information required to be set forth therein is not applicable or is shown in the financial statements or notes thereto.

## **SIGNATURES**

Pursuant to the requirements of the Securities Act of 1933, as amended, the Registrant has duly caused this Registration Statement on Form S-1 to be signed on its behalf by the undersigned, thereunto duly authorized, in Emeryville, California on September 30, 2019.

4D Molecular Therapeutics, Inc.

By: <u>/s/ David Kirn</u>
David Kirn, M.D.
Chief Executive Officer

## **POWER OF ATTORNEY**

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below hereby constitutes and appoints David Kirn, M.D. and August J. Moretti, and each of them acting individually, as his or her true and lawful attorneys-in-fact and agents, each with full power of substitution, for him or her in any and all capacities, to sign any and all amendments to this Registration Statement, including post-effective amendments or any abbreviated registration statement and any amendments thereto filed pursuant to Rule 462(b) increasing the number of securities for which registration is sought, and to file the same, with all exhibits thereto and other documents in connection therewith, with the SEC, granting unto said attorneys-in-fact and agents, with full power of each to act alone, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully for all intents and purposes as he might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or his or their substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act, this Registration Statement has been signed by the following persons in the capacities and on the dates indicated.

Signature	Title	Date
/s/ David Kirn David Kirn, M.D.	Chairman and Chief Executive Officer (Principal Executive Officer)	September 30, 2019
/s/ August J. Moretti August J. Moretti	Chief Financial Officer (Principal Financial and Accounting Officer)	September 30, 2019
/s/ David Schaffer David Schaffer, Ph.D.	Director	September 30, 2019
/s/ William Burkoth William Burkoth	Director	September 30, 2019
/s/ Jacob Chacko Jacob Chacko, M.D.	Director	September 30, 2019
/s/ Charles P. Theuer Charles P. Theuer, M.D., Ph.D.	Director	September 30, 2019
/s/ Tony Yao Tony Yao, M.D., Ph.D.	Director	September 30, 2019

# THIRD AMENDED AND RESTATED CERTIFICATE OF INCORPORATION OF 4D MOLECULAR THERAPEUTICS, INC.

4D Molecular Therapeutics, Inc., a corporation organized and existing under the laws of the State of Delaware, hereby certifies as follows:

- A. The name of the corporation is 4D Molecular Therapeutics, Inc. The corporation was originally incorporated pursuant to the General Corporation Law of the State of Delaware on March 11, 2015 under the same name.
- B. The date of filing of the corporation's original Certificate of Incorporation with the Secretary of State of the State of Delaware was March 11, 2015.
- C. The Third Amended and Restated Certificate of Incorporation of 4D Molecular Therapeutics, Inc. in the form attached hereto as Exhibit A has been duly adopted in accordance with the provisions of Sections 228, 242 and 245 of the General Corporation Law of the State of Delaware by the board of directors and stockholders of the corporation, and prompt written notice was duly given pursuant to Section 228 of the General Corporation Law of the State of Delaware to those stockholders who did not approve the Third Amended and Restated Certificate of Incorporation by written consent.
- D. The Third Amended and Restated Certificate of Incorporation so adopted reads in full as set forth in Exhibit A attached hereto and is incorporated herein by this reference.

IN WITNESS WHEREOF, the corporation has caused the Third Amended and Restated Certificate of Incorporation to be signed by its duly authorized officer.

Dated: August 27, 2018

4D MOLECULAR THERAPEUTICS, INC.

By: /s/ David Kirn
Name: David Kirn

Title: Chief Executive Officer

#### **EXHIBIT A**

## THIRD AMENDED AND RESTATED CERTIFICATE OF INCORPORATION OF 4D MOLECULAR THERAPEUTICS, INC.

#### ARTICLE I

The name of the corporation is 4D Molecular Therapeutics, Inc. (the "Corporation").

## **ARTICLE II**

The name of the Corporation's registered agent in the State of Delaware is The Corporation Trust Company, whose address is 1209 Orange Street, City of Wilmington, County of New Castle, 19801.

## ARTICLE III

The purpose of the Corporation is to engage in any lawful act or activity for which corporations may be organized under the General Corporation Law of the State of Delaware (the "**DGCL**").

## ARTICLE IV

The total number of shares of all classes of stock which the Corporation shall have authority to issue is (i) 50,000,000 shares of Common Stock, \$0.0001 par value per share ("*Common Stock*"), and (ii) 7,375,638 shares of Preferred Stock, \$0.0001 par value per share ("*Preferred Stock*"), (A) 909,312 shares of which shall be designated "*Series A Preferred Stock*," (B) 1,311,687 shares of which shall be designated "*Series A-1 Preferred Stock*" and (C) 5,154,639 shares of which shall be designated "*Series B Preferred Stock*."

The following is a statement of the designations and the powers, preferences and rights, and the qualifications, limitations or restrictions thereof, in respect of each class or series of capital stock of the Corporation:

## A. COMMON STOCK

- 1. <u>General</u>. The voting, dividend and liquidation rights of the holders of Common Stock are subject to and qualified by the powers, preferences and rights of the holders of Preferred Stock set forth herein.
- 2. <u>Voting</u>. On any matter presented to the stockholders of the Corporation for their action or consideration at any meeting of stockholders (or by written consent in lieu of meeting), each holder of outstanding shares of Common Stock shall be entitled, with respect to each outstanding share of Common Stock held by such holder, to cast one vote. There shall be no cumulative voting. The number of authorized shares of Common Stock may be increased or decreased (but not below the number of shares thereof then outstanding) by (in addition to any vote of the holders of one or more series of Preferred Stock that may be required by the terms of the Certificate of Incorporation) the affirmative vote of the holders of shares of capital stock of the Corporation representing a majority of the votes represented by all outstanding shares of capital stock of the Corporation entitled to vote, irrespective of the provisions of Section 242(b)(2) of the DGCL.

## B. PREFERRED STOCK

Unless otherwise indicated, references to "Sections" or "Subsections" in this Part B of this Article IV refer to Sections and Subsections in this Part B of this Article IV.

## 1. Dividends.

The Corporation shall not declare, pay or set aside any dividends on shares of any class or series of capital stock of the Corporation (other than dividends on shares of Common Stock payable in shares of Common Stock ("Common Stock Dividend")) unless (in addition to obtaining any consents required elsewhere in the Certificate of Incorporation) the holders of Preferred Stock then outstanding shall first receive, or simultaneously receive, a dividend on each outstanding share of Preferred Stock in an amount at least equal to (i) in the case of a dividend on Common Stock or any class or series that is convertible into Common Stock, the product of (A) the dividend payable on each share of Common Stock or each share of such class or series that is convertible into Common Stock determined, if applicable, as if all shares of such class or series had been converted into Common Stock and (B) the number of shares of Common Stock issuable upon conversion of a share of Preferred Stock, in each case calculated on the record date for determination of holders entitled to receive such dividend or (ii) in the case of a dividend on any class or series that is not convertible into Common Stock, the amount determined by (A) dividing the amount of the dividend payable on each share of such class or series of capital stock by the original issuance price of such class or series of capital stock (subject to appropriate adjustment for any stock dividend, stock split, combination or other similar recapitalization (collectively, "Recapitalizations") with respect to such class or series) and (B) multiplying such fraction by an amount equal to the applicable Original Issue Price (as defined below); provided, however, that, if the Corporation declares, pays or sets aside, on the same date, a dividend on shares of more than one class or series of capital stock of the Corporation, the dividend payable to the holders of Preferred Stock pursuant to this Section 1 shall be calculated based upon the dividend on the class or series of capital stock that would result in the highest Preferred Stock dividend. "Original Series A Issue Price" means \$7.70, subject to appropriate adjustment for any Recapitalizations with respect to Series A Preferred Stock. "Original Series A-1 Issue Price" means \$8.839, subject to appropriate adjustment for any Recapitalizations with respect to Series A-1 Preferred Stock. "Original Series B Issue Price" means \$17.46, subject to appropriate adjustment for any Recapitalizations with respect to Series B Preferred Stock. The Original Series A Issue Price, Original Series A-1 Issue Price and Original Series B Issue Price are collectively referred to as the "Original Issue Price" herein.

- 2. <u>Liquidation, Dissolution or Winding Up; Certain Mergers, Consolidations and Asset Sales.</u>
  - 2.1 Preferential Payments to Holders of Preferred Stock.

2.1.1 Series B Preferred Stock. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event (as defined in Subsection 2.3.1), subject to the provisions of Subsection 2.3.2(b) in the case of a Deemed Liquidation Event referred to in Subsection 2.3.1(a)(ii) or Subsection 2.3.1(b), the holders of Series B Preferred Stock then outstanding shall be entitled to be paid, out of the Available Assets (as defined below), and prior and in preference to any payment of any Available Assets to the holders of Series A-1 Preferred Stock, Series A Preferred Stock or Common Stock by reason of their ownership thereof, an amount per share equal to the greater of (i) the sum of the Original Series B Issue Price plus all declared but unpaid dividends thereon and (ii) such amount per share as would have been payable had all shares of Series B Preferred Stock been converted into Common Stock pursuant to Section 4 immediately prior to such liquidation, dissolution, winding up of the Corporation or Deemed Liquidation Event (the amount payable pursuant to this sentence, the "Series B Liquidation Amount"). If upon any such liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the Available Assets shall be insufficient to pay the holders of Series B Preferred Stock the full amount to which they shall be entitled under this Subsection 2.1.1, the Available Assets shall be distributed to the holders of Series B Preferred Stock pro rata, on an equal priority, pari passu basis, in proportion to the full preferential amount each such holder is otherwise entitled to receive under this Subsection 2.1.1. "Available Assets" means the funds and assets that may be legally distributed to the stockholders of the Corporation.

2.1.2 <u>Series A-1 Preferred Stock</u>. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event (as defined in <u>Subsection 2.3.1(a)(ii)</u> or <u>Subsection 2.3.1(b)</u>, the holders of shares of Series A-1 Preferred Stock then outstanding shall be entitled to be paid, out of the Available Assets (as defined below), and prior and in preference to any payment of any Available Assets to the holders of Series A Preferred Stock or Common Stock by reason of their ownership thereof, an amount per share equal to the greater of (i) the sum of the Original Series A-1 Issue Price plus all declared but unpaid dividends thereon and (ii) such amount per share as would have been payable had all shares of Series A-1 Preferred Stock been converted into Common Stock pursuant to <u>Section 4</u> immediately prior to such liquidation, dissolution, winding up of the Corporation or Deemed Liquidation Event (the amount payable pursuant to this sentence, the "Series A-1 Liquidation Amount"). If upon any such liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the Available Assets shall be insufficient to pay the holders of Series A-1 Preferred Stock the full amount to which they shall be entitled under this <u>Subsection 2.1.2</u>, the Available Assets shall be distributed to the holders of Series A-1 Preferred Stock pro rata, on an equal priority, pari passu basis, in proportion to the full preferential amount each such holder is otherwise entitled to receive under this Subsection 2.1.2.

2.1.3 <u>Series A Preferred Stock</u>. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event (as defined in <u>Subsection 2.3.1</u>), subject to the provisions of <u>Subsection 2.3.2(b)</u> in the case of a Deemed Liquidation Event referred to in <u>Subsection 2.3.1(a)(ii)</u> or <u>Subsection 2.3.1(b)</u>, the holders of shares of Series A Preferred Stock then outstanding shall be entitled to be paid, out of the remaining Available Assets, and prior and in preference to any payment of any Available

Assets to the holders of Common Stock by reason of their ownership thereof, an amount per share equal to the greater of (i) the sum of the Original Series A Issue Price plus all declared but unpaid dividends thereon and (ii) such amount per share as would have been payable had all shares of Series A Preferred Stock been converted into Common Stock pursuant to Section 4 immediately prior to such liquidation, dissolution, winding up of the Corporation or Deemed Liquidation Event (the amount payable pursuant to this sentence, the "Series A Liquidation Amount"). If upon any such liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the remaining Available Assets shall be insufficient to pay the holders of Series A Preferred Stock the full amount to which they shall be entitled under this Subsection 2.1.3, such remaining Available Assets shall be distributed to the holders of Series A Preferred Stock pro rata, on an equal priority, pari passu basis, in proportion to the full preferential amount each such holder is otherwise entitled to receive under this Subsection 2.1.3.

## 2.1.4 Calculation of Liquidation Amounts.

- (a) For purposes of calculating the Series B Liquidation Amount, Series A-1 Liquidation Amount and the Series A Liquidation Amount, it shall be assumed that all shares of each series of Preferred Stock that would receive a greater per share liquidation payment if converted into Common Stock than if remaining as Preferred Stock shall have been converted into Common Stock.
- (b) Subject to <u>Subsection 2.5</u>, neither the Series B Liquidation Amount nor the Series A-1 Liquidation Amount shall be abrogated or diminished in the event part of the consideration paid in a Deemed Liquidation Event is subject to an escrow holdback or satisfaction of milestones except to the extent that holders of shares of Series B Preferred Stock and Series A-1 Preferred Stock have received an amount per share in excess of the sum of the applicable Original Issue Price plus all declared but unpaid dividends thereon.
- 2.2 <u>Payments to Holders of Common Stock</u>. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, after payment of the full amount required by <u>Subsection 2.1</u>, the remaining Available Assets shall be distributed among the holders of shares of Common Stock pro rata according to the number of shares of Common Stock held by each such holder.

## 2.3 Deemed Liquidation Events.

2.3.1 <u>Definition</u>. Each of the following events shall be considered a "*Deemed Liquidation Event*" unless the holders of (i) a majority of the then outstanding shares of Preferred Stock, voting together as a single class on an as-converted to Common Stock basis, and (ii) a majority of the then outstanding shares of Series B Preferred Stock, elect otherwise by written notice sent to the Corporation at least 10 days prior to the effective date of any such event:

- (a) a merger or consolidation in which
  - (i) the Corporation is a constituent party or

(ii) a subsidiary of the Corporation is a constituent party and the Corporation issues shares of its capital stock pursuant to such merger or consolidation,

except any such merger or consolidation (A) effected exclusively for the purpose of changing the Corporation's domicile or (B) involving the Corporation or a subsidiary of the Corporation in which the shares of capital stock of the Corporation outstanding immediately prior to such merger or consolidation continue to represent, or are converted into or exchanged for shares of capital stock that represent, immediately following such merger or consolidation, at least a majority, by voting power, of the capital stock (to be held in substantially the same proportions and with substantially the same rights, preferences and powers) of (1) the surviving or resulting corporation or (2) if the surviving or resulting corporation is a wholly owned subsidiary of another corporation immediately following such merger or consolidation, the parent corporation of such surviving or resulting corporation; or

(b) the sale, lease, transfer, exclusive license or other disposition, in a single transaction or series of related transactions, by the Corporation or any subsidiary of the Corporation of all or substantially all of the assets or intellectual property of the Corporation and its subsidiaries taken as a whole, or the sale or disposition (whether by merger, consolidation or otherwise) of one or more subsidiaries of the Corporation if substantially all of the assets of the Corporation and its subsidiaries taken as a whole are held by such subsidiary or subsidiaries, except where such sale, lease, transfer, exclusive license or other disposition is to a wholly owned subsidiary of the Corporation.

## 2.3.2 Effecting a Deemed Liquidation Event.

(a) The Corporation shall not have the power to effect a Deemed Liquidation Event referred to in <u>Subsection 2.3.l(a)(i)</u> unless the agreement or plan of merger or consolidation for such transaction provides that the consideration payable to the stockholders of the Corporation shall be allocated among the holders of capital stock of the Corporation in accordance with <u>Subsection 2.1</u> and <u>Subsection 2.2</u>.

(b) In the event of a Deemed Liquidation Event referred to in Subsection 2.3.1(a)(ii) or Subsection 2.3.1(b), if the Corporation does not effect a dissolution of the Corporation under the DGCL within 90 days after such Deemed Liquidation Event (and a subsequent distribution of Available Assets pursuant to Subsection 2.1), then (i) the Corporation shall send a written notice to each holder of record of each series of Preferred Stock no later than the 90th day after such Deemed Liquidation Event advising such holders of the right (and the requirements to be met to secure such right) pursuant to the terms of the following clause (ii) to require the redemption of the shares of such series of Preferred Stock and (ii) if the holders of a majority of the then outstanding shares of such series of Preferred Stock so request in a written instrument delivered to the Corporation no later than 120 days after such Deemed Liquidation Event, the Corporation shall use the consideration received by the Corporation for such Deemed Liquidation Event (net of any retained liabilities associated with the assets sold or technology licensed, as determined in good faith by the board of directors of the Corporation (the "Board")), together with any other assets of the Corporation available for distribution to its

stockholders, all to the extent permitted by Delaware law governing distributions to stockholders (collectively, the "Available Proceeds"), on the 150th day after such Deemed Liquidation Event (the "Redemption Date"), to redeem all outstanding shares of such series of Preferred Stock at a price per share equal to the Series B Liquidation Amount, Series A-1 Liquidation Amount and the Series A Liquidation Amount, as the case may be. Notwithstanding the foregoing, in the event of a redemption pursuant to the preceding sentence, if the Available Proceeds are not sufficient to redeem all outstanding shares of Preferred Stock which have requested such redemption, the Corporation shall ratably redeem each holder's shares of Series B Preferred Stock to the fullest extent of such Available Proceeds; shall thereafter ratably redeem the Series A-1 Preferred Stock to the fullest extent of such Available Proceeds and shall thereafter ratably redeem the Series A Preferred Stock to the fullest extent of any remaining Available Proceeds; and shall thereafter ratably redeem the remaining shares as soon as it may lawfully do so under Delaware law governing distributions to stockholders. Prior to the distribution or redemption provided for in this <u>Subsection 2.3.2(b)</u>, the Corporation shall not expend or dissipate the consideration received for such Deemed Liquidation Event, except to discharge expenses incurred in connection with such Deemed Liquidation Event or in the ordinary course of business. At least 15, but not more than 40, days prior to the Redemption Date, the Corporation shall send a written notice (the "Redemption Notice") to each holder of record of Preferred Stock to be redeemed. The Redemption Notice shall state (i) the number of shares of Preferred Stock held by such holder that the Corporation shall redeem on the Redemption Date specified in the Redemption Notice, (ii) the Redemption Date, (iii) the relevant liquidation amount, and (iv) that such holder is to surrender to the Corporation, in the manner and at the place designated, his, her or its certificate or certificates representing the shares of Preferred Stock to be redeemed. On or before the Redemption Date, each holder of shares of Preferred Stock to be redeemed on the Redemption Date, unless such holder has exercised his, her or its right to convert such shares as provided in Section 4, shall surrender the certificate or certificates for such shares (or, if such holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate) to the Corporation, in the manner and at the place designated in the Redemption Notice, and thereupon the relevant liquidation amount for such shares shall be payable to the order of the person or entity whose name appears on such certificate or certificates as the owner thereof and each surrendered certificate shall be cancelled. If less than all of the shares of Preferred Stock represented by a certificate are redeemed, a new certificate representing the unredeemed shares of Preferred Stock shall promptly be issued to such holder. No transfers of Preferred Stock shall be permitted during the five-day period prior to and including the Redemption Date, and the Corporation shall not recognize any such prohibited transfer on its books and records. If (i) the Redemption Notice shall have been duly given and (ii) on the Redemption Date, the relevant liquidation amount payable upon redemption of the shares of Preferred Stock to be redeemed on the Redemption Date is paid or tendered for payment or deposited with an independent payment agent so as to be available therefor in a timely manner, then, notwithstanding that the certificates evidencing any of the shares of Preferred Stock so called for redemption shall not have been surrendered, after the Redemption Date (A) dividends with respect to such shares of Preferred Stock shall cease to accrue and (B) all rights with respect to such shares (other than the right of the holders of such shares to receive the relevant liquidation amount without interest upon surrender of their certificate or certificates therefor)

shall forthwith terminate, and such shares shall not thereafter be transferred on the books of the Corporation or be deemed to be outstanding for any purpose whatsoever. Any funds deposited with an independent payment agent as described in the preceding sentence ("Deposited Funds") for the redemption of shares of Preferred Stock thereafter converted into shares of Common Stock prior to the Redemption Date shall be returned to the Corporation forthwith upon such conversion, and the balance of any Deposited Funds that remain unclaimed at the end of one year from the Redemption Date shall be released or repaid to the Corporation, after which time the holders of shares of Preferred Stock called for redemption who have not claimed such funds shall be entitled to receive payment of the relevant liquidation amount only from the Corporation.

2.4 <u>Amount Deemed Paid or Distributed</u>. The amount deemed paid or distributed to the holders of capital stock of the Corporation upon any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event (or, if applicable, redemption pursuant to <u>Subsection 2.3.2(b)</u>) shall be the cash or the value of the property, rights or securities paid or distributed to such holders by the Corporation or the acquiring person, firm or other entity. The value of such property, rights or securities shall be determined in good faith by the Board, including the approval of at least one Preferred Director.

2.5 <u>Allocation of Escrow and Contingent Consideration</u>. In the event of a Deemed Liquidation Event pursuant to <u>Subsection 2.3.1</u> (a)(i), if any portion of the consideration payable to the stockholders of the Corporation is payable only upon satisfaction of contingencies (the "Additional Consideration"), the agreement or plan of merger or consolidation for such transaction shall provide that (a) the portion of such consideration that is not Additional Consideration (such portion, the "Initial Consideration") shall be allocated among the holders of capital stock of the Corporation in accordance with <u>Subsections 2.1</u> and <u>2.2</u> as if the Initial Consideration were the only consideration payable in connection with such Deemed Liquidation Event; and (b) any Additional Consideration which becomes payable to the stockholders of the Corporation upon satisfaction of such contingencies shall be allocated among the holders of capital stock of the Corporation in accordance with <u>Subsections 2.1</u> and <u>2.2</u> after taking into account the previous payment of the Initial Consideration as part of the same transaction. For the purposes of this <u>Subsection 2.5</u>, consideration placed into escrow or retained as holdback to be available for satisfaction of indemnification or similar obligations in connection with such Deemed Liquidation Event shall be deemed to be Additional Consideration.

## 3. Voting.

3.1 General. On any matter presented to the stockholders of the Corporation for their action or consideration at any meeting of stockholders (or by written consent in lieu of meeting), each holder of outstanding shares of Preferred Stock shall be entitled, with respect to the outstanding shares of Preferred Stock held by such holder, to cast the number of votes equal to the number of whole shares of Common Stock into which the shares of Preferred Stock held by such holder are convertible as of the record date for determining stockholders entitled to vote on such matter or, if no such record date is established, the date such vote is taken or any written consent of stockholders is solicited. Except as provided by law or by the other provisions of the Certificate of Incorporation, the holders of Preferred Stock shall vote together with the holders of Common Stock as a single class. Subject to Subsections 3.4(b),

3.5(c) and 3.6(b), the number of authorized shares of Preferred Stock (or any series thereof) may be increased or decreased (but not below the number of shares thereof then outstanding), and the rights, preferences and privileges of any newly created series of Preferred Stock may be approved, by the affirmative vote of the holders of shares of capital stock of the Corporation representing a majority of the votes represented by all outstanding shares of capital stock of the Corporation entitled to vote, irrespective of the provisions of Section 242(b)(2) of the DGCL.

## 3.2 Election of Directors; Board Size.

3.2.1 So long as at least 916.380 shares of Series B Preferred Stock remain issued and outstanding, the holders of record of a majority of the then outstanding shares of Series B Preferred Stock, exclusively and as a separate class, shall be entitled to elect one (1) director of the Corporation (the "Series B Director"). The holders of record of shares of Series A-1 Preferred Stock, exclusively and as a separate class, shall be entitled to elect one (1) director of the Corporation (the "Series A-1 Director" and, together with the Series B Director, the "Preferred Directors"). The holders of record of shares of Common Stock, exclusively and as a separate class, shall be entitled to elect two (2) directors of the Corporation (the "Common Directors"), and the holders of record of shares of Common Stock and of any other class or series of voting stock (including Preferred Stock), exclusively and voting together as a single class on an as-converted to Common Stock basis, shall be entitled to elect the balance of the total number of directors of the Corporation (the "Other Directors"). With respect to the election of any director or directors as provided in the preceding sentence, that candidate or those candidates, as applicable, shall be elected who either (1) in the case of any such vote conducted at a meeting, receive(s) the highest number of affirmative votes (on an as-converted to Common Stock basis) of the outstanding shares of the class (or classes) or series of capital stock entitled to elect such director or directors (the "Specified Stock"), up to the number of directors to be elected by such Specified Stock, or (2) in the case of any such vote taken by written consent without a meeting, is or are elected by the written consent of the holders of a majority of the voting power (on an as-converted to Common Stock basis) of the outstanding shares of such Specified Stock. Subject to Section 141(k) of the DGCL, any director elected by the holders of any Specified Stock, or by the Remaining Directors (as defined below) as provided below in this Subsection 3.2.1, may be removed without cause by, and only by, the affirmative vote of shares representing a majority of the voting power (on an as-converted to Common Stock basis) of the outstanding shares of such Specified Stock entitled to vote, given either at a special meeting of such stockholders duly called for that purpose or pursuant to a written consent of stockholders. If the holders of Specified Stock fail to elect a sufficient number of directors to fill all directorships for which they are entitled to elect directors, voting exclusively and as a separate class, pursuant to the first sentence of this Subsection 3.2.1, then any directorship not so filled shall remain vacant until such time as the holders of such Specified Stock elect a person to fill such directorship by vote or written consent in lieu of a meeting; and no such directorship may be filled by stockholders of the Corporation other than by the holders of such Specified Stock, voting exclusively and as a separate class. At any meeting held for the purpose of electing a director, the presence in person or by proxy of (a) the holders of a majority of the shares of Series B Preferred Stock then outstanding, Series A-1 Preferred Stock then outstanding or the holders of a majority of the shares of Common Stock then outstanding shall constitute a quorum for the election of the Series B Director, Series A-1 Director or the Common Directors, respectively, and (b) the holders of a majority of the voting power (on an as converted to

Common Stock basis) of the then outstanding shares of voting stock shall constitute a quorum for the election of the Other Directors. Except as otherwise provided in this <u>Subsection 3.2.1</u>, a vacancy in any directorship elected by the holders of any Specified Stock shall be filled only by either (x) a majority of the remaining director or directors, if any, in office that were so elected by the holders of such Specified Stock (the "*Remaining Directors*"), by the affirmative vote of a majority of such Remaining Directors (or by the sole remaining director elected by the holders of such Specified Stock if there is but one), or (y) the required vote or written consent of holders of the shares of such Specified Stock that are entitled to elect such director.

- 3.2.2 Elections of directors need not be by written ballot unless the Bylaws of the Corporation shall so provide.
- 3.2.3 Subject to any additional vote required by the Certificate of Incorporation, the number of directors of the Corporation shall be determined in the manner set forth in the Bylaws of the Corporation.
- 3.3 <u>Preferred Stock Protective Provisions</u>. At any time shares of Preferred Stock are outstanding, the Corporation shall not (by amendment, merger, consolidation or otherwise), directly or indirectly, do any of the following without (in addition to any other vote required by law or the Certificate of Incorporation) the written consent or affirmative vote, given in writing or by vote at a meeting, of the holders of a majority of Preferred Stock then outstanding, consenting or voting (as the case may be) as a single class on an as-converted to Common Stock basis, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect.
- (a) amend, alter or repeal any provision of the Certificate of Incorporation or Bylaws in a manner that would adversely alter the rights, preferences, privileges or powers of, or restrictions provided for the benefit of the Preferred Stock;
  - (b) liquidate, dissolve or wind up the business and affairs of the Corporation or effect a Deemed Liquidation Event;
  - (c) increase or decrease the authorized number of directors of the Corporation;
- (d) purchase or redeem, or pay or declare any dividend or make any distribution on, any shares of capital stock of the Corporation, except (i) dividends or other distributions payable on shares of Common Stock solely in the form of additional shares of Common Stock, or (ii) the repurchase of shares held by directors, officers, employees, consultants, independent contractors, advisors or other persons performing services for the Corporation (or a subsidiary) that are subject to agreements under which the Corporation has the option to repurchase such shares (A) at cost, upon the occurrence of certain events, such as termination of employment or services, or (B) at any price pursuant to the Corporation's exercise of a right of first refusal to repurchase such shares if approved by the Board, including the approval of at least one Preferred Director;

(e) (i) reclassify, alter or amend any existing security of the Corporation that is *pari passu* with any series of Preferred Stock in respect of the distribution of assets on the liquidation, dissolution or winding up of the Corporation, the payment of dividends or rights of redemption, if such reclassification, alteration or amendment would render such other security senior to any series of Preferred Stock in respect of any such right, preference, or privilege or (ii) reclassify, alter or amend any existing security of the Corporation that is junior to any series of Preferred Stock in respect of the distribution of assets on the liquidation, dissolution or winding up of the Corporation, the payment of dividends or rights of redemption, if such reclassification, alteration or amendment would render such other security senior to or *pari passu* with any series of Preferred Stock in respect of any such right, preference or privilege;

(f) create or hold capital stock in any subsidiary of the Corporation that is not a wholly owned subsidiary of the Corporation or dispose of any equity interest of any subsidiary of the Corporation or all or substantially all of the assets of any subsidiary of the Corporation;

(g) create or authorize any debt security or guarantee (whether or not convertible into shares of capital stock of the Corporation); <u>provided</u> that (i) the Corporation may incur aggregate non-convertible indebtedness of up to \$2,500,000 to banks and other institutional or venture capital investors of national reputation; and (ii) without duplication of clause (i), the Corporation may incur aggregate indebtedness of up to \$250,000 in connection with the financing of equipment (including leases required to be accounted for as capital leases under United States generally accepted accounting principles);

(h) create or authorize the creation of or issue of any equity security (including, without limitation, (i) any other security convertible into or exercisable for any such equity security or (ii) any unit of debt and equity securities) having rights, preferences or privileges senior to or on parity with any series of Preferred Stock, or increase the authorized number of shares of Preferred Stock; or

(i) create any equity incentive plan or increase the number of securities authorized for issuance under any equity incentive plan.

3.4 <u>Series B Preferred Stock Protective Provisions</u>. At any time shares of Series B Preferred Stock are outstanding, the Corporation shall not (by amendment, merger, consolidation or otherwise), directly or indirectly, do any of the following without (in addition to any other vote required by law or the Certificate of Incorporation) the written consent or affirmative vote, given in writing or by vote at a meeting, of the holders of a majority of Series B Preferred Stock then outstanding, consenting or voting (as the case may be) as a single class on an as-converted to Common Stock basis, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect.

(a) amend, alter or repeal any provision of the Certificate of Incorporation or the Bylaws of the Corporation in a manner that adversely alter the rights the rights, preferences, privileges or powers of, or restrictions provided for the benefit of the holders of Series B Preferred Stock; or

- (b) increase or decrease the authorized number of shares of Series B Preferred Stock.
- 3.5 <u>Series A-1 Preferred Stock Protective Provisions</u>. At any time shares of Series A-1 Preferred Stock are outstanding, the Corporation shall not (by amendment, merger, consolidation or otherwise), directly or indirectly, do any of the following without (in addition to any other vote required by law or the Certificate of Incorporation) the written consent or affirmative vote, given in writing or by vote at a meeting, of the holders of a majority of Series A-1 Preferred Stock then outstanding, consenting or voting (as the case may be) as a single class on an as-converted to Common Stock basis, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect.
- (a) amend, alter or repeal any provision of the Certificate of Incorporation or Bylaws in a manner that would adversely alter the rights, preferences, privileges or powers of, or restrictions provided for the benefit of, the holders of Series A-1 Preferred Stock;
  - (b) decrease the authorized number of directors of the Corporation to fewer than six (6) directors; or
  - (c) increase or decrease the authorized number of shares of Series A-1 Preferred Stock.
- 3.6 <u>Series A Preferred Stock Protective Provisions</u>. At any time shares of Series A Preferred Stock are outstanding, the Corporation shall not (by amendment, merger, consolidation or otherwise), directly or indirectly, do any of the following without (in addition to any other vote required by law or the Certificate of Incorporation) the written consent or affirmative vote, given in writing or by vote at a meeting, of the holders of a majority of Series A Preferred Stock then outstanding, consenting or voting (as the case may be) as a single class on an as-converted to Common Stock basis, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect.
- (a) amend, alter or repeal any provision of the Certificate of Incorporation or the Bylaws of the Corporation in a manner that adversely affects the powers, preferences or rights of Series A Preferred Stock; or
  - (b) increase the authorized number of shares of Series A Preferred Stock.
  - 4. Optional Conversion. The holders of shares of Preferred Stock shall have conversion rights as follows (the "Conversion Rights").

## 4.1 Right to Convert.

4.1.1 Conversion Ratio. Each share of Preferred Stock shall be convertible, at the option of the holder thereof, at any time and from time to time (subject to Subsection 4.1.2), and without the payment of additional consideration by the holder thereof, into such number of fully paid and nonassessable shares of Common Stock as is determined by dividing the applicable Original Issue Price by the applicable Conversion Price (determined as described below) in effect on the date the certificate for such share is surrendered for conversion. The "Conversion Price" shall initially be, with respect to Series A Preferred Stock, the Original Series A Issue Price per share of Series A Preferred Stock, with respect to the Series B-referred Stock, the Original Series B Issue Price per share of Series B-referred Stock, and with respect to the Series B-referred Stock, the Original Series B-referred Stock. Such initial Conversion Price, and the rate at which shares of Preferred Stock may be converted into shares of Common Stock, shall be subject to adjustment as provided below. Following each adjustment of the Conversion Price of any series of Preferred Stock, such adjusted Conversion Price shall remain in effect until a further adjustment of such Conversion Price hereunder.

4.1.2 <u>Termination of Conversion Rights</u>. In the event of a liquidation, dissolution or winding up of the Corporation or a Deemed Liquidation Event, the Conversion Rights shall terminate at the close of business on the last full day preceding the date fixed for the payment of any such amounts distributable as a result of such event to the holders of Preferred Stock.

4.2 <u>Fractional Shares</u>. No fractional shares of Common Stock shall be issued upon conversion of Preferred Stock (including any conversion pursuant to <u>Section 4</u> or <u>Section 5</u>). In lieu of any fractional shares to which the holder would otherwise be entitled, the Corporation shall pay cash equal to such fraction multiplied by the fair market value of a share of Common Stock on the date of conversion as determined in good faith by the Board, including the approval of at least one Preferred Director. Whether or not fractional shares would be issuable upon such conversion shall be determined on the basis of the total number of shares of Preferred Stock the holder is at the time converting into Common Stock and the aggregate number of shares of Common Stock issuable upon such conversion.

## 4.3 Mechanics of Conversion.

4.3.1 Notice of Conversion. In order for a holder of Preferred Stock to voluntarily convert shares of Preferred Stock into shares of Common Stock, such holder shall surrender the certificate or certificates for such shares of Preferred Stock (or, if such holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate), at the office of the transfer agent for Preferred Stock (or at the principal office of the Corporation if the Corporation serves as its own transfer agent), together with written notice that such holder elects to convert all or any number of the shares of Preferred Stock represented by such certificates and, if applicable, any event on which such conversion is contingent (the "Conversion Notice"). The Conversion Notice shall state such holder's name or the names of the nominees in which such holder wishes the certificate or certificates for shares of Common Stock to be issued. If required by the Corporation, certificates surrendered for conversion shall be endorsed or accompanied by a written instrument or instruments of transfer, in form satisfactory to the Corporation, duly executed by the registered holder or his, her or its attorney duly authorized in writing. The close of business on the date of receipt by the transfer agent (or by the Corporation if the Corporation serves as its own transfer

agent) of such certificates (or lost certificate affidavit and agreement) and the Conversion Notice shall be the time of conversion (the "Conversion Time"), and the shares of Common Stock issuable upon conversion of the shares represented by such certificate or certificates shall be deemed to be outstanding of record as of such date. The Corporation shall, as soon as practicable after the Conversion Time, (i) issue and deliver to such holder of Preferred Stock, or to his, her or its nominees, a certificate or certificates for the number of full shares of Common Stock issuable upon such conversion in accordance with the provisions hereof and a new certificate for the number of shares, if any, of Preferred Stock represented by the surrendered certificate or certificates that were not converted into Common Stock, (ii) pay in cash such amount as provided in Subsection 4.2 in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion, and (iii) pay all declared but unpaid dividends on the shares of Preferred Stock converted.

4.3.2 <u>Reservation of Shares</u>. The Corporation shall at all times when Preferred Stock shall be outstanding reserve and keep available out of its authorized but unissued capital stock, for the purpose of effecting the conversion of Preferred Stock (including any conversion pursuant to <u>Section 4</u> or <u>Section 5</u>), such number of its duly authorized shares of Common Stock as shall from time to time be sufficient to effect the conversion of all then outstanding shares of Preferred Stock; and if at any time the number of authorized but unissued shares of Common Stock shall not be sufficient to effect the conversion of all then outstanding shares of Preferred Stock, the Corporation shall take such corporate action as may be necessary to increase its authorized but unissued shares of Common Stock to such number of shares as shall be sufficient for such purposes. Before taking any action that would cause an adjustment reducing any Conversion Price below the then par value of the shares of Common Stock issuable upon conversion of Preferred Stock, the Corporation shall take any corporate action which may, in the opinion of its counsel, be necessary in order that the Corporation may validly and legally issue fully paid and nonassessable shares of Common Stock at such adjusted Conversion Price.

4.3.3 Effect of Conversion. All shares of Preferred Stock which shall have been surrendered for conversion as provided in this Section 4 shall no longer be deemed to be outstanding and all rights with respect to such shares shall immediately cease and terminate at the Conversion Time, except only the right of the holders thereof to receive the items provided for in the last sentence of Subsection 4.3.1. Any shares of Preferred Stock so converted shall be retired and cancelled and may not be reissued as shares of such series, and the Corporation may thereafter take such appropriate action (without the need for stockholder action) as may be necessary to reduce the authorized number of shares of Preferred Stock accordingly.

4.3.4 <u>No Further Adjustment</u>. Upon any such conversion, no adjustment to the Conversion Price shall be made for any declared but unpaid dividends on the relevant series of Preferred Stock surrendered for conversion or on Common Stock delivered upon conversion.

4.3.5 <u>Taxes</u>. The Corporation shall pay any and all issue and other similar taxes that may be payable in respect of any issuance or delivery of shares of Common Stock upon conversion of shares of Preferred Stock pursuant to this <u>Section 4</u>. The Corporation shall not, however, be required to pay any tax which may be payable in respect of any transfer involved in the issuance and delivery of shares of Common Stock in a name other than that in which the shares of Preferred Stock so converted were registered, and no such issuance or delivery shall be made unless and until the person or entity requesting such issuance has paid to the Corporation the amount of any such tax or has established, to the satisfaction of the Corporation, that such tax has been paid.

#### 4.4 Adjustments to Conversion Price for Dilutive Issuances.

# 4.4.1 Special Definitions.

(a) "Additional Shares of Common Stock" means all shares of Common Stock issued (or, pursuant to Subsection 4.4.3 below, deemed to be issued) by the Corporation after the Original Series B Issue Date, other than (1) the following shares of Common Stock and (2) shares of Common Stock deemed to be issued pursuant to the following Options and Convertible Securities (clauses (1) and (2), collectively, "Exempted Securities").

- shares of Common Stock, Options or Convertible Securities issued as a dividend or distribution on Preferred Stock;
- (ii) shares of Common Stock, Options or Convertible Securities issued by reason of a dividend, stock split, split-up or other distribution on shares of Common Stock that is covered by <u>Subsection 4.5</u>, <u>Subsection 4.6</u>, <u>Subsection 4.7</u> or <u>Subsection 4.8</u>;
- (iii) shares of Common Stock or Options issued to employees or directors of, or consultants or advisors to, the Corporation or any of its subsidiaries pursuant to a plan, agreement or arrangement approved by the Board, including the approval of at least one Preferred Director;
- (iv) shares of Common Stock or Convertible Securities actually issued upon the exercise of Options or shares of Common Stock actually issued upon the conversion or exchange of Convertible Securities, in each case provided such issuance is pursuant to the terms of such Option or Convertible Security;
- (v) shares of Common Stock, Options or Convertible Securities issued to banks, equipment lessors or other financial institutions, or to real property lessors, pursuant to a debt financing, equipment leasing or real property leasing transaction approved by the Board, including the approval of at least one Preferred Director;

- (vi) shares of Common Stock, Options or Convertible Securities issued to suppliers or third party service
  providers in connection with the provision of goods or services pursuant to transactions approved by the
  Board, including the approval of at least one Preferred Director;
- (vii) shares of Common Stock, Options or Convertible Securities issued in connection with joint venture agreements or the Corporation's acquisition of other corporations or entities by merger, purchase of substantially all of the assets or other reorganization pursuant to transactions approved by the Board, including the approval of at least one Preferred Director; or
- (viii) shares of Common Stock, Options or Convertible Securities issued in connection with sponsored research, collaboration, technology license, development, OEM, marketing or other similar agreements or strategic partnerships approved by the Board, including the approval of at least one Preferred Director.
- (b) "Convertible Securities" means any evidences of indebtedness, shares or other securities directly or indirectly convertible into or exchangeable for Common Stock, but excluding Options.
  - (c) "Options" means rights, options or warrants to subscribe for, purchase or otherwise acquire Common Stock or

Convertible Securities.

(d) "Original Series B Issue Date" means the date on which the first share of Series B Preferred Stock was issued by

the Corporation.

4.4.2 <u>No Adjustment of Conversion Price</u>. Notwithstanding anything herein to the contrary, no adjustment of the Conversion Price with respect to any series of Preferred Stock shall be made as the result of the issuance or deemed issuance of Additional Shares of Common Stock if the Corporation receives written notice from the holders of a majority of the then outstanding shares of Preferred Stock, with all series voting together as a single class, agreeing that no such adjustment shall be made as the result of the issuance or deemed issuance of such Additional Shares of Common Stock; <u>provided</u>, <u>however</u>, that the waiver of any adjustment of the Conversion Price applicable to the Series B Preferred Stock shall require the consent of the holders of a majority of the shares of Series B Preferred Stock then outstanding.

#### 4.4.3 Deemed Issuance of Additional Shares of Common Stock.

(a) If the Corporation at any time or from time to time after the Original Series B Issue Date issues any Options or Convertible Securities (excluding Options or Convertible Securities which are themselves Exempted Securities) or fixes a record date for the determination of holders of any class of securities entitled to receive any such Options or Convertible Securities, then the maximum number of shares of Common Stock (as set forth in the instrument relating thereto, assuming the satisfaction of any conditions to exercisability, convertibility or exchangeability but without regard to any provision contained therein for a subsequent adjustment of such number) issuable upon the exercise of such Options or, in the case of Convertible Securities and Options therefor, upon the conversion or exchange of such Convertible Securities shall be deemed to be Additional Shares of Common Stock issued as of the time of such issuance or, in case such a record date shall have been fixed, as of the close of business on such record date.

(b) If the terms of any Option or Convertible Security, the issuance of which resulted in an adjustment to any Conversion Price pursuant to the terms of <u>Subsection 4.4.4</u>, are revised as a result of an amendment to such terms or any other adjustment pursuant to the provisions of such Option or Convertible Security (but excluding automatic adjustments to such terms pursuant to anti-dilution or similar provisions of such Option or Convertible Security) to provide for either (i) any increase or decrease in the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any such Option or Convertible Security or (ii) any increase or decrease in the consideration payable to the Corporation upon such exercise, conversion and/or exchange, then, effective upon such increase or decrease becoming effective, such Conversion Price computed upon the original date of issuance of such Option or Convertible Security (or upon the occurrence of a record date with respect thereto) shall be readjusted to the Conversion Price that would have been obtained had such revised terms been in effect upon the original date of issuance of such Option or Convertible Security (or upon the occurrence of a record date with respect thereto). Notwithstanding the foregoing, no readjustment pursuant to this clause (b) shall have the effect of increasing the relevant Conversion Price to an amount which exceeds the lower of (1) the relevant Conversion Price in effect immediately prior to the original adjustment made as a result of the issuance of such Option or Convertible Security (or the occurrence of a record date with respect thereto) or (2) the Conversion Price that would have resulted from any issuances of Additional Shares of Common Stock (other than deemed issuances of Additional Shares of Common Stock as a result of the issuance of such Option or Convertible Security) between the original adjustment date.

(c) If the terms of any Option or Convertible Security (excluding Options or Convertible Securities which are themselves Exempted Securities), the issuance of which did not result in an adjustment to any Conversion Price pursuant to the terms of Subsection 4.4.4 (either because the consideration per share (determined pursuant to Subsection 4.4.5) of the Additional Shares of Common Stock subject thereto was equal to or greater than such Conversion Price then in effect or because such Option or Convertible Security was issued before the Original Series B Issue Date), are revised after the Original Series B Issue Date as a result of an amendment to such terms or any other adjustment pursuant to the provisions of such Option or Convertible Security (but excluding automatic adjustments to such terms pursuant to anti-dilution or similar provisions of such Option or Convertible Security) to provide for either (i) any increase in the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any such Option or Convertible Security or (ii) any decrease in the consideration payable to the Corporation upon such exercise, conversion and/or exchange, then such Option or Convertible Security, as so amended or adjusted, and the Additional Shares of Common Stock subject thereto (determined in the manner provided in Subsection 4.4.3(a)), shall be deemed to have been issued effective upon such increase or decrease becoming effective.

(d) Upon the expiration or termination of any unexercised Option (or portion thereof) or unconverted or unexchanged Convertible Security (or portion thereof) that resulted (either upon its original issuance or upon a revision of its terms) in an adjustment to any Conversion Price pursuant to the terms of <u>Subsection 4.4.4</u>, such Conversion Price shall be readjusted to the Conversion Price that would have been obtained had such unexercised Option (or portion thereof) or unconverted or unexchanged Convertible Security (or portion thereof) never been issued.

(e) If the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any Option or Convertible Security, or the consideration payable to the Corporation upon such exercise, conversion and/or exchange, (i) is calculable at the time such Option or Convertible Security is issued or amended but is subject to adjustment based upon subsequent events, any adjustment to any Conversion Price provided for in this Subsection 4.4.3 shall be effected at the time of such issuance or amendment based on such number of shares or amount of consideration without regard to any provisions for subsequent adjustments (and any subsequent adjustments shall be treated as provided in clauses (b) and (c) of this Subsection 4.4.3) or (ii) cannot be calculated at all at the time such Option or Convertible Security is issued or amended, any adjustment to such Conversion Price that would result under the terms of this Subsection 4.4.3 at the time of such issuance or amendment shall instead be effected at the time such number of shares and/or amount of consideration is first calculable (even if subject to subsequent adjustments), assuming for purposes of calculating such adjustment to the Conversion Price that such issuance or amendment took place at the time such calculation can first be made.

4.4.4 <u>Adjustment of Conversion Price Upon Issuance of Additional Shares of Common Stock</u>. If, at any time after the applicable Original Series B Issue Date, the Corporation issues Additional Shares of Common Stock (including Additional Shares of Common Stock deemed to be issued pursuant to <u>Subsection 4.4.3</u>), without consideration or for a consideration per share less than the relevant Conversion Price in effect immediately prior to such issuance of Additional Shares of Common Stock, then such Conversion Price in effect immediately prior to such issuance of Additional Shares of Common Stock ("*CP*<sub>1</sub>") shall be reduced, concurrently with such issuance, to a price (calculated to the nearest one-hundredth of a

cent) determined by multiplying CP<sub>1</sub> by a fraction, the *numerator* of which shall be the sum of (i) the number of shares of Common Stock outstanding immediately prior to such issuance of Additional Shares of Common Stock (treating for this purpose as outstanding all shares of Common Stock issuable upon (A) exercise of Options outstanding immediately prior to such issuance of Additional Shares of Common Stock and (B) conversion or exchange of Convertible Securities (including Preferred Stock) outstanding (assuming exercise of any outstanding Options therefor) immediately prior to such issuance of Additional Shares of Common Stock) (the "*Outstanding Common Stock Equivalents*") and (ii) the number of shares of Common Stock that would have been issued if such Additional Shares of Common Stock had been issued at a price per share equal to CP<sub>1</sub> (determined by dividing (A) the aggregate consideration received by the Corporation in respect of such issuance of Additional Shares of Common Stock by (B) CP<sub>1</sub>), and the *denominator* of which shall be the sum of (i) the Outstanding Common Stock Equivalents and (ii) the number of such Additional Shares of Common Stock issued in such transaction.

4.4.5 <u>Determination of Consideration</u>. For purposes of this <u>Subsection 4.4</u>, the consideration received by the Corporation for the issuance of any Additional Shares of Common Stock shall be computed as follows:

#### (a) Cash and Property: Such consideration shall:

- insofar as it consists of cash, be computed at the aggregate amount of cash received by the Corporation, excluding amounts paid or payable for accrued interest;
- (ii) insofar as it consists of property other than cash, be computed at the fair market value thereof at the time of such issuance, as determined in good faith by the Board, including the approval of at least one Preferred Director; and
- (iii) in the event Additional Shares of Common Stock are issued together with other shares or securities or other assets of the Corporation for consideration which covers both, be the proportion of such consideration so received, computed as provided in clauses (i) and (ii) above, as determined in good faith by the Board, including the approval of at least one Preferred Director.

(b) <u>Options and Convertible Securities</u>. Such consideration shall, in the event that Additional Shares of Common Stock are deemed to have been issued pursuant to <u>Subsection 4.4.3</u> due to the issuance of any Options or Convertible Securities, be determined, on a per share basis, by dividing:

- (i) the total amount, if any, received or receivable by the Corporation as consideration for the issuance of such Options or Convertible Securities, plus the minimum aggregate amount of additional consideration (as set forth in the instruments relating thereto, without regard to any provision contained therein for a subsequent adjustment of such consideration), if any, payable to the Corporation upon the exercise of such Options or the conversion or exchange of such Convertible Securities (or, in the case of Options for Convertible Securities, the exercise of such Options for Convertible Securities and the conversion or exchange of such Convertible Securities); by
- (ii) the maximum number of shares of Common Stock (as set forth in the instruments relating thereto, without regard to any provision contained therein for a subsequent adjustment of such number) issuable upon the exercise of such Options or the conversion or exchange of such Convertible Securities (or, in the case of Options for Convertible Securities, the exercise of such Options for Convertible Securities and the conversion or exchange of such Convertible Securities).

4.4.6 <u>Multiple Closing Dates</u>. If the Corporation issues, on more than one date, Additional Shares of Common Stock that are a part of one transaction or a series of related transactions and that would result in an adjustment to any Conversion Price pursuant to the terms of <u>Subsection 4.4.4</u>, and such issuance dates occur within a period of no more than 90 days from the first such issuance to the final such issuance, then, upon the final such issuance, the such Conversion Price shall be readjusted to give effect to all such issuances as if they occurred on the date of the first such issuance (and without giving effect to any additional adjustments as a result of any such subsequent issuances within such period).

4.5 Adjustment for Stock Splits and Combinations. If the Corporation at any time or from time to time after the Original Series B Issue Date effects a subdivision of the outstanding shares of Common Stock into a greater number of shares of Common Stock, the relevant Conversion Price in effect immediately before the subdivision shall be proportionately decreased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be increased in proportion to such increase in the aggregate number of shares of Common Stock outstanding. If the Corporation at any time or from time to time after the Original Series B Issue Date combines the outstanding shares of Common Stock into a smaller

number of shares of Common Stock, the relevant Conversion Price in effect immediately before the combination shall be proportionately increased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be decreased in proportion to such decrease in the aggregate number of shares of Common Stock outstanding. Any adjustment under this <u>Subsection 4.5</u> shall become effective at the close of business on the date that the subdivision or combination, as applicable, becomes effective. The relevant Conversion Price shall be readjusted in the same manner upon the happening of each subsequent subdivision or combination of the outstanding shares of Common Stock.

4.6 <u>Adjustment for Common Stock Dividends</u>. If the Corporation at any time or from time to time after the Original Series B Issue Date makes or issues, or fixes a record date for the determination of holders of Common Stock entitled to receive, a Common Stock Dividend, then in each such event the relevant Conversion Price in effect immediately before such event shall be decreased as of the time of such issuance or, in the event such a record date shall have been fixed, as of the close of business on such record date, by multiplying the such Conversion Price then in effect by a fraction:

(1) the numerator of which shall be the total number of shares of Common Stock issued and outstanding immediately prior to the time of such issuance or the close of business on such record date; and

(2) the denominator of which shall be the sum of (a) the total number of shares of Common Stock issued and outstanding immediately prior to the time of such issuance or the close of business on such record date and (b) the total number of shares of Common Stock issuable in payment of such Common Stock Dividend.

Notwithstanding the foregoing, (i) if such record date shall have been fixed and such Common Stock Dividend is not fully paid or made on the date fixed therefor, the relevant Conversion Price shall be recomputed accordingly as of the close of business on such record date and thereafter such Conversion Price shall be adjusted pursuant to this <u>Subsection 4.6</u> as of the time of actual payment of such Common Stock Dividend and (ii) no such adjustment shall be made if the holders of the relevant series of Preferred Stock simultaneously receive a dividend or other distribution of shares of Common Stock in an amount equal to the number of shares of Common Stock as they would have received if all outstanding shares of such series of Preferred Stock had been converted into Common Stock on the date of such event or such record date, as applicable. The relevant Conversion Price shall be readjusted in the same manner upon the happening of each subsequent Common Stock Dividend.

4.7 <u>Adjustment for Other Dividends and Distributions</u>. If the Corporation at any time or from time to time after the Original Series B Issue Date makes or issues, or fixes a record date for the determination of holders of Common Stock entitled to receive, a dividend or other distribution payable in securities of the Corporation (other than a Common Stock Dividend), cash or other property and the provisions of <u>Section 1</u> do not apply to such dividend or other distribution, then in each such event provision shall be made so that the holders of Preferred Stock shall receive upon conversion thereof, in addition to the number of shares of Common Stock receivable thereupon, the kind and amount of securities, cash or other property which they would have received had Preferred Stock been converted into Common

Stock on the date of such event or such record date, as applicable, and had they thereafter, during the period from the date of such event or such record date, as applicable, to and including the conversion date, retained such securities during such period, giving application to all adjustments called for during such period under this Section 4 with respect to the rights of the holders of Preferred Stock or with respect to such other securities by their terms; provided, however, that no such provision shall be made if the holders of Preferred Stock receive, simultaneously with the dividend or distribution to the holders of Common Stock, a dividend or other distribution of such securities, cash or other property in an amount equal to the amount of such securities, cash or other property as they would have received if all outstanding shares of Preferred Stock had been converted into Common Stock on the date of such event or such record date, as applicable.

4.8 Adjustment for Reorganization. Subject to the provisions of Subsection 2.3, if there shall occur any reorganization, recapitalization, reclassification, consolidation or merger involving the Corporation in which Common Stock (but not Preferred Stock) is converted into or exchanged for securities, cash or other property (other than a transaction covered by <u>Subsection 4.4</u>, <u>Subsection 4.6</u> or <u>Subsection 4.7</u>) (any such event, a "Reorganization"), then, following any such Reorganization, each share of Preferred Stock shall thereafter be convertible, in lieu of the shares of Common Stock into which it was convertible prior to such event, into the kind and amount of securities, cash or other property which a holder of the number of shares of Common Stock issuable upon conversion of one share of Preferred Stock immediately prior to such Reorganization would have been entitled to receive pursuant to such transaction; and, in such case, appropriate adjustment (as determined in good faith by the Board, including the approval of at least one Preferred Director) shall be made in the application of the provisions of this Section 4 with respect to the rights and interests thereafter of the holders of Preferred Stock, to the end that the provisions of this Section 4 (including adjustment of any Conversion Price then in effect and the number of shares issuable upon conversion of Preferred Stock) shall thereafter be applicable, as nearly as reasonably may be, in relation to any securities or other property thereafter deliverable upon the conversion of Preferred Stock. This Subsection 4.8 shall similarly apply to successive Reorganizations. Notwithstanding anything to the contrary contained in this Subsection 4.8, if any Reorganization is approved by the vote of stockholders required by Subsections 3.4, 3.5 and 3.6, then such Reorganization and the rights of the holders of Common Stock and Preferred Stock pursuant to such Reorganization shall be governed by the documents entered into in connection with such Reorganization and not by the provisions of this Subsection 4.8. For the avoidance of doubt, nothing in this Subsection 4.8 shall be construed as preventing the holders of Preferred Stock from seeking any appraisal rights to which they are otherwise entitled under the DGCL in connection with a Reorganization triggering an adjustment hereunder, nor shall this Subsection 4.8 be deemed conclusive evidence of the fair value of the shares of Preferred Stock in any such appraisal proceeding.

4.9 <u>Certificate as to Adjustments</u>. Upon the occurrence of each adjustment or readjustment of any Conversion Price pursuant to this <u>Section 4</u>, the Corporation, at its expense, shall promptly (but in any event not later than ten days thereafter) compute such adjustment or readjustment in accordance with the terms hereof and furnish, or cause to be furnished, to each holder of the relevant series of Preferred Stock a certificate setting forth such adjustment or readjustment (including the kind and amount of securities, cash or other property into which such series of Preferred Stock is convertible) and showing in detail the facts upon which such adjustment or readjustment is based. Upon the written request at any time of any

holder of any Preferred Stock, the Corporation shall promptly (but in any event not later than ten days thereafter) furnish, or cause to be furnished, to such holder a certificate setting forth (i) the Conversion Price then in effect and (ii) the number of shares of Common Stock and the amount, if any, of other securities, cash or property which then would be received upon the conversion of such series of Preferred Stock.

4.10 Notice of Record Date. In the event (x) the Corporation takes a record of the holders of any class of securities for the purpose of determining the holders thereof who are entitled to receive any dividend or other distribution, or to receive any right to subscribe for or purchase any shares of capital stock of any class or any other securities, (y) of any capital reorganization of the Corporation, any reclassification of Common Stock or any Deemed Liquidation Event, or (z) of the voluntary or involuntary liquidation, dissolution or winding up of the Corporation (the events described in the foregoing clauses (y) and (z), "Other Events"), then in each such case the Corporation shall send, or cause to be sent, to the holders of record of Preferred Stock, a notice specifying, as the case may be, (i) the date on which any such record is to be taken for the purpose of such dividend, distribution or right and (ii) the effective date on which such Other Event is proposed to take place and the time, if any is to be fixed, as of which the holders of record of Common Stock (or such other capital stock or securities at the time issuable upon the conversion of Preferred Stock) shall be entitled to exchange their shares of Common Stock (or such other capital stock or securities at the time issuable upon the conversion of the relevant series of Preferred Stock) for securities or other property deliverable upon such Other Event, and the amount per share and character of such exchange applicable to Common Stock and the relevant series of Preferred Stock. Each notice described in the foregoing sentence shall be sent at least ten days prior to the record date or effective date, as applicable, for the event specified in such notice.

## 5. Mandatory Conversion.

5.1 <u>Trigger Event.</u> All outstanding shares of Preferred Stock shall automatically, and without any further action on the part of the holders thereof, be converted into shares of Common Stock at the then effective Conversion Price, upon either (a) the closing of the sale of shares of Common Stock to the public in a firm-commitment underwritten public offering pursuant to an effective registration statement filed under the Securities Act of 1933, as amended, (i) with a price of at least 1.5 times (1.5X) the Original Series B Issue Price for any such public offering consummated at any time on or prior to the first anniversary of the Series B Original Issue Date, or 1.25 times (1.25X) the Original Series B Issue Price for any such public offering consummated thereafter, in each case, as adjusted for any stock splits, stock dividends, combinations, subdivisions, recapitalizations or the like, and (ii) resulting in at least \$30,000,000 of gross proceeds to the Corporation or (b) the date and time, or the occurrence of an event, specified by vote or written consent of the holders of a majority of the then outstanding shares of Preferred Stock, voting together as a single class on an as-converted to Common Stock basis; <u>provided</u>, <u>however</u>, that any automatic conversion of the Series B Preferred Stock pursuant to <u>Section 5.1(b)</u> shall require the consent of the holders of a majority of the shares of Series B Preferred Stock then outstanding (the time of such closing or the date and time, or the time of the event, specified in such vote or written consent is referred to herein as the "*Mandatory Conversion Time*").

- 5.2 Procedural Requirements. The Corporation shall send, or cause to be sent, to all holders of record of shares of Preferred Stock, written notice of the Mandatory Conversion Time and the place designated for mandatory conversion of all such shares of Preferred Stock pursuant to this Section 5. Such notice need not be sent in advance of the occurrence of the Mandatory Conversion Time. Upon receipt of such notice, each holder of shares of Preferred Stock shall surrender his, her or its certificate or certificates for all such shares (or, if such holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate) to the Corporation, in the manner and at the place designated in such notice. If required by the Corporation, certificates surrendered for conversion shall be endorsed or accompanied by a written instrument or instruments of transfer, in form satisfactory to the Corporation, duly executed by the registered holder or his, her or its attorney duly authorized in writing. All shares of Preferred Stock shall no longer be deemed to be outstanding and all rights with respect to such shares, including the rights, if any, to receive notices and to vote (other than as a holder of Common Stock), shall immediately cease and terminate at the Mandatory Conversion Time (notwithstanding the failure of the registered holder or holders thereof to surrender the certificates for such shares at or prior to such time), except only the right of the registered holders thereof, upon surrender of their certificate or certificates therefor (or lost certificate affidavit and agreement), to receive the items provided for in the next sentence of this Subsection 5.2. The Corporation shall, as soon as practicable after the Mandatory Conversion Time and the surrender of the certificate or certificates (or lost certificate affidavit and agreement) for shares of Preferred Stock so converted, (i) issue and deliver to such holder of Preferred Stock, or to his, her or its nominees, a certificate or certificates for the number of full shares of Common Stock issuable upon such conversion in accordance with the provisions hereof, (ii) pay in cash such amount as provided in Subsection 4.2 in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion, and (iii) pay all declared but unpaid dividends on the shares of Preferred Stock converted. Such converted shares of Preferred Stock shall be retired and cancelled and may not be reissued as shares of such series, and the Corporation may thereafter take such appropriate action (without the need for stockholder action) as may be necessary to reduce the authorized number of shares of Preferred Stock accordingly.
- 6. <u>Redemption</u>. Neither the Corporation nor the holders of Preferred Stock shall have the unilateral right to call or redeem, or cause to have called or redeemed, any shares of Preferred Stock.
- 7. No Reissuance of Preferred Stock. Any shares of Preferred Stock that are acquired by the Corporation or any of its subsidiaries by reason of redemption, purchase, conversion or otherwise shall be automatically and immediately retired and cancelled and shall not be reissued, sold or transferred. Neither the Corporation nor any of its subsidiaries may exercise any voting or other rights granted to the holders of Preferred Stock following redemption.

8. <u>Waiver</u>. Any of the rights, powers, preferences and other terms of Series A Preferred Stock set forth herein may be waived on behalf of all holders of Series A Preferred Stock by the affirmative written consent or vote of the holders of a majority of the shares of Series A Preferred Stock then outstanding. Any of the rights, powers, preferences and other terms of Series A-1 Preferred Stock set forth herein may be waived on behalf of all holders of Series A-1 Preferred Stock by the affirmative written consent or vote of the holders of at least a majority of the shares of Series A-1 Preferred Stock then outstanding. Any of the rights, powers, preferences and other terms of Series B Preferred Stock set forth herein may be waived on behalf of all holders of Series B Preferred Stock by the affirmative written consent or vote of the holders of at least a majority of the shares of Series B Preferred Stock then outstanding.

9. <u>Notices</u>. Any notice required or permitted by the provisions of this Article IV to be given to a holder of shares of Preferred Stock shall be (i) mailed by certified or registered mail, return receipt requested and postage prepaid, or delivered by a recognized express courier, fees prepaid, in each case to the address of such holder last shown on the records of the Corporation, or (ii) given by electronic communication in compliance with the provisions of the DGCL. Any such notice shall be deemed given upon such mailing, delivery or electronic transmission, as applicable.

# ARTICLE V

Subject to any additional vote required by the Certificate of Incorporation or the Bylaws of the Corporation, in furtherance and not in limitation of the powers conferred by statute, the Board is expressly authorized to make, repeal, alter, amend or rescind any or all of the Bylaws of the Corporation.

#### **ARTICLE VI**

Meetings of stockholders may be held within or without the State of Delaware, as the Bylaws of the Corporation may provide. The books of the Corporation may be kept (subject to any provision contained in applicable statutes) outside the State of Delaware at such place or places as may be designated from time to time by the Board or in the Bylaws of the Corporation.

#### **ARTICLE VII**

To the fullest extent permitted by applicable law as it presently exists or may hereafter be amended, a director of the Corporation shall not be personally liable to the Corporation or its stockholders for monetary damages for breach of fiduciary duty as a director, except for liability (i) for any breach of the director's duty of loyalty to the Corporation or its stockholders, (ii) for acts or omissions not in good faith or that involve intentional misconduct or a knowing violation of law, (iii) under Section 174 of the DGCL, or (iv) for any transaction from which the director derived any improper personal benefit. If the DGCL or any other applicable law of the State of Delaware is amended, after approval by the stockholders of this Article VH, to authorize corporate action further eliminating or limiting the personal liability of directors, then the liability of a director of the Corporation shall be eliminated or limited to the fullest extent permitted by the DGCL or other applicable law of the State of Delaware, as so amended.

Any repeal or modification of the foregoing provisions of this Article VII shall not adversely affect any right or protection of any director of the Corporation existing at the time of, or increase the liability of any director of the Corporation with respect to any acts or omissions of such director occurring prior to, such repeal or modification.

#### **ARTICLE VIII**

To the fullest extent permitted by applicable law as it presently exists or may hereafter be amended, the Corporation is authorized to provide indemnification of (and advancement of expenses to) directors, officers, employees and agents of the Corporation (and any other persons to whom the DGCL permits the Corporation to provide indemnification and advancement of expenses) through Bylaw provisions, agreements with such persons, vote of stockholders or disinterested directors or otherwise, in excess of the indemnification and advancement of expenses otherwise permitted by Section 145 of the DGCL.

Any repeal or modification of the foregoing provisions of this Article VIII shall not adversely affect any right or protection of any director, officer, employee or agent of the Corporation existing at the time of such repeal or modification.

# ARTICLE IX

The Corporation renounces, to the fullest extent permitted by law, any interest or expectancy of the Corporation in, or in being offered an opportunity to participate in, any Excluded Opportunity. An "Excluded Opportunity" is any matter, transaction or interest that is presented to, or acquired, created or developed by, or which otherwise comes into the possession of (i) any director of the Corporation who is not an officer or employee of the Corporation or any of its subsidiaries, or (ii) any holder of Preferred Stock or Common Stock issued upon the conversion of the Preferred Stock, or any partner, member, director, stockholder, employee or agent of any such holder, excluding (A) any holder that is affiliated with someone who is an officer or employee of the Corporation or any of its subsidiaries, and (B) anyone who is an officer or employee of the Corporation or any of its subsidiaries (the persons and entities being referred to in clauses (i) and (ii), collectively, "Covered Persons"), unless such matter, transaction or interest is presented to, or acquired, created or developed by, or otherwise comes into the possession of, a Covered Person expressly and solely in such Covered Person's capacity as a director of the Corporation.

#### ARTICLE X

Unless the Corporation consents in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware shall be the sole and exclusive forum for (i) any derivative action or proceeding brought on behalf of the Corporation, (ii) any action asserting a claim for breach of a fiduciary duty owed by any director, officer, employee or agent of the Corporation to the Corporation or any of its stockholders, (iii) any action asserting a claim arising pursuant to any provision of the DGCL or the Certificate of Incorporation or Bylaws of the Corporation, or (iv) any action asserting a claim governed by the internal affairs doctrine, in each case subject to said Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein.

#### **ARTICLE XI**

For purposes of Section 500 of the California Corporations Code (to the extent applicable), in connection with any repurchase of shares of Common Stock permitted under the Certificate of Incorporation from employees, officers, directors or consultants of the Corporation in connection with a termination of employment or services pursuant to agreements or arrangements approved by the Board (in addition to any other consent required under the Certificate of Incorporation), such repurchase may be made without regard to any "preferential dividends arrears amount" or "preferential rights amount" (as those terms are defined in Section 500 of the California Corporations Code). Accordingly, for purposes of making any calculation under Section 500 of the California Corporations Code in connection with such repurchase, the amount of any "preferential dividends arrears amount" or "preferential rights amount" (as those terms are defined therein) shall be deemed to be zero (0).

# **BYLAWS**

# OF 4D MOLECULAR THERAPEUTICS, INC.

Adopted March 20, 2015

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#### **BYLAWS**

#### ARTICLE I — MEETINGS OF STOCKHOLDERS

# 1.1 Place of Meetings

Meetings of stockholders of 4D Molecular Therapeutics, Inc. (the "*Company*") shall be held at any place, within or outside the State of Delaware, as determined by the Company's board of directors (the "*Board*"). The Board may, in its sole discretion, determine that a meeting of stockholders shall not be held at any place, but may instead be held solely by means of remote communication as authorized by Section 211(a)(2) of the Delaware General Corporation Law (the "*DGCL*"). In the absence of any such determination by the Board, meetings of stockholders shall be held at the Company's principal executive office.

#### 1.2 **Annual Meeting**

An annual meeting of stockholders shall be held for the election of directors on such date and at such time as may be designated by resolution of the Board from time to time. Any other proper business may be transacted at the annual meeting of stockholders. Notwithstanding the foregoing, the Company shall not be required to hold an annual meeting of stockholders, provided that (i) the stockholders are permitted to act by written consent under the Company's certificate of incorporation and these bylaws, (ii) the stockholders take action by written consent to elect directors, and (iii) the stockholders unanimously consent to such action or, if such consent is less than unanimous, all of the directorships to which directors could be elected at an annual meeting held at the effective time of such action are vacant and are filled by such action.

# 1.3 Special Meeting

A special meeting of stockholders may be called at any time by the Board, Chairperson of the Board, Chief Executive Officer or President (in the absence of a Chief Executive Officer).

At any time or times that the Company is subject to Section 2115(b) of the California Corporations Code (the "*CCC*"), stockholders holding 5% or more of the outstanding shares shall have the right to call a special meeting of stockholders as set forth in Section 2.4.

If any person(s) other than the Board calls a special meeting, the request shall:

- (i) be in writing;
- (ii) specify the general nature of the business proposed to be transacted; and
- (iii) be delivered personally or sent by registered mail, return receipt requested, or by facsimile transmission to the Chairperson of the Board, Chief Executive Officer, President (in the absence of a Chief Executive Officer) or Secretary.

The officer(s) of the Company receiving the request shall cause notice to be promptly given to the stockholders entitled to vote at such meeting, in accordance with these bylaws, that a meeting will be held at the place and time determined by the Board, which shall not be fewer than 30 nor more than 120 days after the date of receipt of the request. No business may be transacted at such special meeting other than the business specified in such notice to stockholders. Nothing contained in this paragraph of this Section 1.3 shall be construed as limiting, fixing or affecting the time when a meeting of stockholders called by action of the Board may be held.

### 1.4 Notice of Meetings

Whenever stockholders are required or permitted to take any action at a meeting, a written notice of the meeting shall be given which shall state the place, if any, date and hour of the meeting, the means of remote communication, if any, by which stockholders and proxy holders may be deemed to be present in person and vote at such meeting, the record date for determining the stockholders entitled to vote at the meeting (if such date is different from the record date for determining stockholders entitled to notice of the meeting), and, in the case of a special meeting, the purpose or purposes for which the meeting is called. Except as otherwise provided in the DGCL, the certificate of incorporation or these bylaws, the written notice of any meeting of stockholders shall be given not fewer than 10 nor more than 60 days before the date of the meeting to each stockholder entitled to vote at such meeting as of the record date for determining the stockholders entitled to notice of the meeting.

#### 1.5 **Quorum**

Except as otherwise provided in the DGCL, the certificate of incorporation or these bylaws, at each meeting of stockholders the presence in person or by proxy of the holders of shares of stock having a majority of the votes which could be cast by the holders of all outstanding shares of stock entitled to vote at the meeting shall be necessary and sufficient to constitute a quorum; provided that, where a separate vote by a class or series or classes or series is required, a majority of the outstanding shares of such class or series or classes or series, present in person or represented by proxy, shall constitute a quorum entitled to take action with respect to that vote on that matter.

If, however, such quorum is not present or represented at any meeting of stockholders, then either (i) the Chairperson of the Meeting (as defined in Section 1.7) or (ii) the holders of a majority of the shares present or represented at the meeting shall have the power to adjourn the meeting from time to time, in the manner provided in Section 1.6, until a quorum is present or represented. A quorum, once established, shall not be broken by the subsequent withdrawal of enough votes to leave less than a quorum.

# 1.6 Adjourned Meeting; Notice

Any meeting of stockholders may adjourn from time to time to reconvene at the same or some other place, and notice need not be given of the adjourned meeting if the time, place, if any, thereof, and the means of remote communication, if any, by which stockholders and proxy holders may be deemed to be present in person and vote at such adjourned meeting are announced at the meeting at which the adjournment is taken. At the adjourned meeting, the Company may transact

any business which might have been transacted at the original meeting. If the adjournment is for more than 30 days, a notice of the adjourned meeting shall be given to each stockholder of record entitled to vote at the meeting. If, after the adjournment, a new record date for stockholders entitled to vote is fixed for the adjourned meeting, the Board shall fix a new record date for notice of such adjourned meeting in accordance with the DGCL and shall give notice of the adjourned meeting to each stockholder of record entitled to vote at such adjourned meeting as of the record date fixed for notice of such adjourned meeting.

# 1.7 Conduct of Business

Each meeting of stockholders shall be presided over by the Chairperson of the Board, if any, or in his or her absence by the Vice Chairperson of the Board, if any, or in the absence of the foregoing persons by the Chief Executive Officer, or in the absence of the foregoing persons by a Vice President, or in the absence of the foregoing persons by a chairperson of such meeting designated by the Board, or in the absence of such designation by a chairperson chosen at such meeting by the holders of a majority of the shares present or represented at such meeting (such presiding person, the "*Chairperson of the Meeting*"). The Secretary shall act as secretary of each meeting, but in his or her absence the Chairperson of the Meeting may appoint any person to act as secretary of such meeting. The Chairperson of the Meeting shall determine the order of business and the procedure at the meeting, including such regulation of the manner of voting and the conduct of business.

#### 1.8 Voting

The stockholders entitled to vote at any meeting of stockholders shall be determined in accordance with the provisions of Section 1.10, subject to Section 217 of the DGCL (relating to voting rights of fiduciaries, pledgors and joint owners of stock) and Section 218 of the DGCL (relating to voting trusts and other voting agreements).

Unless otherwise provided in the certificate of incorporation and subject to Section 1.10, each stockholder entitled to vote at any meeting of stockholders shall be entitled to one vote for each share of capital stock held by such stockholder which has voting power upon the matter in question. Elections of directors need not be by written ballot and, unless otherwise required by law, need not be conducted by inspectors of election unless so determined by the holders of shares of stock having a majority of the votes which could be cast by the holders of all outstanding shares of stock entitled to vote thereon which are present in person or by proxy at such meeting. If authorized by the Board, such requirement of a written ballot shall be satisfied by a ballot submitted by electronic transmission, provided that any such electronic transmission must either set forth or be submitted with information from which it can be determined that the electronic transmission was authorized by the stockholder or proxy holder.

Except as otherwise provided in the DGCL, the certificate of incorporation or these bylaws, (i) in all matters other than the election of directors, the affirmative vote of the majority of the voting power of the shares present in person or represented by proxy at the meeting and entitled to vote on the subject matter shall be the act of the stockholders, (ii) directors shall be elected by a plurality of the voting power of the shares present in person or represented by proxy at the meeting and entitled

to vote on the election of directors, and (iii) where a separate vote by a class or series or classes or series is required, in all matters other than the election of directors, the affirmative vote of the majority of shares of such class or series or classes or series present in person or represented by proxy at the meeting shall be the act of such class or series or classes or series.

# 1.9 Stockholder Action by Written Consent Without a Meeting

Unless otherwise provided in the certificate of incorporation, any action required by the DGCL to be taken at any annual or special meeting of stockholders, or any action which may be taken at any annual or special meeting of such stockholders, may be taken without a meeting, without prior notice and without a vote, if a consent or consents in writing, setting forth the action so taken, shall be signed by the holders of outstanding stock having not less than the minimum number of votes that would be necessary to authorize or take such action at a meeting at which all shares entitled to vote thereon were present and voted.

Every written consent (other than a written consent by electronic transmission) shall bear the date of signature of each stockholder who signs the consent, and no written consent shall be effective to take the corporate action referred to therein unless, within 60 days of the earliest dated consent delivered to the Company in the manner herein required, written consents signed by a sufficient number of stockholders to take action are delivered to the Company by delivery to its registered office in the State of Delaware, its principal place of business or an officer or agent of the Company having custody of the book in which proceedings of meetings of stockholders are recorded. Delivery made to the Company's registered office shall be by hand or by certified or registered mail, return receipt requested.

An electronic transmission consenting to an action to be taken and transmitted by a stockholder or proxy holder, or by a person or persons authorized to act for a stockholder or proxy holder, shall be deemed to be written, signed and dated for purposes of this Section 1.9, provided that any such electronic transmission sets forth or is delivered with information from which the Company can determine (i) that the electronic transmission was transmitted by the stockholder or proxy holder or by a person or persons authorized to act for the stockholder or proxy holder and (ii) the date on which such stockholder or proxy holder or authorized person or persons transmitted such electronic transmission. The date on which such electronic transmission is transmitted shall be deemed to be the date on which such consent was signed.

Notwithstanding the foregoing, in the event that the Board shall have instructed the officers of the Company to solicit the vote or written consent of the stockholders of the Company, an electronic transmission of a stockholder written consent given pursuant to such solicitation may be delivered to the Secretary or President (or to a person designated by the Secretary or President). The Secretary or President (or a person designated by the Secretary or President) shall cause any such written consent by electronic transmission to be reproduced in paper form and inserted into the Company's corporate records.

## 1.10 Record Date for Stockholder Notice; Voting; Giving Consents

In order that the Company may determine the stockholders entitled to notice of or to vote at any meeting of stockholders or any adjournment thereof, or entitled to consent to corporate action in writing without a meeting, or entitled to receive payment of any dividend or other distribution or allotment of any rights, or entitled to exercise any rights in respect of any change, conversion or exchange of stock or for the purpose of any other lawful action, the Board may fix a record date, which record date shall not precede the date upon which the resolution fixing the record date is adopted by the Board, and which record date:

- (i) in the case of determination of stockholders entitled to notice of or to vote at any meeting of stockholders or any adjournment thereof, shall, unless otherwise required by the DGCL, not be more than 60 nor fewer than 10 days before the date of such meeting;
- (ii) in the case of determination of stockholders entitled to consent to corporate action in writing without a meeting, shall not be more than 10 days after the date upon which the resolution fixing the record date is adopted by the Board; and
  - (iii) in the case of determination of stockholders for any other action, shall not be more than 60 days prior to such action.

If no record date is fixed by the Board:

- (i) the record date for determining stockholders entitled to notice of and to vote at a meeting of stockholders shall be at the close of business on the day next preceding the day on which notice is given, or, if notice is waived, at the close of business on the day next preceding the day on which the meeting is held;
- (ii) the record date for determining stockholders entitled to consent to corporate action in writing without a meeting, when no prior action by the Board is required by the DGCL, shall be the first date on which a signed written consent setting forth the action taken or proposed to be taken is delivered to the Company in accordance with the DGCL, or, if prior action by the Board is required by the DGCL, shall be at the close of business on the day on which the Board adopts the resolution taking such prior action; and
- (iii) the record date for determining stockholders for any other purpose shall be at the close of business on the day on which the Board adopts the resolution relating thereto.

A determination of stockholders of record entitled to notice of or to vote at a meeting of stockholders shall apply to any adjournment of the meeting, provided that the Board may fix a new record date for the determination of stockholders entitled to vote at the adjourned meeting, and in such case shall also fix as the record date for stockholders entitled to notice of such adjourned meeting the same or an earlier date as that fixed for determination of stockholders entitled to vote in accordance with the foregoing provisions of this Section 1.10 at the adjourned meeting.

#### 1.11 Proxies

Each stockholder entitled to vote at a meeting of stockholders or to express consent or dissent to corporate action in writing without a meeting may authorize another person or persons to act for such stockholder by proxy authorized by an instrument in writing or by a transmission permitted by law, but no such proxy shall be voted or acted upon after three years from its date, unless the proxy provides for a longer period. The revocability of a proxy that states on its face that it is irrevocable shall be governed by the provisions of Section 212 of the DGCL.

#### 1.12 List of Stockholders Entitled to Vote

The officer who has charge of the stock ledger of the Company shall prepare and make, at least 10 days before every meeting of stockholders, a complete list of the stockholders entitled to vote at the meeting (unless the record date for determining the stockholders entitled to vote is less than 10 days before the meeting date, in which case the list shall reflect the stockholders entitled to vote as of the tenth day before the meeting date), arranged in alphabetical order, and showing the address of each stockholder and the number of shares registered in the name of each stockholder. The Company shall not be required to include electronic mail addresses or other electronic contact information on such list. Such list shall be open to the examination of any stockholder for any purpose germane to the meeting for a period of at least 10 days prior to the meeting; (i) on a reasonably accessible electronic network, provided that the information required to gain access to such list is provided with the notice of the meeting, or (ii) during ordinary business hours, at the Company's principal place of business. In the event that the Company determines to make the list available on an electronic network, the Company may take reasonable steps to ensure that such information is available only to stockholders of the Company. If the meeting is to be held at a place, then a list of stockholders entitled to vote at the meeting shall be produced and kept at the time and place of the meeting during the whole time thereof and may be examined by any stockholder who is present. If the meeting is to be held solely by means of remote communication, then such list shall also be open to the examination of any stockholder during the whole time of the meeting on a reasonably accessible electronic network, and the information required to access such list shall be provided with the notice of the meeting.

# ARTICLE II — DIRECTORS

# 2.1 Powers

The business and affairs of the Company shall be managed by or under the direction of the Board, except as may be otherwise provided in the DGCL or the certificate of incorporation.

# 2.2 Number of Directors

The Board shall consist of one or more members, each of whom shall be a natural person. Unless the certificate of incorporation fixes the number of directors, the number of directors shall be determined from time to time by resolution of the Board. No reduction of the authorized number of directors shall have the effect of removing any director before that director's term of office expires.

#### 2.3 Election, Qualification and Term of Office of Directors

Except as provided in Section 2.4, and subject to Section 1.2 and Section 1.9, directors shall be elected at each annual meeting of stockholders. Directors need not be stockholders unless so required by the certificate of incorporation or these bylaws. The certificate of incorporation or these bylaws may prescribe other qualifications for directors. Each director shall hold office until such director's successor is elected and qualified or until such director's earlier death, resignation or removal.

No stockholder entitled to vote at an election for directors may cumulate votes to which such stockholder is entitled, unless, at the time of such election, the Company is subject to Section 2115(b) of the CCC. During such time that the Company is subject to Section 2115 of the CCC, the Company's stockholders shall have the right to cumulate their votes in connection with the election of directors as provided by subdivisions (a), (b) and (c) of Section 708 of the CCC.

# 2.4 Resignation and Vacancies

Any director may resign at any time upon notice given in writing or by electronic transmission to the Company. A resignation is effective when the resignation is delivered unless the resignation specifies a later effective date or an effective date determined upon the happening of an event or events. A resignation which is conditioned upon the director failing to receive a specified vote for reelection as a director may provide that it is irrevocable. Unless otherwise provided in the certificate of incorporation or these bylaws, when one or more directors shall resign from the Board, effective at a future date, a majority of the directors then in office, including those who have so resigned, shall have power to fill such vacancy or vacancies, the vote thereon to take effect when such resignation or resignations shall become effective.

Unless otherwise provided in the certificate of incorporation or these bylaws, vacancies and newly created directorships resulting from any increase in the authorized number of directors may be filled by a majority of the directors then in office, although less than a quorum, or by a sole remaining director.

If at any time, by reason of death or resignation or other cause, the Company should have no directors in office, then any officer or any stockholder or an executor, administrator, trustee or guardian of a stockholder, or other fiduciary entrusted with like responsibility for the person or estate of a stockholder, may call a special meeting of stockholders in accordance with the certificate of incorporation or these bylaws, or may apply to the Court of Chancery for a decree summarily ordering an election as provided in Section 211 of the DGCL.

If, at the time of filling any vacancy or any newly created directorship, the directors then in office shall constitute less than a majority of the whole Board (as constituted immediately prior to any such increase), the Court of Chancery may, upon application of any stockholder or stockholders holding at least 10% of the voting stock at the time outstanding having the right to vote for such directors, summarily order an election to be held to fill any such vacancies or newly created directorships, or to replace the directors chosen by the directors then in office as aforesaid, which election shall be governed by Section 211 of the DGCL as far as applicable.

At any time or times that the Company is subject to Section 2115(b) of the CCC, if, after the filling of any vacancy by the directors, the directors then in office who have been elected by the stockholders shall constitute less than a majority of the directors then in office, then (i) any holder or holders of an aggregate of five percent (5%) or more of the total number of shares at the time outstanding having the right to vote for those directors may call a special meeting of stockholders or (ii) the superior court of the proper county shall, upon application of such stockholder or stockholders, summarily order a special meeting of stockholders, to be held to elect the entire Board, all in accordance with Section 305(c) of the CCC, and the term of office of any director shall terminate upon that election of a successor.

A director elected to fill a vacancy shall be elected for the unexpired term of his or her predecessor in office and until such director's successor is elected and qualified or until such director's earlier death, resignation or removal.

# 2.5 Place of Meetings; Meetings by Telephone

Unless otherwise restricted by the certificate of incorporation or these bylaws, the Board may hold meetings within or outside the State of Delaware.

Unless otherwise restricted by the certificate of incorporation or these bylaws, members of the Board, or any committee designated by the Board, may participate in a meeting of the Board, or any committee, by means of conference telephone or other communications equipment by means of which all persons participating in the meeting can hear each other, and such participation in a meeting shall constitute presence in person at the meeting.

#### 2.6 Conduct of Business

Each meeting of the Board shall be presided over by the Chairperson of the Board, if any, or in his or her absence by the Vice Chairperson of the Board, if any, or in the absence of the foregoing persons by a chairperson of such meeting designated by the Board, or in the absence of such designation by a chairperson chosen at such meeting. The Secretary shall act as secretary of the meeting, but in his or her absence the chairperson of the meeting may appoint any person to act as secretary of such meeting.

# 2.7 Regular Meetings

Regular meetings of the Board may be held without notice at such time and at such place as shall from time to time be determined by the Board.

#### 2.8 Special Meetings; Notice

Special meetings of the Board for any purpose or purposes may be called at any time by the Chairperson of the Board, the Chief Executive Officer, the President, the Secretary or any two directors.

Notice of the time and place of all special meetings shall be:

- (i) delivered personally by hand, by courier or by telephone (including a voice messaging system or other technology designed to record and communicate messages) during normal business hours at least 24 hours before the date and time of the meeting;
  - (ii) sent by United States first-class mail, postage prepaid, deposited at least four days before the date and time of the meeting;
  - (iii) sent by facsimile during normal business hours at least 24 hours before the date and time of the meeting; or
  - (iv) sent by electronic mail during normal business hours at least 24 hours before the date and time of the meeting,

directed to each director at that director's address, telephone number, facsimile number or electronic mail address, as the case may be, as shown on the Company's records.

Any oral notice may be communicated to the director. The notice need not specify the place of the meeting (if the meeting is to be held at the Company's principal executive office) nor the purpose of the meeting.

# 2.9 Quorum; Voting

At all meetings of the Board, a majority of the total number of directors shall constitute a quorum for the transaction of business, unless the certificate of incorporation or these bylaws require a greater number. If a quorum is not present at any meeting of the Board, then the directors present thereat may adjourn the meeting from time to time, without notice other than announcement at the meeting, until a quorum is present. A meeting at which a quorum is initially present may continue to transact business notwithstanding the withdrawal of directors if any action taken is approved by at least a majority of the required quorum for that meeting.

The vote of the majority of the directors present at a meeting at which a quorum is present shall be the act of the Board, unless the certificate of incorporation or these bylaws requires a vote of a greater number.

If the certificate of incorporation provides that one or more directors shall have greater or fewer than one vote per director on any matter, every reference in these bylaws to a majority or other proportion of the directors.

#### 2.10 Board Action by Written Consent Without a Meeting

Unless otherwise restricted by the certificate of incorporation or these bylaws, any action required or permitted to be taken at any meeting of the Board, or of any committee thereof, may be taken without a meeting if all members of the Board or committee, as the case may be, consent thereto in writing (or by electronic transmission) and the writing or writings (or electronic transmissions) are filed with the minutes of proceedings of the Board or committee.

Such filing shall be in paper form if the minutes are maintained in paper form and shall be in electronic form if the minutes are maintained in electronic form.

#### 2.11 Fees and Compensation of Directors

Unless otherwise restricted by the certificate of incorporation or these bylaws, the Board shall have the authority to fix the compensation of directors.

# 2.12 Removal of Directors

Unless otherwise restricted by the DGCL, the certificate of incorporation or these bylaws, and assuming that the Company is not subject to Section 2115(b) of the CCC, any director or the entire Board may be removed, with or without cause, by the holders of a majority of the shares then entitled to vote at an election of directors. Whenever the holders of any class or series are entitled to elect one or more directors by the certificate of incorporation, this Section 2.12 shall apply, in respect to the removal without cause of a director or directors so elected, to the vote of the holders of the outstanding shares of that class or series and not to the vote of the outstanding shares as a whole.

During such time that the Company is subject to Section 2115(b) of the CCC, unless otherwise provided in the Certificate of Incorporation, and subject to applicable law and the rights of the holders of any series of Preferred Stock of the Company, any or all of the directors may be removed without cause by the affirmative vote of the holders of at least a majority of the outstanding shares entitled to vote on such removal, provided that, unless the entire Board is removed, no individual director may be removed when the votes cast against such director's removal, or not consenting in writing to such removal, would be sufficient to elect that director if voted cumulatively at an election at which the same total number of votes were cast (or, if such action is taken by written consent, all shares entitled to vote were voted) and the entire number of directors authorized at the time of such director's most recent election were then being elected.

#### ARTICLE III — COMMITTEES

# 3.1 Committees of Directors

The Board may designate one or more committees, each committee to consist of one or more of the directors of the Company. The Board may at any time increase or decrease the number of members of any committee or terminate the existence of any committee. The membership of a committee member shall terminate on the date of his or her death, resignation from such committee or the Board or removal from such committee or the Board. The Board may at any time and for any reason remove any individual committee member, and the Board may fill any committee vacancy created by a committee member's death, resignation or removal. The Board may designate one or more directors as alternate members of any committee, who may replace any absent or disqualified member at any meeting of the committee. In the absence or disqualification of a member of a committee, the member or members present at any meeting and not disqualified from voting, whether or not such member or members constitute a quorum, may unanimously appoint another member of the Board to act at the meeting in the place of any such absent or disqualified member. Any such committee, to the extent provided in the resolution of the Board or in these bylaws, shall

have and may exercise all the powers and authority of the Board in the management of the business and affairs of the Company, and may authorize the seal of the Company to be affixed to all papers that may require it; but no such committee shall have the power or authority to (i) approve or adopt, or recommend to the stockholders, any action or matter (other than the election or removal of directors) expressly required by the DGCL to be submitted to stockholders for approval or (ii) adopt, amend or repeal any bylaw of the Company.

# 3.2 **Committee Minutes**

Each committee shall keep regular minutes of its meetings and report the same to the Board when required.

#### 3.3 Meetings and Actions of Committees

Meetings and actions of committees shall be governed by, and be held and taken in accordance with, the provisions of:

- (i) Section 2.5 (Place of Meetings; Meetings by Telephone);
- (ii) Section 2.7 (Regular Meetings);
- (iii) Section 2.8 (Special Meetings; Notice);
- (iv) Section 2.9 (Quorum; Voting);
- (v) Section 2.10 (Board Action by Written Consent Without a Meeting); and
- (vi) Section 7.5 (Waiver of Notice),

with such changes in the context of those bylaws as are necessary to substitute the committee and its members for the Board and its members. However:

- (i) the time of regular meetings of a committee may be determined either by resolution of the Board or by resolution of such committee;
- (ii) special meetings of committees may also be called by resolution of the Board; and
- (iii) notice of special meetings of any committee shall also be given to all alternate members of such committee, who shall have the right to attend all meetings of such committee. The Board may adopt rules for the government of any committee not inconsistent with the provisions of these bylaws.

Any provision in the certificate of incorporation providing that one or more directors shall have more or less than one vote per director on any matter shall apply to voting in any committee or subcommittee, unless otherwise provided in the certificate of incorporation or these bylaws.

#### 3.4 Subcommittees

Unless otherwise provided in the certificate of incorporation, these bylaws or the resolution of the Board designating the committee, a committee may create one or more subcommittees, each subcommittee to consist of one or more members of the committee, and delegate to a subcommittee any or all of the powers and authority of the committee.

#### ARTICLE IV — OFFICERS

#### 4.1 Officers

The officers of the Company shall be a President and a Secretary. The Company may also have, at the discretion of the Board, a Chairperson of the Board (or Co-Chairpersons of the Board, each of whom acting alone shall, unless otherwise directed by the Board, have all the powers and duties of a Chairperson of the Board), a Vice Chairperson of the Board, a Chief Executive Officer, one or more Vice Presidents, a Chief Financial Officer, a Treasurer, one or more Assistant Treasurers, one or more Assistant Secretaries, and any such other officers as may be appointed in accordance with the provisions of these bylaws. Any number of offices may be held by the same person.

# 4.2 Appointment of Officers

The Board shall appoint the officers of the Company, except such officers as may be appointed in accordance with the provisions of Section 4.3.

#### 4.3 Subordinate Officers

The Board may appoint, or empower the Chief Executive Officer or President (in the absence of a Chief Executive Officer) to appoint, such other officers and agents as the business of the Company may require. Each of such other officers and agents shall hold office for such period, have such authority, and perform such duties as are provided in these bylaws or as the Board may from time to time determine.

# 4.4 Removal and Resignation of Officers

Any officer may be removed, with or without cause, by an affirmative vote of the majority of the Board or, except in the case of an officer chosen by the Board, by any officer upon whom such power of removal may be conferred by the Board.

Any officer may resign at any time by giving written notice to the Company. Any officer's resignation shall take effect at the date of the Company's receipt of such notice or at any later time specified in such notice. Unless otherwise specified in an officer's notice of resignation, the acceptance of the resignation shall not be necessary to make it effective. Any resignation is without prejudice to the rights, if any, of the Company under any contract to which the resigning officer is a party.

#### 4.5 Vacancies in Offices

Any vacancy occurring in any office of the Company shall be filled by the Board or as provided in Section 4.3.

## 4.6 Representation of Shares of Other Corporations

Unless otherwise directed by the Board, the President (or any other person authorized by the Board or the President) is authorized to vote, represent and exercise, on behalf of the Company, all rights incident to any and all shares or other securities or interests in, or issued by, any other corporation standing in the name of the Company. The authority granted herein may be exercised either by such person directly or by any other person authorized to do so by proxy or power of attorney duly executed by such person having the authority.

### 4.7 Authority and Duties of Officers

Except as otherwise provided in these bylaws, the officers of the Company shall have such powers and duties in the management of the Company as may be designated from time to time by the Board and, to the extent not so provided, as generally pertain to their respective offices, subject to the control of the Board.

#### ARTICLE V — INDEMNIFICATION

# 5.1 Indemnification of Directors and Officers in Third Party Proceedings

Subject to the other provisions of this Article V, the Company shall indemnify, to the fullest extent permitted by applicable law as it presently exists or may hereafter be amended, any director or officer of the Company who was or is a party or is threatened to be made a party to any threatened, pending or completed action, suit or proceeding other than an action by or in the right of the Company, whether civil, criminal, administrative or investigative (a "*Proceeding*"), by reason of the fact that such person is or was a director or officer of the Company, or is or was serving at the request of the Company as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by such person in connection with such Proceeding if such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the Company, and, with respect to any criminal action or proceeding, had no reasonable cause to believe such person's conduct was unlawful. The termination of any such Proceeding by judgment, order, settlement, conviction, or upon a plea of *nolo contendere* or its equivalent, shall not, of itself, create a presumption that such person did not act in good faith and in a manner which such person reasonably believed to be in or not opposed to the best interests of the Company, and, with respect to any criminal action or proceeding, had reasonable cause to believe that such person's conduct was unlawful.

# 5.2 <u>Indemnification of Directors and Officers in Actions by or in the Right of the Company</u>

Subject to the other provisions of this Article V, the Company shall indemnify, to the fullest extent permitted by applicable law as it presently exists or may hereafter be amended, any director or officer of the Company who was or is a party or is threatened to be made a party to any threatened, pending or completed action or suit by or in the right of the Company to procure a judgment in its favor by reason of the fact that such person is or was a director or officer of the Company, or is or was serving at the request of the Company as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys' fees) actually and reasonably incurred by such person in connection with the defense or settlement of any such action or suit if such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the Company, except that no indemnification shall be made in respect of any claim, issue or matter as to which such person shall have been adjudged to be liable to the Company unless and only to the extent that the Court of Chancery or the court in which such action or suit was brought shall determine upon application that, despite the adjudication of liability but in view of all the circumstances of the case, such person is fairly and reasonably entitled to indemnity for such expenses which the Court of Chancery or such other court shall deem proper.

#### 5.3 Successful Defense

To the extent that a present or former director or officer of the Company has been successful on the merits or otherwise in defense of any action, suit or proceeding described in Section 5.1 or Section 5.2, or in defense of any claim, issue or matter therein, such person shall be indemnified against expenses (including attorneys' fees) actually and reasonably incurred by such person in connection therewith.

# 5.4 Indemnification of Others

Subject to the other provisions of this Article V, the Company shall have the power to indemnify its employees and agents to the extent not prohibited by the DGCL or other applicable law. The Board shall have the power to delegate to such person or persons the determination of whether employees or agents shall be indemnified.

# 5.5 Advancement of Expenses

Expenses (including attorneys' fees) incurred by a director or officer of the Company in defending any civil, criminal, administrative or investigative action, suit or proceeding shall be paid by the Company in advance of the final disposition of such action, suit or proceeding upon receipt of a written request therefor (together with documentation reasonably evidencing such expenses) and an undertaking by or on behalf of such director or officer to repay such amount if it shall ultimately be determined that such person is not entitled to be indemnified by the Company under this Article V or the DGCL. Such expenses (including attorneys' fees) incurred by former directors and officers or other employees and agents of the Company may be so paid upon such terms and conditions, if any, as the Company deems appropriate. The right to advancement of expenses shall not apply to any action, suit or proceeding for which indemnity is excluded pursuant to these bylaws, but shall apply to any action, suit or proceeding referenced in Section 5.6(ii) prior to a determination that the person is not entitled to be indemnified by the Company.

#### 5.6 Limitation on Indemnification

Subject to the requirements in Section 5.3 and the DGCL, the Company shall not be obligated to indemnify any person pursuant to this Article V in connection with any action, suit or proceeding (or any part of any action, suit or proceeding):

- (i) for which payment has actually been made to or on behalf of such person under any statute, insurance policy, indemnity provision, vote or otherwise, except with respect to any excess beyond the amount paid;
- (ii) initiated by such person, including any action, suit or proceeding (or any part of any action, suit or proceeding) initiated by such person against the Company or any of its directors, officers, employees, agents or other indemnitees, unless (a) the Board authorized the action, suit or proceeding (or the relevant part of the action, suit or proceeding) prior to its initiation, (b) the Company provides the indemnification, in its sole discretion, pursuant to the powers vested in the Company under applicable law, (c) otherwise required to be made under Section 5.7, or (d) otherwise required by applicable law; or
  - (iii) if prohibited by applicable law.

#### 5.7 Determination; Claim

If a claim for indemnification or advancement of expenses under this Article V is not paid by the Company or on its behalf within 90 days after the Company's receipt of a written claim therefor, the claimant shall be entitled to an adjudication by a court of competent jurisdiction of his or her entitlement to such indemnification or advancement of expenses. To the extent not prohibited by applicable law, the Company shall indemnify such claimant against all expenses he or she actually and reasonably incurred in connection with any action for indemnification or advancement of expenses from the Company under this Article V, to the extent such claimant is successful in such action. In any such action, the Company shall, to the fullest extent no prohibited by law, have the burden of proving that the claimant is not entitled to the requested indemnification or advancement of expenses.

#### 5.8 Non-Exclusivity of Rights

The indemnification and advancement of expenses provided by, or granted pursuant to, this Article V shall not be deemed exclusive of any other rights to which those seeking indemnification or advancement of expenses may be entitled under the certificate of incorporation or any statute, bylaw, agreement, vote of stockholders or disinterested directors or otherwise, both as to action in such person's official capacity and as to action in another capacity while holding such office. The Company is specifically authorized to enter into individual contracts with any or all of its directors, officers, employees or agents respecting indemnification and advancement of expenses, to the fullest extent not prohibited by the DGCL or other applicable law.

#### 5.9 Insurance

The Company may purchase and maintain insurance on behalf of any person who is or was a director, officer, employee or agent of the Company, or is or was serving at the request of the Company as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise against any liability asserted against such person and incurred by such person in any such capacity, or arising out of such person's status as such, whether or not the Company would have the power to indemnify such person against such liability under the provisions of the DGCL.

#### 5.10 Survival

Subject to the terms contained in any indemnification agreement entered into between the Company and a director, officer, employee or agent, the indemnification and advancement of expenses provided by this Article V shall continue as to a person who has ceased to be a director, officer, employee or agent and shall inure to the benefit of the heirs, executors and administrators of such a person.

#### 5.11 Effect of Repeal or Modification

A right to indemnification or to advancement of expenses arising hereunder shall not be eliminated or impaired by an amendment hereof after the occurrence of the act or omission that is the subject of the civil, criminal, administrative or investigative action, suit or proceeding for which indemnification or advancement of expenses is sought.

# 5.12 **Certain Definitions**

For purposes of this Article V, references to the "Company" shall include, in addition to the resulting corporation, any constituent corporation (including any constituent of a constituent) absorbed in a consolidation or merger which, if its separate existence had continued, would have had power and authority to indemnify its directors, officers, employees or agents, so that any person who is or was a director, officer, employee or agent of such constituent corporation, or is or was serving at the request of such constituent corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, shall stand in the same position under this Article V with respect to the resulting or surviving corporation as such person would have with respect to such constituent corporation if its separate existence had continued. For purposes of this Article V, references to "other enterprises" shall include employee benefit plans; references to "fines" shall include any excise taxes assessed on a person with respect to an employee benefit plan; and references to "serving at the request of the Company" shall include any service as a director, officer, employee or agent of the Company which imposes duties on, or involves services by, such director, officer, employee or agent with respect to an employee benefit plan, its participants or beneficiaries; and a person who acted in good faith and in a manner such person reasonably believed to be in the interest of the participants and beneficiaries of an employee benefit plan shall be deemed to have acted in a manner "not opposed to the best interests of the Company" as referred to in this Article V.

#### ARTICLE VI - STOCK

#### 6.1 Stock Certificates; Partly Paid Shares

The shares of the Company shall be represented by certificates, provided that the Board may provide by resolution or resolutions that some or all of any or all classes or series of its stock shall be uncertificated shares. Any such resolution shall not apply to shares represented by a certificate until such certificate is surrendered to the Company. Every holder of stock represented by certificates shall be entitled to have a certificate signed by, or in the name of, the Company by the Chairperson of the Board or Vice-Chairperson of the Board, or the President or a Vice President, and by the Treasurer or an Assistant Treasurer, or the Secretary or an Assistant Secretary of the Company representing the number of shares registered in certificate form. Any or all of the signatures on the certificate may be a facsimile. In case any officer, transfer agent or registrar who has signed or whose facsimile signature has been placed upon a certificate has ceased to be such officer, transfer agent or registrar before such certificate is issued, it may be issued by the Company with the same effect as if such person were such officer, transfer agent or registrar at the date of issue. The Company shall not have power to issue a certificate in bearer form.

The Company may issue the whole or any part of its shares as partly paid and subject to call for the remainder of the consideration to be paid therefor. Upon the face or back of each stock certificate issued to represent any such partly paid shares, or upon the books and records of the Company in the case of uncertificated partly paid shares, the total amount of the consideration to be paid therefor and the amount paid thereon shall be stated. Upon the declaration of any dividend on fully paid shares, the Company shall declare a dividend upon partly paid shares of the same class, but only upon the basis of the percentage of the consideration actually paid thereon.

## 6.2 Special Designation on Certificates

If the Company is authorized to issue more than one class of stock or more than one series of any class, then the powers, designations, preferences and relative, participating, optional or other special rights of each class of stock or series thereof and the qualifications, limitations or restrictions of such preferences and/or rights shall be set forth in full or summarized on the face or back of the certificate that the Company shall issue to represent such class or series of stock, provided that, except as otherwise provided in Section 202 of the DGCL, in lieu of the foregoing requirements, there may be set forth on the face or back of the certificate which the Company shall issue to represent such class or series of stock, a statement that the Company will furnish without charge to each stockholder who so requests the powers, designations, preferences and relative, participating, optional or other special rights of each class of stock or series thereof and the qualifications, limitations or restrictions of such preferences and/or rights. Within a reasonable time after the issuance or transfer of uncertificated stock, the Company shall send to the registered owner thereof a written notice containing the information required to be set forth or stated on certificates pursuant to this Section 6.2 or Section 156, Section 202(a) or Section 218(a) of the DGCL or with respect to this

Section 6.2 a statement that the Company will furnish without charge to each stockholder who so requests the powers, designations, preferences and relative, participating, optional or other special rights of each class of stock or series thereof and the qualifications, limitations or restrictions of such preferences and/or rights. Except as otherwise expressly provided by law, the rights and obligations of the holders of uncertificated stock and the rights and obligations of the holders of certificates representing stock of the same class and series shall be identical.

#### **6.3 Lost Certificates**

Except as provided in this Section 6.3, no new certificates for shares shall be issued to replace a previously issued certificate unless the previously issued certificate is surrendered to the Company and cancelled at the same time. The Company may issue a new certificate of stock or uncertificated shares in place of any certificate theretofore issued by it, alleged to have been lost, stolen or destroyed, and the Company may require the owner of the lost, stolen or destroyed certificate, or such owner's legal representative, to give the Company a bond sufficient to indemnify it against any claim that may be made against it on account of the alleged loss, theft or destruction of any such certificate or the issuance of such new certificate or uncertificated shares.

### 6.4 Dividends

The Board, subject to applicable law and any restrictions contained in the certificate of incorporation, may declare and pay dividends upon the shares of the Company's capital stock. Dividends may be paid in cash, in property or in shares of the Company's capital stock, subject to applicable law and the certificate of incorporation.

The Board may set apart out of any of the funds of the Company available for dividends a reserve or reserves for any proper purpose and may abolish any such reserve.

# 6.5 Stock Transfer Agreements

The Company shall have power to enter into and perform any agreement with any number of stockholders of any one or more classes of stock of the Company to restrict the transfer of shares of stock of the Company of any one or more classes owned by such stockholders in any manner not prohibited by the DGCL.

## 6.6 Registered Stockholders

The Company:

- (i) shall be entitled to recognize the exclusive right of a person registered on its books as the owner of shares to receive dividends and to vote as such owner;
  - (ii) shall be entitled to hold liable for calls and assessments the person registered on its books as the owner of shares; and

(iii) shall not be bound to recognize any equitable or other claim to or interest in such share or shares on the part of another person, whether or not it shall have express or other notice thereof, except as otherwise provided by the laws of Delaware.

# 6.7 Transfers

Stock of the Company shall be transferable in the manner prescribed by law and in these bylaws. Transfers of stock of the Company shall be made on its books only by the person named as the holder thereof on the company's stock records, in person or by an attorney duly authorized in writing, and, if such stock is certificated, upon the surrender of the certificate thereof, which shall be cancelled before a new certificate or uncertificated shares shall be issued. No transfer of stock of the Company shall be valid as against the Company for any purpose until it shall have been entered in the Company's stock records by an entry showing from and to whom transferred.

#### ARTICLE VII — MANNER OF GIVING NOTICE AND WAIVER

#### 7.1 Notice of Stockholder Meetings

Notice of any meeting of stockholders, if mailed, is given when deposited in the United States mail, postage prepaid, directed to the stockholder at such stockholder's address as it appears on the Company's records. An affidavit of the Secretary or an Assistant Secretary or of the transfer agent or other agent of the Company that the notice has been given shall, in the absence of fraud, be *prima facie* evidence of the facts stated therein.

# 7.2 Notice by Electronic Transmission

Without limiting the manner by which notice otherwise may be given effectively to stockholders, any notice to stockholders given by the Company under any provision of the DGCL, the certificate of incorporation or these bylaws shall be effective if given by a form of electronic transmission consented to by the stockholder to whom the notice is given. Any such consent shall be revocable by the stockholder by written notice to the Company. Any such consent shall be deemed revoked if:

- (i) the Company is unable to deliver by electronic transmission two consecutive notices given by the Company in accordance with such consent; and
- (ii) such inability becomes known to the Secretary or an Assistant Secretary or to the transfer agent or other person responsible for the giving of notice.

However, the inadvertent failure to treat such inability as a revocation shall not invalidate any meeting or other action.

Any notice given pursuant to the preceding paragraph of this Section 7.2 shall be deemed given:

- (i) if by facsimile telecommunication, when directed to a number at which the stockholder has consented to receive notice;
- (ii) if by electronic mail, when directed to an electronic mail address at which the stockholder has consented to receive notice;
- (iii) if by a posting on an electronic network together with separate notice to the stockholder of such specific posting, upon the later of (A) such posting and (B) the giving of such separate notice; and
  - (iv) if by any other form of electronic transmission, when directed to the stockholder.

An affidavit of the Secretary or an Assistant Secretary or of the transfer agent or other agent of the Company that the notice has been given by a form of electronic transmission shall, in the absence of fraud, be *prima facie* evidence of the facts stated therein.

As used in these bylaws, an "electronic transmission" means any form of communication, not directly involving the physical transmission of paper, that creates a record that may be retained, retrieved and reviewed by a recipient thereof, and that may be directly reproduced in paper form by such a recipient through an automated process.

This Section 7.2 shall not apply to Section 164, Section 296, Section 311, Section 312 or Section 324 of the DGCL.

## 7.3 Notice to Stockholders Sharing an Address

Without limiting the manner by which notice otherwise may be given effectively to stockholders, any notice to stockholders given by the Company under any provision of the DGCL, the certificate of incorporation or these bylaws shall be effective if given by a single written notice to stockholders who share an address if consented to by the stockholders at that address to whom such notice is given. Any such consent shall be revocable by the stockholder by written notice to the Company. Any stockholder who fails to object in writing to the Company, within 60 days of having been given written notice by the Company of its intention to send the single notice permitted under this Section 7.3, shall be deemed to have consented to receiving such single written notice. This Section 7.3 shall not apply to Section 164, Section 296, Section 311, Section 312 or Section 324 of the DGCL.

#### 7.4 Notice to Person with Whom Communication is Unlawful

Whenever notice is required to be given, under the DGCL, the certificate of incorporation or these bylaws, to any person with whom communication is unlawful, the giving of such notice to such person shall not be required and there shall be no duty to apply to any governmental authority or

agency for a license or permit to give such notice to such person. Any action or meeting which shall be taken or held without notice to any such person with whom communication is unlawful shall have the same force and effect as if such notice had been duly given. In the event that the action taken by the Company is such as to require the filing of a certificate under the DGCL, the certificate shall state, if such is the fact and if notice is required, that notice was given to all persons entitled to receive notice except such persons with whom communication is unlawful.

#### 7.5 Waiver of Notice

Whenever notice is required to be given under any provision of the DGCL, the certificate of incorporation or these bylaws, a written waiver, signed by the person entitled to notice, or a waiver by electronic transmission by the person entitled to notice, whether before or after the time stated therein, shall be deemed equivalent to notice. Attendance of a person at a meeting shall constitute a waiver of notice of such meeting, except when the person attends a meeting for the express purpose of objecting, at the beginning of the meeting, to the transaction of any business because the meeting is not lawfully called or convened. Neither the business to be transacted at, nor the purpose of, any regular or special meeting of stockholders, directors or members of a committee of directors need be specified in any written waiver of notice or any waiver by electronic transmission unless so required by the certificate of incorporation or these bylaws.

#### ARTICLE VIII — GENERAL MATTERS

#### 8.1 Fiscal Year

The fiscal year of the Company shall be fixed by resolution of the Board and may be changed by the Board.

## 8.2 **Seal**

The Company may adopt a corporate seal, which shall be in such form as may be approved from time to time by the Board. The Company may use the corporate seal by causing it or a facsimile thereof to be impressed or affixed or in any other manner reproduced.

#### 8.3 Annual Report

The Company shall cause an annual report to be sent to the stockholders of the Company to the extent required by applicable law. If and so long as there are fewer than 100 holders of record of the Company's shares, the requirement of sending an annual report to the stockholders of the Company is expressly waived to the extent permitted by applicable law.

## 8.4 Construction; Definitions

Unless the context requires otherwise, the general provisions, rules of construction and definitions in the DGCL shall govern the construction of these bylaws. Without limiting the generality of this provision, the singular number includes the plural, the plural number includes the singular, and the term "person" includes both a corporation and a natural person.

## 8.5 Conflict with Applicable Law or Certificate of Incorporation

These bylaws are adopted subject to any applicable law and the certificate of incorporation. Whenever these bylaws may conflict with any applicable law or the certificate of incorporation, such conflict shall be resolved in favor of such law or the certificate of incorporation.

## ARTICLE IX — AMENDMENTS

These bylaws may be adopted, amended or repealed by the stockholders entitled to vote. However, the Company may, in its certificate of incorporation, confer the power to adopt, amend or repeal bylaws upon the directors. The fact that such power has been so conferred upon the directors shall not divest the stockholders of the power, nor limit their power, to adopt, amend or repeal bylaws.

A bylaw amendment adopted by stockholders which specifies the votes that shall be necessary for the election of directors shall not be further amended or repealed by the Board.

Execution Version CONFIDENTIAL

# 4D MOLECULAR THERAPEUTICS, INC.

## SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT

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Schedule 1 - Investors

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## 4D MOLECULAR THERAPEUTICS, INC.

#### SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT

This Second Amended and Restated Investors' Rights Agreement, dated as of August 27, 2018 (this "*Agreement*"), is entered into by and among 4D Molecular Therapeutics, Inc., a Delaware corporation (the "*Company*"), each holder of the Company's Preferred Stock, par value \$0.0001 per share (the "*Preferred Stock*"), listed on <u>Schedule 1</u> attached hereto (each, an "*Investor*" and, collectively, the "*Investors*"), and each Person (as defined below) listed on <u>Schedule 2</u> attached hereto (each, a "*Key Holder*" and collectively, the "*Key Holders*" and together with the Investors, the "*Stockholders*").

WHEREAS, certain of the Investors (the "*Prior Investors*") hold shares of Series A Preferred Stock, \$0.0001 par value per share, of the Company ("*Series A Preferred Stock*") and/or shares of Series A-1 Preferred Stock, \$0.0001 par value per share, of the Company ("*Series A-1 Preferred Stock*") (and/or shares of Common Stock (as defined below) issued upon conversion thereof), and possess registration rights, rights of first refusal and other rights pursuant to that certain Investors' Rights Agreement, dated as of October 6, 2015 (the "*Prior Agreement*"), by and among the Company, the Key Holders and the Prior Investors; and

WHEREAS, the Prior Investors are holders of at least a majority of the Registrable Securities then outstanding (as defined in the Prior Agreement) and the Key Holders hold a majority of the shares of capital stock of the Company held by all of the Key Holders (as defined in the Prior Agreement) who are providing services to the Company as directors, officers, employees or consultants, and the Prior Investors and the Key Holders desire to amend and restate the Prior Agreement in its entirety and accept the rights created pursuant to this Agreement in lieu of the rights granted to them under the Prior Agreement;

WHEREAS, the Company and certain of the Investors are parties to that certain Series B Preferred Stock Purchase Agreement, dated of even date herewith (the "Series B Purchase Agreement"), pursuant to which such Investors have agreed to purchase shares of Series B Preferred Stock, \$0.0001 par value per share, of the Company ("Series B Preferred Stock" and, together with the Series A Preferred Stock and Series A-1 Preferred Stock, the "Preferred Stock") and under which certain of the Company's and such Investors' obligations are conditioned upon the execution and delivery of this Agreement by the parties hereto;

WHEREAS, in order to induce the Company to enter into the Series B Purchase Agreement and to induce certain of the Investors to invest funds in the Company pursuant to the Series B Purchase Agreement, the parties hereby agree that this Agreement shall govern the rights of the Investors to cause the Company to register shares of Common Stock (as defined below), to receive certain information from the Company and to participate in future equity offerings by the Company, and shall govern certain other matters, all as set forth in this Agreement; and

WHEREAS, the parties also desire to enter into this Agreement to set forth their agreements and understandings with respect to how shares of the Company's capital stock held by them will be voted on, or tendered in connection with, certain matters.

NOW, THEREFORE, the parties to this Agreement hereby agree as follows:

- 1. <u>Definitions</u>. In addition to the terms defined elsewhere in this Agreement, the following terms used herein shall be construed to have the meanings set forth or referenced below:
- "Affiliate" means, with respect to any specified Person, any other Person who (i) directly or indirectly, controls, is controlled by, or is under common control with such specified Person, including any general partner, managing member, officer or director of such specified Person or any venture capital, private equity or similar investment fund now or hereafter existing which is controlled by one or more general partners or managing members of, or shares the same management company with, such specified Person or (ii) is an Immediate Family Member of or associated with such Person, including their respective trusts and other controlled entities.
  - "Board" means the Company's board of directors.
  - "Common Stock" means shares of Common Stock, par value \$0.0001 per share, of the Company.
- "Company IPO" means the Company's first underwritten public offering of Common Stock under the Securities Act that includes securities to be sold on behalf of the Company to the public.
- "Damages" means any loss, damage, claim or liability (joint or several) to which a Holder Indemnified Person (as defined in Section 2.8(a)) or a Company Indemnified Person (as defined in Section 2.8(b)) may become subject under the Securities Act, the Exchange Act or any state securities law in connection with a registration statement filed pursuant to Section 2, insofar as such loss, damage, claim or liability (or any action, claim or proceeding in respect thereof) arises out of or is based upon (i) any untrue statement or alleged untrue statement of a material fact contained in any such registration statement of the Company, including any preliminary prospectus or final prospectus contained therein or any amendments or supplements thereto, (ii) an omission or alleged omission to state therein a material fact required to be stated therein, or necessary to make the statements therein not misleading, or (iii) any violation or alleged violation by the indemnifying party (or any of its agents or Affiliates) of the Securities Act, the Exchange Act or any state securities law.
  - "Deemed Liquidation Event" has the meaning given to that term in the Restated Charter.
  - "Exchange Act" means the Securities Exchange Act of 1934, as amended.
- "Excluded Registration" means (i) a registration relating to the sale of securities to employees of the Company or any of its subsidiaries pursuant to a stock option, stock purchase or similar plan, (ii) a registration relating to a Rule 145 transaction, (iii) a registration on any form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of the Registrable Securities, or (iv) a registration in which the only Common Stock being registered is Common Stock issuable upon conversion of debt securities that are also being registered.

- "Form S-1" means such form registration statement under the Securities Act as in effect on the date of this Agreement or any successor form registration statement under the Securities Act subsequently adopted by the SEC.
- "Form S-3" means such form registration statement under the Securities Act as in effect on the date of this Agreement or any form registration statement under the Securities Act subsequently adopted by the SEC that permits incorporation of substantial information by reference to other documents filed by the Company with the SEC.
- "*Holder*" means any holder of Registrable Securities who is a party to this Agreement or any assignee of record of such Registrable Securities to whom registration rights set forth in Section 2 have been duly assigned in accordance with Section 2.13.
- "Immediate Family Member" means a spouse (or former spouse) or domestic partner, child or stepchild, grandchild, parent, stepparent, sibling, father-in-law, mother-in-law, son-in-law, daughter-or-law, brother-in-law, sister-in-law, grandparent, niece or nephew, including adoptive relationships, of a natural person referred to herein. A person shall be deemed to be a "domestic partner" of another person if the two persons (i) reside in the same residence and plan to do so indefinitely, (ii) have resided together for at least one year, (iii) are each at least 18 years of age and mentally competent to consent to contract, (iv) are not blood relatives closer than would prohibit legal marriage in the state in which they reside, (v) are financially interdependent, as demonstrated to the Company's reasonable satisfaction, and (vi) have each been the sole spousal equivalent of the other for the year prior to the determination of "domestic partner" status and plan to remain so indefinitely; provided, however, that a person shall not be deemed to be a "domestic partner" if he or she is married to another person or has any other spousal equivalent.
  - "Initiating Holders" means, collectively, Holders who properly initiate a registration request under this Agreement.
- "Major Investor" means any Investor that, individually or together with such Investor's Affiliates, holds at least 450,000 shares of Registrable Securities.
- "New Securities" means, collectively, (i) equity securities of the Company, whether or not currently authorized, (ii) rights, options or warrants to purchase equity securities of the Company, and (iii) securities of any type whatsoever that are, or may become, convertible or exchangeable into or exercisable for equity securities of the Company.
- "Other Shares" means shares of Common Stock, other than Registrable Securities, with respect to which registration rights have been granted by the Company.
- "*Person*" means an individual, a partnership, a corporation (including a business trust), a joint stock company, a limited liability company, an unincorporated association, a joint venture or other entity or a governmental authority.
- "*Preferred Directors*" means the Series A-1 Director (as defined below), so long as Pfizer is entitled to elect a Series A-1 Director, and the Series B Director (as defined below), so long as the holders of Series B Preferred Stock are entitled to elect a Series B Director.

"*Pfizer*" means (i) Pfizer Manufacturing LLC, a Delaware limited liability company, and Pfizer Production LLC, a Delaware limited liability company, acting for and on behalf of C.P. Pharmaceuticals International C.V., a Netherlands limited partnership (commanditaire vennootschap), and (ii) Pfizer Inc.

"Registrable Securities" means (i) the Common Stock issued or issuable upon conversion of the Preferred Stock and (ii) any Common Stock issued as (or issuable upon the conversion or exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange for or in replacement of, the shares referenced in clause (i) above; excluding, in all cases, however, (x) any Registrable Securities Transferred by a Person in a transaction in which the applicable rights under this Agreement are not assigned in accordance with the terms and provisions of this Agreement, (y) any Registrable Securities that have been previously registered, and (z) any Registrable Securities that have been sold to the public either pursuant to a registration statement or Rule 144, and excluding, for purposes of Section 2, any shares for which registration rights have terminated pursuant to Section 2.14.

"Registrable Securities then outstanding" means the number of shares determined by adding (i) the number of shares of outstanding Common Stock which are Registrable Securities that are then issued and outstanding and (ii) the number of shares of Common Stock issuable (directly or indirectly) pursuant to then exercisable and/or then convertible securities that are Registrable Securities.

- "Restated Charter" means the Company's Third Amended and Restated Certificate of Incorporation, as amended from time to time.
- "Restricted Securities" means (i) the Preferred Stock, (ii) the Registrable Securities, and (iii) any other securities issued in respect of the securities referenced in clauses (i) and (ii) upon any stock combination, stock split, stock dividend, recapitalization or other similar transaction.
- "ROFR and Co-Sale Agreement" means that certain Second Amended and Restated Right of First Refusal and Co-Sale Agreement, dated of even date herewith, by and among the Company and the other parties specified therein.
- "*Rule 144*" means Rule 144 promulgated by the SEC under the Securities Act, as amended from time to time, or any similar rule that may be promulgated by the SEC.
- "*Rule 145*" means Rule 145 promulgated by the SEC under the Securities Act, as amended from time to time, or any similar rule that may be promulgated by the SEC.
  - "SEC" means the U.S. Securities and Exchange Commission. "Securities Act" means the Securities Act of 1933, as amended.

"Selling Expenses" means, collectively, (i) all underwriting discounts, selling commissions and stock transfer taxes applicable to the sale of Registrable Securities and (ii) the fees and disbursements of legal counsel for any Holder, except for the fees and disbursements of the Selling Holder Counsel (as defined in Section 2.6) borne and paid by the Company as provided in Section 2.6.

"*Transfer*" means any sale, transfer, assignment, pledge, encumbrance or other disposition; provided, that any customary arrangement in connection with the deposit of Registrable Securities in a non-margin custodial account shall not be deemed a sale, transfer or pledge for purposes of this Agreement so long as such Registrable Securities are in certificated form (it being understood that the Company may require the exchange of any such certificated securities for book-entry shares upon the Company IPO).

"Viking" means Viking Global Opportunities Illiquid Investments Sub-Master LP and its Affiliates.

"Voting Shares" means and includes any securities of the Company the holders of which are entitled to vote for members of the Board, including all shares of Common Stock and Preferred Stock, by whatever name called, now owned or subsequently acquired by a Stockholder, however acquired, whether through stock splits, stock dividends, reclassifications, recapitalizations, similar events or otherwise.

2. Registration Rights. The Company covenants and agrees as follows:

## 2.1 Demand Registration.

(a) Form S-1 Demand. If, at any time after 180 days after the effective date of the registration statement for the Company IPO (or the subsequent date on which all lock-up periods applicable to the Company IPO have terminated), the Company receives a written request from Holders of at least 50% of the Registrable Securities then outstanding that the Company file a Form S-1 with respect to at least 50% of the Registrable Securities then outstanding (or a lesser percent if the anticipated aggregate offering price, net of Selling Expenses, would exceed \$30,000,000), then the Company shall (A) within 20 days after the Company's receipt of such request, give written notice thereof (the "Form S-1 Demand Notice") to all Holders other than the Initiating Holders and (B) as soon as practicable, and in any event within 60 days after the Company's receipt of such request, file a Form S-1 under the Securities Act covering all Registrable Securities that the Initiating Holders requested to be registered and any additional Registrable Securities requested to be included in such registration by any other Holders, as specified in a written notice given by each such Holder to the Company by no later than the 20th day after the date on which the Form S-1 Demand Notice is, pursuant to Section 8.5, deemed to have been delivered to such Holder, and, in each case, subject to the limitations of this Section 2. Any registration statement filed pursuant to this Section 2.1(a) may, subject to the provisions of Section 2.3, include Company Shares (as defined in Section 2.3(a)) or Other Shares.

(b) <u>Form S-3 Demand</u>. If, at any time when the Company is eligible to use a Form S-3, the Company receives a written request from Holders of at least 30% of the Registrable Securities then outstanding that the Company file a Form S-3 with respect to Registrable Securities then outstanding of such Holders having an anticipated aggregate offering price, net of Selling Expenses, of at least \$5,000,000, then the Company shall (i) within 20 days after the Company's receipt of such request, give a written notice thereof (the "Form S-3 Demand Notice") to all Holders other than the Initiating Holders and (ii) as soon as practicable, and in any event within 45 days after the Company's receipt of such request, file a Form S-3

under the Securities Act covering all Registrable Securities that the Initiating Holders requested to be registered and any additional Registrable Securities requested to be included in such registration by any other Holders, as specified in a written notice given by each such Holder to the Company by no later than the tenth day after the date on which the Form S-3 Demand Notice is, pursuant to Section 8.5, deemed to have been delivered to such Holder, and, in each case, subject to the limitations of this Section 2. Any registration statement filed pursuant to this Section 2.1(b) may, subject to the provisions of Section 2.3, include Company Shares (as defined in Section 2.3(a)) or Other Shares.

- (c) <u>Deferral</u>. Notwithstanding the foregoing obligations in Section 2.1(a) and Section 2.1(b), if the Company furnishes to the Holders requesting a registration pursuant to this Section 2.1 a certificate signed by the Company's Chief Executive Officer or President stating that, in the good faith judgment of the Board, it would be materially detrimental to the Company and its stockholders for such registration statement to be filed and it is therefore necessary to defer the filing of such registration statement, then the Company shall have the right to defer taking action with respect to such filing, and any time periods with respect to filing or effectiveness thereof shall be tolled correspondingly, for a period of not more than 120 days after delivery to the Company by the Initiating Holders of such registration request; <u>provided</u>, <u>however</u>, that the Company may not invoke this right more than once in any 12-month period; <u>provided</u>, <u>further</u>, that the Company shall not register any securities for its own account or that of any other stockholder during such 120-day period other than pursuant to an Excluded Registration.
- (d) Limitations. The Company shall not be obligated to effect, or to take any action to effect, any registration pursuant to Section 2.1(a): (i) during the period that is 60 days before the Company's good faith estimate of the date of filing of, and ending on a date that is 180 days after the effective date of, a Company-initiated registration, provided that the Company is actively employing, in good faith, commercially reasonable efforts to cause such registration statement to become effective; (ii) if the Company has already effected two (2) registrations pursuant to Section 2.1(a); or (iii) if the Initiating Holders propose to dispose of shares of Registrable Securities that may be immediately registered on Form S-3 pursuant to a request made pursuant to Section 2.1(b). The Company shall not be obligated to effect, or to take any action to effect, any registration pursuant to Section 2.1(b): (i) during the period that is 30 days before the Company's good faith estimate of the date of filing of, and ending on a date that is 90 days after the effective date of, a Company-initiated registration, provided that the Company is actively employing, in good faith, commercially reasonable efforts to cause such registration statement to become effective; or (ii) if the Company has already effected two (2) registrations pursuant to Section 2.1(b) within the 12-month period immediately preceding the date of such request. A registration shall not be counted as "effected" for purposes of this Section 2.1(d) until such time as the applicable registration statement has been declared effective by the SEC; provided, however, that if (i) Holders of a majority of the Registrable Securities to be registered withdraw the request for such registration or a sufficient number of Holders withdraw from such registration so that the minimum offering conditions set forth in Section 2.1(a) or Section 2.1(b), as applicable, are no longer satisfied and (ii) the Holders of a majority of the Registrable Securities agree to forfeit their right to one registration pursuant to Section 2.1(a) or Section 2.1(b), as the case may be, as described in Section 2.6, with the Company paying the Withdrawn Registration Expenses (as defined in Section 2.6), then such withdrawn registration statement shall be counted as "effected" for purposes of this Section 2.1(d).

2.2 <u>Company Registration</u>. If the Company proposes to register (including, for this purpose, a registration effected by the Company for stockholders other than the Holders) any of its Common Stock under the Securities Act in connection with the public offering of such securities solely for cash (other than in an Excluded Registration), then the Company shall, at such time, promptly give each Holder written notice of such registration (the "*Company Notice*"). Upon the written request of each Holder given to the Company by no later than the 20th day after the date on which the Company Notice is, pursuant to Section 8.5, deemed to have been delivered to such Holder, the Company shall, subject to the provisions of Section 2.3, cause to be registered all of the Registrable Securities that each such Holder has so requested to be included in such registration. The Company shall have the right to terminate or withdraw any registration initiated by it under this Section 2.2 before the effective date of such registration, whether or not any Holder has elected to include Registrable Securities in such registration. The expenses of such withdrawn registration (other than Selling Expenses) shall be borne by the Company in accordance with Section 2.6.

## 2.3 <u>Underwriting Requirements</u>.

(a) If, pursuant to Section 2.1, the Initiating Holders intend to distribute the Registrable Securities covered by their request by means of an underwriting, they shall so advise the Company as part of their request made pursuant to Section 2.1, and the Company shall include such information in the Form S-1 Demand Notice. The underwriter(s) will be selected by the Company and shall be reasonably acceptable to a majority in interest of the Initiating Holders. If, within 20 days after the Company's receipt of a written registration request pursuant to Section 2.1, the Company delivers to the Initiating Holders a written request to include in such registration (x) securities being sold for the Company's own account ("Company Shares") or (y) Other Shares, then the Initiating Holders shall, on behalf of all Holders, offer to include the Company Shares and such Other Shares in the underwriting. All Holders proposing to distribute their securities through such underwriting shall, together with the Company as provided in Section 2.4(e) and the holders of any Other Shares that are to be included in such underwriting and registration (such holders, the "Other Holders"), enter into an underwriting agreement in customary form with the underwriter(s) selected for such underwriting. Notwithstanding any other provision of this Section 2, if the managing underwriter(s) advise(s) the Initiating Holders, in writing, that marketing factors require a limitation on the number of shares to be underwritten, then the Initiating Holders shall so notify the Company, all Holders of Registrable Securities that otherwise would be underwritten and registered and all Other Holders, in writing, and the number of Registrable Securities, Company Shares and Other Shares that may be included in such underwriting and registration shall be allocated as follows: (i) first, among all Holders that requested inclusion of any Registrable Securities in such registration statement, including the Initiating Holders, in proportion (as nearly as practicable) to the number of Registrable Securities held by each selling Holder (or in such other proportion as shall mutually be agreed to, in writing, by all such selling Holders), provided, however, that the number of Registrable Securities held by the Holders to be included in such underwriting shall not be reduced unless all other securities are first entirely excluded from the underwriting; (ii) second, to the Other Holders; and (iii) third, to the Company. To facilitate the allocation of shares in accordance with the foregoing provisions of this Section 2.3(a), the Company or the underwriter(s) may round the number of shares allocated to any Holder or Other Holder, as the case may be, to the nearest 100 shares.

(b) In connection with any offering involving an underwriting of shares of the Company's capital stock pursuant to Section 2.2 (each, a "Company Offering"), the Company will not be required to include any Registrable Securities in such Company Offering unless the Holders of the Registrable Securities to be included in such Company Offering accept the terms of the underwriting as agreed upon between the Company and the underwriter(s) selected by the Company (and enter into an underwriting agreement in customary form with the underwriter(s)selected for such Company Offering), and then only in such quantity, as determined in the sole discretion of the underwriter(s) and the Company, as will not jeopardize the success of such Company Offering. If the total number of securities, including Registrable Securities, requested by stockholders of the Company to be included in a Company Offering exceeds the number of securities to be sold (other than by the Company) that the underwriter(s) and the Company determine is compatible with the success of such Company Offering, then the Company will be required to include in such Company Offering only that number of such securities, including Registrable Securities, which the underwriter(s) and the Company in their sole discretion determine will not jeopardize the success of such Company Offering. If the underwriter(s) and the Company determine that less than all of the Registrable Securities requested to be registered can be included in a Company Offering, then the Registrable Securities that are included in such Company Offering shall be allocated among the selling Holders in proportion (as nearly as practicable) to the number of Registrable Securities held by each selling Holder (or in such other proportion as shall mutually be agreed to, in writing, by all such selling Holders). To facilitate the allocation of shares in accordance with the foregoing provisions of this Section 2.3(b), the Company or the underwriter(s) may round the number of shares allocated to any Holder or Other Holder, as the case may be, to the nearest 100 shares. Notwithstanding the foregoing, in no event shall the number of Registrable Securities to be included in a Company Offering (i) be reduced unless all other securities (other than securities to be sold by the Company) are first entirely excluded from such Company Offering or (ii) be reduced below 30% of the total number of securities included in such Company Offering, unless such Company Offering is the Company IPO, in which case the selling Holders may be excluded further if the underwriter(s) and the Company make the determination described above and no other stockholder's securities are included in such Company Offering. For purposes of the provisions in this Section 2.3(b) concerning apportionment, for any selling Holder that is a partnership, limited liability company or corporation, the partners, members, retired partners, retired members, stockholders and Affiliates of such Holder, or the estates and Immediate Family Members of any such partners, members, retired partners and retired members and any trusts for the benefit of any of the foregoing Persons, shall be deemed to be a single "selling Holder," and any pro rata reduction with respect to such "selling Holder" shall be based upon the aggregate number of Registrable Securities owned by all Persons included in such "selling Holder," as defined in this sentence.

(c) For purposes of <u>Section 2.1</u>, a registration shall not be counted as "effected" if, as a result of an exercise of the underwriters' and the Company's cutback in <u>Section 2.3(a)</u>, fewer than fifty percent (50%) of the total number of Registrable Securities that Holders have requested to be included in such registration statement are actually included.

- 2.4 <u>Obligations of the Company</u>. Whenever required under this Section 2 to effect the registration of any Registrable Securities, the Company shall use its commercially reasonable efforts to:
- (a) prepare and file with the SEC a registration statement with respect to such Registrable Securities and cause such registration statement to become effective and, upon the request of the Holders of a majority of the Registrable Securities registered thereunder, keep such registration statement effective for a period of up to 90 days or, if shorter, until the distribution contemplated therein has been completed; <u>provided</u>, <u>however</u>, that such 90-day period shall be extended for a period of time equal to the period that the Holders refrain, at the request of an underwriter of Common Stock (or other securities) of the Company, from selling any securities included in such registration;
- (b) prepare and file with the SEC such amendments and supplements to the registration statement with respect to such Registrable Securities, or prospectus forming a part thereto, as may be necessary to comply with the Securities Act in order to enable the disposition of all Registrable Securities covered by such registration statement for the period set forth in Section 2.4(a);
- (c) furnish to the selling Holders such number of copies of a prospectus (including a preliminary prospectus) as required by the Securities Act and such other documents incident thereto as such Holders may reasonably request in order to facilitate the disposition of their Registrable Securities included in such registration;
- (d) register and qualify the Registrable Securities covered by the registration statement under such other securities or blue sky laws of such jurisdictions as shall be reasonably requested by the selling Holders; <u>provided</u>, <u>however</u>, that the Company shall not be required to qualify to do business, or to file a general consent to service of process, in any such states or jurisdictions;
- (e) in the event of any underwritten public offering, enter into an underwriting agreement, in usual and customary form, with the underwriter(s) of such offering, provided that each Holder participating in such underwriting also enters into such agreement;
- (f) cause all Registrable Securities covered by the registration statement to be listed on a national securities exchange or trading system and each securities exchange and trading system (if any) on which similar securities issued by the Company are then listed;
- (g) provide a transfer agent and registrar for all Registrable Securities registered pursuant to this Agreement and provide a CUSIP number for all such Registrable Securities, in each case not later than the effective date of such registration;
- (h) promptly make all financial and other records, pertinent corporate documents and properties of the Company available for inspection by the selling Holders, any managing underwriter(s) participating in any disposition pursuant to the registration statement with respect to such Registrable Securities and any attorney, accountant or other agent retained by any such selling Holders or underwriter(s) and cause the Company's directors, officers, employees and independent accountants to supply all information reasonably requested by any such selling Holder, underwriter, attorney, accountant or agent, in each case, as necessary or advisable to verify the accuracy of the information in such registration statement and to conduct appropriate due diligence in connection therewith;

- (i) notify each selling Holder, promptly after the Company receives notice thereof, of the time when the registration statement with respect to such Registrable Securities has been declared effective or a supplement to any prospectus forming a part thereto has been filed; and
- (j) notify each selling Holder, after a registration statement with respect to such Registrable Securities becomes effective, of any request by the SEC that the Company amend or supplement such registration statement or prospectus forming a part thereto.

In addition, the Company shall ensure that, at all times after any registration statement covering a public offering of the Company's securities under the Securities Act shall have become effective, its insider trading policy shall provide that the Company's directors may implement a trading program under Rule 10b5-1 of the Exchange Act.

- 2.5 <u>Furnish Information</u>. It shall be a condition precedent to the Company's obligation to take any action pursuant to this Section 2 with respect to the Registrable Securities of any selling Holder that such Holder shall have furnished to the Company such information regarding himself/herself/itself, the Registrable Securities held by him/her/it, and the intended method of disposition of such Registrable Securities as is reasonably required in connection with any registration, qualification or compliance referred to in this Section 2.
- 2.6 Expenses of Registration. All expenses (other than Selling Expenses) incurred in connection with registrations pursuant to Section 2.1(a) and Section 2.2 and the first registration pursuant to Section 2.1(b), including all registration, filing and qualification fees, printers' and accounting fees, fees and disbursements of legal counsel for the Company, and the reasonable fees and disbursements (not to exceed \$25,000) of one legal counsel for the selling Holders (the "Selling Holder Counsel"), shall be borne and paid by the Company; provided, however, that the Company shall not be required to pay for any expenses of any registration proceeding begun pursuant to Section 2.1 if such registration request is subsequently withdrawn at the request of the Holders of a majority of the Registrable Securities to be registered or because a sufficient number of Holders have withdrawn from such registration so that the minimum offering conditions set forth in Section 2.1(a) or Section 2.1(b), as applicable, are no longer satisfied (such expenses, "Withdrawn Registration Expenses") (in which case, all participating Holders shall bear such Withdrawn Registration Expenses pro rata based upon the number of Registrable Securities that were to be included by each such Holder in such withdrawn registration), unless the Holders of a majority of the Registrable Securities agree to forfeit their right to one registration pursuant to Section 2.1(a) or Section 2.1(b), as the case may be (in which case, such right shall be forfeited by all Holders and the Company shall pay for such Withdrawn Registration Expenses); provided, further, that, if, at the time of such withdrawal, the Holders requesting withdrawal (x) shall have learned of a material adverse change in the condition, business or prospects of the Company from that known to the Holders at the time of their registration request and (y) have withdrawn their request with reasonable promptness after learning of such material adverse change, then the Holders shall not be required

such Withdrawn Registration Expenses and shall not forfeit their right to one registration pursuant to Section 2.1(a) or Section 2.1(b), as the case may be. Notwithstanding anything to the contrary contained herein, (i) all Selling Expenses and fees and disbursements of the Selling Holder Counsel in excess of \$25,000 shall be borne and paid by the Holders pro rata based upon the number of Registrable Securities registered on their behalf (or, in the case of a withdrawn registration, the number of Registrable Securities that were to be included on their behalf) and (ii) all expenses incurred in connection with any registration pursuant to Section 2.1(b) after the first such registration shall be borne and paid by the Holders who participate in such registration pro rata based upon the number of Registrable Securities registered on their behalf (or, in the case of a withdrawn registration, the number of Registrable Securities that were to be included on their behalf).

2.7 <u>Delay of Registration</u>. No Holder shall have any right to take any action to restrain, enjoin or otherwise delay any registration pursuant to this Agreement as the result of any controversy that might arise with respect to the interpretation or implementation of this Section 2.

## 2.8 Indemnification.

- (a) By the Company. If any Registrable Securities are included in a registration statement under this Section 2, then, to the extent permitted by law, the Company will indemnify and hold harmless each selling Holder, the partners, members, directors, officers and stockholders of each such Holder, legal counsel and accountants for each such Holder, any underwriter (as defined in the Securities Act) for each such Holder and each Person, if any, who controls such Holder or underwriter within the meaning of the Securities Act or the Exchange Act (each, a "Holder Indemnified Person") against any Damages, and the Company will pay to each Holder Indemnified Person any legal or other expenses reasonably incurred by him/her/it, within three months after a request for reimbursement has been received by the Company, in connection with investigating or defending any action, claim or proceeding from which Damages may result; provided, however, that the indemnity agreement contained in this Section 2.8(a) shall not apply to amounts paid in settlement of any such action, claim or proceeding if such settlement is effected without the Company's prior written consent, which consent shall not be unreasonably withheld, nor shall the Company be liable for any Damages to the extent that they arise out of or are based upon statements, actions, omissions or violations made in reliance upon, and in conformity with, written information furnished by or on behalf of any such Holder Indemnified Person expressly for use in connection with such registration.
- (b) <u>By Selling Holders</u>. If any Registrable Securities are included in a registration statement under this Section 2, then, to the extent permitted by law, each selling Holder, severally and not jointly, will indemnify and hold harmless the Company, the directors, officers and partners of the Company, legal counsel and accountants for the Company, any underwriter (as defined in the Securities Act) and each Person, if any, who controls the Company or such underwriter within the meaning of the Securities Act or the Exchange Act (each, a "*Company Indemnified Person*") against any Damages, and such selling Holder will pay to each Company Indemnified Person any legal or other expenses reasonably incurred by him/her/it, within three months after a request for reimbursement has been received by such selling Holder, in connection with investigating or defending any action, claim or proceeding from which

Damages may result, in each case only to the extent that such Damages arise out of or are based upon statements, actions, omissions or violations made in reliance upon, and in conformity with, written information furnished by or on behalf of such selling Holder expressly for use in connection with such registration; provided, however, that the indemnity agreement contained in this Section 2.8(b) shall not apply to amounts paid in settlement of any such action, claim or proceeding if such settlement is effected without such selling Holder's prior written consent, which consent shall not be unreasonably withheld; provided, further, that in no event shall the aggregate amounts payable by any selling Holder by way of indemnity under this Section 2.8(b) or contribution under Section 2.8(d) exceed the proceeds from the offering received by such Holder (net of any Selling Expenses paid by such Holder), except in the case of fraud or willful misconduct by such Holder.

(c) Notice. Promptly after (i) receipt by a Holder Indemnified Person or a Company Indemnified Person (each, an "Indemnified Party") of notice of the commencement of any action, claim or proceeding (including any governmental action, claim or proceeding) for which a party may be entitled to indemnification hereunder or (ii) an Indemnified Party has actual knowledge of any claim as to which indemnity may be sought hereunder, such Indemnified Party shall, if a claim in respect thereof is to be made against any indemnifying party under this Section 2.8 (each, an "Indemnifying Party"), give the Indemnifying Party written notice thereof. The Indemnifying Party shall have the right to (x) participate in such action, claim or proceeding and, to the extent the Indemnifying Party so desires, participate jointly with any other Indemnifying Party to which written notice has been given and (y) assume the defense thereof with legal counsel approved by the Indemnified Party (whose approval shall not be unreasonably withheld); provided, however, that the Indemnified Party shall have the right to retain one separate counsel, with the fees and expense to be paid by the Indemnifying Party, if representation of such Indemnified Party by the counsel retained by the Indemnifying Party would be inappropriate due to actual or potential differing interests between such Indemnified Party and any other party represented by such counsel in such action. The failure to give timely written notice to the Indemnifying Party as provided in this Section 2.8(c) (1) shall relieve such Indemnifying Party of any liability to the Indemnified Party of any liability to the Extent that such failure materially prejudices the Indemnifying Party of any liability to the Indemnified Party otherwise than under this Section 2.8. Each Indemnified Party shall furnish such information regarding such Indemnified Party or the claim in question as the Indemnifying Party may reasonably request in writing and as shall be reasonably required in connection with the def

(d) <u>Contribution</u>. To provide for just and equitable contribution to joint liability under the Securities Act in any case in which either (i) any party otherwise entitled to indemnification hereunder makes a claim for indemnification pursuant to this Section 2.8 but it is judicially determined (by the entry of a final judgment or decree by a court of competent jurisdiction and the expiration of time to appeal or the denial of the last right of appeal) that such indemnification may not be enforced in such case, notwithstanding the fact that this Section 2.8 provides for indemnification in such case, or (ii) contribution under the Securities Act may be required on the part of any party hereto for which indemnification is provided under this Section 2.8, then, and in each such case, such parties shall contribute to the aggregate losses, damages, claims, liabilities or expenses to which they may be subject (after contribution from others) in

such proportion as is appropriate to reflect the relative fault of each of the Indemnifying Party and the Indemnified Party in connection with the statements, actions, omissions or violations that resulted in such loss, damage, claim, liability or expense, as well as to reflect any other relevant equitable considerations. The relative fault of the Indemnifying Party and of the Indemnified Party shall be determined by reference to, among other things, whether the untrue or alleged untrue statement of a material fact, or the omission or alleged omission of a material fact, relates to information supplied by the Indemnifying Party or by the Indemnified Party and the parties' relative intent, knowledge, access to information and opportunity to correct or prevent such statement or omission; provided, however, that, (x) no Holder will be required to contribute any amount in excess of the public offering price of all such Registrable Securities offered and sold by such Holder pursuant to such registration statement, and (y) in any such case, no Person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) will be entitled to contribution from any Person who was not guilty of such fraudulent misrepresentation; and provided further that in no event shall a Holder's liability pursuant to this Section 2.8(d), when combined with the amounts paid or payable by such Holder pursuant to Section 2.8(b), exceed the proceeds from the offering received by such Holder, except in the case of willful misconduct or fraud by such Holder.

- (e) <u>Conflict with Underwriting Agreement</u>. Notwithstanding the foregoing provisions of this Section 2.8, to the extent that the provisions on indemnification and contribution contained in the underwriting agreement entered into in connection with an underwritten public offering under this Section 2 are in conflict with the foregoing provisions of this Section 2.8, the provisions in the underwriting agreement shall control.
- (f) <u>Survival</u>. Unless otherwise superseded by an underwriting agreement entered into in connection with an underwritten public offering under this Section 2, the obligations of the Company and the Holders pursuant to this Section 2.8 shall survive the completion of any offering of Registrable Securities in a registration under this Section 2 and otherwise shall survive the termination of this Agreement.
- 2.9 <u>Reports Under Exchange Act</u>. With a view to making available to the Holders the benefits of Rule 144 and any other rule or regulation of the SEC that may at any time permit a Holder to sell Registrable Securities to the public pursuant to a registration on Form S-3 or without registration, the Company shall use its commercially reasonable efforts to:
- (a) at any time from and after 90 days following the effective date of the registration statement filed by the Company for the Company IPO, make and keep available adequate current public information (as those terms are understood and defined in Rule 144) with respect to the Company;
- (b) at any time after the Company has become subject to such reporting requirements, file with the SEC in a timely manner all reports and other documents required of the Company under the Securities Act and the Exchange Act; and

- (c) so long as a Holder owns any Registrable Securities, furnish to such Holder upon his/her/its written request (i) to the extent accurate, a written statement by the Company that (A) it has complied with the reporting requirements of Rule 144 (at any time from and after 90 days following the effective date of the registration statement filed by the Company for the Company IPO), the Securities Act and the Exchange Act (at any time after the Company has become subject to such reporting requirements) or (B) it qualifies as a registrant whose securities may be resold pursuant to Form S-3 (at any time after the Company so qualifies to use such form) and (ii) such other information as may be reasonably requested by such Holder in availing himself/herself/itself of any rule or regulation of the SEC that permits the selling of such securities of the Company to the public pursuant to a registration on Form S-3 (at any time after the Company so qualifies to use such form) or without registration (at any time after the Company has become subject to the reporting requirements under the Exchange Act).
- 2.10 <u>Limitations on Subsequent Registration Rights</u>. From and after the date of this Agreement, the Company shall not, without the prior written consent of the holders of at least a majority of the Registrable Securities, enter into any agreement with any holder or prospective holder of any securities of the Company giving such holder or prospective holder any registration rights the terms of which are pari passu with or senior to the registration rights granted to the Holders hereunder; <u>provided</u> that this limitation shall not apply to Registrable Securities acquired by any additional Investor that becomes a party to this Agreement in accordance with Subsection 8.13(a).
- 2.11 Lock-Up Period. Each Holder hereby agrees that such Holder will not, during the period commencing on the date of the final prospectus relating to the Company IPO and ending on the date specified by the Company and the managing underwriter(s) (such period not to exceed 180 days, or such other period as may be requested by the Company or the underwriter(s) to accommodate regulatory restrictions on (i) the publication or other distribution of research reports and (ii) analyst recommendations and opinions, including the restrictions contained in FINRA Rule 2241 (or any successor provisions or amendments thereto), as applicable), (a) sell, dispose of, make any short sale of, offer, hypothecate, pledge, contract to sell, grant or sell any option or contract to purchase, purchase any option or contract to sell, grant any right or warrant to purchase, lend or otherwise transfer or encumber, directly or indirectly, any shares of Common Stock or other securities convertible into or exercisable or exchangeable (directly or indirectly) for shares of Common Stock held immediately prior to the effectiveness of the Registration Statement for such Offering (such shares and other securities, the "Lock-Up Shares") or (b) enter into any swap, hedging or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of any Lock-Up Shares, whether any such transaction described in clause (a) or (b) above is to be settled by delivery of Common Stock or other securities, in cash or otherwise. The foregoing provisions of this Section 2.11 (1) shall not apply to the sale of any Lock-Up Shares to an underwriter pursuant to an underwriting agreement and (2) shall be applicable to the Holders only if all directors and officers of the Company are subject to similar restrictions and the Company uses commercially reasonable effort to obtain a similar agreement from all stockholders individually owning more than one percent (1%) of the Company's outstanding Common Stock (after giving effect to conversion into Common Stock of all outstanding shares of Preferred Stock). The underwriters for any registered offering described in this Section 2.11 are intended third party beneficiaries of this Section 2.11 and shall have the right, power and authority to enforce the provisions of this Section 2.11 as though they were parties hereto. Each Holder further agrees to execute such agreements as may be reasonably requested by the underwriter(s) in connection with any registered offering described in this Section 2.11 and that are consistent with this Section 2.11 or necessary to give further effect thereto. Any discretionary waiver or termination of the restrictions of any or all of such agreements by the Company or the underwriter(s) shall apply pro rata to all Holders subject to such agreements based on the number of shares subject to such agreements.

#### 2.12 Restrictions on Transfer.

- (a) The Restricted Securities, and any beneficial interest therein, shall not be Transferred, and the Company will not recognize, and will issue stop-transfer instructions to its transfer agent with respect to, any such Transfer, except upon the conditions specified in this Agreement, which conditions are intended to ensure compliance with the provisions of the Securities Act. Each Holder shall cause any proposed purchaser, pledgee or transferee of any Restricted Securities held by such Holder to agree, in a written instrument delivered to the Company, to take and hold such securities subject to the provisions, and upon the conditions, specified in this Agreement (including the obligations set forth in Section 2.11).
- (b) Each certificate evidencing any Restricted Securities shall (unless otherwise permitted by the provisions of Section 2.12(c)) bear the following legends (or substantially equivalent legends) in addition to any legends required under applicable securities laws:

THE SECURITIES REPRESENTED BY THIS CERTIFICATE HAVE BEEN ACQUIRED FOR INVESTMENT AND NOT WITH A VIEW TO, OR IN CONNECTION WITH, THE SALE OR DISTRIBUTION THEREOF AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED (THE "ACT"), OR ANY STATE SECURITIES LAWS. THEY MAY NOT BE OFFERED, SOLD OR OTHERWISE TRANSFERRED, PLEDGED OR HYPOTHECATED EXCEPT AS PERMITTED UNDER THE ACT AND APPLICABLE STATE SECURITIES LAWS PURSUANT TO AN EFFECTIVE REGISTRATION STATEMENT OR AN EXEMPTION THEREFROM. THE ISSUER OF THESE SECURITIES MAY REQUIRE AN OPINION OF COUNSEL SATISFACTORY TO THE ISSUER THAT SUCH OFFER, SALE, TRANSFER, PLEDGE OR HYPOTHECATION OTHERWISE COMPLIES WITH THE ACT AND ANY APPLICABLE STATE SECURITIES LAWS.

THE SECURITIES REPRESENTED BY THIS CERTIFICATE ARE SUBJECT TO THE TERMS OF AGREEMENTS BETWEEN THE ISSUER AND THE ORIGINAL HOLDER OF SUCH SECURITIES (COPIES OF WHICH ARE ON FILE WITH THE SECRETARY OF THE ISSUER) AND BY ACCEPTING ANY INTEREST IN SUCH SECURITIES THE PERSON ACCEPTING SUCH INTEREST SHALL BE DEEMED TO AGREE TO, AND SHALL BECOME BOUND BY, ALL OF THE PROVISIONS OF THOSE AGREEMENTS, INCLUDING CERTAIN RESTRICTIONS ON TRANSFER AND OWNERSHIP SET FORTH THEREIN.

The parties hereby agree that the failure to cause the certificates, if any, evidencing Restricted Securities to bear the legends required by this Section 2.12(b) shall not affect the validity or enforcement of this Agreement. In order to enforce the provisions hereof, the Company may issue appropriate stop-transfer instructions to its transfer agent, if any, and if the Company transfers its own securities, it may make appropriate notations to the same effect in its own records.

- (c) Each holder of Restricted Securities, by acceptance thereof, agrees to comply in all respects with the provisions of this Section 2. Before any proposed Transfer of any Restricted Securities, unless there is in effect a registration statement under the Securities Act covering the proposed transaction (and the proposed transaction is made in accordance with such registration statement), the holder thereof shall give written notice to the Company of such holder's intention to effect such Transfer ("Transfer Notice"). Each such Transfer Notice shall describe the manner and circumstances of the proposed Transfer in sufficient detail and, if reasonably requested by the Company, shall be accompanied, at such holder's expense, by either (x) a written opinion of legal counsel who shall, and whose legal opinion shall, be reasonably satisfactory to the Company, addressed to the Company, to the effect that the proposed Transfer may be effected without registration under the Securities Act, (y) a "no action" letter from the SEC to the effect that the proposed Transfer without registration will not result in a recommendation by the staff of the SEC that action be taken with respect thereto, or (z) any other evidence reasonably satisfactory to legal counsel for the Company to the effect that the proposed Transfer may be effected without registration under the Securities Act, whereupon the holder of such Restricted Securities shall be entitled to Transfer such Restricted Securities in accordance with the terms of the applicable Transfer Notice given by such holder to the Company. The Company will not require such a legal opinion or "no action" letter (x) in any transaction in compliance with Rule 144; or (y) in any transaction in which such Holder distributes Restricted Securities to an Affiliate of such Holder for no consideration. Each certificate evidencing any Restricted Securities that are Transferred as provided in this Section 2.12(c) shall bear, except if such Transfer is made pursuant to Rule 144, the appropriate restrictive legends set forth or described in Section 2.12(b), except that such certificate shall not bear such restrictive legends if, in the opinion of legal counsel for such Transferring holder and legal counsel for the Company, such legends are not required in order to establish compliance with any provisions of applicable securities laws, including the Securities Act.
- 2.13 <u>Assignment of Registration Rights</u>. The right of any Holder to request registration or inclusion of Registrable Securities in any registration pursuant to Section 2.1 or Section 2.2 (collectively, "*Registration Rights*") may be assigned (but only with all related obligations) by such Holder to a transferee of Registrable Securities that (x) after such Transfer, holds at least 450,000 shares of Registrable Securities, (y) is an Affiliate of such Holder, or (z) is such Holder's Immediate Family Member or trust for the benefit of such individual Holder (or one or more of his or her Immediate Family Members); <u>provided</u>, <u>however</u>, that (i) such Transfer of Registrable Securities is effected in accordance with Sections 2.12 and 8.14 and all applicable securities laws, (ii) before such Transfer of Registrable Securities, such Holder gives the

Company written notice stating the name and address of such transferee and identifying the securities of the Company with respect to which Registration Rights are intended to be assigned, (iii) such transferee of Registrable Securities agrees, in a written instrument delivered to the Company, to receive such assigned Registration Rights subject to all of the terms and conditions hereof, including the provisions of Section 2.11, and (iv) such transferee of Registrable Securities is not deemed by the Board, in its reasonable judgment, to be a competitor of the Company or a director, officer, employee or holder of more than 10% of a competitor of the Company, <u>provided</u>, <u>however</u>, that none of Pfizer, Viking, or any of their respective Affiliates shall be deemed to be a competitor of the Company for purposes of this Agreement.

2.14 <u>Termination of Registration Rights</u>. The Registration Rights shall automatically terminate and be of no further force or effect upon the earliest to occur of: (i) the dissolution or winding up of the Company; (ii) immediately before the consummation of a Deemed Liquidation Event; (iii) such time as Rule 144 or another similar exemption under the Securities Act is available for the sale of all Registrable Securities proposed to be sold by such Holder without limitation during a three-month period; and (iv) the 5-year anniversary of the Company IPO.

## 3. Information Rights.

- 3.1 <u>Delivery of Financial Statements</u>. The Company shall deliver to each Major Investor:
- (a) as soon as practicable, but in any event within one hundred twenty (120) days after the end of each fiscal year of the Company (i) a balance sheet as of the end of such year, (ii) statements of income and of cash flows for such year, and (iii) a statement of stockholders' equity as of the end of such fiscal year, all such financial statements to be in reasonable detail, and prepared in accordance with generally accepted accounting principles ("GAAP"), and audited and certified by an independent public accounting firm of nationally recognized standing approved by the Board (including at least one Preferred Director);
- (b) as soon as practicable, but in any event within forty-five (45) days after the end of each quarter of each fiscal year of the Company, unaudited statements of income and cash flows for such fiscal quarter, and an unaudited balance sheet as of the end of such fiscal quarter, all prepared in accordance with GAAP (except that such financial statements may (i) be subject to normal year-end audit adjustments; and (ii) not contain all notes thereto that may be required in accordance with GAAP);
- (c) as soon as practicable, but in any event at least thirty (30) days prior to the end of each fiscal year, a budget and business plan for the next fiscal year, prepared on a monthly basis, including balance sheets, income statements and statements of cash flows for such months and, as soon as prepared, any other budgets or revised budgets prepared by the Company;
- (d) such other information relating to the financial condition, business, prospects, or corporate affairs of the Company as any Major Investor may from time to time reasonably request; provided, however, that the Company shall not be obligated under this <u>Subsection 3.1</u> to provide information (i) that the Company reasonably determines in good faith to be a trade secret or confidential or commercially sensitive information (unless covered by a confidentiality agreement, in a form reasonably acceptable to the Company, including in a form as set forth in Section 3.4); or (ii) the disclosure of which would adversely affect the attorney-client privilege between the Company and its counsel.

If, for any period, the Company has any subsidiary whose accounts are consolidated with those of the Company, then in respect of such period the financial statements delivered pursuant to the foregoing sections shall be the consolidated and consolidating financial statements of the Company and all such consolidated subsidiaries.

Notwithstanding anything else in this <u>Subsection 3.1</u> to the contrary, the Company may cease providing the information set forth in this <u>Subsection 3.1</u> during the period starting with the date sixty (60) days before the Company's good-faith estimate of the date of filing of a registration statement if it reasonably concludes it must do so to comply with the SEC rules applicable to such registration statement and related offering; <u>provided</u> that the Company's covenants under this <u>Subsection 3.1</u> shall be reinstated at such time as the Company is no longer actively employing its commercially reasonable efforts to cause such registration statement to become effective.

- 3.2 <u>Inspection</u>. The Company shall permit each Major Investor, at such Major Investor's expense, to visit and inspect the Company's properties; examine its books of account and records; and discuss the Company's affairs, finances, and accounts with its officers, during normal business hours of the Company as may be reasonably requested by the Major Investor; <u>provided</u>, <u>however</u>, that the Company shall not be obligated pursuant to this <u>Subsection 3.2</u> to provide access to any information that it reasonably and in good faith considers to be a trade secret or confidential or commercially sensitive information (unless covered by a confidentiality agreement, in form reasonably acceptable to the Company, including in a form as set forth in Section 3.4) or the disclosure of which would adversely affect the attorney-client privilege between the Company and its counsel.
- 3.3 <u>Termination of Information</u>. The covenants set forth in <u>Subsection 3.1</u> and <u>Subsection 3.2</u> shall terminate and be of no further force or effect (i) immediately before the consummation of the Company IPO; (ii) upon the consummation of a Deemed Liquidation Event; or (iii) when the Company first becomes subject to the periodic reporting requirements of Section 12(g) or 15(d) of the Exchange Act, whichever event occurs first.
- 3.4 <u>Confidentiality</u>. Each Investor agrees that such Investor will keep confidential and will not disclose, divulge or use for any purpose (other than to monitor such Investor's investment in the Company) any confidential information obtained from the Company pursuant to this Agreement (including notice of the Company's intention to file a registration statement), unless such confidential information (x) is known or becomes known to the public in general (other than as a result of a breach or violation by such Investor or any of its Affiliates or representatives of this Section 3.4 or any other non-use or confidentiality obligation), (y) is or has been independently developed or conceived by such Investor without use of, derivation from, reference to or reliance upon any of the Company's confidential information and without violating any of the confidentiality obligations hereunder or any other non-use or confidentiality

obligation, or (z) is or has been made known or disclosed to such Investor by a third party without a breach of any legal, fiduciary, contractual or other obligation of confidentiality such third party may have to the Company; provided, however, that an Investor may disclose the Company's confidential information (i) to such Investor's attorneys, accountants, consultants, advisors and other professionals to the extent necessary to obtain their services in connection with monitoring such Investor's investment in the Company, provided that such Investor informs each such individual that such information is confidential and that by receiving such information such individual is agreeing to maintain the confidentiality of such information, (ii) to any Affiliate, current and prospective partner, member, stockholder or wholly owned subsidiary of such Investor in the ordinary course of business, provided that such Investor informs such Person that such information is confidential and directs such Person to maintain the confidentiality of such information, (iii) with prior notification to the Company, to any prospective purchaser of any Registrable Securities from such Investor, provided that such prospective purchaser agrees, in writing, to be bound by provisions not less restrictive than those set forth in this Section 3.4, or (iv) as may be required by applicable law, provided that such Investor delivers to the Company advance written notice of such disclosure and exercises commercially reasonable efforts to minimize the extent of any such required disclosure and obtain assurance that confidential treatment will be accorded to the disclosed information.

3.5 <u>Auditor Independence</u>. The Company shall be reasonably responsive to requests for information from the Investor relating to issues that may impact auditor independence rules applicable to the Investor.

## 4. Rights to Future Stock Issuances.

- 4.1 <u>Right of First Refusal</u>. If the Company proposes to offer or sell any New Securities, the Company shall first offer such New Securities to the Major Investors in accordance with the terms and conditions of this Section 4.1 and subject to applicable securities laws (the "*Right of First Refusal*").
- (a) The Company shall give written notice (the "Offer Notice") to each Major Investor, stating (i) its bona fide intention to offer such New Securities, (ii) the type and number of such New Securities to be offered (the "Offered Shares"), and (iii) the price and general terms, if any, upon which it proposes to offer such New Securities.
- (b) Each Major Investor, by written notice to the Company (the "*Election Notice*") given no later than the twentieth day after the date on which the Offer Notice is, pursuant to Section 8.5, deemed to have been delivered to such Major Investor (such twentieth day, the "*Initial Offer Deadline*"), may elect to purchase or acquire, at the price and on the general terms specified in the Offer Notice, up to that portion of the Offered Shares which equals the proportion that the Common Stock issued and then held, or issuable (directly or indirectly) upon conversion and/or exercise, as applicable, of the Preferred Stock then held by such Major Investor bears to the total Common Stock then outstanding (assuming full conversion and/or exercise, as applicable, of all Preferred Stock). Each Election Notice shall specify the number of Offered Shares that such Major Investor is electing to purchase or acquire. Promptly after the Initial Offer Deadline, the Company shall give written notice to each Major Investor that has elected to purchase or acquire all of the Offered Shares available to such Major Investor (each, a

"Fully Exercising Investor") of any other Major Investor's failure to do likewise (the "Second Offer Notice"). Each Fully Exercising Investor may, by giving written notice to the Company (the "Second Election Notice") during the ten-day period commencing on the date on which the Second Offer Notice is, pursuant to Section 8.5, deemed to have been delivered to such Fully Exercising Investor (such ten-day period, the "Second Offer Period"), elect to purchase or acquire, in addition to the number of Offered Shares such Fully Exercising Investor has already elected to purchase or acquire and at the same price and on the general terms specified in the Offer Notice, up to that portion of the Offered Shares for which Major Investors were entitled to subscribe but that were not subscribed for by the Major Investors by the Initial Offer Deadline (the "Unsubscribed Shares") which equals the proportion that the Common Stock issued and then held, or issuable (directly or indirectly) upon conversion and/or exercise, as applicable, of the Preferred Stock then held, by such Fully Exercising Investor bears to the total Common Stock issued and then held, or issuable (directly or indirectly) upon conversion and/or exercise, as applicable, of the Preferred Stock then held by all of the Fully Exercising Investors who elect to purchase or acquire Unsubscribed Shares. Each Second Election Notice shall specify the number of Unsubscribed Shares that such Fully Exercising Investor is electing to purchase or acquire. The closing of any sale of New Securities pursuant to this Section 4.1(b) shall occur on or before the later of (i) 90 days after the last date on which the Offer Notice is, pursuant to Section 8.5, deemed to have been delivered to all Major Investors and (ii) the date of initial sale of New Securities pursuant to Section 4.1(c).

(c) If all Offered Shares are not elected to be purchased or acquired as provided in Section 4.1(b), the Company may, during the 90-day period following the expiration of the Second Offer Period for all Fully Exercising Investors, offer and sell the remaining unsubscribed portion of such Offered Shares to any Person or Persons (the "Offerees") at a price not less than, and upon terms not more favorable than, specified in the Offer Notice. If the Company does not enter into a written agreement with the Offerees for the sale of New Securities within such 90-day period, or if the sale of such New Securities pursuant to such agreement is not consummated within 30 days after the execution thereof, the Right of First Refusal shall be deemed to be revived and such New Securities shall not be offered or sold to any Person or Persons unless first reoffered to the Major Investors in accordance with this Section 4.1.

(d) The Right of First Refusal shall not be applicable to: (i) Exempted Securities (as defined in the Restated Charter); (ii) securities of the Company which are otherwise excluded from the Right of First Refusal by the affirmative vote or consent of the holders of a majority of all shares of Preferred Stock then outstanding; (iii) shares of Common Stock issued in the Company IPO, or (iv) the issuance of shares of Series B Preferred Stock to Additional Purchasers pursuant to Subsection 1.2(c) of the Purchase Agreement. In the event the Right of First Refusal under this Section 4 is waived with respect to an offering of Equity Securities without a Major Investor's prior written consent and any party that participated in waiving such rights actually purchases New Securities in such offering, the Company shall grant to any such non-waiving Major Investor the right to purchase, in a subsequent closing of such issuance on substantially the same terms and conditions, the same percentage of its full pro rata share of such New Securities as the highest percentage of any such purchasing waiving party.

- (e) Notwithstanding any provision hereof to the contrary, no Major Investor shall have any right to purchase or acquire any New Securities pursuant to this Section 4.1 if such Major Investor cannot demonstrate to the Company's reasonable satisfaction that such Major Investor is, at the time of the proposed issuance of such New Securities, an "accredited investor" within the meaning of SEC Rule 501 of Regulation D, as then in effect.
- 4.2 <u>Assignment of Right of First Refusal</u>. The Right of First Refusal may be assigned (but only with all related obligations) by any Major Investor to a transferee of Registrable Securities that (x) after such Transfer, holds at least 450,000 shares of Registrable Securities, (y) is an Affiliate of such Major Investor, or (z) is such Major Investor's Immediate Family Member or trust for the benefit of such individual Major Investor (or one or more of his or her Immediate Family Members); <u>provided, however,</u> that (i) such Transfer of Registrable Securities is effected in accordance with Sections 2.12 and 8.14 and all applicable securities laws, (ii) before such Transfer of Registrable Securities, such Major Investor gives the Company written notice stating the name and address of such transferee and identifying the securities of the Company with respect to which the Right of First Refusal is intended to be assigned, (iii) such transferee of Registrable Securities agrees, in a written instrument delivered to the Company, to receive such assigned Right of First Refusal subject to all of the terms and conditions hereof and (iv) such transferee of Registrable Securities is not deemed by the Board, in its reasonable judgment, to be a competitor of the Company or a director, officer, employee or holder of more than 10% of a competitor of the Company; provided, however, none of Viking, Pfizer or any of their respective Affiliates shall be deemed to be a competitor of the Company for purposes of this Agreement.
- 4.3 <u>Termination of Right of First Refusal</u>. The covenants set forth in Section 4.1 shall automatically terminate and be of no further force or effect upon the earliest to occur of: (i) immediately before the consummation of the Company IPO; (ii) when the Company first becomes subject to the periodic reporting requirements of Section 12(g) or Section 15(d) of the Exchange Act; (iii) the dissolution or winding up of the Company; and (iv) immediately before the consummation of a Deemed Liquidation Event.

#### 5. Additional Covenants.

- 5.1 <u>Employee Agreements</u>. The Company shall cause each individual now or hereafter employed by it or any of its subsidiaries (or engaged by the Company or any of its subsidiaries as a consultant or independent contractor) with access to the Company's trade secrets and/or confidential information to enter into a confidential information and invention assignment agreement, substantially in the form made available to the Investors.
- 5.2 <u>Matters Requiring Preferred Director Approval</u>. So long as there is at least one Preferred Director serving on the Board, the Company hereby covenants and agrees with each Investor that it shall not, without approval of the Board, which approval must include the affirmative vote of at least one Preferred Director:
- (a) make, or permit any of its subsidiaries to make, any loan or advance to, or own any stock or other securities of, any subsidiary or other corporation, partnership or other entity unless it is wholly owned by the Company;

- (b) make, or permit any of its subsidiaries to make, any loan or advance to any person or entity, including any director or employee, except advances and similar expenditures in the ordinary course of business or under the terms of an employee stock or option plan approved by the Board;
- (c) guarantee (directly or indirectly), or permit any of its subsidiaries to guarantee (directly or indirectly), any indebtedness, except for trade accounts of the Company or any of its subsidiaries arising in the ordinary course of business;
- (d) enter into, or be a party to, any transaction with any director, officer or employee of the Company or any "associate" (as defined in Rule 12b 2 promulgated under the Exchange Act) of any such person or entity, except for (x) transactions contemplated by the Transaction Agreements (as defined in the Series B Purchase Agreement) or (y) transactions made in the ordinary course of business and pursuant to reasonable requirements of the Company's business and upon fair and reasonable terms that are approved by a majority of the Board;
- (e) hire, terminate or change the compensation of the Company's executive officers, including approving any option grants or stock awards to such executive officers, or paying bonuses in excess of 20% of base compensation (such approval not to be unreasonably withheld);
  - (f) change the Company's principal business, enter new lines of business or exit the Company's current lines of business; or
- (g) sell, assign, license, pledge or encumber material technology or intellectual property, other than (i) licenses granted in the ordinary course of business, (ii) in connection with a Deemed Liquidation Event or (iii) in connection with equipment leasing transactions of less than \$100,000 in the aggregate, in each case as approved by the Board.
- 5.3 <u>D&O Insurance</u>. The Company shall use its best efforts to maintain in full force and effect directors and officers insurance in the amount of at least three million dollars (\$3,000,000) (or such greater amount as determined by the Board), as determined by the Board and covering such risks as are adequate and customary for its size and business, each with financially sound and reputable insurance companies or associations; <u>provided</u>, <u>however</u>, that the Company shall not terminate or reduce such directors and officers insurance to less than three million dollars (\$3,000,000) without the prior written consent of Pfizer.
- 5.4 <u>Termination</u>. The covenants set forth in this Section 5 shall terminate and be of no further force or effect upon the earliest to occur of: (i) immediately before the consummation of the Company IPO; (ii) the dissolution or winding up of the Company; and (iii) immediately before the consummation of a Deemed Liquidation Event.

## 6. Voting Provisions Regarding Board of Directors.

- 6.1 <u>Board Composition</u>. Each Stockholder agrees to vote, or cause to be voted, all Voting Shares owned by such Stockholder, or over which such Stockholder has voting control, from time to time and at all times, in whatever manner as shall be necessary to ensure that, at each annual or special meeting of the Company's stockholders at which an election of directors is held or pursuant to any written consent of the Company's stockholders, the following individuals shall be elected to the Board:
- (a) one individual designated by Pfizer (the "Series A-1 Director"), which individual shall initially be Margi McLoughlin, for so long as Pfizer and its Affiliates continue to own beneficially 25% of the shares of Series A-1 Preferred Stock originally issued pursuant to the Series A-1 Purchase Agreement;

- (b) one individual designated by the holders of a majority of the Series B Preferred Stock (the "*Series B Director*," together with the Series A-1 Director, the "*Preferred Directors*"), which individual shall initially be Tony Yao, for so long as at least 916,380 shares of Series B Preferred Stock remain issued and outstanding;
- (c) two individuals designated by the holders of a majority of the outstanding shares of the Common Stock (other than (i) any Common Stock issued or issuable upon conversion of the Preferred Stock or (ii) any Common Stock issued as (or issuable upon the conversion or exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange for or in replacement of, the shares referenced in clause (i) above) (the "Common Directors"), which individuals shall initially be David Kirn and David Schaffer; and
- (d) three individuals who are not otherwise Affiliates (as defined below) of the Company or of any Investor (the "Independent Directors"), (i) the first of whom shall be mutually agreed upon by a majority of the other members of the Board, which approval shall not be unreasonably withheld or delayed, which individual shall initially be Hoyoung Huh, (ii) the second of whom shall be proposed by the Company subject to the approval of a majority of the other members of the Board, which approval shall not be unreasonably withheld or delayed, which individual shall initially be Charles Theuer, and (iii) the third of whom shall be an individual that satisfies the independence, financial literacy and financial expertise requirements to serve as an audit committee chairperson pursuant to relevant SEC, New York Stock Exchange and Nasdaq laws and regulations, and mutually acceptable to a majority of the other members of the Board.

To the extent that any of clauses (a) through (d) above shall not be applicable, any member of the Board who would otherwise have been designated in accordance with the terms thereof shall instead be elected by all of the Company's stockholders entitled to vote thereon in accordance with, and pursuant to, the Restated Charter. The Company will fill vacancies on the Board as soon as practicable and in any event within twelve (12) months after the Initial Closing (as defined in the Series B Purchase Agreement). The parties acknowledge that additional seats on the Board shall be determined in connection with future financing and other strategic transactions involving the Company, and to satisfy other needs of the Company for independent directors, and as determined by the Board, may provide for rights to designate directors similar to the rights provided to Pfizer and the holders of a majority of the outstanding shares of Series B Preferred Stock under this Section 6.

- 6.2 <u>Failure to Designate a Board Member</u>. In the absence of any designation from the Persons or groups with the right to designate a director as specified in Section 6.1, the director previously designated by them and then serving shall be reelected if still eligible to serve as provided herein.
- 6.3 <u>Removal of Board Members</u>. Each Stockholder agrees to vote, or cause to be voted, all Voting Shares owned by such Stockholder, or over which such Stockholder has voting control, from time to time and at all times, in whatever manner as shall be necessary to ensure that:
- (a) no director elected pursuant to Section 6.1 or Section 6.2 may be removed from office unless (i) such removal is directed or approved by the affirmative vote of the Person, or of the holders of a majority of the shares of stock, entitled under Section 6.1 to designate that director or (ii) the Person(s) originally entitled to designate or approve such director;
- (b) any vacancies created by the resignation, removal or death of a director elected pursuant to Section 6.1 or Section 6.2 shall be filled pursuant to the provisions of this Section 6; and
- (c) upon the written request of any party entitled to designate a director as provided in Section 6.1(a), Section 6.1(b) to remove such director, such director shall be removed.

All Stockholders agree to execute any written consents required to perform their obligations as set forth in this Agreement, and the Company agrees, at the written request of any party entitled to designate directors, to call a special meeting of the Company's stockholders for the purpose of electing directors.

- 6.4 <u>No Liability for Election of Recommended Directors</u>. No Stockholder, nor any Affiliate of any Stockholder, shall have any liability as a result of designating an individual for election as a director for any act or omission by such designated individual in his or her capacity as a director of the Company, nor shall any Stockholder have any liability as a result of voting for any such designee in accordance with the provisions of this Agreement.
- 6.5 No "Bad Actor" Designees. Each Person or group with the right to designate, or participate in the designation of, a director as specified in Section 6.1 (each, a "Designator") hereby represents and warrants to the Company that, to such Designator's knowledge, none of the "bad actor" disqualifying events described in Rule 506(d)(1)(i)-(viii) promulgated under the Securities Act (each, a "Disqualification Event"), is applicable to such Designator's initial designee named in Section 6.1, except, if applicable, for a Disqualification Event as to which Rule 506(d)(2)(ii) or (iii) or (d)(3) is applicable. Any director designee to whom any Disqualification Event is applicable, except for a Disqualification Event as to which Rule 506(d)(2)(ii) or (iii) or (d)(3) is applicable, is hereinafter referred to as a "Disqualified Designee." Each Designator hereby covenants and agrees (i) not to designate, or participate in the designation of, any director designee who, to such Designator's knowledge, is a Disqualified Designee and (ii) that, in the event such Designator becomes aware that any individual previously designated by any such Designator is or has become a Disqualified Designee, such Designator shall, as promptly as practicable, take such actions as are necessary to remove such Disqualified Designee from the Board and designate a replacement designee who is not a Disqualified Designee.

#### 7. Drag-Along Right; Vote to Increase Common Stock.

#### 7.1 Definitions

- (a) "Sale of the Company" means either (i) a Stock Sale (as defined below) or (ii) a Deemed Liquidation Event.
- (b) "Stock Sale" means a change in ownership of the Company, other than a Deemed Liquidation Event, that occurs when one Person, or more than one Person acting as a group, acquires ownership of stock of the Company, in a stock sale or exchange, that, together with the stock held by such Person or group, constitutes more than 50% of the total voting power of the stock of the Company; provided, however, that a Stock Sale will not occur if any Person, or more than one Person acting as a group, owns more than 50% of the total voting power of the stock of the Company and acquires additional stock of the Company; provided, further, that any change in the ownership of the stock of the Company as a result of a bona fide equity financing of the Company that is approved by the Board will not be considered a Stock Sale.
- (c) "Subject Shares" means all securities of the Company, including securities of the Company acquired upon exercise or conversion of any options, warrants or other convertible securities.
- 7.2 Actions to be Taken. In the event that the holders of (i) a majority of the then outstanding shares of Common Stock and (ii) a majority of the then outstanding shares of Preferred Stock (voting together as a single class on an as converted to Common Stock basis) (the "Selling Stockholders") approve a Sale of the Company, in writing, specifying that this Section 7 shall apply to such transaction, then the Company and each Stockholder hereby agrees:
- (a) if such transaction requires stockholder approval, with respect to all Voting Shares owned by such Stockholder, or over which such Stockholder has voting control, to vote (in person, by proxy or by written consent, as applicable) such Voting Shares in favor of such Sale of the Company (together with any related amendment to the Restated Charter required in order to implement such Sale of the Company) and in opposition to any and all other proposals that could reasonably be expected to delay or impair the ability of the Company or its stockholders to consummate such Sale of the Company;
- (b) to sell or exchange all Subject Shares that such Stockholder then beneficially holds pursuant to the terms and conditions of such Deemed Liquidation Event or, in the case of a Stock Sale, to sell or otherwise transfer to the acquiring Person all Subject Shares that such Stockholder then beneficially holds (or in the event that the Selling Stockholders are selling fewer than all of their Subject Shares, shares in the same proportion as the Selling Stockholders are selling to the acquiring Person) for the same per share consideration in accordance with the provisions of the Restated Charter, and on the same terms and conditions (except as otherwise permitted by Section 7.3), as the Selling Stockholders;

- (c) to refrain from exercising any dissenters' rights or rights of appraisal under applicable law (if any) with respect to such Sale of the Company;
- (d) to execute and deliver all related documentation and take such other actions as may be reasonably requested, in writing, by the Company or the Selling Stockholders in support of such Sale of the Company, including executing and delivering instruments of conveyance and transfer, any purchase agreement, merger agreement, indemnity agreement, escrow agreement, consent, waiver, governmental filing and share certificates duly endorsed for transfer (free and clear of impermissible liens, claims and encumbrances), and any similar or related documents;
- (e) to refrain from entering into any agreement or understanding (including any proxy or voting trust) that would be inconsistent with, or violate, the provisions of this Section 7, unless specifically requested to do so, in writing, by the acquiring Person in connection with such Sale of the Company; and
- (f) in the event that a stockholder representative (the "Stockholder Representative") is appointed with respect to matters affecting the Company's stockholders under the applicable definitive transaction agreements relating to such Sale of the Company, (i) to consent to (x) the appointment of such Stockholder Representative, (y) the establishment of any applicable escrow, expense or similar fund in connection with any indemnification or similar obligations, and (z) the payment of such Stockholder's pro rata portion (from the applicable escrow or expense fund or otherwise) of any and all of such Stockholder Representative's reasonable fees and expenses arising out of such Stockholder Representative's services and duties as the representative of the Company's stockholders in connection with such Sale of the Company, and (ii) not to assert any claim, or commence any suit, against the Stockholder Representative or any other stockholder of the Company with respect to any action or inaction by the Stockholder Representative in connection with his or her service as the Stockholder Representative, absent fraud, gross negligence or willful misconduct.

The provisions of this Section 7 shall (i) (x) with respect to Theresa Janke, supersede and replace the provisions of Section 12 of that certain Contribution Agreement made and entered into as of March 20, 2015 by and between the Company and Theresa Janke and (y) with respect to Melissa Kotterman, supersede and replace the provisions of Section 12 of that certain Contribution Agreement made and entered into as of March 20, 2015 by and between the Company and Melissa Kotterman and (ii) not be deemed to require any Stockholder to approve any amendment or waiver of any provision of the Restated Charter or otherwise approve a Sale of the Company that would not allocate the consideration in accordance with the Restated Charter.

- 7.3 Exceptions. Notwithstanding the foregoing, a Stockholder's obligations pursuant to Section 7.2 in connection with any proposed Sale of the Company (the "*Proposed Sale*") shall be subject to the following conditions:
- (a) any representations and warranties to be made by such Stockholder in connection with the Proposed Sale are limited to representations and warranties related to authority, ownership and the ability to convey title to the Stockholder's Shares, including, but not limited to, representations and warranties that (i) the Stockholder holds all right, title and

interest in and to the Shares such Stockholder purports to hold, free and clear of all liens and encumbrances, (ii) the obligations of the Stockholder in connection with the transaction have been duly authorized, if applicable, (iii) the documents to be entered into by the Stockholder have been duly executed by the Stockholder and delivered to the acquirer and are enforceable against the Stockholder in accordance with their respective terms; and (iv) neither the execution and delivery of documents to be entered into in connection with the Proposed Sale, nor the performance of the Stockholder's obligations thereunder, will cause a breach or violation of the terms of any agreement, law or judgment, order or decree of any court or governmental agency;

- (b) the Stockholder shall not be liable for the inaccuracy of any representation or warranty made by any other Person in connection with the Proposed Sale, other than the Company (except to the extent that funds may be paid out of an escrow established to cover breach of representations, warranties and covenants of the Company as well as breach by any stockholder of any of identical representations, warranties and covenants provided by all stockholders);
- (c) the liability for indemnification, if any, of such Stockholder in the Proposed Sale, and for the inaccuracy of any representations and warranties made by the Company or its stockholders in connection with the Proposed Sale, shall be several and not joint with any other Person (except to the extent that funds may be paid out of an escrow established to cover breach of representations, warranties and covenants of the Company as well as breach by any stockholder of any identical representations, warranties and covenants provided by all stockholders) and, subject to any provisions of the Restated Charter relating to the allocation of the escrow, shall be pro rata in proportion to, and shall not exceed, the amount of consideration paid to such Stockholder in connection with the Proposed Sale.
- (d) other than liability in respect of actions or omissions of, or representations and warranties made solely by and with respect to, such Stockholder, liability shall be limited to such Stockholder's applicable share (determined based on the respective proceeds payable to each Stockholder in connection with such Proposed Sale in accordance with the provisions of the Restated Certificate) of a negotiated aggregate indemnification amount that applies equally to all Stockholders but that in no event exceeds the amount of consideration otherwise payable to such Stockholder in connection with such Proposed Sale, except with respect to claims related to fraud by such Stockholder, the liability for which need not be limited as to such Stockholder;
- (e) as a result of the Proposed Sale, (i) each holder of each class or series of capital stock of the Company shall be entitled to receive the same form of consideration (and be subject to the same indemnity and escrow provisions) for their shares of such class or series as is received by other holders with respect to their shares of such same class or series of stock, (ii) each holder of a series of Preferred Stock of the Company shall receive the same amount of consideration per share of such series of Preferred Stock of the Company as is received by other holders with respect to their shares of such same series, (iii) each holder of Common Stock shall receive the same amount of consideration per share of Common Stock as is received by other holders with respect to their shares of Common Stock, and (iv) unless the holders of each series of Preferred Stock agree otherwise by legally sufficient amendment or waiver of the provisions of the Restated Charter then in effect, the aggregate consideration receivable by all holders of Common Stock and Preferred Stock of the Company shall be allocated among the holders of

Common Stock and Preferred Stock of the Company on the basis of the relative liquidation preferences to which the holders of each respective series of Preferred Stock of the Company and the holders of Common Stock are entitled in a Deemed Liquidation Event (assuming for this purpose that the Proposed Sale is a Deemed Liquidation Event) in accordance with the Restated Charter in effect immediately prior to the Proposed Sale; provided, however, that, notwithstanding the foregoing, if the consideration to be paid in exchange for the Subject Shares pursuant to the Proposed Sale includes any securities and due receipt thereof by such Stockholder would require, under applicable law, (x) the registration or qualification of such securities or of any Person as a broker, dealer or agent with respect to such securities or (y) the provision to such Stockholder of any information other than such information as a prudent issuer would generally furnish in an offering made solely to "accredited investors" as defined in Regulation D promulgated under the Securities Act, then the Company may cause to be paid to such Stockholder, in lieu of such securities, against surrender of the Subject Shares which would have otherwise been sold by such Stockholder, an amount in cash equal to the fair market value (as determined in good faith by the Company) of the securities that such Stockholder would otherwise receive as of the date of issuance of such securities in exchange for such Stockholder's Subject Shares;

- (f) subject to clause (e) above, requiring the same form of consideration to be available to the holders of any single class or series of capital stock, if any holders of any capital stock of the Company are given an option as to the form and amount of consideration to be received as a result of the Proposed Sale, all holders of such capital stock will be given the same option; provided, however, that nothing in this Section 7.3(f) shall entitle any holder to receive any form of consideration that such holder would be ineligible to receive as a result of such holder's failure to satisfy any condition, requirement or limitation that is generally applicable to the Company's stockholders;
- (g) No Stockholder or its affiliates shall be required to agree (unless such Stockholder is a Company officer or employee) to any restrictive covenant in connection with the Proposed Sale (including without limitation any covenant not to compete or covenant not to solicit customers, employees or suppliers of any party to the Proposed Sale);
- (h) No Stockholder or its affiliates shall be required to amend, extend or terminate any commercial, contractual or other relationship with the Company, the acquirer or their respective affiliates, except that the Stockholder may be required to agree to terminate the investment-related documents between or among such Stockholder, the Company and/or other stockholders of the Company.
- 7.4 Restrictions on Sales of Control of the Company. No Stockholder shall be a party to any Stock Sale unless all holders of Preferred Stock are allowed to participate in such transaction and the consideration received pursuant to such transaction is allocated among the parties thereto in the manner specified in the Restated Certificate in effect immediately prior to the Stock Sale (as if such transaction were a Deemed Liquidation Event), unless (i) a majority of the then outstanding shares of Preferred Stock, voting together as a single class on an as-converted to Common Stock basis, and (ii) a majority of the then outstanding shares of Series B Preferred Stock, elect otherwise by written notice given to the Company at least ten (10) days prior to the effective date of any such transaction or series of related transactions.

7.5 <u>Vote to Increase Authorized Common Stock</u>. Each Stockholder agrees to vote, or cause to be voted, all Voting Shares owned by such Stockholder, or over which such Stockholder has voting control, from time to time and at all times, in whatever manner as shall be necessary to increase the number of authorized shares of Common Stock from time to time to ensure that there will be sufficient shares of Common Stock available for conversion of all shares of Preferred Stock outstanding at any given time.

#### 7.6 Remedies.

(a) Irrevocable Proxy and Power of Attorney, Each Stockholder hereby constitutes and appoints as such Stockholder's proxy, and hereby grants a power of attorney to, the Company's Chief Executive Officers with full power of substitution, to represent and to vote or act by written consent with respect to all securities of the Company that such Stockholder beneficially holds, either as of the date of this Agreement or at any time thereafter (collectively, the "Proxy Shares"), in accordance with Section 6 or this Section 7, if and only if such Stockholder or any transferee of any Proxy Shares (i) fails to vote all of the Proxy Shares in accordance with this Section 6 or Section 7, (ii) attempts to vote any of the Proxy Shares (whether in person, by proxy or by written consent) in a manner that is inconsistent with Section 6 or this Section 7, or (iii) fails to take any action necessary to effect the provisions of Section 6 or this Section 7. Each proxy and power of attorney granted pursuant to the immediately preceding sentence is given to secure the performance of each Stockholder's duties under Section 6 and this Section 7, and each Stockholder shall take such further action and execute such other instruments as may be necessary to effectuate the intent of such Stockholder's proxy. Each proxy and power of attorney is coupled with an interest, shall be irrevocable and shall be valid for so long as any of the Proxy Shares are outstanding until the covenants set forth in this Section 7 terminate or expire pursuant to Section 7.8. The authority vested in each proxy shall run with the Proxy Shares regardless of any change in legal ownership thereof. The power of attorney granted by each Stockholder herein is a durable power of attorney and shall survive such Stockholder's bankruptcy, death or incapacity. Each Stockholder hereby revokes any and all previous proxies and powers of attorney with respect to the Proxy Shares. Each Stockholder and each subsequent holder of any Proxy Shares shall not hereafter, unless and until the covenants set forth in Section 6 and this Section 7 terminate or expire pursuant to Section 7.8, (x) purport to grant any other proxy or power of attorney with respect to any of the Proxy Shares, (b) deposit any of the Proxy Shares into a voting trust, or (z) enter into any agreement, arrangement or understanding with any Person (other than the Company), directly or indirectly, to vote, grant any proxy or give instructions with respect to the voting of any of the Proxy Shares, in each case, with respect to any of the matters set forth in Section 6 or this Section 7.

7.7 Equitable Relief. Each party acknowledges and agrees that any breach or threatened breach of the covenants set forth in Section 6 or this Section 7 will cause irreparable injury and that money damages will not provide an adequate remedy. Accordingly, it is agreed that each party hereto shall be entitled to an injunction to prevent breaches of the covenants set forth in Section 6 or this Section 7 or other equitable relief (including specific performance in any action instituted in any court of the United States or any state having subject matter jurisdiction). The aforementioned equitable relief shall be in addition to, and not in lieu of, legal remedies, monetary damages or other available forms of relief.

7.8 <u>Termination</u>. The covenants set forth in Section 6 and this Section 7 shall automatically terminate and be of no further force or effect upon the earliest to occur of: (i) immediately before the consummation of the Company IPO; (ii) the dissolution or winding up of the Company; (iii) the consummation of a Deemed Liquidation Event; and (iv) the consummation of a Sale of the Company and distribution of proceeds to, or escrow for the benefit of, the Company's stockholders in accordance with the Restated Charter; <u>provided</u> that the provisions of this Section 7 will continue after the closing of any Sale of the Company to the extent necessary to enforce the provisions of this Section 7 with respect to such Sale of the Company.

#### 8. Miscellaneous.

- 8.1 <u>Successors and Assigns</u>. Except as otherwise provided herein, this Agreement, and any and all rights, duties and obligations hereunder, shall not be assigned, transferred, delegated or sublicensed by any Stockholder without the Company's prior written consent, and any attempt by any Stockholder to assign, transfer, delegate or sublicense any right, duty or obligation hereunder without such prior written consent of the Company shall be void. Subject to the foregoing and except as otherwise provided herein, the provisions hereof shall inure to the benefit of, and be binding upon, the respective successors, permitted assigns, heirs, executors and administrators of the parties hereto. Nothing herein, express or implied, is intended to confer upon any party other than the parties hereto or their respective successors, permitted assigns, heirs, executors and administrators any rights, duties or obligations under or by reason of this Agreement, except as expressly provided herein.
- 8.2 Governing Law; Venue; Jury Trial Waiver. This Agreement shall be governed by and construed in accordance with the internal laws of the State of Delaware without regard to conflict-of-law principles. Each party hereto hereby (i) irrevocably and unconditionally submits to the jurisdiction of the courts of the State of California located in Alameda County and of the United States of America for the Northern District of California for the purpose of any action, suit or proceeding based upon, arising out of or relating to this Agreement, (ii) agrees not to commence any action, suit or proceeding based upon, arising out of or relating to this Agreement, (iii) agrees not to commence any action, suit or proceeding based upon, arising out of or relating to this Agreement, (ii) agrees not to commence any action, suit or proceeding based upon, arising out of or relating to this Agreement, (iii) agrees not to commence any action, suit or proceeding based upon, arising out of or relating to this Agreement, (ii) agrees not to commence any action, suit or proceeding based upon, arising out of or relating to this Agreement, (iii) agrees not to commence any action, suit or proceeding based upon, arising out of or relating to this Agreement, (iii) agrees not to commence any action, suit or proceeding any action, suit or proceeding, any claim that such party is not subject personally to the jurisdiction of the aforesaid courts, that such party's property is exempt or immune from attachment or execution, that such party is property is exempt or immune from attachment or execution, that such party is property is exempt or immune from attachment or execution, that such party is property is exempt or immune from attachment or execution, that such party is property is exempt or immune from attachment or execution, that such party is property is exempt or proceeding is improper or that this Agreement or the subject matter hereof may not be enforced in or by the aforesaid courts. EACH PARTY HERETO REPRESENTS AND WARRANTS THAT SUCH P

FILED IN ANY COURT AND THAT RELATE TO THE SUBJECT MATTER OF THIS TRANSACTION, INCLUDING CONTRACT CLAIMS, TORT CLAIMS (INCLUDING NEGLIGENCE), BREACH OF DUTY CLAIMS AND ALL OTHER COMMON LAW AND STATUTORY CLAIMS. THIS SECTION 8.2 HAS BEEN FULLY DISCUSSED BY EACH OF THE PARTIES HERETO, AND THE PROVISIONS OF THIS SECTION 8.2 WILL NOT BE SUBJECT TO ANY EXCEPTIONS.

- 8.3 <u>Counterparts</u>. This Agreement may be executed in counterparts, each of which shall be deemed an original, but all of which together shall be deemed to be one and the same agreement. The exchange of copies hereof, including signature pages hereto, by facsimile, e-mail or other means of electronic transmission shall constitute effective execution and delivery hereof as to the parties and may be used in lieu of the original Agreement for all purposes. Signatures transmitted by facsimile, e-mail or other means of electronic transmission shall be deemed to be original signatures for all purposes.
- 8.4 Interpretation. Capitalized terms shall have the meanings as defined herein, and the meaning of defined terms shall be equally applicable to both the singular and plural forms of the terms defined. For purposes of this Agreement, (i) the words "include," "includes" and "including" shall be deemed to be followed by the words "without limitation," (ii) the word "or" is not exclusive, (iii) the words "herein," "hereof," "hereby," "hereto," "hereunder" and words of similar import refer to this Agreement as a whole, and (iv) with respect to the determination of any period of time, "from" means "from and including" and "to" means "to but excluding." Unless the context otherwise requires, references herein: (a) to a Section, a Schedule or an Exhibit mean a Section, a Schedule or an Exhibit of, or attached to, this Agreement; (b) to agreements, instruments and other documents shall be deemed to include all subsequent amendments, supplements and other modifications thereto; (c) to statutes or regulations are to be construed as including all statutory and regulatory provisions consolidating, amending or replacing the statute or regulation referred to; (d) to any Person includes such Person's successors and assigns, but, if applicable, only if such successors and assigns are not prohibited by this Agreement; and (e) to any gender includes each other gender. The Exhibits attached hereto shall be construed with, and as an integral part of, this Agreement to the same extent as if they were set forth verbatim herein. The titles, captions and headings herein are for convenience of reference only and shall not affect the meaning or interpretation hereof. This Agreement shall be construed without regard to any presumption or rule requiring construction or interpretation against the party drafting an instrument or causing any instrument to be drafted.
- 8.5 Notices. Except as may be otherwise provided herein, all notices, requests, consents, claims, demands, waivers and other communications required or permitted hereunder shall be in writing and shall be deemed given or delivered (i) when delivered personally, (ii) one business day after being deposited with an overnight courier service (costs prepaid), (iii) when sent by facsimile or e-mail if sent during the recipient's normal business hours and on the next business day if sent after the recipient's normal business hours, in each case with confirmation of transmission by the transmitting equipment, or (iv) when received or rejected by the addressee, if sent by certified or registered mail, return receipt requested, postage prepaid, in each case to the addresses, facsimile numbers and e-mail addresses and marked to the attention of the person (by name or title) designated on Schedule 1 or Schedule 2 attached hereto (or to such address, facsimile number and e-mail address and marked to the attention of the person (by name or title)

- (a) indicated in the Company's records, in the case of any other holder of capital stock of the Company, and (b) on the signature page(s) hereto, in the case of the Company) or to such other address, facsimile number, e-mail address or person as such party may designate by a notice delivered to the other parties hereto. In addition, all notices, requests, consents, claims, demands, waivers and other communications given or delivered to the Company shall include a mandatory copy (which shall not constitute notice) to Latham & Watkins LLP, 140 Scott Drive, Menlo Park, CA 94025, Attn: Alan Mendelson and Ben Potter, Facsimile (650-463-2600), E-mail (or such other Person as the Company may designate by a notice delivered to the other parties hereto).
- 8.6 <u>Attorneys' Fees</u>. If any action, suit or proceeding is necessary to enforce or interpret the terms of this Agreement, the prevailing party shall be entitled to reasonable attorneys' fees, costs and necessary disbursements in addition to any other relief to which such party may be entitled.
- 8.7 Amendments and Waivers; Termination. In addition to automatic termination of specific rights and restrictions as provided in Section 2.14, Section 4.3, and Section 7.8, this Agreement may be amended, modified, terminated or supplemented and the observance of any provision hereof may be waived (either generally or in a particular instance, and either retroactively or prospectively) by a writing signed by (i) the Company, (ii) the holders of a majority of the Registrable Securities then outstanding (the "Requisite Investors") and (iii) the Key Holders holding a majority of the shares of capital stock of the Company held by all of the Key Holders who are then providing services to the Company as directors, officers, employees or consultants (the "Requisite Key Holders"); provided, however, that the Company may, in its sole discretion, waive compliance with Section 2.12(c) (and the Company's failure to object promptly in writing after receiving written notification of a proposed assignment allegedly in violation of Section 2.12(c) shall be deemed to be a waiver); provided, further, that, notwithstanding anything to the contrary in this Section 8.7, any provision hereof may be waived by a party, on such party's own behalf, without the consent of any other party. Any amendment, modification, termination, supplement or waiver effected in accordance with this Section 8.7 shall be binding on all parties hereto and all of their respective successors, permitted assigns, heirs, executors and administrators whether or not such party, successor, permitted assignee, heir, executor or administrator entered into or approved such amendment, modification, termination, supplement or waiver. Each Key Holder and each Investor acknowledges that, by operation of this Section 8.7, the Requisite Key Holders and the Requisite Investors will have the right and power to diminish or eliminate all rights of such Key Holder or Investor, as applicable, hereunder. No waivers of, or exceptions to, any term, condition or provision hereof, in any one or more instances, shall be deemed to be, or construed as, a further or continuing waiver of, or exception to, any such term, condition or provision. Notwithstanding the foregoing:
- (a) <u>Schedule 1</u> and <u>Schedule 2</u> attached hereto may be amended by the Company from time to time in accordance with Section 8.13 to add information regarding additional Investors or Key Holders, as applicable, without the consent of the other parties hereto;

- (b) the consent of the Key Holders shall not be required for any amendment, modification, termination, supplement or waiver of any provision hereof if such amendment, modification, termination, supplement or waiver is not directly applicable to the rights of the Key Holders hereunder;
- (c) Section 6.1(a), this Section 8.7(c) and Section 8.20 shall not be amended, modified, terminated, supplemented or waived (i) without the written consent of Pfizer, for so long as Pfizer and its Affiliates (as defined below) continue to own beneficially a majority of the shares of Series A-1 Preferred Stock originally issued pursuant to the Series A-1 Purchase Agreement, or (ii) in connection with a bona fide preferred stock equity financing pursuant to which this Agreement is amended and restated but such provisions are not otherwise substantially modified;
- (d) Section 6.1(b) and this Section 8.7(d) shall not be amended, modified, terminated, supplemented or waived without the written consent of the holders of a majority of the outstanding shares of Series B Preferred Stock, for so long as at least 916,380 shares of Series B Preferred Stock remain issued and outstanding;
- (e) Section 6.1(c) shall not be amended, modified, terminated, supplemented or waived without the written consent of the holders of a majority of the outstanding shares of Common Stock (other than (i) any Common Stock issued or issuable upon conversion of the Preferred Stock or (ii) any Common Stock issued as (or issuable upon the conversion or exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange for or in replacement of, the shares referenced in clause (i) above); and
- (f) Any amendment, modification, termination or waiver of Subsection 7.3 that (i) materially increases the obligations of any Investor under this Agreement, or (ii) materially adversely affects the rights of any Investor, shall require the consent of such Investor; <u>provided</u>, <u>however</u>, that in no circumstances shall this Subsection 8.7(f) be interpreted in such a manner to, except as otherwise provided for herein, require the consent of any Investor to (i) the termination of this Agreement in accordance with this Section 8.7, (ii) the termination of Section 7 in its entirety in accordance with Section 7.8 hereof or this Section 8.7, or (iii) any amendment or modification of Subsection 7.3 in connection with a bona fide equity financing pursuant to which this Agreement is amended and/or restated but such provisions are not amended or modified in a manner that is materially adverse to such Investor.
- 8.8 <u>Severability</u>. Should any provision contained herein be held as invalid, illegal or unenforceable, such holding shall not affect the validity of the remainder of this Agreement, the balance of which shall continue to be binding upon the parties with any such modification to become a part hereof and treated as though originally set forth herein.
- 8.9 <u>Delays or Omissions</u>. Except as otherwise provided herein, no delay or omission to exercise any right, power or remedy accruing to any party hereunder upon any breach or default of any other party hereunder shall impair any such right, power or remedy of such non- breaching or non-defaulting party nor shall it be construed to be a waiver of any such breach or default, or an acquiescence therein, or of (or in) any similar breach or default thereafter occurring; nor shall any waiver of any single breach or default be deemed a waiver of any other

breach or default theretofore or thereafter occurring. Subject to Section 8.5 and Section 8.7, any waiver, permit, consent or approval, of any kind or character on the part of any party, of any breach or default hereunder (or any waiver on the part of any party of any provisions or conditions hereof) must be in writing and shall be effective only to the extent specifically set forth in such writing. All remedies, either hereunder or by law or otherwise afforded to any party, shall be cumulative and not alternative.

- 8.10 Entire Agreement. This Agreement (including the Schedule(s) and Exhibit(s) attached hereto) constitutes the full and entire understanding and agreement of the parties hereto with respect to the subject matter contained herein and supersedes any and all other communications, representations, agreements, understandings and letters of intent, whether written or oral, between or among any of the parties hereto with respect to the subject matter contained herein.
- 8.11 <u>Further Assurances</u>. Each party hereto agrees to cooperate fully with the other parties hereto and to execute and deliver, or cause to be executed and delivered, such further agreements, instruments and documents and to give such further written assurance and take such further acts as may be reasonably requested by any other party hereto to evidence and reflect the transactions contemplated by this Agreement and to carry into effect the intents and purposes of this Agreement.
- 8.12 <u>Adjustments for Stock Splits, Etc.</u> All references herein to numbers of shares shall automatically be proportionally adjusted to reflect any stock combination, stock split, stock dividend, recapitalization or other similar transaction affecting the capital stock of the Company occurring after the date of this Agreement.

#### 8.13 Additional Parties.

- (a) If the Company issues additional shares of Preferred Stock after the date hereof, the Company shall, as a condition to the issuance of such shares, require the purchaser of such shares to become a party to this Agreement by executing and delivering to the Company (i) the Adoption Agreement, in substantially the form attached hereto as <a href="Exhibit A">Exhibit A</a> (the "Adoption Agreement"), or (ii) a counterpart signature page hereto agreeing to be bound by, and subject to, the terms hereof as an Investor and Stockholder hereunder. In either event, each such Person shall thereafter be deemed an Investor and a Stockholder for all purposes hereunder.
- (b) If the Company issues additional shares of Common Stock after the date hereof representing 1% or more of the Company's fully-diluted capitalization, the Company shall, as a condition to the issuance of such shares, require the purchaser or recipient of such shares to become a party to this Agreement by executing and delivering to the Company (i) the Adoption Agreement, or (ii) a counterpart signature page hereto agreeing to be bound by, and subject to, the terms hereof as a Key Holder and Stockholder hereunder. In either event, each such Person shall thereafter be deemed a Key Holder and a Stockholder for all purposes hereunder.

- 8.14 <u>Transfers</u>. Each transferee or assignee of any shares of capital stock of the Company subject hereto shall continue to be bound by, and subject to, the terms and conditions hereof, and, as a condition precedent to any such transfer or assignment of shares, each such transferee or assignee shall agree in writing to be bound by, and subject to, all of the terms and conditions hereof by executing the Adoption Agreement and delivering it to the Company. Upon the execution of the Adoption Agreement (and delivery thereof to the Company) by any transferee or assignee of any shares of capital stock of the Company subject hereto, such transferee or assignee shall be deemed to be (i) a party hereto as if such transferee or assignee were the transferor or assignor and such transferee's or assignee's signature appeared on the signature pages of this Agreement and (ii) an Investor and a Stockholder, or a Key Holder and a Stockholder, as applicable. The Company shall not permit the transfer, on its books, of any shares of capital stock of the Company subject hereto or issue a new certificate representing any such shares unless and until such transferee or assignee shall have complied with the terms of this Section 8.14.
- 8.15 Aggregation of Stock. All shares of capital stock of the Company held or acquired by a Stockholder and/or its Affiliates shall be aggregated together for the purpose of determining the availability of any rights hereunder, and such Affiliated Persons may apportion such rights as among themselves in any manner they deem appropriate. For purposes of determining the number of shares of Registrable Securities held by a transferee, the holdings of a transferee that is (x) an Affiliate or stockholder of a Holder, (y) a Holder's Immediate Family Member, or (z) a trust for the benefit of an individual Holder (or one or more of his or her Immediate Family Members) shall be aggregated together and with those of such transferring Holder; provided, however, that all transferees who would not qualify individually for assignment of rights shall have a single attorney-in-fact for the purpose of exercising any rights, receiving notices or taking any action under this Agreement. Notwithstanding anything to the contrary, the shares held by JANUS HENDERSON GLOBAL LIFE SCIENCES FUND or JANUS HENDERSON CAPITAL FUNDS PLC on behalf of its series, JANUS HENDERSON GLOBAL LIFE SCIENCES FUND will be aggregated solely for the purpose of determining whether either JANUS HENDERSON GLOBAL LIFE SCIENCES FUND (or their respective transferees) is a "Major Investor" under this Agreement.
- 8.16 <u>Calculations</u>. All calculations hereunder, including calculations of pro rata shares, shall be made by the Company and shall be binding upon the parties hereto absent fraud or manifest error. No fractional shares shall be Transferred hereunder.
- 8.17 <u>Conflict</u>. In the event of any conflict between this Agreement and the Restated Charter or the Company's Bylaws, the terms of the Restated Charter or the Company's Bylaws, as the case may be, shall control. In the event of any conflict between this Agreement (or any notice delivered hereunder) and the Company's books and records, the Company's books and records shall control absent fraud or error.
- 8.18 <u>Spousal Consent</u>. If any individual Stockholder is married on the date of this Agreement or the date such Stockholder becomes a party to this Agreement pursuant to Section 8.13 or Section 8.14, such Stockholder's spouse shall, concurrently with the execution of this Agreement by such Stockholder, execute and deliver to the Company a spousal consent in substantially the form of <u>Exhibit B</u> attached hereto ("*Spousal Consent*"). Notwithstanding the execution and delivery thereof, such Spousal Consent shall not be deemed to confer or convey to

such Stockholder's spouse any rights in such Stockholder's shares of capital stock of the Company that do not otherwise exist by operation of law or the agreement of the parties. If any individual Stockholder marries (or remarries) after the date of this Agreement or the date such Stockholder becomes a party to this Agreement pursuant to Section 8.13 or Section 8.14, such Stockholder shall, within 30 days thereafter, obtain his or her new spouse's acknowledgement of, and consent to, the existence and binding effect of all restrictions contained in this Agreement by causing such spouse to execute and deliver a Spousal Consent.

8.19 <u>Prior Agreement Superseded</u>. Pursuant to Section 8.7 of the Prior Agreement, the undersigned parties who are parties to the Prior Agreement hereby amend and restate the Prior Agreement to read in its entirety as set forth in this Agreement, such that the Prior Agreement shall be of no further force or effect and is hereby entirely replaced and superseded by this Agreement.

8.20 Other Business Activities of Investors. The Company acknowledges that the Investors, and each of their respective Affiliates are in the business of investing and therefore review the business plans and related proprietary information of many enterprises, including enterprises that may have products or services that compete directly or indirectly with those of the Company. Nothing in this Agreement or any other agreement related to the transactions contemplated by this Agreement (collectively, the "Transaction Agreements") shall preclude or in any way restrict the Investors from investing or participating in any particular enterprise, whether or not such enterprise has products or services that compete with those of the Company. Further, the Company, each Investor and each Key Holder acknowledge and agree that (i) the Investors, and each of their respective Affiliates may presently have, or may engage in the future in, internal development programs, or may receive information from third parties that relates to, and may develop and commercialize products independently or in cooperation with such third parties, that are similar to or that are directly or indirectly competitive with, the Company's development programs, products or services, and (ii) any employee of any Investor, or any of their respective Affiliates serving on the Board or as an observer thereon is serving in such capacity at the request, and for the benefit, of the Company. Accordingly, the Investors', or any of their respective Affiliate's designation of any individual to the Board or as an observer to the Board, the service of such individual on the Board or as an observer thereon on behalf of any Investor, or the exercise by any Investor or any of their respective Affiliates of any rights under this Agreement or any of the Transaction Agreements, shall not in any way preclude or restrict any Investor or their respective Affiliates from conducting any development program, commercializing any product or service or otherwise engaging in any enterprise, whether or not such development program, product, service or enterprise competes with those of the Company, so long as such activities do not result in a violation of applicable law, the confidentiality provisions of this Agreement, any other Transaction Agreement, or any other agreement between the Company, on the one hand, and such Investor or any of their respective Affiliates, on the other hand. Nothing herein or in any other Transaction Agreement shall be construed to impose on any Investor or any of their respective Affiliates or any their respective Board Designees or observers any restriction, duty or obligation other than as expressly set forth herein or therein.

[signature pages follow]

# 4D MOLECULAR THERAPEUTICS, INC.

By: /s/ David Kirn

Name: David Kirn

Title: Chief Executive Officer

Address:

5980 Horton Street

Suite 460

Emeryville, CA 94608

Attention: Chief Executive Officer

# VIKING GLOBAL OPPORTUNITIES ILLIQUID INVESTMENTS SUB-MASTER LP

By: Viking Global Opportunities GP LLC, its general partner

By: /s/ Mathew Bloom

Name: Mathew Bloom Title: Authorized Signatory

#### C.P. PHARMACEUTICALS INTERNATIONAL C.V.

#### Pfizer Manufacturing LLC,

as general partner for and on behalf of C.P. Pharmaceuticals International C.V.

By: /s/ Brian McMohan
Name: Brian McMohan
Title: Senior Vice President

## Pfizer Production LLC,

as general partner for and on behalf of C.P. Pharmaceuticals International C.V.

By: /s/ Colum Lane
Name: Colum Lane
Title: Senior Vice President

#### PFIZER INC.

By: /s/ Barbara Dalton

Name: Barbara Dalton

Title: Vice President, Pfizer Venture

# REPLEON, LLC

By: <u>/s/ Greg Betterton</u>
Name: Greg Betterton

Title: Manager

# CHM VENTURE I, LLC

By: GeneraBio, Inc. Its: Manager

By: /s/ Randy M. Wheelock Name: Randy M. Wheelock

Title: President

# MIRAEASSET-CELLTRION NEW GROWTH FUND I

By: MiraeAsset Capital co., Ltd, its general partner

By: /s/ KuBeom LEE

Name: KuBeom LEE

Title: CEO of MiraeAsset Capital co., Ltd

# MIRAEASSET CAPITAL CO., LTD

By: /s/ KuBeom LEE

Name: KuBeom LEE

Title: CEO of MiraeAsset Capital co., Ltd

## MIRAEASSET-NAVER NEW GROWTH FUND I

By: MiraeAsset Capital co., Ltd, its general partner

By: /s/ KuBeom LEE

Name: KuBeom LEE

Title: CEO of MiraeAsset Capital co., Ltd

# MIRAE ASSET HI-TECH FRONTIER INVESTMENT FUND

By: MiraeAsset Venture Investment, Co, Ltd, its general partner

By: /s/ EungSuk KIM

Name: EungSuk KIM

Title: CEO of MiraeAsset Venture Investment, Co, Ltd

# MIRAE ASSET GOOD COMPANY SECONDARY FUND #18-1

By: MiraeAsset Venture Investment, Co, Ltd, its general partner

By: /s/ EungSuk KIM

Name: EungSuk KIM

Title: CEO of MiraeAsset Venture Investment, Co, Ltd

# MIRAEASSET VENTURE INVESTMENT, CO, LTD

By: /s/ EungSuk KIM

Name: EungSuk KIM

Title: CEO of MiraeAsset Venture Investment, Co, Ltd

## RIDGEBACK CAPITAL INVESTMENTS LP

By: Ridgeback Capital Management LP; its Investment Manager

By: /s/ Christopher A. Nonas Name: Christopher A. Nonas Title: Chief Financial Officer

# A.M. PAPPAS LIFE SCIENCE VENTURES V, LP

By: AMP&A Management V, LLC, its General Partner

By: /s/ Ford S. Worthy Name: Ford S. Worthy Title: Partner and CFO

# PV V CEO FUND, LP

By: AMP&A Management V, LLC, its General Partner

By: /s/ Ford S. Worthy Name: Ford S. Worthy Title: Partner and CFO

# CHIESI VENTURES, LP

By: Chiesi Ventures, Inc., its General Partner

By: Pappas Capital, LLC, its Management Company

By: /s/ Ford S. Worthy

Name: Ford S. Worthy Title: Partner and CFO

## **CUREDUCHENNE VENTURES**

By: /s/ Debra Miller Name: Debra Miller

Title: CEO

## BERKELEY CATALYST FUND I LP

By: BCF I LLC Its: General Partner

By: Berkeley Catalyst Fund Management LLC

Its: General Partner

By: /s/ Laura A. Smoliar
Name: Laura A. Smoliar
Title: Managing Member

# PERCEPTIVE LIFE SCIENCES MASTER FUND LTD

51 Astor Place, 10th Floor New York, NY 10003

By: /s/ James H Mannix
Name: James H Mannix
Title: Chief Operating Officer

# JANUS HENDERSON GLOBAL LIFE SCIENCES FUND

By: /s/ Andrew Acker

Name: Andrew Acker

Title: Portfolio Manager and Authorized Signatory

JANUS HENDERSON CAPITAL FUNDS PLC on behalf of its series,

JANUS GLOBAL LIFE SCIENCES FUND

By: /s/ Andrew Acker

Name: Andrew Acker

Title: Portfolio Manager and Authorized Signatory

#### MERIDIAN SMALL CAP GROWTH FUND

By: its Investment Adviser

ArrowMark Colorado Holdings, LLC

By: /s/ David Corkins

Name: David Corkins Title: Managing Member

## ARROWMARK LIFE SCIENCE FUND, LP

By: its General Partner AMP Life Science GP, LLC

By: /s/ David Corkins

Name: David Corkins Title: Managing Member

# ARROWMARK FUNDAMENTAL OPPORTUNITY FUND, L.P.

By: its General Partner ArrowMark Partners GP, LLC

By: /s/ David Corkins

Name: David Corkins Title: Managing Member

#### LOOKFAR INVESTMENTS, LLC

By: /s/ David Corkins

Name: David Corkins Title: Managing Member

# CF ASCENT LLC

By: /s/ David Corkins

Name: David Corkins Title: Managing Member

#### **PURCHASER:**

#### THB IRON ROSE LLC

By: its Investment Adviser ArrowMark Colorado Holdings, LLC

By: /s/ David Corkins

Name: David Corkins Title: Managing Member

## IRON HORSE INVESTMENT, LLC

By: its Investment Adviser

ArrowMark Colorado Holdings, LLC

By: /s/ David Corkins

Name: David Corkins Title: Managing Member

# THB IRON ROSE LLC, LIFE SCIENCE PORTFOLIO

By: its Investment Adviser

ArrowMark Colorado Holdings, LLC

By: /s/ David Corkins

Name: David Corkins Title: Managing Member

#### BIOTECHNOLOGY VALUE FUND, L.P.

By: /s/ Mark Lampert

Name: Mark Lampert

Title: President BVF Inc., General Partner of BVF Partners

L.P., itself GP of Biotechnology Value Fund, L.P.

## BIOTECHNOLOGY VALUE FUND II, LP

By: /s/ Mark Lampert

Name: Mark Lampert

Title: President BVF Inc., General Partner of BVF Partners

L.P., itself GP of Biotechnology Value Fund II, L.P.

# BIOTECHNOLOGY VALUE TRADING FUND OS, L.P.

By: /s/ Mark Lampert

Name: Mark Lampert

Title: President BVF Inc., General Partner of BVF Partners L.P., itself sole member of BVF Partners OS Ltd., itself GP

of Biotechnology Trading Fund OS, L.P.

#### **INVESTMENT 10, L.L.C.**

By: /s/ Mark Lampert

Name: Mark Lampert

Title: President BVF Inc., General Partner of BVF Partners L.P., itself attorney-in-fact for Investment 10, L.L.C.

## MSI BVF SPV, L.L.C. c/o Magnitude Capital B

By: /s/ Mark Lampert

Name: Mark Lampert

Title: President BVF Inc., itself General Partner of BVF Partners L.P., itself attorney-in-fact for MSI BVF SPV,

L.L.C.

## **ARROWMARK**

By: /s/ Tony Yao Name: Tony Yao

	IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors	' Rights Agreement	as of the date first	writter
above				

KEV	HOI	DER:

By: /s/ David Kirn
Name: David Kirn

#### **EXHIBIT B**

#### CONSENT OF SPOUSE

I, Kristin Ahlquist, spouse of David Kirn, acknowledge that I have read the Amended and Restated Investors' Rights Agreement, dated as of August 27, 2018, to which this Consent is attached as <a href="Exhibit B">Exhibit B</a> (the "**Agreement**"), and that I know the contents of the Agreement. I am aware that the Agreement contains provisions regarding the voting and transfer of shares of capital stock of the Company that my spouse may own, including any interest I might have therein.

I hereby agree that my interest, if any, in any shares of capital stock of the Company subject to the Agreement shall be irrevocably bound by the Agreement and further understand and agree that any community property interest I may have in such shares of capital stock of the Company shall be similarly bound by the Agreement.

I am aware that the legal, financial and related matters contained in the Agreement are complex and that I am free to seek independent professional guidance or counsel with respect to this Consent. I have either sought such guidance or counsel or determined after reviewing the Agreement carefully that I will waive such right.

Dated: August 27, 2018 /s/ Kristin Ahlquist

Name: Kristin Ahlquist

# **KEY HOLDER:**

By: /s/ Theresa Janke

Name: Theresa Janke

#### **EXHIBIT B**

#### CONSENT OF SPOUSE

I, Bradley Janke, spouse of Theresa Janke, acknowledge that I have read the Amended and Restated Investors' Rights Agreement, dated as of August 27, 2018, to which this Consent is attached as <a href="Exhibit B">Exhibit B</a> (the "**Agreement**"), and that I know the contents of the Agreement. I am aware that the Agreement contains provisions regarding the voting and transfer of shares of capital stock of the Company that my spouse may own, including any interest I might have therein.

I hereby agree that my interest, if any, in any shares of capital stock of the Company subject to the Agreement shall be irrevocably bound by the Agreement and further understand and agree that any community property interest I may have in such shares of capital stock of the Company shall be similarly bound by the Agreement.

I am aware that the legal, financial and related matters contained in the Agreement are complex and that I am free to seek independent professional guidance or counsel with respect to this Consent. I have either sought such guidance or counsel or determined after reviewing the Agreement carefully that I will waive such right.

Dated: August 27, 2018 /s/ Bradley Janke

Name: Bradley Janke

## **KEY HOLDER:**

By: /s/ Melissa Kotterman

Name: Melissa Kotterman

#### **EXHIBIT B**

#### CONSENT OF SPOUSE

I, William L Kotterman, spouse of Melissa Kotterman, acknowledge that I have read the Amended and Restated Investors' Rights Agreement, dated as of August 27, 2018, to which this Consent is attached as <u>Exhibit B</u> (the "**Agreement**"), and that I know the contents of the Agreement. I am aware that the Agreement contains provisions regarding the voting and transfer of shares of capital stock of the Company that my spouse may own, including any interest I might have therein.

I hereby agree that my interest, if any, in any shares of capital stock of the Company subject to the Agreement shall be irrevocably bound by the Agreement and further understand and agree that any community property interest I may have in such shares of capital stock of the Company shall be similarly bound by the Agreement.

I am aware that the legal, financial and related matters contained in the Agreement are complex and that I am free to seek independent professional guidance or counsel with respect to this Consent. I have either sought such guidance or counsel or determined after reviewing the Agreement carefully that I will waive such right.

Dated: August 27, 2018 /s/ William L Kotterman

Name: William L Kotterman

## **KEY HOLDER:**

# SCHAFFER-HINH FAMILY TRUST

By: /s/ David Schaffer Name: David Schaffer

Title: Trustee

By: /s/ Linda Hinh Name: Linda Hinh Title: Trustee

### **SCHEDULE 1**

## SCHEDULE OF INVESTORS

# VIKING GLOBAL OPPORTUNITIES ILLIQUID INVESTMENTS SUB-MASTER LP

c/o Viking Global Investors LP

55 Railroad Avenue

Greenwich, CT 06830

# JANUS HENDERSON GLOBAL LIFE SCIENCES FUND

c/o Janus Capital Management LLC

151 Detroit Street

Denver, CO 80206

## JANUS HENDERSON CAPITAL FUNDS PLC

c/o Janus Capital Management LLC

151 Detroit Street

Denver, CO 80206

## BIOTECHNOLOGY VALUE FUND, L.P.

44 Montgomery Street 40th Floor

San Francisco, CA 94104

## BIOTECHNOLOGY VALUE FUND II, LP

44 Montgomery Street 40th Floor

San Francisco, CA 94104

## BIOTECHNOLOGY VALUE TRADING FUND OS, L.P.

PO Box 309 Ugland House,

Grand Cayman, KY1-1104, Cayman Islands

# INVESTMENT 10, L.L.C.

Address: 900 N Michigan Ave, Suite 1100 Chicago, IL 60611

# MSI BVF SPV, L.L.C. c/o Magnitude Capital

200 Park Avenue, 56th Floor

New York, NY 10166

## MERIDIAN SMALL CAP GROWTH FUND

c/o ArrowMark Colorado Holdings, LLC 100 Fillmore Street, Suite 325 Denver, CO 80206

# ARROWMARK LIFE SCIENCE FUND, LP

c/o ArrowMark Colorado Holdings, LLC 100 Fillmore Street, Suite 325 Denver, CO 80206

## ARROWMARK FUNDAMENTAL OPPORTUNITY FUND, L.P.

c/o ArrowMark Colorado Holdings, LLC 100 Fillmore Street, Suite 325 Denver, CO 80206

## LOOKFAR INVESTMENTS, LLC

c/o ArrowMark Colorado Holdings, LLC 100 Fillmore Street, Suite 325 Denver, CO 80206

### CF ASCENT LLC

c/o ArrowMark Colorado Holdings, LLC 100 Fillmore Street, Suite 325 Denver, CO 80206

## THB IRON ROSE LLC

c/o ArrowMark Colorado Holdings, LLC 100 Fillmore Street, Suite 325 Denver, CO 80206

## IRON HORSE INVESTMENTS, LLC

c/o ArrowMark Colorado Holdings, LLC 100 Fillmore Street, Suite 325 Denver, CO 80206

## THB IRON ROSE LLC, LIFE SCIENCE PORTFOLIO

c/o ArrowMark Colorado Holdings, LLC 100 Fillmore Street, Suite 325 Denver, CO 80206

## TONY YAO

c/o ArrowMark Colorado Holdings, LLC 100 Fillmore Street, Suite 325 Denver, CO 80206

# A.M. PAPPAS LIFE SCIENCE VENTURES V, LP

c/o Pappas Capital, LLC 2520 Meridian Parkway, Suite 400 Durham, NC 27713

# PV V CEO FUND, LP

c/o Pappas Capital, LLC 2520 Meridian Parkway, Suite 400 Durham, NC 27713

## CHIESI VENTURES, LP

c/o Pappas Capital, LLC 2520 Meridian Parkway, Suite 400 Durham, NC 27713

## PFIZER INC.

230 East Grand South San Francisco, CA 94080 With a copy to: Pfizer Inc. 235 E. 42nd Street New York, NY 10017

## PERCEPTIVE LIFE SCIENCES MASTER FUND LTD

51 Astor Place 10th floor New York NY 10003

## RIDGEBACK CAPITAL INVESTMENTS LP

75 Ninth Avenue, 5th Floor New York, NY 10011

## **CUREDUCHENNE VENTURES**

1400 Quail St. #110, Newport Beach, CA 92660

# BERKELEY CATALYST FUND I LP

19925 Stevens Creek Blvd., Suite 100 Cupertino, CA 95014

# C.P. PHARMACEUTICALS INTERNATIONAL C.V.

c/o its General Partners, Pfizer Manufacturing LLC and Pfizer Production LLC 235 East 42nd Street New York, NY 10017 United States of America With a copy to:
Pfizer Inc.
230 East Grand
South San Francisco, CA 94080

Pfizer Inc. 235 E. 42nd Street New York, NY 10017

## CHM VENTURE I, LLC

PO Box 5331

Johnson City, TN 37602-5331

## REPLEON, LLC

735 E. Venice Avenue, Ste 200 Venice, FL 34285

# MIRAEASSET-CELLTRION NEW GROWTH FUND I

2F, GlassTower Bldg, 534, Teheran-ro, Gangnam-gu, Seoul, 06181, Korea

# MIRAEASSET CAPITAL CO., LTD

 $2F\!,$  Glass Tower Bldg, 534, Teheran-ro, Gangnam-gu, Seoul, 06181, Korea

# MIRAEASSET-NAVER NEW GROWTH FUND I

2F, GlassTower Bldg, 534, Teheran-ro, Gangnam-gu, Seoul, 06181, Korea

## MIRAE ASSET HI-TECH FRONTIER INVESTMENT FUND

Mirae Asset Venture Tower

B1F20, Pangyoyeok-ro 241 beongil, Bundang-gu, Seongnam-si, Gyeonggi-do, 13494, Republic of Korea

# MIRAE ASSET GOOD COMPANY SECONDARY FUND #18-1

Mirae Asset Venture Tower

B1F20, Pangyoyeok-ro 241 beongil, Bundang-gu, Seongnam-si, Gyeonggi-do, 13494, Republic of Korea

# MIRAEASSET VENTURE INVESTMENT, CO, LTD

Mirae Asset Venture Tower

B1F20, Pangyoyeok-ro 241 beongil, Bundang-gu, Seongnam-si, Gyeonggi-do, 13494, Republic of Korea

# SCHEDULE 2

# **KEY HOLDERS**

David Kirn

**Schaffer-Hinh Family Trust** 

Theresa Janke

Melissa Kotterman

# EXHIBIT A

# ADOPTION AGREEMENT

the terms of Company a in this Ado	Adoption Agreement ("Adoption Agreement") is executed on, 20, by the undersigned (the "Holder") pursuant to f that certain Amended and Restated Investors' Rights Agreement dated as of August 27, 2018 (the "Agreement"), by and among the und certain of its Stockholders, as such Agreement may be amended or amended and restated hereafter. Capitalized terms used but not defined ption Agreement shall have the respective meanings ascribed to such terms in the Agreement. By the execution of this Adoption Agreement, agrees as follows.
	acknowledgement. Holder acknowledges that Holder is acquiring certain shares of the capital stock of the Company (the " <b>Stock</b> "), for one of ng reasons (Check the correct box):
	As a transferee of Shares from a party in such party's capacity as an "Investor" bound by the Agreement, and after such transfer, Holder shall be considered an "Investor" and a "Stockholder" for all purposes of the Agreement.
	As a transferee of Shares from a party in such party's capacity as a "Key Holder" bound by the Agreement, and after such transfer, Holder shall be considered a "Key Holder" and a "Stockholder" for all purposes of the Agreement.
	As a new Investor in accordance with <u>Subsection 8.13(a)</u> of the Agreement, in which case Holder will be an "Investor" and a "Stockholder" for all purposes of the Agreement.
	In accordance with <u>Subsection 8.13(b)</u> of the Agreement, as a new party who is not a new Investor, in which case Holder will be a "Stockholder" for all purposes of the Agreement.
thereby, sha	agreement. Holder hereby (a) agrees that the Stock, and any other shares of capital stock or securities required by the Agreement to be bound all be bound by and subject to the terms of the Agreement and (b) adopts the Agreement with the same force and effect as if Holder were a party thereto.
1.3 <u>N</u> signature h	Notice. Any notice required or permitted by the Agreement shall be given to Holder at the address or facsimile number listed below Holder's ereto.
HOLDER:	: ACCEPTED AND AGREED:
By: Name and	Title of Signatory 4D MOLECULAR THERAPEUTICS, INC.
Address:	By:
	Title:

Facsimile Number:

# EXHIBIT B

# CONSENT OF SPOUSE

I, [], spouse of [], acknowledge that I have read the Amended and Restated Investors' Rigingreement, dated as of August 27, 2018, to which this Consent is attached as Exhibit B (the "Agreement"), and that I know the contents of the greement. I am aware that the Agreement contains provisions regarding the voting and transfer of shares of capital stock of the Company that my bouse may own, including any interest I might have therein.	
I hereby agree that my interest, if any, in any shares of capital stock of the Company subject to the Agreement shall be irrevocably by the Agreement and further understand and agree that any community property interest I may have in such shares of capital stock of the Companial be similarly bound by the Agreement.	
I am aware that the legal, financial and related matters contained in the Agreement are complex and that I am free to seek independent of the contained of the contained after reviewing the greement carefully that I will waive such right.	nt
ated:	
[Name of Key Holder's Spouse]	

### 4D MOLECULAR THERAPEUTICS, INC.

### 2015 EQUITY INCENTIVE PLAN

Adopted by Board and Stockholders on March 20, 2015, as amended on March 10, 2016 and August 27, 2018 Termination Date: March 20, 2025

## 1. **Purposes of the Plan**. The purposes of the Plan are:

- to attract and retain the best available personnel for positions of substantial responsibility;
- · to provide incentives that align the interests of Employees, Directors and Consultants with those of the Company's stockholders; and
- to promote the success of the Company's business.

The Plan permits the grant of Incentive Stock Options, Nonstatutory Stock Options, Stock Appreciation Rights, Restricted Stock and Restricted Stock Units.

# 2. **<u>Definitions</u>**. As used herein, the following definitions will apply:

- (a) "*Administrator*" means the Committee; <u>provided</u>, <u>however</u>, that "*Administrator*" means the Board if (i) no Committee is appointed by the Board or (ii) the Board terminates the Committee's responsibilities hereunder and revests in the Board the administration of the Plan; <u>provided</u>, <u>further</u>, that "*Administrator*" may be comprised of different Committees with respect to different groups of Service Providers.
- (b) "*Affiliate*" means a parent corporation (or other entity) of the Company, a majority-owned subsidiary of the Company or a majority-owned subsidiary of the Company's parent corporation (or other entity).
- (c) "*Applicable Laws*" means the requirements related to or implicated by the administration of the Plan under U.S. state corporate laws, U.S. federal and state securities laws, the Code, state and local tax laws, any stock exchange or quotation system on which the Common Stock is listed or quoted and the applicable laws of any foreign country or jurisdiction where Awards are granted.
- (d) "Award" means any right granted under the Plan, including an Incentive Stock Option, a Nonstatutory Stock Option, a Stock Appreciation Right, Restricted Stock or a Restricted Stock Unit.
- (e) "*Award Agreement*" means a written or electronic agreement, contract, certificate or other instrument or document setting forth the terms and conditions applicable to an Award. Each Award Agreement will be subject to the terms and conditions of the Plan.
  - (f) "Board" means the board of directors of the Company.

- (g) "Certificate of Incorporation" means the Company's Certificate of Incorporation, as may be amended from time to time.
- (h) "Change in Control" means the occurrence of either of the following events:
- (i) a merger or consolidation in which the Company is a constituent party (or a subsidiary of the Company is a constituent party and the Company issues shares of its capital stock pursuant to such merger or consolidation), except any such merger or consolidation (A) effected exclusively for the purpose of changing the Company's domicile or (B) involving the Company (or a subsidiary of the Company) in which the shares of capital stock of the Company outstanding immediately prior to such merger or consolidation continue to represent, or are converted into or exchanged for shares of capital stock that represent, immediately following such merger or consolidation, at least a majority, by voting power, of the capital stock of (x) the surviving or resulting corporation or (y) if the surviving or resulting corporation is a wholly owned subsidiary of another corporation immediately following such merger or consolidation, the parent corporation of such surviving or resulting corporation; or
- (ii) the sale, lease, transfer, exclusive license or other disposition, in a single transaction or series of related transactions, by the Company (or any subsidiary of the Company) of all or substantially all of the assets of the Company and its subsidiaries taken as a whole or the sale or disposition (whether by merger or otherwise) of one or more subsidiaries of the Company if substantially all of the assets of the Company and its subsidiaries taken as a whole are held by such subsidiary or subsidiaries, except where such sale, lease, transfer, exclusive license or other disposition is to a wholly owned subsidiary of the Company;

<u>provided</u>, <u>however</u>, that any transaction or series of transactions principally for bona fide equity financing purposes in which cash is received by the Company or indebtedness of the Company is cancelled or converted (or a combination thereof) will not be deemed to be a Change in Control.

- (i) "*Code*" means the Internal Revenue Code of 1986, as it may be amended from time to time. Any reference to a section of the Code will be deemed to include a reference to any regulations and Internal Revenue Service guidance promulgated thereunder.
- (j) "*Committee*" means the compensation committee of the Board or other committee of one or more Directors appointed by the Board to administer the Plan in accordance with Section 4.
  - (k) " Common Stock ' means the common stock, par value \$0.0001 per share, of the Company.
  - (1) "Company" means 4D Molecular Therapeutics, Inc., a Delaware corporation, and any successor thereto.

- (m) "Consultant" means any Person (i) engaged by the Company or any Affiliate to render consulting or advisory services to such entity and who is compensated for such services or (ii) serving as a member of the board of directors of any Affiliate and who is compensated for such services; provided, however, that the term "Consultant" will not include Directors who are not compensated by the Company for their services as Directors, and the payment of a director's fee by the Company for services as a Director will not cause a Director to be considered a "Consultant" for purposes of the Plan.
  - (n) "Director" means a member of the Board.
- (o) "*Disability*" has the meaning ascribed to that term under Section 22(e)(3) of the Code; <u>provided</u>, <u>however</u>, that, in the case of Awards other than Incentive Stock Options or Awards subject to Section 409A of the Code, the Administrator, in its sole discretion, may determine whether a Disability exists in accordance with uniform and non-discriminatory standards adopted by the Administrator from time to time.
- (p) "*Employee*" means any individual, including an officer or director, employed by the Company or any Affiliate; <u>provided</u>, <u>however</u>, that, for purposes of determining eligibility to receive Incentive Stock Options, "*Employee*" means an employee of the Company or a parent or subsidiary corporation within the meaning of Section 424 of the Code. Neither service as a Director nor payment of a director's fee by the Company for such service or for service as a member of the board of directors of any Affiliate will be sufficient to constitute "*employment*" by the Company or any Affiliate.
  - (q) "Exchange Act" means the Securities Exchange Act of 1934, as amended.
- (r) "*Exchange Program*" means a program under which (i) outstanding Awards are surrendered or cancelled in exchange for cash, Awards of the same type (which may have higher or lower purchase, exercise or base prices and different terms) or Awards of a different type, and/or (ii) Participants would have the opportunity to transfer any outstanding Awards to a financial institution or other Person selected by the Administrator, and/or (iii) the purchase, exercise or base price of an outstanding Award is reduced or increased.
- (s) "*Fair Market Value*" means, as of any date, the value of the Common Stock determined in good faith by the Administrator and in a manner consistent with Section 260.140.50 of Title 10 of the California Code of Regulations and, with respect to Nonstatutory Stock Options and Stock Appreciation Rights, in compliance with Section 409A of the Code.
- (t) "*Incentive Stock Option*" means a stock option granted pursuant to the Plan that by its terms qualifies and is otherwise intended to qualify as an incentive stock option within the meaning of Section 422 of the Code.
- (u) "Nonstatutory Stock Option" means a stock option granted pursuant to the Plan that by its terms does not qualify or is not intended to qualify as an Incentive Stock Option.
  - (v) "Option" means an Incentive Stock Option or a Nonstatutory Stock Option.
- (w) "Participant" means an eligible person to whom an Award is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Award.

- (x) "*Period of Restriction*" means the period during which the transfer of Restricted Stock is subject to restrictions and, therefore, the Restricted Stock is subject to a substantial risk of forfeiture. Such restrictions may be based on the passage of time, the achievement of target levels of performance or the occurrence of other events as determined by the Administrator.
- (y) "*Person*" means any individual, firm, corporation, association, partnership, limited liability company, trust, joint venture, governmental entity or other entity.
  - (z) "Plan" means this 2015 Equity Incentive Plan, as amended from time to time in accordance herewith.
- (aa) "Restricted Stock" means Shares issued pursuant to an Award granted under Section 8 or issued pursuant to the early exercise of an Option.
- (bb) "*Restricted Stock Unit*" means a bookkeeping entry representing an amount equal to the Fair Market Value of one Share granted under Section 9 and payable in cash, in Shares or in a combination thereof, as specified in the applicable Award Agreement. Each Restricted Stock Unit represents an unfunded and unsecured obligation of the Company.
  - (cc) "Securities Act" means Securities Act of 1933, as amended.
  - (dd) "Service Provider" means an Employee, Director or Consultant.
  - (ee) "Share" means a share of the Common Stock, as adjusted in accordance with Section 13.
- (ff) "Stock Appreciation Right" means the right pursuant to an Award granted under Section 7 to receive, upon exercise, payment from the Company (in cash, in Shares or in a combination thereof, as specified in the applicable Award Agreement) in an amount determined by multiplying (i) the difference between the Fair Market Value of a Share on the date of exercise over the base price by (ii) the number of Shares with respect to which the Stock Appreciation Right is exercised.
- (gg) "Stock Sale" means a change in ownership of the Company, other than a Change in Control, that occurs when one Person, or more than one Person acting as a group, acquires ownership of stock of the Company, in a stock sale or exchange, that, together with the stock held by such Person or group, constitutes more than 50% of the total voting power of the Stock of the Company; provided, however, that a Stock Sale will not occur if any Person, or more than one Person acting as a group, owns more than 50% of the total voting power of the stock of the Company and acquires additional stock of the Company; provided, further, that any change in the ownership of the stock of the Company as a result of a private financing of the Company that is approved by the Board will not be considered a Stock Sale.

### 3. Shares Subject to the Plan.

- (a) <u>Share Reserve; Source of Shares</u>. Subject to adjustment as provided in Section 13, the maximum aggregate number of Shares that are available for all Awards is Two Million Six Hundred Ninety-Four Thousand Five Hundred Twenty-Eight (2,694,528) Shares. During the term of the Awards, the Company shall at all times reserve and keep available such number of Shares as will be sufficient to satisfy such Awards. The Shares may be authorized but unissued Shares, reacquired Shares or a combination thereof.
- (b) Reversion of Shares to the Share Reserve. If Shares subject to an outstanding Award are not issued or delivered or are returned to the Company by reason of (i) the expiration, termination, cancellation or forfeiture of such Award, (ii) the settlement of such Award in cash, or (iii) the delivery or withholding of Shares to pay all or a portion of the exercise price of an Award, if any, or to satisfy all or a portion of the tax withholding obligations relating to an Award, then such Shares will revert to and again become available for issuance under the Plan. If the exercise price of any Award is satisfied by tendering Shares held by the Participant, then the number of such tendered Shares will revert to and again become available for issuance under the Plan. With respect to Stock Appreciation Rights, only Shares actually issued pursuant to a Stock Appreciation Right will cease to be available under the Plan; all remaining Shares under Stock Appreciation Rights will remain available for future grant or sale under the Plan. Shares that have actually been issued under any Award will not be returned to the Plan and will not again become available for Awards; provided, however, that if Shares issued pursuant to Awards of Restricted Stock or Restricted Stock Units are repurchased by, or forfeited to, the Company due to the failure to vest, such Shares will become available for future grant under the Plan.
- (c) <u>Other Limits</u>. Subject to adjustment as provided in Section 13, the maximum aggregate number of Shares that may be issued upon the exercise of Incentive Stock Options will equal the aggregate Share number stated in Section 3(a) plus, to the extent allowable under Section 422 of the Code, any Shares that become available for issuance under the Plan pursuant to Section 3(b) by reason of the expiration, termination, cancellation or forfeiture of an Award.

## 4. Administration of the Plan.

- (a) **Procedure**. The Plan shall be administered by the Administrator. The Board may terminate the Committee's responsibilities hereunder at any time and revest in the Board the administration of the Plan. The members of the Committee will be appointed by, and serve at the pleasure of, the Board. From time to time, the Board may increase or decrease the size of the Committee and add additional members to, remove members (with or without cause) from, appoint new members in substitution therefor and fill vacancies, however caused, in the Committee. The Committee will act pursuant to a vote (or written consent) of the majority of its members or, if the Committee is comprised of only two members, the unanimous vote (or written consent) of its members, whether present or not. Minutes will be kept of all of meetings of the Committee, and copies thereof will be provided to the Board.
- (b) <u>Powers of the Administrator</u>. Subject to the provisions of the Plan and Applicable Laws and, if applicable, specific duties delegated by the Board to the relevant Committee, the Administrator will have the authority:
  - (i) to determine the Fair Market Value;

- (ii) to select the Service Providers to whom Awards will be granted and the type of Award that will be granted;
- (iii) to determine when Awards are to be granted, the applicable grant date and the number of Shares to be covered by each Award;
- (iv) to approve forms of Award Agreements for use under the Plan;
- (v) to determine the terms and conditions of any Award, including the purchase, exercise or base price, the time or times when Awards may be exercised (which may be based on performance criteria), any forfeiture events, any vesting acceleration or waiver of forfeiture restrictions, and any restriction or limitation regarding any Award or the Shares relating thereto, based in each case on such factors as the Administrator determines;
  - (vi) to institute and determine the terms and conditions of any Exchange Program;
  - (vii) to construe and interpret the terms of the Plan and Awards;
- (viii) to establish sub-plans under the Plan, containing such limitations and other terms and conditions as the Administrator determines are necessary or desirable, for the purpose of satisfying blue sky, securities, tax or other laws of various jurisdictions in which the Company intends to grant Awards or qualifying for favorable tax treatment under applicable foreign laws;
  - (ix) to prescribe, amend and rescind rules and regulations relating to the Plan, including rules and regulations relating to sub-plans;
- (x) to correct any defect, omission or inconsistency in the Plan or any Award Agreement, in a manner and to the extent it deems necessary or advisable to make the Plan fully effective;
- (xi) to amend any outstanding Award, including the discretionary authority to accelerate the time at which an Award may first be exercised or the time during which an Award or any part thereof will vest in accordance with the Plan or to extend the post-termination exercisability period of Awards (subject to Section 409A of the Code) and to extend the maximum term of an Option;
  - (xii) to allow Participants to satisfy tax withholding obligations in a manner prescribed by Section 14;
  - (xiii) to authorize any individual to execute, on behalf of the Company, any instrument required to carry out the purposes of the Plan;
- (xiv) to allow a Participant to defer the receipt of the payment of cash or the delivery of Shares that otherwise would be due to such Participant under an Award (subject to Section 409A of the Code); and

- (xv) to make all other determinations deemed necessary or advisable for administering the Plan.
- (c) <u>Effect of Administrator's Decision</u>. Subject to Section 4(a), the Administrator's decisions, determinations and interpretations will be final, binding and conclusive on all Persons.
- 5. <u>Eligibility</u>. Nonstatutory Stock Options, Stock Appreciation Rights, Restricted Stock and Restricted Stock Units may be granted to Service Providers. Incentive Stock Options may be granted only to Employees.

### 6. Stock Options.

- (a) <u>Grant of Options</u>. Subject to the terms and conditions of the Plan, the Administrator, at any time and from time to time, may grant Options in such amounts as the Administrator, in its sole discretion, will determine.
- (b) **Option Agreement**. Each Award of an Option will be evidenced by an Award Agreement that will specify the exercise price, the term of the Option, the number of Shares subject to the Option, the exercise restrictions, if any, applicable to the Option, and such other terms and conditions as the Administrator, in its sole discretion, will determine.
- (c) <u>Limitations</u>. Each Option will be designated in the applicable Award Agreement as either an Incentive Stock Option or a Nonstatutory Stock Option. Notwithstanding such designation, however, to the extent that the aggregate Fair Market Value (determined at the time of grant) of the Shares with respect to which Incentive Stock Options are exercisable for the first time by any Participant during any calendar year (under all plans of the Company and any Affiliate) exceeds \$100,000, such Options or portions thereof that exceed such limit (according to the order in which they were granted) will be treated as Nonstatutory Stock Options. For purposes of this Section 6(c), calculations will be performed in accordance with Section 422 of the Code.
- (d) <u>Term of Option</u>. The term of each Option will be stated in the applicable Award Agreement; <u>provided</u>, <u>however</u>, that the term will be no more than ten years from the date of grant thereof; <u>provided</u>, <u>further</u>, that, in the case of an Incentive Stock Option granted to an Employee who, at the time the Incentive Stock Option is granted, owns (or, pursuant to Section 424(d) of the Code, is deemed to own) stock representing more than 10% of the total combined voting power of all classes of stock of the Company or any Affiliate, the term of the Incentive Stock Option will be five years from the date of grant thereof or such shorter term as may be provided in the applicable Award Agreement.

## (e) Exercise Price and Consideration.

(i) Exercise Price. The per Share exercise price for the Shares to be issued pursuant to the exercise of an Option will be determined by the Administrator, but will be no less than 100% of the Fair Market Value per Share on the date of grant. In addition, in the case of an Incentive Stock Option granted to an Employee who, at the time the Incentive Stock Option is granted, owns (or, pursuant to Section 424(d) of the Code, is deemed to own) stock representing more than 10% of the total combined voting power of all classes of stock of the Company or any Affiliate, the per Share exercise price will be no less than 110% of the Fair Market Value per Share on the date of grant.

- (ii) <u>Waiting Period and Exercise Dates</u>. The period during which an Option may be exercised will be determined by the Administrator at the time such Option is granted; <u>provided</u>, <u>however</u>, that no Option may be exercised after the expiration of its term. The Administrator may, in its sole discretion, determine any other conditions that must be satisfied before an Option may be exercised.
- (iii) Form of Consideration. The Administrator will determine the acceptable form of consideration for exercising an Option, including the method of payment. In the case of an Incentive Stock Option, the Administrator will determine the acceptable form of consideration at the time of grant. To the extent permitted by Applicable Laws, such consideration, in the Administrator's sole discretion, may consist entirely of: (1) cash; (2) check; (3) promissory note; (4) other Shares owned by the Participant free and clear of any liens, claims, encumbrances or security interests, provided that such Shares have a Fair Market Value on the date of surrender equal to the aggregate exercise price of the Shares as to which such Option will be exercised and provided further that accepting such Shares will not result in any adverse accounting consequences to the Company, as the Administrator determines in its sole discretion; (5) consideration received by the Company under a cashless exercise program (whether through a broker or otherwise) implemented by the Company in connection with the Plan; (6) a net exercise; (7) such other consideration and method of payment for the issuance of Shares; or (8) any combination of the foregoing methods of payment. In making its determination as to the type of consideration to accept, the Administrator will consider if acceptance of such consideration may be reasonably expected to benefit the Company.

## (f) Exercise of Option.

(i) **Procedure for Exercise**. Any Option will be exercisable according to the terms of the Plan and at such times and under such conditions as determined by the Administrator and set forth in the applicable Award Agreement.

An Option will be deemed exercised when the Company receives: (1) a notice of exercise (in such form as the Administrator may specify from time to time) from the person entitled to exercise the Option; and (2) full payment for the Shares with respect to which the Option is exercised (together with applicable tax withholding). Full payment will consist of any consideration and method of payment authorized by the Administrator and permitted by the applicable Award Agreement and the Plan. Shares issued upon exercise of an Option will be issued in the name of the Participant or, if requested by the Participant, in the name of the Participant and his or her spouse. The Company will issue (or cause to be issued) such Shares as soon as practicable after the Option is exercised.

Exercising an Option in any manner will decrease the number of Shares thereafter available, both for purposes of the Plan and for sale under the Option, by the number of Shares as to which the Option is exercised.

- (ii) <u>Termination of Relationship as a Service Provider</u>. If a Participant ceases to be a Service Provider, other than upon the Participant's termination as the result of his or her death or Disability, the Participant may exercise his or her Option, to the extent it is vested on the date of termination, within 30 days following termination or such longer period of time as is specified in the applicable Award Agreement (but in no event later than the expiration of the term of such Option as set forth in the applicable Award Agreement). If, after such termination, the Participant does not exercise his or her Option within the time specified in the preceding sentence, the Option will terminate and the Shares covered by such Option will revert to the Plan. Unless otherwise provided by the Administrator, if on the date of termination the Participant is not vested as to his or her entire Option, the Shares covered by the unvested portion of the Option will revert to the Plan.
- (iii) <u>Disability of Participant</u>. If a Participant ceases to be a Service Provider as a result of his or her Disability, the Participant may exercise his or her Option, to the extent it is vested on the date of termination, within six months following termination or such longer period of time as is specified in the applicable Award Agreement (but in no event later than the expiration of the term of such Option as set forth in the applicable Award Agreement). If, after such termination, the Participant does not exercise his or her Option within the time specified in the preceding sentence, the Option will terminate and the Shares covered by such Option will revert to the Plan. Unless otherwise provided by the Administrator, if on the date of termination the Participant is not vested as to his or her entire Option, the Shares covered by the unvested portion of the Option will revert to the Plan.
- (iv) **Death of Participant**. If a Participant dies while he or she is a Service Provider, the Participant's designated beneficiary (provided such beneficiary has been designated, in a form acceptable to the Administrator, prior to the Participant's death) may exercise the Participant's Option, to the extent it is vested on the date of death, within six months following the Participant's death or such longer period of time as is specified in the applicable Award Agreement (but in no event later than the expiration of the term of such Option as set forth in the applicable Award Agreement). If no such beneficiary was designated by the Participant prior to his or her death, then the Participant's Option may be exercised by the personal representative of the Participant's estate or by the person(s) to whom the Option is transferred pursuant to the Participant's will or in accordance with the laws of descent and distribution. If, after death, the Participant's Option is not so exercised within the time specified in this Section 6(f)(iv), the Option will terminate and the Shares covered by such Option will revert to the Plan. Unless otherwise provided by the Administrator, if at the time of death the Participant is not vested as to his or her entire Option, the Shares covered by the unvested portion of the Option will revert to the Plan.

## 7. Stock Appreciation Rights.

(a) <u>Grant of Stock Appreciation Rights</u>. Subject to the terms and conditions of the Plan, the Administrator, at any time and from time to time, may grant Stock Appreciation Rights in such amounts as the Administrator, in its sole discretion, will determine.

- (b) <u>Stock Appreciation Rights Agreement</u>. Each Award of a Stock Appreciation Right will be evidenced by an Award Agreement that will specify the base price, the term of the Stock Appreciation Right, the number of Shares subject to the Award, the conditions of exercise (including vesting criteria), whether the Award is settled in cash, in Shares or in a combination thereof, and such other terms and conditions as the Administrator, in its sole discretion, will determine.
- (c) <u>Term and Exercise of Stock Appreciation Rights</u>. The term of each Stock Appreciation Right will be stated in the applicable Award Agreement. Notwithstanding the foregoing, the rules of Section 6(d) relating to the maximum term of an Option and Section 6(f) relating to the exercise of Options also will apply to Stock Appreciation Rights.
- (d) **Base Price**. The per Share base price for the Shares that will determine the amount of the payment to be received upon exercise of a Stock Appreciation Right as set forth in Section 7(e) will be determined by the Administrator at the time of grant of the Stock Appreciation Right, but will be no less than 100% of the Fair Market Value per Share on the date of grant.
- (e) <u>Payment of Stock Appreciation Right Amount</u>. Upon a Participant's exercise of a Stock Appreciation Right in accordance with the applicable Award Agreement, the Participant will be entitled to receive payment from the Company in an amount determined by multiplying:
- (i) the difference between the Fair Market Value of a Share on the date of exercise over the per Share base price determined by the Administrator in accordance with Section 7(d); by
  - (ii) the number of vested Shares with respect to which the Stock Appreciation Right is exercised.

The payment upon exercise of a Stock Appreciation Right may, in the Administrator's sole discretion, be in cash, in Shares of equivalent value or in some combination thereof, as set forth in the applicable Award Agreement.

### 8. Restricted Stock.

- (a) <u>Grant of Restricted Stock</u>. Subject to the terms and conditions of the Plan, the Administrator, at any time and from time to time, may grant Restricted Stock in such amounts as the Administrator, in its sole discretion, will determine.
- (b) <u>Restricted Stock Agreement</u>. Each Award of Restricted Stock will be evidenced by an Award Agreement that will specify the Period of Restriction, the number of Shares granted, and such other terms and conditions as the Administrator, in its sole discretion, will determine. Unless the Administrator determines otherwise, as set forth in the applicable Award Agreement, the Company will hold Restricted Stock, as escrow agent, until the restrictions on such Shares have lapsed.
- (c) <u>Removal of Restrictions</u>. Except as otherwise provided in this Section 8, Shares covered by each grant of Restricted Stock will be released from escrow as soon as practicable after the last day of the applicable Period of Restriction or at such other time as the Administrator may determine. The Administrator, in its sole discretion, may accelerate the time at which any restrictions will lapse or be removed.

(d) <u>Return of Restricted Stock to Company</u>. On the date set forth in the applicable Award Agreement, the Restricted Stock for which restrictions have not lapsed will revert to the Company and will again become available for grant under the Plan.

## 9. Restricted Stock Units.

- (a) <u>Grant of Restricted Stock Units</u>. Subject to the terms and conditions of the Plan, the Administrator, at any time and from time to time, may grant Restricted Stock Units in such amounts as the Administrator, in its sole discretion, will determine. No Shares will be issued at the time a Restricted Stock Unit is granted, and the Company will not be required to set aside a fund for the payment of any such Award.
- (b) <u>Restricted Stock Unit Agreement</u>. Each Award of a Restricted Stock Unit will be evidenced by an Award Agreement that will specify the number of Shares subject to the Award, the vesting criteria, whether the Award is settled in cash, in Shares or in a combination thereof, and such other terms and conditions as the Administrator, in its sole discretion, will determine.
- (c) <u>Vesting Criteria</u>. The Administrator, in its sole discretion, will set vesting criteria which, depending on the extent to which the criteria are met, will determine the number of Restricted Stock Units that will be paid out to the Participant. The Administrator, in its sole discretion, may set vesting criteria based upon the achievement of Company-wide, business unit or individual goals (including continued employment or service), or any other basis determined by the Administrator.
- (d) <u>Earning Restricted Stock Units</u>. Upon meeting the applicable vesting criteria, the Participant will be entitled to receive a payout as determined by the Administrator. Notwithstanding the foregoing, at any time after the grant of a Restricted Stock Unit, the Administrator, in its sole discretion, may reduce or waive any vesting criteria that must be met to receive a payout.
- (e) <u>Timing and Form of Payment</u>. Payment of earned Restricted Stock Units will be made at the time, and in the form, set forth in the applicable Award Agreement, but in no event later than the 15<sup>th</sup> day of the third month following the end of the calendar year in which such Restricted Stock Units became vested, except to the extent payment is deferred under an arrangement approved by the Administrator, in accordance with Section 409A of the Code. Settlement of earned Restricted Stock Units may, in the Administrator's sole discretion, be in cash, in Shares or in some combination thereof.
- (f) <u>Return of Restricted Stock Units to Company</u>. On the date set forth in the applicable Award Agreement, all unearned Restricted Stock Units will revert to the Company and will again become available for grant under the Plan.
- 10. <u>Compliance With Section 409A of the Code</u>. Awards will be designed and operated in such a manner that they are either exempt from the application of, or comply with, the requirements of Section 409A of the Code. The Plan and each Award Agreement are intended to meet the requirements of Section 409A of the Code and will be construed and interpreted in accordance with such intent, except as otherwise determined in the Administrator's sole discretion.

- 11. Leaves of Absence/Transfer Between Locations. Unless the Administrator provides otherwise, vesting of a Participant's Awards will be suspended during his or her unpaid leave of absence from the Company or any Affiliate. A Participant will not cease to be an Employee in the case of (i) any leave of absence approved by the Company or (ii) transfers between locations of the Company or between the Company and any Affiliate. For purposes of Incentive Stock Options, no such leave of absence may exceed three months, unless reemployment upon expiration of such leave is guaranteed by statute or contract. If reemployment upon expiration of a leave of absence approved by the Company is not so guaranteed, then six months following the 1st day of such leave, any Incentive Stock Option held by the Participant will cease to be treated as an Incentive Stock Option and will be treated for tax purposes as a Nonstatutory Stock Option.
- 12. <u>Limited Transferability of Awards</u>. Unless determined otherwise by the Administrator, (i) Awards (and, in the case of Options, the Shares subject to such Options prior to exercise) may not be sold, pledged, assigned, hypothecated or otherwise transferred in any manner, including by entering into any short position, any "put equivalent position" or any "call equivalent position" (as defined in Rule 16a-1(h) and Rule 16a-1(b), respectively, of the Exchange Act), whether by operation of law or otherwise, other than by will or by the laws of descent and distribution, and may be exercised, during the lifetime of the Participant, only by the Participant and (ii) Restricted Stock may not be sold, pledged, assigned, hypothecated or otherwise transferred in any manner until the end of the applicable Period of Restriction. If the Administrator makes an Award transferable, such Award may only be transferred (1) by will, (2) by the laws of descent and distribution, (3) to a revocable trust, or (4) as permitted by Rule 701 of the Securities Act. The terms of the Plan will be binding upon the executors, administrators, heirs, successors and assigns of the Participants. Notwithstanding the foregoing, the Administrator, in its sole discretion, may determine to permit transfers to the Company or in connection with a Change in Control, a Stock Sale or other acquisition transaction involving the Company.

## 13. Adjustments; Dissolution or Liquidation; Merger, Change in Control or Stock Sale.

(a) Adjustments. In the event of any dividend or other distribution (whether in the form of cash, Shares, other securities or other property), recapitalization, reincorporation, stock split, reverse stock split, reorganization, merger, consolidation, split-up, spin-off, combination, reclassification, repurchase or exchange of Shares or other securities of the Company, or other change in the corporate structure of the Company affecting the Shares, the Administrator, in order to prevent diminution or enlargement of the benefits or potential benefits intended to be made available under the Plan, will appropriately adjust the number and class of Shares available under the Plan and the number, class and price of Shares subject to each outstanding Award; provided, however, that (i) the Administrator will make such adjustments to an Award required by Section 25102(o) of the California Corporations Code to the extent the Company is relying upon the exemption afforded thereby with respect to the Award and (ii) in the case of outstanding Awards consisting of Options and Stock Appreciation Rights, the Administrator will make such adjustments in accordance with Section 409A of the Code.

- (b) <u>Dissolution or Liquidation</u>. In the event of the proposed dissolution or liquidation of the Company, the Administrator will notify each Participant as soon as practicable prior to the effective date of such proposed transaction. To the extent it has not been previously exercised, the Administrator may cause an Award to terminate immediately prior to the consummation of such proposed dissolution or liquidation of the Company.
- (c) <u>Merger, Change in Control or Stock Sale</u>. In the event of a merger involving the Company, a Change in Control or a Stock Sale, each outstanding Award will be treated as the Administrator (as constituted prior to such merger, Change in Control or Stock Sale) may determine without the Participant's consent, subject to such Participant's Award Agreement. Without limiting the generality of the foregoing sentence, in the event of a merger involving the Company, a Change in Control or a Stock Sale, the Administrator may, in its sole discretion but subject to such Participant's Award Agreement, provide that:
- (i) Awards will be assumed, or substantially equivalent Awards will be substituted, by the acquiring or succeeding entity (or any affiliate thereof) with appropriate adjustments as to the number and kind of shares and prices;
- (ii) all outstanding Awards, in whole or in part, will be surrendered to the Company by the holder thereof and immediately cancelled by the Company, with the holder thereof receiving (A) an amount of cash, if any, equal to the amount that would have been attained upon the exercise of such Award or realization of the Participant's rights as of the date of the occurrence of such merger, Change in Control or Stock Sale (and, for the avoidance of doubt, if as of the date of the occurrence of such merger, Change in Control or Stock Sale the Administrator determines in good faith that no amount would have been attained upon the exercise of such Award or realization of the Participant's rights, then such Award may be terminated by the Company without payment), (B) such other rights or property selected by the Administrator in its sole discretion, or (C) a combination of (A) and (B);
- (iii) all outstanding Options and Stock Appreciation Rights will immediately vest and become exercisable, in whole or in part, all restrictions on Restricted Stock and Restricted Stock Units will lapse, and, with respect to Awards subject to performance-based vesting, all performance goals or other vesting criteria will be deemed achieved at 100% of target level (or such other level specified by the Administrator) and all other terms and conditions met, in whole or in part, prior to or upon consummation of such merger, Change in Control or Stock Sale; and/or
  - (iv) any combination of the foregoing.

In taking any of the actions permitted under this Section 13(c), the Administrator will not be obligated to treat all Awards, all Awards held by a Participant, or all Awards of the same type, similarly.

Notwithstanding anything in this Section 13(c) to the contrary, if a payment under an Award Agreement is subject to Section 409A of the Code and if the Change in Control or Stock Sale does not constitute a "change in control event" as defined in Section 409A of the Code, then any payment of an amount that is otherwise accelerated under this Section 13(c) will be delayed until the earliest time that such payment would be permissible under Section 409A of the Code without triggering any penalties applicable under Section 409A of the Code.

### 14. Tax Withholding.

- (a) <u>Withholding Requirements</u>. Prior to the delivery of any Shares or cash pursuant to an Award (or exercise thereof), the Company will have the power and the right to deduct or withhold, or require a Participant to remit to the Company, an amount sufficient to satisfy federal, state, local, foreign or other taxes (including the Participant's FICA obligation) required to be withheld with respect to such Award (or exercise thereof).
- (b) <u>Withholding Arrangements</u>. The Administrator, in its sole discretion and pursuant to such procedures as it may specify from time to time, may permit a Participant to satisfy the tax withholding obligations described in Section 14(a), in whole or in part, by (without limitation) (i) paying cash, (ii) electing to have the Company withhold otherwise deliverable Shares having a Fair Market Value equal to the minimum statutory amount required to be withheld, (iii) delivering to the Company previously owned and unencumbered Shares having a Fair Market Value equal to the minimum statutory amount required to be withheld, provided the delivery of such Shares will not result in any adverse accounting consequences to the Company, as determined by the Administrator in its sole discretion, or (iv) selling a sufficient number of otherwise deliverable Shares through such means as the Administrator may determine in its sole discretion (whether through a broker or otherwise) equal to the minimum statutory amount required to be withheld. The Fair Market Value of the Shares to be withheld or delivered will be determined as of the date that the taxes are required to be withheld. Any fraction of a Share that would be required to satisfy such an obligation will be disregarded, and the remaining amount due will be paid in cash by the Participant.
- 15. No Effect on Employment or Service. Nothing in the Plan or any instrument executed, or Award granted, pursuant to the Plan, including any Award Agreement, will confer upon any Participant any right with respect to continuing his or her relationship as a Service Provider, nor will they interfere in any way with the Participant's or the Company's right to terminate such relationship at any time, with or without cause or notice, to the extent permitted by Applicable Laws.
- 16. **Date of Grant**. The date of grant of an Award will be, for all purposes, the date on which the Administrator makes the determination granting such Award, or such other later date as is determined by the Administrator. Notice of the determination will be provided to the Participant within a reasonable time after the date of his or her grant.
- 17. **Term of Plan**. Subject to Section 21, the Plan will become effective upon its adoption by the Board. Unless sooner terminated under Section 18, the Plan will continue in effect for a term of ten years from the date the Plan is adopted or the date the Plan is approved by the Company's stockholders, whichever is earlier. No Awards may be granted under the Plan while the Plan is suspended or after it is terminated.

## 18. Amendment and Termination of the Plan.

(a) Amendment and Termination. Subject to Section 18(b), the Board may at any time amend, alter, suspend or terminate the Plan.

- (b) <u>Stockholder Approval</u>. The Company will obtain stockholder approval of any Plan amendment to the extent necessary and desirable to comply with Applicable Laws.
- (c) <u>Effect of Amendment or Termination</u>. Unless the Participant and the Administrator mutually agree otherwise in a written agreement signed by the Participant and the Company, no amendment, alteration, suspension or termination of the Plan will impair the Participant's rights under his or her Award. Termination of the Plan will not affect the Administrator's ability to exercise the powers granted to it hereunder with respect to Awards granted prior to the date of such termination.

### 19. Conditions Upon Issuance of Shares.

- (a) **Legal Compliance**. Shares will not be issued pursuant to the exercise of an Award unless the exercise of such Award and the issuance and delivery of such Shares will comply with Applicable Laws and will be further subject to the approval of the Company's counsel with respect to such compliance.
- (b) <u>Investment Representations</u>. As a condition to the exercise of an Award, the Company may require the person exercising such Award to represent and warrant at the time of any such exercise that the Shares are being purchased only for investment and without any present intention to sell or distribute such Shares if, in the opinion of the Company's counsel, such a representation is required.
- 20. <u>Inability to Obtain Authority</u>. The Company's inability to obtain, after reasonable efforts, authority from any regulatory body having jurisdiction, which authority is deemed by the Company's counsel to be necessary for the lawful issuance and sale of any Shares hereunder, will relieve the Company of any liability for failure to issue or sell such Shares as to which such requisite authority has not been obtained. The Company will not be required to register under the Securities Act the Plan, any Award or any Share issued or issuable pursuant to any Award.
- 21. <u>Stockholder Approval of Plan</u>. The Plan must be approved by a majority of the outstanding securities entitled to vote by the later of (i) within 12 months before or after the date the Plan is adopted by the Board or (ii) prior to or within 12 months of the granting of any Option or issuance of any Share under the Plan in the State of California. Such stockholder approval will be obtained in the manner, and to the degree, required under Applicable Laws.

### 22. Miscellaneous.

(a) <u>Stockholder Rights</u>; <u>Voting Rights</u>. Except as otherwise provided in the Plan or the applicable Award Agreement, no Participant will be deemed to be the holder of, or to have any of the rights of a holder with respect to, any Shares subject to his or her Award unless and until the Shares underlying the Award are actually issued (as evidenced by the appropriate entry on the books of the Company or of a duly authorized transfer agent of the Company) notwithstanding the exercise of the Award. During the Period of Restriction, Participants holding Restricted Stock may exercise full voting rights with respect to such Shares, unless the Administrator, in its sole discretion, determines otherwise. A Participant will have no voting rights with respect to any Restricted Stock Units.

- (b) <u>Dividends and Other Distributions</u>. Unless and until the Shares underlying an Award are actually issued (as evidenced by the appropriate entry on the books of the Company or of a duly authorized transfer agent of the Company), no right to receive dividends or other distributions will exist with respect to the Shares underlying such Award, notwithstanding the exercise of the Award. During the Period of Restriction, Participants holding Restricted Stock will be entitled to receive all dividends and other distributions paid with respect to such Shares, unless the Administrator, in its sole discretion, determines otherwise. If any such dividends or distributions are paid in Shares, such Shares will be subject to the same restrictions on transferability and forfeitability as the Restricted Stock with respect to which they were paid.
- (c) <u>No Fractional Shares</u>. No fractional Shares will be issued or delivered pursuant to the Plan. Except as otherwise provided in the Plan or applicable Award Agreement, the Administrator will determine whether cash, additional Awards or other securities or property will be issued or paid in lieu of fractional Shares or whether any fractional Shares should be rounded, forfeited or otherwise eliminated.
- (d) <u>Severability</u>. If any provision of the Plan is held to be invalid, illegal or unenforceable, in whole or in part, such provision will be deemed modified to the extent, but only to the extent, of such invalidity, illegality or unenforceability, and the remaining provisions will not be affected thereby.
- (e) <u>Headings</u>. The headings contained herein are for purposes of convenience only and are not intended to define or limit the construction of the provisions hereof.
- (f) **Non-Uniform Treatment**. The Administrator's determinations under the Plan need not be uniform and may be made by it selectively among persons who are eligible to receive, or actually receive, Awards. Without limiting the generality of the foregoing, the Administrator will be entitled to make non-uniform and selective determinations, amendments and adjustments and to enter into non-uniform and selective Award Agreements.
- (g) <u>Governing Law</u>. The laws of the State of California will govern all questions concerning the construction, validity and interpretation of the Plan, without regard to such state's conflict of law rules.

### 4D MOLECULAR THERAPEUTICS, INC.

### 2015 EQUITY INCENTIVE PLAN

### STOCK OPTION AGREEMENT

This Stock Option Agreement (this "Agreement") is made and entered into as of "Date\_of\_Grant" by and between 4D Molecular Therapeutics, Inc., a Delaware corporation (the "Company"), and "Participant" ("Participant"). Unless otherwise defined herein, capitalized terms used herein shall have the same defined meanings as set forth in the 4D Molecular Therapeutics, Inc. 2015 Equity Incentive Plan attached hereto as <u>Exhibit A</u> (the "Plan").

### I. NOTICE OF STOCK OPTION GRANT

Participant has been granted an option to purchase Common Stock, subject to the terms and conditions of the Plan and this Agreement, as follows:

Participant: «Participant»
Address: «Address»
«City»

Grant Number: «Grant\_Number»
Grant Date: «Date\_of\_Grant»

Vesting Commencement Date: «Vesting\_Commencement\_Date»
Exercise Price per Share: \$«Exercise\_Price\_per\_Share»

Number of Shares Subject to Option: «Total\_Shares»

Total Exercise Price: \$\(\)\$ \(\)\$ Total\_Exercise\_Price>

Type of Option:  $\square$  ISO  $\square$  NSO

Term/Expiration Date: «Expiration Date», or earlier as provided in the Plan or this Agreement

## **Vesting Schedule:**

This Option shall become vested and exercisable, in whole or in part, according to the following vesting schedule:

25% of the Shares subject to this Option on the Grant Date shall vest on the one-year anniversary of the Vesting Commencement Date, and 1/48<sup>th</sup> of the Shares subject to this Option on the Grant Date shall vest each month thereafter on the same day of the month as the Vesting Commencement Date (or if there is no corresponding day, on the last day of such month), subject to Participant continuing to be a Service Provider through each such date.

## **Termination Period:**

This Option shall be exercisable for three months after Participant ceases to be a Service Provider, unless such termination is due to Participant's death or Disability, in which case this Option shall be exercisable for 12 months after Participant ceases to be a Service Provider. Notwithstanding the foregoing sentence, in no event may this Option be exercised after the Term/Expiration Date as provided above, and this Option may be subject to earlier termination as provided in the Plan.

### II. AGREEMENT

1. <u>Grant of Option</u>. In consideration of the services to be rendered by Participant to the Company or any Affiliate and subject to the terms and conditions of the Plan and this Agreement, the Administrator hereby grants to Participant an option (this "*Option*") to purchase the number of Shares set forth in the Notice of Stock Option Grant in Part I of this Agreement, at the Exercise Price per Share set forth in the Notice of Stock Option Grant in Part I of this Agreement (the "*Exercise Price*").

If designated as an ISO in the Notice of Stock Option Grant in Part I of this Agreement, this Option is intended to qualify as an Incentive Stock Option; provided, however, that, to the extent that the aggregate Fair Market Value (determined at the time of grant) of the Shares with respect to which Incentive Stock Options are exercisable for the first time by Participant during any calendar year (under all plans of the Company and any Affiliate) exceeds \$100,000, such Options or portions thereof that exceed such limit (according to the order in which they were granted) shall be treated as Nonstatutory Stock Options. Further, if for any reason this Option (or portion thereof) shall not qualify as an Incentive Stock Option, then, to the extent of such nonqualification, this Option (or portion thereof) shall be regarded as a Nonstatutory Stock Option. In no event shall the Administrator, the Company or any Affiliate, or any of their respective employees or directors, have any liability to Participant (or any other Person) due to the failure of this Option (or portion thereof) to qualify for any reason as an Incentive Stock Option.

## 2. Exercise of Option.

- (a) <u>Right to Exercise</u>. This Option shall be exercisable during its term in accordance with (i) the Vesting Schedule set out in the Notice of Stock Option Grant in Part I of this Agreement and (ii) the applicable provisions of the Plan and this Agreement. This Option may not be exercised for a fraction of a Share.
- (b) <u>Method of Exercise</u>. This Option shall be exercisable by delivery of an option exercise notice in the form attached hereto as <u>Exhibit B</u> (the "*Option Exercise Notice*") or in a manner and pursuant to such procedures as the Administrator may determine, which shall state the election to exercise this Option, the whole number of Shares with respect to which this Option is being exercised, and such other representations and agreements as may be required by the Company. If someone other than Participant exercises this Option, as permitted by the Plan, then such Person must submit documentation reasonably acceptable to the Company verifying that such Person has the legal right to exercise this Option. The Option Exercise Notice shall be accompanied by payment of the aggregate Exercise Price as to all exercised Shares, together with any applicable tax withholding. This Option shall be deemed to be exercised upon receipt by the Company of such fully executed Option Exercise Notice accompanied by the aggregate Exercise Price, together with any applicable tax withholding.
- 3. <u>Participant's Representations</u>. If the Common Stock has not been registered under the Securities Act at the time this Option is exercised, Participant shall concurrently with the exercise of all or any portion of this Option, if required by the Company, deliver to the Company Participant's Investment Representation Statement in the form attached hereto as **Exhibit C**.

- 4. Lock-Up Period. Participant will not, during the period commencing on the date of the final prospectus relating to the registration by the Company for its own behalf of shares of its Common Stock or any other equity securities under the Securities Act on a Form S-1 (excluding a registration relating solely to employee benefit plans on Form S-1) or Form S-3 and ending on the date specified by the Company and the underwriter(s) (such period not to exceed 180 days in the case of the Company's IPO or 90 days in the case of any registration other than the Company's IPO, or such other period as may be requested by the Company or the underwriters to accommodate regulatory restrictions on (i) the publication or other distribution of research reports and (ii) analyst recommendations and opinions, including the restrictions contained in NASD Rule 2711(f)(4) or NYSE Rule 472(f) (4) (or any successor provisions or amendments thereto), as applicable), (A) sell, dispose of, make any short sale of, offer, hypothecate, pledge, contract to sell, grant any option or contract to purchase, purchase any option or contract to sell, grant any right or warrant to purchase, lend or otherwise transfer or encumber, directly or indirectly, any Shares or other securities convertible into or exercisable or exchangeable (directly or indirectly) for shares of Common Stock (whether such Shares or other securities are then held by Participant or thereafter acquired) (such Shares and other securities, the "Lock-Up Shares") or (B) enter into any swap, hedging or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of any Lock-Up Shares. The foregoing provisions of this Section II.4 shall not prevent the exercise of any repurchase option in favor of the Company or apply to the sale of any Lock-Up Shares to an underwriter pursuant to an underwriting agreement or to the Transfer (as defined in Section II.7) of any Lock-Up Shares by Participant to any trust for the direct or indirect benefit of Participant or an Immediate Family Member (as defined in the Option Exercise Notice) of Participant (provided that the trustee of the trust agrees, in writing, to be bound by the restrictions set forth herein and provided further that any such Transfer (as defined in Section II.7) does not involve a disposition for value). Participant shall execute such documents as may be reasonably requested by the Company or the underwriters in connection with any registered offering described in this Section II.4 and that are consistent with this Section II.4 or necessary to give further effect thereto.
- 5. <u>Method of Payment</u>. To the extent permitted by Applicable Laws, payment of the aggregate Exercise Price as to all exercised Shares shall be by any of the following methods, or a combination thereof, at Participant's election:
  - (a) cash;
  - (b) check;
- (c) surrender of other Shares which (i) shall be valued at their Fair Market Value on the date of exercise and (ii) must be owned by Participant free and clear of any liens, claims, encumbrances or security interests, if accepting such Shares, in the Administrator's sole discretion, will not result in any adverse accounting consequences to the Company; or

(d) consideration received by the Company under a cashless exercise program (whether through a broker or otherwise) implemented by the Company in connection with the Plan.

Any fraction of a Share which would be required to pay such aggregate Exercise Price shall be disregarded, and the remaining amount due shall be paid in cash by Participant.

- 6. <u>Restrictions on Exercise</u>. This Option may not be exercised unless the issuance of Shares upon such exercise, or the method of payment of consideration for such Shares, complies with Applicable Laws. Assuming such compliance, Shares shall be considered transferred to Participant, for income tax purposes, on the date on which this Option is exercised with respect to such Shares.
- 7. Non-Transferability of Option. This Option (or, prior to exercise, the Shares subject to this Option) may not be sold, pledged, assigned, hypothecated or otherwise transferred in any manner, including by entering into any short position, any "put equivalent position" or any "call equivalent position" (as defined in Rule 16a-1(h) and Rule 16a-1(b), respectively, of the Exchange Act), whether by operation of law or otherwise ("Transfer"), other than by will or by the laws of descent and distribution, and may be exercised, during the lifetime of Participant, only by Participant. The terms of the Plan and this Agreement shall be binding upon the executors, administrators, heirs, successors and assigns of Participant.
- 8. **Term of Option**. This Option may be exercised only (i) within the term set out in the Notice of Stock Option Grant in Part I of this Agreement and (ii) in accordance with the terms and conditions of the Plan and this Agreement.

## 9. Tax Obligations.

- (a) <u>Tax Withholding</u>. Participant agrees to make appropriate arrangements satisfactory to the Company to pay or provide for the satisfaction of all federal, state, local, foreign and other taxes (including Participant's FICA obligation) required to be withheld with respect to the exercise of this Option. Participant acknowledges and agrees that the Company may refuse to honor the exercise of this Option, and refuse to deliver the Shares, if such withholding amounts are not delivered by Participant at the time of exercise.
- (b) Notice of Disqualifying Disposition of ISO Shares. If this Option is an Incentive Stock Option, and if Participant makes a "disposition" (as defined in Section 424 of the Code) of all or any portion of the Shares acquired upon exercise of this Option within two years from the Grant Date set out in the Notice of Stock Option Grant in Part I of this Agreement or within one year after issuance of the Shares acquired upon exercise of this Option, then Participant shall immediately notify the Company in writing as to the occurrence of, and the price realized upon, such disposition. Participant agrees that Participant may be subject to income tax withholding by the Company on the compensation income recognized by Participant.
- (c) <u>Section 409A of the Code</u>. Under Section 409A of the Code, an Option that was granted with a per Share exercise price that is determined by the U.S. Internal Revenue Service (the "*IRS*") to be less than the Fair Market Value of a Share on the date of grant (a "*discount option*") may be considered "*deferred compensation*." An Option that is a "*discount option*" may result in (i) income recognition by Participant prior to the exercise of this Option,

(ii) an additional 20% federal income tax, (iii) potential penalty and interest charges, and (iv) additional state income, penalty and interest tax to Participant (collectively, "409A Penalties"). Participant acknowledges that the Company cannot guarantee, and has not guaranteed, that the IRS will agree, in a later examination, that the per Share exercise price of this Option equals or exceeds the Fair Market Value of a Share on the date of grant. Participant agrees that, if the IRS determines that this Option is a "discount option," Participant shall be solely responsible for Participant's costs related to such a determination, including any 409A Penalties.

## 10. Agreement to Participate.

(a) Each Holder (as defined in the Option Exercise Notice) agrees that, in the event a Change in Control or Stock Sale is approved by the Board (if required) and the requisite vote of the Company's stockholders, and upon receipt from the Company of a Participation Notice (as defined below), such Holder shall (1) vote (in person, by proxy or by written consent, as applicable) all securities of the Company the holders of which are entitled to vote for members of the Board, including securities of the Company acquired upon exercise or conversion of any options (including this Option), warrants or other convertible securities, owned by such Holder, or over which such Holder has voting control, in favor of such Change in Control or Stock Sale (together with any related amendment to the Certificate of Incorporation required in order to implement such Change in Control or Stock Sale) and in opposition to any and all other proposals that could delay or impair the ability of the Company or its stockholders to consummate such Change in Control or Stock Sale and (2) sell or exchange all securities of the Company, including securities of the Company acquired upon exercise or conversion of any options (including this Option), warrants or other convertible securities (collectively, "Subject Shares"), that such Holder then beneficially holds pursuant to the terms and conditions of such Change in Control or, in the case of a Stock Sale, sell or otherwise transfer to the acquiring Person all Subject Shares that such Holder then beneficially holds (or in the event that the Company's stockholders that approved such Stock Sale are selling fewer than all of their Subject Shares, shares in the same proportion as such stockholders are selling to the acquiring Person) for the same per share consideration in accordance with the provisions of the Certificate of Incorporation, and on the same terms and conditions (except as otherwise permitted by this Section II.10(a)), as such stockholders, subject to the following conditions:

(i) such Holder shall not be required in his or her capacity as a stockholder to make any representation or warranty in connection with such Change in Control or Stock Sale, other than as to such Holder's ownership and authority to sell, exchange or otherwise transfer such Holder's Subject Shares in such Change in Control or Stock Sale, free and clear of all claims, rights, obligations, liens and encumbrances of any nature whatsoever:

(ii) such Holder shall not be liable for the inaccuracy of any representation or warranty made by any other Person in connection with such Change in Control or Stock Sale, other than the Company (except to the extent that funds may be paid out of an escrow established to cover breach of representations, warranties and covenants of the Company as well as breach by any stockholder of any identical representations, warranties and covenants provided by all stockholders);

(iii) the liability for indemnification, if any, of such Holder in such Change in Control or Stock Sale, and for the inaccuracy of any representations and warranties made by the Company or its stockholders in connection with such Change in Control or Stock Sale, shall be several and not joint with any other Person (except to the extent that funds may be paid out of an escrow established to cover breach of representations, warranties and covenants of the Company as well as breach by any stockholder of any identical representations, warranties and covenants provided by all stockholders) and, subject to any provisions of the Certificate of Incorporation relating to the allocation of the escrow, shall be pro rata in proportion to, and shall not exceed, the amount of consideration paid to such Holder in connection with such Change in Control or Stock Sale;

(iv) liability shall be limited to such Holder's applicable share (determined based on the respective proceeds payable to each stockholder of the Company in connection with such Change in Control or Stock Sale in accordance with the Certificate of Incorporation) of a negotiated aggregate indemnification amount that applies equally to all stockholders of the Company but that in no event exceeds the amount of consideration otherwise payable to such Holder in connection with such Change in Control or Stock Sale, except with respect to claims related to fraud by such Holder, the liability for which need not be limited as to such Holder;

(v) as a result of such Change in Control or Stock Sale, (A) each holder of each class or series of capital stock of the Company shall be entitled to receive the same form of consideration (and be subject to the same indemnity and escrow provisions) for their shares of such class or series as is received by other holders with respect to their shares of such same class or series of stock, (B) each holder of a series of Preferred Stock of the Company shall receive the same amount of consideration per share of such series of Preferred Stock of the Company as is received by other holders with respect to their shares of such same series, (C) each holder of Common Stock shall receive the same amount of consideration per share of Common Stock as is received by other holders with respect to their shares of Common Stock, and (D) unless the holders of each series of Preferred Stock of the Company agree otherwise by legally sufficient amendment or waiver of the provisions of the Certificate of Incorporation then in effect, the aggregate consideration receivable by all holders of Common Stock and Preferred Stock of the Company shall be allocated among the holders of Common Stock and Preferred Stock of the Company on the basis of the relative liquidation preferences to which the holders of each respective series of Preferred Stock of the Company and the holders of Common Stock are entitled in a Deemed Liquidation Event (assuming for this purpose that such Change in Control or Stock Sale is a Deemed Liquidation Event (as defined in the Certificate of Incorporation)) in accordance with the Certificate of Incorporation in effect immediately prior to such Change in Control or Stock Sale; provided, however, that, notwithstanding the foregoing, if the consideration to be paid in exchange for the Subject Shares pursuant to such Change in Control or Stock Sale includes any securities and due receipt thereof by such Holder would require, under applicable law, (x) the registration or qualification of such securities or of any Person as a broker, dealer or agent with respect to such securities or (v) the provision to such Holder of any information other than such information as a prudent issuer would generally furnish in an offering made solely to "accredited investors" as defined in Regulation D promulgated under the Securities Act, then the Company may cause to be paid to such Holder, in lieu of such securities, against surrender of the Subject Shares which would have otherwise been sold by such Holder, an amount in cash equal to the fair market value (as determined in good faith by the Company) of the securities that such Holder would otherwise receive as of the date of issuance of such securities in exchange for such Holder's Subject Shares; and

(vi) subject to clause (v) above requiring the same form of consideration to be available to the holders of any single class or series of capital stock, if any holders of any capital stock of the Company are given an option as to the form and amount of consideration to be received as a result of such Change in Control or Stock Sale, all holders of such capital stock shall be given the same option; provided, however, that nothing in this Section II.10(a)(vi) shall entitle any holder to receive any form of consideration that such holder would be ineligible to receive as a result of such holder's failure to satisfy any condition, requirement or limitation that is generally applicable to the Company's stockholders.

The provisions of this Section II.10 shall not be deemed to require any Holder to approve any amendment or waiver of any provision of the Certificate of Incorporation or otherwise approve a Change in Control or Stock Sale that would not allocate the consideration in accordance with the Certificate of Incorporation.

- "Participation Notice" means a written notice from the Company to such Holder, which notice includes: (x) a summary of the material terms of the proposed Change in Control or Stock Sale; (y) information relating to any stockholder meeting or action by written consent and instructions with respect to the voting of such Holder's Subject Shares as required by this Section II.10(a); and (z) such other information as the Board shall determine is necessary or appropriate.
- (b) Each Holder further agrees, with respect to any Change in Control or Stock Sale approved in accordance with Section II.10(a), to (i) refrain from exercising any dissenters' rights or rights of appraisal under applicable law (if any), (ii) execute and deliver all related documentation and take such other actions as may be reasonably requested, in writing, by the Company or the acquiring Person in support of such Change in Control or Stock Sale, including executing and delivering instruments of conveyance and transfer, any purchase agreement, merger agreement, indemnity agreement, escrow agreement, consent, waiver, governmental filing and share certificates duly endorsed for transfer (free and clear of impermissible liens, claims and encumbrances), and any similar or related documents, and (iii) refrain from entering into any agreement or understanding (including any proxy or voting trust) that would be inconsistent with, or violate, the provisions of this Section II.10, unless specifically requested to do so, in writing, by the acquiring Person in connection with such Change in Control or Stock Sale.
- (c) In the event that a stockholder representative (the "Stockholder Representative") is appointed with respect to matters affecting the Company's stockholders under the applicable definitive transaction agreements relating to any Change in Control or Stock Sale approved in accordance with Section II.10(a), each Holder agrees (i) to consent to (x) the appointment of such Stockholder Representative, (y) the establishment of any applicable escrow, expense or similar fund in connection with any indemnification or similar obligations, and (z) the payment of such Holder's pro rata portion (from the applicable escrow or expense fund or otherwise) of any and all of such Stockholder Representative's reasonable fees and expenses arising out of such Stockholder Representative's services and duties as the representative of the

Company's stockholders in connection with such Change in Control or Stock Sale, and (ii) not to assert any claim, or commence any suit, against the Stockholder Representative or any other stockholder of the Company with respect to any action or inaction by the Stockholder Representative in connection with his or her service as the Stockholder Representative, absent fraud, gross negligence or willful misconduct.

(d) Participant hereby constitutes and appoints as his or her proxies, and hereby grants a power of attorney to, the Company's President and Chief Executive Officer, and each of them, with full power of substitution, to represent and to vote or act by written consent with respect to all securities of the Company that Participant beneficially holds, either as of the date of this Agreement or at any time thereafter (collectively, the "Proxy Shares"), in accordance with this Section II.10, if and only if Participant or any transferee of any Proxy Shares (i) fails to vote all of the Proxy Shares in accordance with this Section II.10, (ii) attempts to vote any of the Proxy Shares (whether in person, by proxy or by written consent) in a manner that is inconsistent with this Section II.10, or (iii) fails to take any action necessary to effect the provisions of this Section II.10. This proxy and power of attorney is given to secure the performance of each Holder's duties under this Section II.10, and each Holder shall take such further action and execute such other instruments as may be necessary to effectuate the intent of this proxy. This proxy and power of attorney is coupled with an interest, shall be irrevocable and shall be valid for so long as any of the Proxy Shares are outstanding until the closing date of the first sale of the Common Stock to the general public pursuant to a registration statement filed with and declared effective by the U.S. Securities and Exchange Commission under the Securities Act (the "Company IPO"). The authority vested in this proxy shall run with the Proxy Shares regardless of any change in legal ownership thereof. The power of attorney granted by Participant herein is a durable power of attorney and shall survive Participant's bankruptcy, death or incapacity. Each party hereto hereby revokes any and all previous proxies and powers of attorney with respect to the Proxy Shares. Each party hereto and each Holder shall not hereafter or after consummation of the Transfer of Exercised Shares (as defined in the Option Exercise Notice) to such Holder in accordance with Section 5 of the Option Exercise Notice, as applicable, until the Company IPO, (x) purport to grant any other proxy or power of attorney with respect to any of the Proxy Shares, (y) deposit any of the Proxy Shares into a voting trust, or (z) enter into any agreement, arrangement or understanding with any Person (other than the Company), directly or indirectly, to vote, grant any proxy or give instructions with respect to the voting of any of the Proxy Shares, in each case, with respect to any of the matters set forth in this Section II.10

### 11. General Provisions.

(a) <u>Power and Authority</u>. Participant hereby represents to the Company that (i) Participant has full power and authority and legal capacity to enter into, execute and deliver this Agreement and to perform fully Participant's obligations hereunder (including the proxy appointment described in Section II.10), (ii) the execution, delivery and performance of this Agreement by Participant does not conflict with, constitute a breach of or violate any arrangement, understanding or agreement to which Participant is a party or by which Participant is bound, and (iii) this Agreement has been duly and validly executed and delivered by Participant and constitutes the legal, valid and binding obligation of Participant, enforceable against Participant in accordance with its terms.

- (b) <u>Survival</u>. The representations, warranties, covenants and agreements made in or pursuant to this Agreement shall survive the execution and delivery hereof and shall not be affected by any investigation made by or on behalf of any party hereto.
- (c) <u>Governing Law</u>. This Agreement shall be governed by and construed in accordance with the laws of the State of California without regard to conflict-of-law principles; <u>provided</u>, <u>however</u>, that Section II.10 shall be governed by the laws of the State of Delaware without regard to conflict-of-law principles.
- (d) Entire Agreement. This Agreement, together with the attached Exhibits, sets forth the entire agreement and understanding between the parties hereto relating to the subject matter hereof and supersedes all prior and contemporaneous understandings, agreements, discussions, representations and warranties, both written and oral, between the parties hereto, including any representations made during any interviews or relocation negotiations, with respect to such subject matter. In the event of a conflict between the terms and conditions of the Plan and this Agreement, the terms and conditions of the Plan shall prevail.
- (e) Notices. All notices or other communications required or permitted hereunder shall be in writing and shall be deemed given or delivered (i) when delivered personally, (ii) one business day after being deposited with an overnight courier service (costs prepaid), (iii) when sent by facsimile or e-mail if sent during normal business hours and on the next business day if sent after normal business hours, in each case with confirmation of transmission by the transmitting equipment, or (iv) when received or rejected by the addressee, if sent by certified mail, return receipt requested, postage prepaid, in each case to the addresses, facsimile numbers or e-mail addresses and marked to the attention of the persons designated (by name or title) on the signature page hereto, as applicable, or to such other address, facsimile number, e-mail address or person as such party may designate by a notice delivered to the other party hereto.
- (f) <u>Successors and Assigns; Transfers</u>. The Company may assign this Agreement, and its rights and obligations hereunder, in whole or in part, to any successor or assign (whether direct or indirect, by purchase, merger, consolidation, sale of assets or stock or otherwise). Except as set forth herein, (x) neither this Agreement nor any rights, duties and obligations hereunder shall be assigned, transferred, delegated or sublicensed by Participant without the Company's prior written consent and (y) any attempt by Participant to assign, transfer, delegate or sublicense this Agreement or any rights, duties or obligations hereunder, without the Company's prior written consent, shall be void. Subject to any restrictions on transfer set forth herein, this Agreement shall be binding upon, and enforceable against, (i) the Company and its successors and assigns and (ii) Participant and his or her heirs, executors, successors, assigns, administrators and other legal representatives. Except as set forth herein, any transfer in violation of any restriction upon transfer contained in any provision hereof shall be void, unless such restriction is waived in accordance with the terms hereof.
- (g) <u>Modification and Waiver</u>. This Agreement may not be amended, modified or supplemented except by a written instrument signed by an authorized representative of each party hereto. Any term or provision hereof may be waived, or the time for its performance may be extended, by the party or parties entitled to the benefit thereof. Any such waiver or extension shall be validly and sufficiently authorized for the purposes hereof if, as to any party, it

is authorized in writing by an authorized representative of such party. The failure or delay of any party to enforce at any time any provision hereof shall not be construed to be a waiver of such provision, nor in any way to affect the validity of this Agreement or any part hereof or the right of any party thereafter to enforce each and every such provision. No waiver of any breach hereof shall be held to constitute a waiver of any other or subsequent breach.

- (h) <u>Further Assurances</u>. Participant shall execute and deliver such additional documents, instruments, conveyances and assurances and take such further actions as may reasonably be necessary or desirable in the view of the Company to carry out the purposes or intent hereof, including the applicable Exhibits attached hereto.
- (i) <u>Severability</u>. Should any provision contained herein be held as invalid, illegal or unenforceable, such holding shall not affect the validity of the remainder of this Agreement, the balance of which shall continue to be binding upon the parties with any such modification to become a part hereof and treated as though originally set forth herein.
- (j) Interpretation. For purposes of this Agreement, (i) the words "include," "includes" and "including" shall be deemed to be followed by the words "without limitation," (ii) the word "or" is not exclusive, (iii) the words "herein," "hereof," "hereby," "hereto," "hereunder" and words of similar import refer to this Agreement as a whole, and (iv) with respect to the determination of any period of time, "from" means "from and including" and "to" means "to but excluding." Unless the context otherwise requires, references herein: (A) to a Section or an Exhibit mean a Section or an Exhibit of, or attached to, this Agreement; (B) to agreements, instruments and other documents shall be deemed to include all subsequent amendments, supplements and other modifications thereto; (C) to statutes or regulations are to be construed as including all statutory and regulatory provisions consolidating, amending or replacing the statute or regulation referred to; (D) to any Person includes such Person's successors and assigns, but, if applicable, only if such successors and assigns are not prohibited by this Agreement; and (E) to any gender includes each other gender. The Exhibits attached hereto shall be construed with, and as an integral part of, this Agreement to the same extent as if they were set forth verbatim herein. The titles, captions and headings herein are for convenience of reference only and shall not affect the meaning or interpretation hereof. This Agreement shall be construed without regard to any presumption or rule requiring construction or interpretation against the party drafting an instrument or causing any instrument to be drafted.
- (k) <u>Counterparts</u>. This Agreement may be executed in counterparts, each of which shall be considered an original, but all of which, when taken together, shall be considered one and the same agreement, and shall become binding when one or more counterparts have been signed by each party hereto and delivered to the other party hereto. Delivery of an executed counterpart of a signature page to this Agreement shall be as effective as delivery of a manually executed counterpart of this Agreement. The exchange of copies of this Agreement and of signature pages hereto by facsimile transmission or e-mail shall constitute effective execution and delivery of this Agreement and may be used in lieu of the original Agreement for all purposes. Signatures transmitted by facsimile or e-mail shall be deemed to be original signatures for all purposes.

- (l) <u>Service Relationship At Will</u>. Participant acknowledges and agrees that the vesting of this Option pursuant hereto is earned only by his or her continuing service as a Service Provider at will (and not through the act of being hired, being granted this Option or acquiring Shares hereunder). Participant further acknowledges and agrees that this Agreement, the transactions contemplated hereby and the vesting schedule set forth herein do not constitute an express or implied promise of continued engagement as a Service Provider for the vesting period, or for any period at all, and shall not interfere with the right of either the Company or Participant to terminate Participant's relationship as a Service Provider at any time, with or without cause or notice.
- (m) Third Party Beneficiary Rights. No provisions hereof are intended, nor shall be interpreted, to provide or create any third party beneficiary rights or any other rights of any kind in any client, customer, affiliate, stockholder, partner or employee of any party hereto or any other Person, unless specifically provided otherwise herein; provided, however, that Section II.4 is intended to benefit the underwriters for any registered offering described in Section II.4, and such underwriters shall have the right, power and authority to enforce the provisions of Section II.4 as though they were parties hereto.
- (n) <u>Adjustments</u>. In the event of any dividend or other distribution (whether in the form of cash, Shares, other securities or other property), recapitalization, reincorporation, stock split, reverse stock split, reorganization, merger, consolidation, split-up, spin-off, combination, reclassification, repurchase or exchange of Shares or other securities of the Company, or other change in the corporate structure of the Company affecting the Shares, the Administrator will appropriately adjust the number, class and price of Shares subject to this Option, with such adjustment to be made in accordance with Section 25102(o) of the California Corporations Code (to the extent the Company is relying upon the exemption afforded thereby with respect thereto) and Section 409A of the Code.
- (o) No Impact on Other Benefits. The value of this Option is not part of Participant's normal or expected compensation for purposes of calculating any severance, retirement, welfare, insurance or similar employee benefit.
- (p) Acceptance. Participant acknowledges receipt of a copy of the Plan and represents that he or she is familiar with the terms and provisions thereof and hereby accepts this Option subject to all of the terms and provisions of the Plan and this Agreement (including all Exhibits attached hereto). Participant has reviewed, and fully understands all provisions of, the Plan and this Agreement in their entirety (including all Exhibits attached hereto) and has had an opportunity to obtain the advice of his or her own legal counsel, tax advisors and other advisors prior to executing this Agreement. Any questions or disputes regarding the interpretation of the Plan or this Agreement (including all Exhibits attached hereto), or arising hereunder or thereunder, shall be submitted by the Company or Participant to the Administrator, and Participant hereby agrees to accept as final, binding and conclusive all decisions, determinations and interpretations of the Administrator upon any such questions or disputes.

(q) Equitable Relief. In the event of a breach or threatened breach by Participant of any provision hereof, Participant hereby consents and agrees that the Company may seek, in addition to other available remedies, injunctive or other equitable relief from any court of competent jurisdiction, without the necessity of showing any actual damages or that money damages would not afford an adequate remedy, and without the necessity of posting any bond or other security. Participant understands that any breach or threatened breach of this Agreement will cause irreparable injury and that money damages will not provide an adequate remedy therefor, and Participant hereby consents to the issuance of an injunction or other equitable relief. The aforementioned equitable relief shall be in addition to, and not in lieu of, legal remedies, monetary damages or other available forms of relief.

(signature page follows)

IN WITNESS WHEREOF, the undersigned have executed this Stock Option Agreement as of the date first above written.
COMPANY
4D MOLECULAR THERAPEUTICS, INC.

Title: Chief Executive Officer		
Notice Address:	5980 Horton Street Suite 460 Emeryville, CA 94608	
Facsimile:		
E-mail:		
Attention:	Chief Executive Officer	
PARTICIPANT		
«Participant»		
Notice Address:		
Facsimile: E-mail: Attention:		
Exhibits:		

By:

Name: David Kirn

A – 2015 Equity Incentive Plan B – Option Exercise Notice C – Investment Representation Statement

[Signature Page to Stock Option Agreement]

## **EXHIBIT A**

# 4D MOLECULAR THERAPEUTICS, INC.

# 2015 EQUITY INCENTIVE PLAN

### **EXHIBIT B**

### **OPTION EXERCISE NOTICE**

4D Molecular Therapeutics, Inc. 5980 Horton Street, Suite 460 Emeryville, CA 94608 Attention: Secretary

1. Exercise of Option. Effective as of today,	, the undersigned ("Participant") hereby elects to exercise Participant's option (the
"Option") to purchase shares (the "Exercised Sa	hares") of the common stock of 4D Molecular Therapeutics, Inc., a Delaware corporation
(the "Company"), under and pursuant to the Company's 2015	5 Equity Incentive Plan (the " <i>Plan</i> ") and that certain Stock Option Agreement made and
entered into as of «Date_of_Grant» by and between the Com	pany and Participant (the "Option Agreement").

- 2. <u>Delivery of Payment</u>. Participant herewith delivers to the Company the full exercise price of the Exercised Shares, as set forth in the Option Agreement, and any and all withholding taxes due in connection with the exercise of the Option.
- 3. **Representations of Participant**. Participant acknowledges that Participant has received, read and understood the Plan and the Option Agreement and agrees to abide, and be bound, by their terms and conditions.
- 4. <u>Rights as Stockholder</u>. Until the issuance of the Exercised Shares (as evidenced by the appropriate entry on the books of the Company or of a duly authorized transfer agent of the Company), no right to vote or receive dividends or other distributions or any other rights as a stockholder shall exist with respect to the Exercised Shares, notwithstanding the exercise of the Option. The Exercised Shares shall be issued to Participant as soon as practicable after the Option is exercised in accordance with the Option Agreement. No adjustment shall be made for a dividend or distribution or other right for which the record date is prior to the date of issuance, except as provided in Section 13 of the Plan.
- 5. <u>Company's Right of First Refusal</u>. Before any Exercised Shares (or any beneficial interest therein) may be Transferred by Participant or any subsequent transferee (each, a "*Holder*"), such Holder must first offer such Exercised Shares to the Company and/or its assignee(s) as follows:
- (a) <u>Notice of Proposed Transfer</u>. The Holder shall deliver to the Company a written notice pursuant to Section II.11(e) of the Option Agreement (a "*Transfer Notice*") stating: (i) the Holder's bona fide intention to Transfer the Exercised Shares; (ii) the name and address of the proposed transferee; (iii) the number of Exercised Shares to be Transferred to the proposed transferee; (iv) the bona fide cash price or other consideration for which the Holder proposes to Transfer the Exercised Shares; and (v) that, by delivering the Transfer Notice, the Holder offers all such Exercised Shares to the Company and/or its assignee(s) pursuant to this Section 5 and on the same terms described in the Transfer Notice.

- (b) <u>Exercise of Right of First Refusal</u>. At any time within 30 days after receipt of a Transfer Notice, the Company and/or its assignee(s) may, by giving written notice pursuant to Section II.11(e) of the Option Agreement (the "*Exercise Notice*") to the Holder, elect to purchase any or all of the Exercised Shares proposed to be Transferred to the proposed transferree, at the purchase price determined in accordance with Section 5(c).
- (c) <u>Purchase Price</u>. The per share purchase price for any Exercised Shares purchased by the Company and/or its assignee(s) under this Section 5 (the "*Company Shares*") shall be the per share price listed in the applicable Transfer Notice. If the price listed in such Transfer Notice includes consideration other than cash, the cash equivalent value of the non-cash consideration shall be determined by the Board in its sole discretion.
- (d) <u>Payment</u>. Payment of the purchase price for any Company Shares shall be made, at the option of the Company and/or its assignee(s), in cash (by check), by cancellation of all or a portion of any outstanding indebtedness of the Holder to the Company and/or its assignee(s), or by any combination thereof, in any such case within ten days after delivery to the Holder of the applicable Exercise Notice and, at such time, if the Company Shares are certificated shares, the Holder shall deliver to the Company and/or its assignee(s) the certificate(s) representing the Company Shares, each certificate to be properly endorsed for transfer.
- (e) <u>Holder's Right to Transfer</u>. If the Company and/or its assignee(s) elects to purchase less than all of the Exercised Shares proposed in a Transfer Notice to be Transferred to a given proposed transferee, then the Holder (x) may Transfer to the proposed transferee any of such Exercised Shares that the Company and/or its assignee(s) elected not to purchase (the "Non-Company Shares"); provided, however, that: (i) the Transfer is made only on the terms provided for in the applicable Transfer Notice, with the exception of the purchase price, which may be either the price listed in such Transfer Notice or any higher price; (ii) the Transfer is consummated within 60 days after the date the applicable Transfer Notice was delivered to the Company; (iii) the Transfer is effected in accordance with any applicable securities laws and, if requested (in writing) by the Company, the Holder shall have delivered a written opinion of counsel acceptable to the Company to that effect; and (iv) the proposed transferee agrees in writing to receive and hold the Non-Company Shares so Transferred subject to all of the provisions hereof (including this Section 5) and of the Option Agreement (including Section II.4 and Section II.10 thereof), and there shall be no further Transfer of any Exercised Shares except in accordance with this Section 5, and (y) shall, in accordance with the other provisions of this Section 5, sell to the Company and/or its assignee(s) the portion of such Exercised Shares that the Company and/or its assignee(s) has elected to purchase. If any Non-Company Shares are not Transferred to the proposed transferee within the period provided in clause (ii) above, then, before any such shares may be Transferred, a new Transfer Notice shall be given to the Company, and the Company and/or its assignee(s) shall again be offered the right of first refusal described in this Section 5.
- (f) Exception for Certain Family Transfers. Notwithstanding anything to the contrary contained elsewhere in this Section 5, the Transfer of any or all of the Exercised Shares during the Holder's lifetime, or on the Holder's death by will or intestacy, to the Holder's spouse (or former spouse) or domestic partner, child or stepchild, grandchild, parent, stepparent, sibling, father-in-law, mother-in-law, son-in-law, daughter-in-law, brother-in-law, sister-in-law, grandparent, niece or nephew, including adoptive relationships (each such person, an "Immediate"

Family Member"), or to a trust or other similar estate planning vehicle for the benefit of the Holder or any such Immediate Family Member, shall be exempt from the provisions of this Section 5; provided, however, that, in each such case, the transferee agrees in writing to receive and hold the Exercised Shares so Transferred subject to all of the provisions hereof (including this Section 5) and of the Option Agreement (including Section II.4 and Section II.10 thereof), and there shall be no further Transfer of such Exercised Shares except in accordance with this Section 5; provided, further, that, without the Company's prior written consent, which may be withheld in its sole discretion, no more than three Transfers may be made pursuant to this Section 5(f), including all Transfers by the Holder and all Transfers by any transferee. For purposes hereof, a person shall be deemed to be a "domestic partner" of another person if the two persons (i) reside in the same residence and plan to do so indefinitely, (ii) have resided together for at least one year, (iii) are each at least 18 years of age and mentally competent to consent to contract, (iv) are not blood relatives closer than would prohibit legal marriage in the state in which they reside, (v) are financially interdependent, as demonstrated to the Company's reasonable satisfaction, and (vi) have each been the sole spousal equivalent of the other for the year prior to the Transfer and plan to remain so indefinitely; provided, however, that a person shall not be deemed to be a "domestic partner" if he or she is married to another person or has any other spousal equivalent.

- (g) <u>Termination of Right of First Refusal</u>. The right of first refusal contained in this Section 5 shall terminate as to all Exercised Shares upon the earlier of: (i) the Company IPO; and (ii) the closing date of a Change in Control or Stock Sale pursuant to which the holders of the outstanding voting securities of the Company receive securities of a class registered pursuant to Section 12 of the Exchange Act.
- 6. <u>Tax Consultation</u>. Participant understands that Participant may suffer adverse tax consequences as a result of Participant's purchase or disposition of the Exercised Shares. Participant represents that Participant has consulted with any tax consultants Participant deems advisable in connection with the purchase or disposition of the Exercised Shares and that Participant is not relying on the Company for any tax advice.

### 7. Restrictive Legends and Stop-Transfer Orders.

(a) <u>Legends</u>. Participant understands and agrees that the Company shall cause the legends set forth below, or substantially equivalent legends, to be placed upon any certificate(s) evidencing ownership of the Exercised Shares, together with any other legends that may be required by the Company or by applicable federal or state securities laws:

THE SECURITIES REPRESENTED BY THIS CERTIFICATE HAVE BEEN ACQUIRED FOR INVESTMENT AND NOT WITH A VIEW TO, OR IN CONNECTION WITH, THE SALE OR DISTRIBUTION THEREOF AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED (THE "ACT"), OR ANY STATE SECURITIES LAWS. THEY MAY NOT BE OFFERED, SOLD OR OTHERWISE TRANSFERRED, PLEDGED OR HYPOTHECATED EXCEPT AS PERMITTED UNDER THE ACT AND APPLICABLE STATE SECURITIES LAWS PURSUANT TO AN EFFECTIVE REGISTRATION STATEMENT OR AN EXEMPTION THEREFROM. THE ISSUER OF THESE SECURITIES MAY REQUIRE AN OPINION OF COUNSEL SATISFACTORY TO THE ISSUER THAT SUCH OFFER, SALE, TRANSFER, PLEDGE OR HYPOTHECATION OTHERWISE COMPLIES WITH THE ACT AND ANY APPLICABLE STATE SECURITIES LAWS.

THE SECURITIES REPRESENTED BY THIS CERTIFICATE ARE SUBJECT TO CERTAIN RESTRICTIONS ON TRANSFER, A RIGHT OF FIRST REFUSAL, AN AGREEMENT TO PARTICIPATE AND A LOCK-UP PERIOD IN THE EVENT OF A PUBLIC OFFERING AS SET FORTH IN AGREEMENTS BETWEEN THE ISSUER AND THE ORIGINAL HOLDER OF THESE SECURITIES, COPIES OF WHICH MAY BE OBTAINED AT THE PRINCIPAL OFFICE OF THE ISSUER. SUCH RESTRICTIONS ON TRANSFER, RIGHT OF FIRST REFUSAL, AGREEMENT TO PARTICIPATE AND LOCK-UP PERIOD ARE BINDING ON TRANSFERES OF THESE SECURITIES.

- (b) <u>Stop-Transfer Notices</u>. In order to ensure compliance with the restrictions referred to herein and in the Option Agreement, including the provisions of Section II.4 of the Option Agreement, the Company may issue appropriate stop-transfer instructions to its transfer agent, if any, and, if the Company transfers its own securities, it may make appropriate notations to the same effect in its own records.
- (c) **Refusal to Transfer**. The Company shall not be required to transfer on its books any Exercised Shares that have been Transferred in violation of any provision hereof or to treat as owner of such Exercised Shares, or otherwise to accord voting or dividend rights to, any purchaser or other transferee to whom such Exercised Shares shall have been so Transferred. Any attempt to Transfer Exercised Shares in violation hereof shall be null and void and shall be disregarded by the Company.
- 8. <u>Capitalized Terms</u>. Unless otherwise defined herein, capitalized terms used herein shall have the same defined meanings as set forth in the Plan or, if not defined therein, in the Option Agreement.
- 9. <u>Governing Law; Severability</u>. This Option Exercise Notice shall be governed by and construed in accordance with the laws of the State of California without regard to conflict-of-law principles. Should any provision contained herein be held as invalid, illegal or unenforceable, such holding shall not affect the validity of the remainder of this Option Exercise Notice, the balance of which shall continue to be binding upon the parties with any such modification to become a part hereof and treated as though originally set forth herein.
- 10. Consent to Notices by Electronic Transmission. Upon becoming a stockholder of the Company and without limiting the manner by which notice otherwise may be given effectively to Participant, Participant hereby consents in accordance with Section 232 of the Delaware General Corporation Law to stockholder notices given by the Company to Participant by any of the following forms of electronic transmission: (i) by facsimile telecommunications to the facsimile number set forth on the signature page hereto or to such other facsimile number as Participant may designate by a written notice delivered to the Company; (ii) by electronic mail to the e-mail address set forth on the signature page hereto or to such other e-mail address as Participant may designate by a written notice delivered to the Company; (iii) by a posting on an electronic network together with separate notice to Participant of such specific posting; and (iv) by any other form of electronic transmission when directed to Participant.

Submitted by:	Accepted by:
	COMPANY
PARTICIPANT	4D MOLECULAR THERAPEUTICS, INC.
Signature	By:
	Name:
«Participant»	Title:
Print Name	
	Date Received:
	5

#### **EXHIBIT C**

### INVESTMENT REPRESENTATION STATEMENT

PARTICIPANT : «Participant» ("Participant")

COMPANY : 4D Molecular Therapeutics, Inc. (the "Company")

SECURITY : COMMON STOCK

AMOUNT : \_\_\_\_\_ shares (the "Shares")

DATE :

Unless otherwise defined in this Investment Representation Statement (this "*Statement*"), capitalized terms used herein shall have the same defined meanings as set forth in the 4D Molecular Therapeutics, Inc. 2015 Equity Incentive Plan or, if not defined therein, in that certain Stock Option Agreement made and entered into as of "Date\_of\_Stock\_Option\_Agreement» by and between the Company and Participant.

In connection with Participant's purchase of the Shares, Participant hereby makes the following representations to the Company:

- 1. *Investment Intent*. Participant is purchasing the Shares solely for investment for his or her own account, not as a nominee or agent, and not with a view to the resale or distribution (within the meaning of the Securities Act) of any part thereof, except to the extent Participant intends to hold the Shares jointly with his or her spouse. Participant has no present intention of selling, transferring, granting any participation in, or otherwise distributing any Shares. Participant does not have, with respect to any of the Shares, any contract or other arrangement with any Person to sell, transfer, grant participations or otherwise distribute the same to such Person or to any third Person. Participant's investment intent is not limited to Participant's present intention to hold the Shares for the minimum capital gains period specified under any applicable tax law, for a deferred sale, for a specified increase or decrease in the market price of the Shares or for any other fixed period in the future.
- 2. Participant is Informed About the Company. Participant is sufficiently aware of the Company's business affairs and financial condition, and has acquired sufficient information he or she considers necessary or appropriate, to make an informed and knowledgeable investment decision with respect to the Shares.
- 3. Participant Can Protect Participant's Own Interests. Participant can properly evaluate the merits and risks of an investment in the Shares and can protect his or her own interests in this regard, whether by reason of Participant's own business and financial expertise, the business and financial expertise of certain professional advisors unaffiliated with the Company (and not compensated by the Company, directly or indirectly) with whom Participant has consulted, or Participant's preexisting business or personal relationship with the Company or any of its directors, officers or controlling persons. Participant acknowledges that the purchase of the Shares involves an extremely high degree of risk and that the Company's future prospects are uncertain. Participant is able, without materially impairing his or her financial condition, to hold the Shares for an indefinite period of time and to suffer a complete loss of his or her entire investment. Participant's purchase of the Shares was not accomplished by a general solicitation or general advertising.

- 4. Participant Knows the Shares are Restricted Securities. Participant understands that the Shares are "restricted securities" under applicable federal and state securities laws inasmuch as they have not been (i) registered under the Securities Act in reliance upon a specific exemption therefrom, which exemption depends upon, among other things, the bona fide nature of Participant's investment intent as expressed in this Statement or (ii) registered or qualified in any state in which they are offered. In this regard, Participant further understands and agrees that: (i) Participant must hold the Shares indefinitely unless they are registered with the U.S. Securities and Exchange Commission and qualified by state authorities or an exemption from such registration and qualification requirements is available; (ii) the Company has no obligation to register or qualify Shares for resale; and (iii) the certificate(s) evidencing the Shares, if certificated, will be imprinted with a legend which prohibits the transfer of the Shares unless the Shares are registered under the Securities Act and applicable state securities laws or such registration is not required in the written opinion of counsel for the Company.
- 5. Participant is Familiar With Rule 701 and Rule 144. Participant is familiar with Rule 701 and Rule 144, each promulgated under the Securities Act ("Rule 701" and "Rule 144", respectively), which in some circumstances permit limited public resales of "restricted securities" like the Shares acquired from an issuer in a non-public offering. Rule 701 provides that if the issuer of the Shares qualifies under Rule 701 at the time of grant of the option to purchase the Shares (the "Option"), the exercise of the Option will be exempt from registration under the Securities Act. If the Company becomes subject to the reporting requirements of Section 13 or 15(d) of the Exchange Act, 90 days thereafter (or such longer period as any market stand-off agreement may require) the Shares exempt under Rule 701 may be resold, subject to the satisfaction of the applicable conditions specified by Rule 144, including, in the case of affiliates, (i) the availability of certain current public information about the Company, (ii) the amount of Shares being sold during any three-month period not exceeding specified limitations, (iii) the sale being made in an unsolicited "broker's transaction," transactions directly with a "market maker" or "riskless principal transactions," as those terms are defined under the Exchange Act, and (iv) the timely filing of a notice of proposed sale on Form 144, if applicable. If the Company does not qualify under Rule 701 at the time of grant of the Option, then the Shares may be resold in certain limited circumstances subject to the provisions of Rule 144, which may require, among other things, (i) the availability of certain current public information about the Company, (ii) the resale occurring more than a specified period after the purchase and full payment (within the meaning of Rule 144) for the Shares, and (iii) in the case of the sale of Shares by an affiliate, the satisfaction of the conditions set forth in clauses (ii), (iii) and (iv) of the preceding sentence. Participant understands that (x) current public information about the Company is not now available, and the Company has no present plans to make such information available, (y) the requirements of Rule 144 may never be met and the Shares may never be saleable under Rule 144, and (z) at the time Participant wishes to sell the Shares, there may be no public market for the Company's stock upon which to make such a sale or the current public information requirements of Rule 144 may not be satisfied, any of which may preclude Participant from selling the Shares under Rule 144 even if the relevant holding period has been satisfied.
- 6. Participant Knows that Participant is Subject to Further Restrictions on Resale. Participant understands that, if Rule 701 or Rule 144 is not available, any future proposed disposition of any of the Shares will not be possible without prior registration under the Securities Act or compliance with some other registration exemption (which may or may not be available).

Participant understands that, although Rule 701 and Rule 144 are not exclusive, the Staff of the U.S. Securities and Exchange Commission has stated that Persons proposing to sell private placement securities other than in a registered offering or pursuant to Rule 701 or Rule 144 will have a substantial burden of proof in establishing that an exemption from registration is available for such offers or sales and that such Persons and their respective brokers who participate in such transactions do so at their own risk.

7. *Address*. The address of Participant's principal residence is set forth on the signature page of this Statement. Participant shall promptly inform the Company, in writing, of any change in Participant's principal residence.

By signing below, Participant acknowledges Participant's agreement with each of the statements contained in this Statement as of the date first set forth above and Participant's intent for the Company to rely on such statements in issuing the Shares to Participant.

Participant's Address:	
	Participant's Signature
	«Participant»
	Print Name

### CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We hereby consent to the use in this Registration Statement on Form S-1 of 4D Molecular Therapeutics, Inc. of our report dated August 2, 2019 relating to the financial statements of 4D Molecular Therapeutics, Inc., which appears in this Registration Statement. We also consent to the reference to us under the heading "Experts" in such Registration Statement.

/s/ PricewaterhouseCoopers LLP

San Jose, California September 30, 2019