4D Molecular Therapeutics Reports Full Year 2021 Financial Results and Operational Highlights

March 28, 2022

- *Provided clinical data updates on three programs, including first clinical data on 4D-310 for Fabry disease and 4D-125 and 4D-110 for inherited retinal dystrophies, each demonstrating preliminary evidence of clinical activity*

- *Advanced two additional programs into clinical development: R100 vector-based 4D-150 for wet AMD, and A101 vector-based 4D-710 for cystic fibrosis*

- *Initiated expansion of our GMP manufacturing facilities for potential commercial-scale clinical manufacturing capabilities*

- *$315 million in cash, cash equivalents and investments at end of 2021 expected to fund operations into the second half of 2024*

EMERYVILLE, Calif., March 28, 2022 (GLOBE NEWSWIRE) -- 4D Molecular Therapeutics, Inc. (Nasdaq: FDMT), a clinical-stage biotherapeutics company harnessing the power of directed evolution for targeted genetic medicines, announced full year 2021 financial results and provided operational highlights.

“In our first full year as a publicly traded company, 4DMT continued to advance our diverse pipeline of five clinical-stage product candidates in three distinct therapeutic areas for both rare and large market diseases, all of which utilize targeted and evolved vectors from our Therapeutic Vector Evolution platform. With the release of promising initial clinical data on three programs and the IND clearances of two additional programs, we delivered on our goals and guidance for 2021,” said David Kirn, M.D., Co-founder and Chief Executive Officer of 4DMT. “We also continued to build a strong clinical and scientific team that is focused not only on our clinical programs but also on continuing to advance our Therapeutic Vector Evolution platform and preclinical product pipeline. We’ve entered 2022 with a strong cash runway into the second half of 2024. By harnessing the power of our Therapeutic Vector Evolution and GMP manufacturing platforms, 4DMT has built a robust product development engine to continue our mission to unlock the full potential of genetic medicines for countless patients.”

2021 Operational Highlights

- Provided clinical data updates on three programs, including first clinical activity data from two 4DMT targeted and evolved vectors: C102 for systemic low dose cardiovascular delivery and R100 for routine intravitreal delivery.
  - Presented Phase 1/2 clinical data on 4D-310 for Fabry disease that demonstrated mean AGA enzyme activity was within, or significantly above, the normal range in all of the first three patients including patients with pre-existing high titer antibodies to AGA. In addition, 4D-310 had a manageable safety profile, without dose-limiting toxicity.
  - Presented Phase 1/2 clinical data on 4D-125 for XLRP and 4D-110 for choroideremia that demonstrated that both intravitreal product candidates were well tolerated at planned doses and were associated with signs of clinical activity, including reduced photoreceptor loss by ellipsoid zone area (4D-125) and reduced retinal pigment epithelium loss by fundus autofluorescence (4D-110).
- Received clearance from the U.S. Food and Drug Administration (FDA) for the 4DMT Investigational New Drug (IND) applications for the Phase 1/2 clinical trials of 4D-150 for wet age-related macular degeneration (wet AMD) and of 4D-710 for cystic fibrosis lung disease. In January 2022, the company announced dosing of the first patient in the Phase 1/2 clinical trial of 4D-150.
- Initiated the build out of a commercial-scale GMP manufacturing facility which is expected to provide clinical trial material for our five product candidates across three therapeutic areas. The clinical trial material produced out of this facility will support each product candidate’s pivotal studies and provide a wide range of analytical capabilities, including potency assay development and validation. These added capabilities are designed to shorten product development timelines, reduce costs, improve quality, and ensure internal control.

2022 Expected Milestones and Objectives

- First patient dosing in the 4D-710 Phase 1/2 clinical trial for cystic fibrosis expected in the first half of 2022
- Continue enrollment and follow-up in both the United States and Asia-Pacific (APAC) Phase 1/2 clinical trials of 4D-310 in Fabry disease
- Continue enrollment and follow-up in the 4D-150 Phase 1/2 clinical trial for wet AMD
- Continue enrollment and follow-up in the Phase 1/2 clinical trials for both 4D-125 for X-linked retinitis pigmentosa and 4D-110 for choroideremia

Full Year 2021 Financial Results

Cash and Cash Equivalents and Marketable Securities: Cash and cash equivalents and marketable securities were $315.4 million as of December 31, 2021, as compared to $276.7 million as of December 31, 2020. The increase in cash was primarily a result of the receipt of net proceeds of approximately $111 million from our October 2021 public offering, which was partially offset by cash used in operations. We expect cash and cash equivalents and marketable securities to be sufficient to fund operations into the second half of 2024.

Revenue: Total revenue was $18.0 million for 2021, as compared to $13.6 million for 2020. This increase was primarily driven by the completion of revenue recognized under the Roche collaboration agreement, which was terminated in September 2021.

R&D Expenses: Research and development expenses were $61.4 million for 2021, as compared to $53.0 million for 2020. This increase was primarily driven by the progression of our existing clinical trials for 4D-310, 4D-125 and 4D-110, in addition to startup costs for our clinical trials of 4D-150 and 4D-710 along with increased payroll and stock-based compensation expense.

G&A Expenses: General and administrative expenses were $28.0 million for 2021, as compared to $17.2 million for 2020. This increase was primarily due to increased payroll, stock-based compensation, insurance, and professional service expenses.

Net Loss: Net loss was $71.3 million for 2021, as compared to $56.7 million for 2020.

About 4DMT

4DMT is a clinical-stage biotherapeutics company harnessing the power of directed evolution for targeted genetic medicines. 4DMT seeks to unlock the full potential of genetic medicines using its platform, Therapeutic Vector Evolution, which combines the power of directed evolution with approximately one billion synthetic capsid sequences to invent targeted and evolved vectors for use in our products. The company is initially focused on five clinical-stage products in three therapeutic areas for both rare and large market diseases: ophthalmology, cardiology (including Fabry disease) and pulmonology. The 4DMT targeted and evolved vectors are invented with the goal of being delivered at relatively low doses 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. The five 4DMT product candidates in clinical development are: 4D-310 for Fabry disease, 4D-150 for wet AMD, 4D-125 for XLRP, 4D-110 for choroideremia and 4D-710 for cystic fibrosis.

4D-310, 4D-150, 4D-125, 4D-110 and 4D-710 are 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-150, 4D-125, 4D-110 or 4D-710 for the therapeutic use for which they are being studied. 4D Molecular Therapeutics™, 4DMT™, Therapeutic Vector Evolution™, and the 4DMT logo are trademarks of 4DM.

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; expectations on how long 4D Molecular Therapeutics' cash, cash equivalents and investments can fund its operations; whether the expansion of 4D Molecular Therapeutics' manufacturing facilities will support commercial-scale production and expand our analytical development capabilities; the timing of the first patient dosing in 4D Molecular Therapeutics' planned 4D-710 Phase 1/2 clinical trial; the ability to continue to enroll 4D Molecular Therapeutics' ongoing clinical trials; 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' Annual Report on Form 10-K to be filed on or about 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 Molecular Therapeutics, Inc.
Statements of Operations
(*in thousands, except share and per share amounts*)

	Year Ended

	December 31,	
	2021	2020
Statements of Operations Data:		
Revenue:		
Collaboration and license revenue	$ 18,038	$ 13,363
Collaboration and license revenue, related parties	—	249
Total revenue	18,038	13,612
Operating expenses:		
Research and development	61,360	53,038
General and administrative	28,011	17,238
Total operating expenses	89,371	70,276
Loss from operations	(71,333)	(56,664)
Other income (expense)	16	(29)
Net loss	$ (71,317)	$ (56,693)
Net loss per share, basic and diluted	$ (2.57)	$ (8.82)
Weighted-average shares outstanding used in computing net loss per share, basic and diluted	27,730,420	6,430,555

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

	As of December 31,	
	2021	2020
Balance Sheet Data:		
Cash and cash equivalents and marketable securities	$ 315,429	$ 276,726
Working capital	239,942	265,912
Total assets	353,487	288,331
Total liabilities	34,380	31,944
Accumulated deficit	(206,996)	(135,679)
Total stockholders' equity	319,107	256,387

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