4D Molecular Therapeutics Reports Financial Results for the Year Ended December 31, 2020 and Provides Operational Highlights

March 25, 2021

- First patient dosed in the 4D-310 Phase 1/2 clinical trial in Fabry disease

- Intravitreal product candidates, 4D-125 for the treatment of XLRP and 4D-110 for the treatment of choroideremia, completed dose escalation portion of Phase 1/2 clinical trials (n=12 patients)

- Intravitreal product candidate 4D-150 for the treatment of wet AMD and DME on track to initiate clinical trial in the second half of 2021

- Cash and cash equivalents of approximately $277M as of Dec 31, 2020

EMERYVILLE, Calif., March 25, 2021 (GLOBE NEWSWIRE) -- 4D Molecular Therapeutics (Nasdaq: FDMT), a clinical-stage gene therapy company harnessing the power of directed evolution for targeted gene therapies, announced financial results for the year ended December 31, 2020 and provided operational highlights.

“2020 was a transformational year for 4D Molecular Therapeutics,” said David Kirn, M.D., Co-founder and Chief Executive Officer of 4DMT. “We transitioned into a clinical-stage company, with three product candidates currently in clinical development: 4D-125 for X-linked retinitis pigmentosa, 4D-110 for choroideremia, and 4D-310 for Fabry disease. In addition, we strengthened our leadership team and corporate governance, with the addition of key clinical development executives and four experienced board members, including our Executive Chairman John Milligan, Ph.D. With the proceeds from our IPO, we raised the capital necessary to expand our vision for developing transformative next-generation gene therapies in multiple therapeutic areas for both rare and large market diseases.”

Recent Operational Highlights

- **Dosed the first patient in the Phase 1/2 clinical trial of 4D-310** for the treatment of Fabry disease in March 2021. The Phase 1/2 open-label, dose-escalation and dose-expansion clinical trial is expected to enroll up to 18 Fabry disease patients. The primary endpoints of this trial are to evaluate safety and to define the maximum-tolerated/-feasible dose.
- **Completed the dose escalation portion of the Phase 1/2 clinical trial of 4D-125**, an intravitreal R100 vector-based product candidate, in adult patients with X-linked retinitis pigmentosa (XLRP). Dose escalation was completed following the dosing of six patients in two cohorts. The dose expansion portion of the trial will enroll patients at the highest dose tested of 1E12 vg/eye. To date, 4D-125 has been well-tolerated and has not resulted in dose-limiting toxicity or serious adverse events. The primary endpoints of this trial are to evaluate safety and to define the maximum-tolerated/-feasible dose.
- **Completed the dose escalation portion of the Phase 1/2 clinical trial of 4D-110,** an intravitreal R100 vector-based product candidate, in adult patients with choroideremia. Dose escalation was completed following the dosing of six patients in two cohorts. The dose expansion portion of the trial will enroll patients at the highest dose tested of 1E12 vg/eye. To date, 4D-110 has been well-tolerated and has not resulted in dose-limiting toxicity or serious adverse events. The primary endpoints of this trial are to evaluate safety and to define the maximum-tolerated/-feasible dose.
- **Initiated IND-enabling studies for 4D-150, an intravitreal R100 vector-based product candidate, and submitted preclinical dataset for presentation at upcoming medical meeting.** 4D-150 is a wholly owned product candidate for wet age-related macular degeneration (wet AMD) and diabetic macular edema (DME) and is engineered for three distinct mechanisms-of-action.
- **Initiated 4D-710 IND-enabling studies and submitted preclinical dataset for presentation at upcoming medical meeting.** 4D-710 is a product candidate for cystic fibrosis lung disease which is designed for efficient single dose aerosol delivery in a broad range of patients.
- **Completed upsized initial public offering** in December 2020 which raised $222 million in gross proceeds, including the full exercise of the underwriters option to purchase additional shares. Together with the completion of a Series C financing earlier in the year, the company raised approximately $298 million in gross proceeds in 2020.
- **Strengthened corporate governance** with appointments to its board of directors including John Milligan Ph.D., as Executive Chairman, and Susannah Gray, Nancy Miller-Rich and Shawn Tomasello as independent members. As a result of becoming a publicly-traded company, the Series B investor representative, Tony Yao, M.D., Ph.D., notified the board of his intent to resign, effective March 20, 2021. Likewise, the Series A investor representative, William Burkoth, notified the board on March 19, 2021 of his intent to not stand for reelection at the company’s 2021 annual meeting of stockholders.
- **Strengthened leadership team** with key appointments in clinical research and development, including Chief Medical Officer & Therapeutic Area Head, Pulmonology Robert Fishman, M.D.; Senior Vice President & Therapeutic Area Head, Cardiology Raphael Schiffmann, M.D.; and Senior Vice President & Clinical Therapeutic Area Head, Ophthalmology Robert Kim, M.D., M.B.A.

Expected Upcoming Milestones

- Initial clinical data from the Phase 1/2 clinical trial of 4D-125 in XLRP expected in the second half of 2021
- Initial clinical data from the Phase 1/2 clinical trial of 4D-310 in Fabry disease expected in the second half of 2021
- Presentation of 4D-150 preclinical data expected at upcoming medical meeting and initiation of clinical trial with 4D-150 in wet AMD and diabetic macular edema expected in the second half of 2021

- Presentation of 4D-710 preclinical data expected at upcoming medical meeting and initiation of clinical trial with 4D-710 in cystic fibrosis lung disease expected in the second half of 2021

Financial Results for the Year Ended December 31, 2020

Cash and Cash Equivalents: Cash and cash equivalents was $276.7 million as of December 31, 2020, as compared to $49.7 million as of December 31, 2019. The increase in cash and cash equivalents was primarily a result of the proceeds received from the December 2020 initial public offering and the issuance of our Series C redeemable convertible preferred stock in the second quarter of 2020, which was partially offset by cash used in operations. We expect cash and cash equivalents to be sufficient to fund operations into mid-2023.

Revenue: Total revenue was $13.6 million for the year ended December 31, 2020, as compared to $7.0 million for the year ended December 31, 2019. The increase was primarily driven by the recognition of revenue under the Roche collaboration agreement entered into in 2017.

R&D Expenses: Research and development expenses were $53.0 million for the year ended December 31, 2020, as compared to $38.7 million for the year ended December 31, 2019. This increase was primarily due to higher external costs incurred to advance our clinical and preclinical programs and higher payroll and stock-based compensation expenses.

G&A Expenses: General and administrative expenses were $17.2 million for the year ended December 31, 2020, as compared to $13.9 million for the year ended December 31, 2019. This increase was primarily due to higher payroll and stock-based compensation expenses and higher professional service expenses.

Net Loss: Net loss was $56.7 million for the year ended December 31, 2020, as compared to $49.3 million for the year ended December 31, 2019.

About 4DMT

4DMT is a clinical-stage company harnessing the power of directed evolution for targeted gene therapies. 4DMT seeks to unlock the full potential of gene therapy using its platform, Therapeutic Vector Evolution, which combines the power of directed evolution with approximately one billion synthetic capsid sequences to invent evolved vectors for use in targeted gene therapy products. The company is initially focused in three therapeutic areas: ophthalmology, cardiology, and pulmonology. The 4DMT targeted and evolved vectors are invented with the goal of being delivered through clinically routine, well-tolerated and minimally invasive routes of administration, transducing diseased cells in target tissues efficiently, having reduced immunogenicity and, where relevant, having resistance to pre-existing antibodies. 4DMT is currently conducting three clinical trials: 4D-125 is in a Phase 1/2 clinical trial for XLRP patients, 4D-110 is in a Phase 1 clinical trial for choroideremia patients and 4D-310 is in a Phase 1/2 clinical trial for Fabry disease patients.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, implied and express statements regarding plans and timelines for the clinical development of 4D-310, 4D-125, 4D-110, 4D-150 and 4D-710, including the therapeutic potential and clinical benefits thereof; the estimated timing of clinical data being available for 4D-125's Phase 1/2 clinical trial and 4D-310's Phase 1/2 clinical trial; the estimated timing of initiating the clinical trials for 4D-150 and 4D-710; expectations on how long our cash and cash equivalents can fund operations; expectations regarding current and future interactions with the U.S. Food and Drug Administration (FDA); and 4D Molecular Therapeutics' strategy, business plans and focus. The words "may," "might," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "expect," "estimate," "seek," "predict," "future," "project," "potential," "continue," "target" and similar words or expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, risks associated with: the impact of COVID-19 on countries or regions in which we have operations or do business, as well as on the timing and anticipated results of our clinical trials, strategy and future operations; the delay of any current or planned clinical trials for the development of 4D Molecular Therapeutics' drug candidates, the risk that the results of our clinical trials may not be predictive of future results in connection with future clinical trials; 4D Molecular Therapeutics' ability to successfully demonstrate the safety and efficacy of its drug candidates; the timing and outcome of our planned interactions with regulatory authorities; and obtaining, maintaining and protecting our intellectual property. These and other risks and uncertainties are described in greater detail in the section entitled "Risk Factors" in 4D Molecular Therapeutics' most recent Annual Report on Form 10-K to be filed on the date hereof, as well as any subsequent filings with the Securities and Exchange Commission. In addition, any forward-looking statements represent 4D Molecular Therapeutics' views only as of today and should not be relied upon as representing its views as of any subsequent date. 4D Molecular Therapeutics explicitly disclaims any obligation to update any forward-looking statements. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements.

4D-310, 4D-125 and 4D-110 are our product candidates in clinical trials and have not yet been approved for marketing by the US FDA or any other regulatory authority. No representation is made as to the safety or effectiveness of 4D-310, 4D-125, or 4D-110 for the therapeutic use for which they are being studied.

4D Molecular Therapeutics, Inc.
Condensed Statements of Operations and Comprehensive Loss
(*in thousands, except share and per share amounts*)

	Year Ended December 31,	
	2020	**2019**
	(in thousands, except share and per share data)	
Statements of Operations and Comprehensive Loss Data:		
Revenue:		
Collaboration and license revenue	$ 13,363	$ 6,960
Collaboration and license revenue, related parties	249	26
Total revenue	13,612	6,986
Operating expenses:		

Research and development	53,038	38,718
Acquired in-process research and development	—	5,137
General and administrative	17,238	13,895
Total operating expenses	70,276	57,750
Loss from operations	(56,664)	(50,764)
Other income (expense)	(29)	1,458
Net loss and comprehensive loss	$ (56,693)	$ (49,306)
Net loss per share attributable to common stockholders, basic and diluted	$ (8.82)	$ (9.59)
Weighted-average shares outstanding used in computing net loss per share attributable to common stockholders, basic and diluted	6,430,555	5,142,560

4D Molecular Therapeutics, Inc.
Condensed Balance Sheet Data
(*in thousands*)

	As of December 31,	
	2020	**2019**
	(in thousands)	
Balance Sheet Data:		
Cash and cash equivalents	$ 276,726	$ 49,652
Working capital	265,912	39,553
Total assets	288,331	58,234
Accumulated deficit	(135,679)	(79,025)
Total stockholders' equity (deficit)	256,387	(72,970)

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