



4DMT to Present Interim Clinical Data from INGLAXA Phase 1/2 Trials of 4D-310 for Fabry Disease Cardiomyopathy at 19th Annual WORLDSymposium™

February 16, 2023

- *Company to host live webcast on Wednesday, February 22, at 4:30 p.m. EST*
- *Platform presentation to be presented at WORLDSymposium™ on Saturday, February 25*

EMERYVILLE, Calif., Feb. 16, 2023 (GLOBE NEWSWIRE) -- 4D Molecular Therapeutics (Nasdaq: FDMT, '4DMT'), a clinical-stage biotherapeutics company harnessing the power of directed evolution for targeted genetic medicines, today announced that the Company will host a live webcast on February 22 to present interim clinical data from its 4D-310 INGLAXA Phase 1/2 clinical trials for the treatment of Fabry disease cardiomyopathy and will give an update on clinical development plans.

The webcast will include a summary of safety and efficacy data, including 12-month cardiac data on imaging, function, and quality of life endpoints. In addition, detailed analysis of a cardiac biopsy will be presented. The Company will also be joined by Paul J. Utz, M.D., Ph.D., Professor, Medicine / Immunology & Rheumatology at Stanford University, to discuss the mechanisms causing atypical hemolytic uremic syndrome (aHUS) with intravenous AAV therapy, pre-existing risk factors for aHUS in patients, and the use of the rituximab/sirolimus immune regimen for the prevention of aHUS.

Webcast Information:

Title: 4D-310 INGLAXA Phase 1/2 Interim Clinical Data Webcast and Q&A
Date/Time: Wednesday, February 22, 2023, 4:30 p.m. EST
Registration: [Link](#)

An archived copy of the webcast will be available for up to one year by visiting the "Investors & Media" section of the 4DMT website at <https://ir.4dmolecularterapeutics.com/events>.

The Company also announced cardiac efficacy data from the 4D-310 INGLAXA Phase 1/2 clinical trials will be presented in a platform presentation at the 19th annual WORLDSymposium™ in Orlando, Florida.

WORLDSymposium™ Platform Presentation Details:

Title: Cardiac effects of 4D-310 in adults with Fabry disease in a phase 1/2 clinical trial: Functional, quality of life, and imaging endpoints in patients with 12 months of follow up
Presenter: Raphael Schiffmann, MD, MHSc, FAAN; 4D Molecular Therapeutics Inc.
Date/Time: Saturday, February 25, 2023, 10:30 a.m. EST

The presentation from WORLDSymposium™ will also be available on the 4D Molecular Therapeutics website under Scientific Presentations: <https://4dmolecularterapeutics.com/technology/scientific-presentations>.

About 4D-310 and Fabry Disease Cardiomyopathy

4D-310 utilizes the targeted and evolved C102 vector to deliver a functional copy of the GLA gene and was designed for a unique mechanism of action, specifically to directly correct the AGA enzyme function within cardiomyocytes (heart muscle cells) after a single IV administration. C102 was invented at 4DMT through our proprietary Therapeutic Vector Evolution platform; we created this platform utilizing principles of directed evolution, a Nobel Prize-winning technology. The product is designed to generate both high local production of AGA directly within critically affected organs, including heart, blood vessels, and kidney, as well as the potential for sustained blood levels of AGA for systemic cross-correction. This product design has the potential to address the cardiomyopathy in these patients that is the leading cause of death, as well as other significant unmet medical needs in patients with Fabry disease.

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 biotherapeutics company harnessing the power of directed evolution for targeted genetic medicines. 4DMT seeks to unlock the full potential of genetic medicines using its proprietary invention platform, Therapeutic Vector Evolution, which combines the power of the Nobel Prize-winning technology, directed evolution, with approximately one billion synthetic AAV capsid-derived sequences to invent targeted and evolved vectors for use in our product candidates. All of our vectors are proprietary to 4DMT and were invented at 4DMT, including the vectors utilized in our clinical-stage and preclinical pipeline product candidates: R100, A101, and C102. The Company is initially focused on five clinical-stage product candidates in three therapeutic areas for both rare and large market diseases: ophthalmology, pulmonology, and cardiology (Fabry disease).

cardiomyopathy). The 4DMT targeted and evolved vectors were 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. 4DMT is currently advancing five product candidates in clinical development: 4D-150 for wet AMD and DME, 4D-710 for cystic fibrosis lung disease, 4D-310 for Fabry disease cardiomyopathy, 4D-125 for XLRP, and 4D-110 for choroideremia. The 4D preclinical product candidates in development are: 4D-175 for geographic atrophy and 4D-725 for alpha-1 antitrypsin deficiency.

4D-150, 4D-710, 4D-310, 4D-125, and 4D-110 are our product candidates in clinical development 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-150, 4D-710, 4D-310, 4D-125, or 4D-110 for the therapeutic uses for which they are being studied.

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