# 4DMT Announces Presentation on Aerosolized 4D-710 for Treatment of Cystic Fibrosis at the American Society of Gene & Cell Therapy (ASGCT) 26th Annual Meeting

May 11, 2023

EMERYVILLE, Calif., May 11, 2023 (GLOBE NEWSWIRE) -- 4D Molecular Therapeutics (Nasdaq: FDMT), a clinical-stage biotherapeutics company harnessing the power of directed evolution for targeted genetic medicines, today announced a presentation on 4D-710 for the treatment of cystic fibrosis lung disease at the American Society of Gene & Cell Therapy (ASGCT) 26th Annual Meeting held in Los Angeles, California on May 16-20, 2023.

The oral presentation will be given by Jennifer L. Taylor-Cousar, M.D., MSCS, Professor, Departments of Medicine and Pediatrics, and Co-Director, Adult Cystic Fibrosis Program, Director, Cystic Fibrosis Foundation Therapeutics Development Center, National Jewish Health. The presentation will include biomarker data on expression of the cystic fibrosis transmembrane regulator (CFTR) transgene protein from the endobronchial lung biopsies & brushings collected at week 4-8 for patients in Cohort 1 (n=3; dose 1E15 vg) of the Phase 1/2 AEROW clinical trial. In situ hybridization biomarker data, demonstrating transgene RNA expression from lung samples from Cohort 1 patients, was previously presented by Dr. Taylor-Cousar at the North American Cystic Fibrosis Conference (NACFC), November 3, 2022.

Additional clinical and biomarker data for Cohort 1 patients (n=3; 9-12 months follow-up) are expected to be presented at the European Cystic Fibrosis Conference in June 2023. Clinical data will focus on safety, tolerability, pulmonary function testing (e.g., percent predicted $FEV_1$) and respiratory-related quality of life assessments.

**Oral Presentation Details:**

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| Presentation Title: | AAV Mediated Gene Therapy for Cystic Fibrosis (4D-710) |
| Session Title: | Advances in Respiratory and GI Tract Gene and Cell Therapy |
| Presenter: | Jennifer L. Taylor-Cousar, M.D., MSCS, Professor, Departments of Medicine and Pediatrics, and Co-Director, Adult Cystic Fibrosis Program, Director, Cystic Fibrosis Foundation Therapeutics Development Center, National Jewish Health; Lead Principal Investigator, 4D-710 Phase 1/2 Clinical Trial |
| Date/Time: | Thursday, May 18, 2023, 9:15 a.m. - 9:40 a.m. PT |

The presentation from the ASGCT annual meeting will also be available on the 4D Molecular Therapeutics website under Scientific Presentations: https://4dmoleculartherapeutics.com/pipeline/#posters-and-publications.

**About 4D-710 and Cystic Fibrosis Lung Disease**

4D-710 is comprised of our targeted and evolved vector, A101, and a codon-optimized CFTRΔR transgene. 4D-710 has the potential to treat a broad range of patients with cystic fibrosis, independent of the specific CFTR mutation, and is designed for aerosol delivery to achieve CFTR expression within lung airway epithelial cells. 4D-710 is being initially developed in the approximately 15% of patients whose disease is not amenable to existing CFTR modulator medicines targeting the CFTR protein. In patients with CFTR mutations whose disease is amenable to modulator medicines, the improvement in lung function is incomplete and is variable. We therefore expect to potentially develop 4D-710 in this broader patient population, as a single agent and/or in combination with CFTR modulator small molecule medicines.

Cystic fibrosis is a major inherited disease caused by mutations in the CFTR gene. According to the CF Foundation, approximately 40,000 people in the United States and more than 70,000 people worldwide are living with cystic fibrosis, with approximately 1,000 new cases of cystic fibrosis diagnosed in the United States each year. Cystic fibrosis is a multisystem disorder affecting the lungs, digestive system and reproductive tract. Lung disease is the leading cause of morbidity and mortality. Cystic fibrosis causes impaired lung function, inflammation and bronchiectasis and is commonly associated with persistent lung infections and repeated exacerbations due to the inability to clear thickened mucus from the lungs. Patients with cystic fibrosis require lifelong treatment with multiple daily medications. The complications of the disease result in progressive loss of lung function and hospitalizations, and ultimately lead to end-stage respiratory failure.

**About 4DMT**

4DMT is a clinical-stage biotherapeutics company harnessing the power of directed evolution for genetic medicines targeting large market diseases. 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 customized 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 customized 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 AATLD.

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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**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 therapeutic potential, and clinical benefits, as well as the plans and related timing for the clinical development of 4D-710. 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 risks and uncertainties that are described in greater detail in the section entitled "Risk Factors" in 4D Molecular Therapeutics’ most recent Annual Report on Form 10-K, 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.

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