PRESS RELEASES

4D Molecular Therapeutics Receives FDA Fast Track Designation for 4D-310 Gene Therapy for Treatment of Fabry Disease

Emeryville, CA – August 13, 2020 – 4D Molecular Therapeutics (4DMT), a clinical-stage leader in the development of precision-guided AAV gene medicines based on directed evolution, announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to 4D-310 for Fabry disease, a debilitating lysosomal storage disorder caused by a deficiency of alpha-galactosidase A (AGA) enzyme activity. 4D-310 is a gene medicine engineered with a proprietary optimized AAV capsid discovered by 4DMT through its proprietary Therapeutic Vector Evolution platform.

“We’re pleased that the FDA has granted Fast Track designation for 4D-310,” said David Kirn, MD, co-founder, chairman and chief executive officer of 4DMT. “Patients with Fabry currently have significant unmet medical needs, despite enzyme replacement therapies [ERT]. In contrast to ERT and other medicines in development, 4D-310 is designed to drive high level AGA enzyme expression directly within the diseased target tissues themselves, including the heart, kidney and blood vessels, in addition to driving high and stable blood concentrations. We believe this differentiation has the potential to benefit a broad range of patients with Fabry disease, including those with significant cardiomyopathy, the leading cause of death in these patients.”

Raphael Schiffman, MD, MH Sc, Principal Investigator at the Baylor Scott & [illegible] Institute and 4DMT Senior Clinical Advisor, Lysosomal Storage Diseases, said "To date, ERT correction of the enzyme deficiency in critical tissues, beyond endothelial cells, has been a challenge. A one time therapy that corrects AGA deficiency directly in organs and tissues where substrate accumulates may more completely and durably address the disease process in Fabry, particularly in the heart."

The FDA's Fast Track designation is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. Once a drug receives Fast Track designation, early and frequent communication between the FDA and a drug company is encouraged throughout the entire drug development and review process. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients.

About Fabry Disease and 4D-310

Affecting more than 50,000 people in the United States and European Union, Fabry disease is a rare genetic disorder that results in the body's inability to produce an enzyme called AGA, causing the accumulation of globotriaosylceramide-3 (Gb3) in critical organs, including the heart and kidney. Such substrate accumulation can lead to life-threatening heart failure, arrhythmias, vascular blockages, and various degrees of kidney dysfunction. Progression of the disease causes significant reduction in the quality of life and significant economic burden associated with greater patient needs for supportive care.

4D-310 holds promise for the treatment of Fabry by using a proprietary and optimized AAV vector to deliver a functional copy of the GLA gene, resulting in AGA production in target tissues. The proprietary vector in 4D-310 is designed for efficient, low-dose intravenous delivery to key affected tissues in Fabry disease, including cardiac tissue, kidney, and vascular smooth muscle tissues.

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 of administration, 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 AAV vectors. 4DMT vectors

are designed to exhibit improved therapeutic profiles that enable the potential to treat previously untreatable patient populations and to address a broad range of rare and large market disease markets.

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