4D Molecular Therapeutics Announces New Agreement with Cystic Fibrosis Foundation

June 17, 2020

EMERYVILLE, CA. – June 17, 2020 – 4D Molecular Therapeutics (4DMT), a clinical-stage leader in the development of precision-guided AAV gene medicines based on directed evolution, today announced a new agreement with Cystic Fibrosis Foundation (CF Foundation) to develop precision gene medicines for cystic fibrosis (CF). The CF Foundation's investment builds upon previous work with 4DMT to advance new therapies for CF. Under this agreement, the CF Foundation will support the completion of IND-enabling research and development activities, and the planned Phase 1/2 clinical study of 4D-710, 4DMT's wholly-owned product candidate for the aerosol treatment of CF lung disease.

As part of this agreement, the CF Foundation has committed $14 million in new funding, consisting of a $10 million equity investment in 4DMT's recently completed Series C financing and additional funding contingent upon achievement of a pre-clinical development milestone. 4DMT will match the funds provided and allocate these funds to the ongoing development of 4D-710. 4DMT retains worldwide rights to develop and commercialize 4D-710 and any other potential medicines. The CF Foundation previously awarded 4DMT over $3 million in funding to support discovery and development of AAV gene delivery to the lung in 2016.

The CF Foundation's investment is being offered as part of its $500 million Path to a Cure initiative to accelerate development of treatments that address the underlying cause of the disease and a cure for CF.

"We are incredibly pleased to expand our strong relationship with the CF Foundation, a world leader in cystic fibrosis research, development and thought leadership," said David Kirn, MD, co-founder, chairman and chief executive officer of 4DMT. "This investment will accelerate development activities for 4D-710, our AAV gene therapy product candidate designed to deliver a functional copy of the CFTR gene via one-time aerosol administration. Support from the CF Foundation will help accelerate our Therapeutic Vector Evolution platform and of 4D-710, our gene therapy candidate for CF lung disease."

About Cystic Fibrosis and 4D-710

Affecting more than 30,000 patients in the United States and 70,000 worldwide, CF is a genetic disease that impacts multiple tissues, including primarily the lung. CF causes chronic lung infections that 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.

4DMT's gene therapy approach holds promise for the treatment of CF by using a proprietary and optimized AAV vector to deliver a functional copy of the CFTR gene to the airway and lungs to restore function and alleviate disease symptoms. 4D-710 is 4DMT's wholly-owned product candidate for the treatment of CF lung disease. 4D-710 is comprised of a transgene insert encoding for the CFTR gene and 4DMT's proprietary vector 4D-A101, a vector that is designed for an efficient, single dose aerosol delivery to the lung airways and with resistance to pre-existing antibodies.

About 4DMT

4DMT is a clinical-stage precision gene medicines company harnessing the power of directed evolution to unlock the full potential of gene therapy for rare and large market diseases in lysosomal storage diseases, ophthalmology, neuromuscular diseases and cystic fibrosis. 4DMT's proprietary Therapeutic Vector Evolution platform enables a "disease first" approach to product discovery and development, thereby empowering customization of AAV vectors to target specific tissue types associated with the underlying disease. These proprietary and optimized AAV vectors are designed to provide targeted delivery by routine clinical routes, efficient transduction, reduced immunogenicity and resistance to pre-existing antibodies — attributes that could enable the development of gene therapies that overcome known limitations of conventional AAVs. 4DMT vectors are designed to exhibit improved therapeutic profiles that enable the company to pursue previously untreatable patient populations and to address a broad range of rare and large market disease markets.

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