4D Molecular Therapeutics Announces FDA Orphan Drug Designation Granted to 4D-110 for the Treatment of Choroideremia

November 16, 2018

Emeryville, CA – November 16, 2018 – 4D Molecular Therapeutics (4DMT), a world-leader in Therapeutic Vector Evolution for adeno-associated virus (AAV) gene therapy vector discovery and product development today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to 4D-110 for the treatment of choroideremia. Choroideremia (CHM) is an X-linked monogenic recessive disease (caused by mutation of the REP-1 gene) starting with reduced night vision and progressive loss of peripheral vision and eventually leading to loss of visual acuity and blindness.

"Our product for CHM patients, 4D-110, will be administered by intravitreal injection, and is designed to result in widespread expression of the REP-1 protein within cells in the retina," said Peter Francis, SVP Clinical, Translational R&D Program Leader, Retina Therapeutic Area. "In contrast to other AAV gene therapy agents for CHM patients, which are administered by subretinal surgical injection, 4D-110 is designed to treat the entire retina, and to treat patients with all stages of the disease."

The company has enrolled more than 50 CHM patients in an on-going natural history study. This study is expected to support the rapid enrollment of the company's planned clinical studies and to provide baseline characteristics for pre and post-treatment analysis. 4DMT expects to initiate the Phase 1 trial in 2019.

The FDA's Office of Orphan Drug Products grants orphan drug designation to support the development of medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the United States. Orphan drug designation provides to 4D Molecular Therapeutics certain benefits, including market exclusivity upon regulatory approval, if received, exemption of FDA application fees and tax credits for qualified clinical trials.

About 4D Molecular Therapeutics (4DMT)

4DMT is focused on the discovery and development of targeted, customized and proprietary next-generation AAV gene therapy products for use in patients with severe genetic diseases with high unmet medical need. Our robust discovery platform, termed Therapeutic Vector Evolution, empowers us to create customized gene delivery vehicles to deliver genes specifically to any tissue or organ in the body, by optimal clinical routes of administration, at lower doses and with resistance to 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 and deep product pipeline through its own internal 4D products, as well as partnered programs.

About 4DMT's Therapeutic Vector Evolution

4DMT is advancing the field of targeted and optimized AAV vector technology by deploying principles of evolution and natural selection to create vectors that efficiently and selectively target the desired cells within the diseased human organ via clinically optimal routes of administration, at manageable doses and with resistance to pre-existing antibodies in the population. Our Therapeutic Vector Evolution platform deploys over 100 million unique AAV variants from over 35 unique and proprietary 4DMT AAV libraries with extensive diversity. After defining the Target Product Profile, and the associated Target Vector Profile, 4DMT then applies proprietary methods to identify lead vectors from within our AAV libraries. The result is a customized, novel, and proprietary pharmaceutical-grade product uniquely designed for targeted therapeutic gene delivery and efficacy in humans.

###

Contacts:

Media:

Jamie Firmage
jfirmage@4dmt.com
415 860-6301

Investors:

Pete Rahmer
Endurance Advisors
prahmer@enduranceadvisors.com
415-515-9763

Mike Zanoni
Endurance Advisors
mzanoni@enduranceadvisors.com
610-442-8570