



## 4D Molecular Therapeutics to Present Updated Clinical Data with 4D-310 in Fabry Disease Patients in Platform Presentation at the 18th Annual WORLDSymposium

February 2, 2022

EMERYVILLE, Calif., Feb. 02, 2022 (GLOBE NEWSWIRE) -- 4D Molecular Therapeutics (Nasdaq: FDMT), a clinical-stage gene therapy company harnessing the power of directed evolution for targeted gene therapies, announced updated interim clinical data from the on-going 4D-310 Phase 1/2 trial in patients with Fabry disease will be presented at the upcoming 18<sup>th</sup> Annual WORLDSymposium that will take place in San Diego, CA and virtually from February 7-11, 2022.

### Platform Presentation Details for 4D-310 at WORLDSymposium:

**Title:** An open-label, phase 1/2 trial of gene therapy 4D-310 in adult males with Fabry disease

**Speaker:** Jerry Vockley, M.D., PhD, Chief of Genetic and Genomic Medicine at the University of Pittsburgh School of Medicine

**Session:** Clinical Applications

**Date and Time:** Wednesday, February 9, 10:30 a.m. PT

The presentation will be made available shortly after being presented on the 4DMT website at <https://ir.4dmolecularterapeutics.com/events>.

### About 4D-310 and Fabry Disease

4D-310 utilizes the targeted and evolved C102 vector to deliver a functional copy of the *GLA* gene and was designed for a unique dual mechanism of action after a single IV administration. The product is designed to generate both high sustained blood levels of AGA for systemic cross-correction of tissues, as well as for a complementary high local production of AGA directly within critically affected organs, including heart, blood vessels and kidney. This product design has the potential to address the significant unmet medical needs in patients with Fabry disease, and we believe either mechanism would represent a significant clinical advancement on its own, and together, these mechanisms could be synergistic.

Affecting more than 50,000 people in the United States and European Union, Fabry disease is a genetic disorder of the *GLA* gene that results in the body's inability to produce an enzyme called alpha-galactosidase or AGA, causing the accumulation of the substrate globotriaosylceramide (Gb3) in critical organs, including the heart, kidney and blood vessels. Such substrate accumulation can lead to life-threatening hypertrophic cardiomyopathy, heart failure, arrhythmias, various degrees of kidney dysfunction and cerebrovascular stroke. Fabry disease progression results in increased morbidity, mortality and cost of care.

Significant unmet medical needs remain for these patients despite enzyme replacement therapy (ERT), the current standard of care. ERT requires biweekly intravenous dosing which markedly decreases patients' quality of life. In addition, while benefit has been demonstrated in the kidney, ERT has not been shown to clearly benefit the heart. Cardiovascular disease remains the leading cause of death and disability in Fabry disease patients.

### 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 on five clinical-stage products in three therapeutic areas: ophthalmology, cardiology (including Fabry disease) 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. 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.

### 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 the development of 4D-310, 4D-150, 4D-125, 4D-710 and 4D-110, including the therapeutic potential and clinical benefits thereof; 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 Quarterly Report on Form 10-Q, 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-150, 4D-125 and 4D-110 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 or 4D-110 for the therapeutic use for which they are

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