4D Molecular Therapeutics Receives $3 Million Follow-On Funding from Cystic Fibrosis Foundation Therapeutics for Gene Therapy IND Candidate for Cystic Fibrosis

September 19, 2017

New Funding Brings Total IND-Enabling Research and Development Support for 4D-710 to over $3.5 Million

Emeryville, CA — 4D Molecular Therapeutics (4DMT), a leader in adeno-associated virus (AAV) gene therapy vector discovery and product development, today announced $3 million in additional funding from Cystic Fibrosis Foundation Therapeutics Inc., (CFFT) the non-profit drug discovery and development affiliate of the Cystic Fibrosis (CF) Foundation, to expand ongoing efforts to develop an IND candidate gene therapeutic for CF using 4DMT's lead proprietary AAV vector targeting lung airway cells after aerosolized delivery.

The new funding from CFFT combined with matching funds from 4DMT is expected to enable completion of remaining studies needed to file an Investigational New Drug application with the Food and Drug Administration to begin clinical trials with 4D-710. This award from CFFT is in addition to a nearly $525,000 grant from CFFT in September 2016, bringing CFFT's commitment to 4DMT's technology to over $3.5 million.

"We are very pleased and honored to have been selected to receive additional IND candidate development funding from Cystic Fibrosis Foundation Therapeutics Inc., a world leader in CF research and development. The CF Foundation's support for our CF product development further validates the potential of our Therapeutic Vector Evolution platform, and the products engineered from our optimized and proprietary AAV vectors. This additional investment will accelerate IND enabling studies and initiation of clinical testing in CF patients with high unmet medical needs," said David Kirn, MD, co-founder and CEO of 4DMT.

"The CFFT is a critical partner for 4DMT's efforts to bring our transformative gene therapy product to CF patients. In addition to financial support, CFFT clinicians, scientists and patient advocates will work closely with 4DMT to advance 4D-710 as efficiently and effectively as possible," said David Schaffer, PhD, co-founder and acting CSO of 4DMT, and Professor of Bioengineering and Chemical Engineering at the University of California, Berkeley.

About Cystic Fibrosis and AAV Gene Therapy

Affecting more than 30,000 patients in the US and 70,000 worldwide, CF is a genetic disease that impacts multiple tissues, including primarily the lung. Chronic lung infections result in progressive lung damage and premature death. CF is caused by mutations in the gene encoding for a protein known as CFTR, rendering it defective. Gene therapy holds promise for the treatment of CF by using a proprietary AAV vector to deliver a functional copy of the CFTR gene to the airway and lungs to restore function and alleviate disease symptoms.

About 4D Molecular Therapeutics

4DMT is focused on the discovery and development of targeted and proprietary AAV gene therapy vectors and therapeutic products. Our robust discovery platform, termed Therapeutic Vector Evolution, empowers us to create customized gene delivery vehicles to deliver genes to any tissue or organ in the body, by optimal clinical routes of administration and evading pre-existing antibodies. These proprietary and targeted products allow us to treat both rare genetic diseases and complex large market diseases. 4DMT is creating a diverse product pipeline both through partnerships and by progressing internal 4DMT products toward clinical trials in parallel. 4DMT partners include: Pfizer (PFE), Roche (SIX: ROG; OTCQX: RHHBY), uniQure (QURE), AGTC and Benitec.

About 4DMT's Therapeutic Vector Evolution

Current clinical stage AAV gene therapy products are based on vectors that are generally "wild-type" or primitive vectors, meaning they were found in nature as laboratory contaminants or as monkey infections. These first-generation AAV vectors, while generally safe and well-tolerated in patients, do not have optimized delivery properties and often require aggressive and/or invasive dosing to attempt even minimal transduction of target cells. 4DMT is advancing the field of AAV vector technology by deploying principles of evolution and selection to create vectors that efficiently target the desired cells within the diseased human organ via clinically optimal routes of administration. Our Therapeutic Vector Evolution platform deploys approximately 100 million unique AAV variants from proprietary 4DMT AAV libraries with extensive diversity. 4DMT then applies proprietary "natural selection" methods to identify lead vectors that are highly optimized for a specific target cell and organ, route of therapeutic administration, and capacity to evade pre-existing antibodies in patients. The result is a panel of customized, novel, and proprietary vectors uniquely designed for gene therapy in humans.

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