4D Molecular Therapeutics Enrolls First Patient in Natural History Study in Lead Clinical Program to Develop Gene Therapy Treatment for Choroideremia

December 23, 2016

Intravitreal delivery to the retina is critical to Choroideremia treatment

Emeryville, CA | December 23, 2016 —4D Molecular Therapeutics (4DMT), a leader in gene therapy product discovery and development, today announced the enrollment of the first participant in its Choroideremia Natural History Study (NHS). This study is an important step in developing a groundbreaking gene therapy product optimized for intravitreal administration to treat Choroideremia (CHM) patients. 4DMT has deployed its proprietary AAV vector discovery platform, Therapeutic Vector Evolution, to create and optimize a proprietary AAV vector for intravitreal delivery to the retina. This vector is designated to be the basis for the first 4DMT experimental gene therapeutic targeted for the treatment of CHM. 4DMT is working in close collaboration with the Choroideremia Research Foundation on CHM product development and the Natural History Study.

Choroideremia Natural History Study (NHS) Purpose
The NHS is a multi-center US-based study designed to evaluate disease progression in a wide variety of individuals with CHM. Understanding more about clinical endpoints as the disease progresses will aid in identifying both potential participants and the best clinical measures for upcoming 4DMT clinical trials. Participants in the study will be assessed periodically over two years. At each visit, they will undergo a series of photographic, imaging and clinical evaluations.

Two clinical sites are actively recruiting for the study:

1. Retina Foundation of the Southwest, Dallas, Texas (Principal Investigator Dr. David Birch)
2. Retina Vitreous Associates of Los Angeles (Principal Investigator Dr. David Liao).

Additional information including contact numbers may be found at clinicaltrials.gov, NCT 02994368.

“The Choroideremia Research Foundation and its community are thrilled at the initiation of 4DMT’s Natural History Study. This milestone brings us one step closer to a treatment that could end blindness from CHM,” said Christopher Moen, MD, President of the Choroideremia Research Foundation.

“This clinical study is a significant milestone for the company and a critical step forward for our lead program to treat choroideremia. We are convinced the data generated in this study will accelerate the clinical development of our lead product,” said Dr. David Kirn, co-founder and CEO, 4D Molecular Therapeutics.

About Choroideremia
Choroideremia is an X-linked retinal disease that begins as night blindness in childhood and progresses to complete blindness. It affects 1 in 50,000 people in the United States, predominantly males, and has no effective treatment.

About the Choroideremia Research Foundation
The CRF was founded in 2000 as a fundraising and patient advocacy organization to stimulate research on CHM. Since its inception, the CRF has provided over $2 million in research awards and is the largest financial supporter of CHM research worldwide. Research funded by the CRF has led to the development of a CHM animal model, the pre-clinical production of gene therapy vectors currently in clinical trials, and the CRF Biobank which stores tissue and stem cell samples donated by CHM patients.

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 with evasion of 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 partnerships, while progressing internal 4DMT products toward clinical trials in parallel. 4D partners include: Pfizer (PFE), Roche (SIX: ROG; OTCQX: RHHBY), uniQure (QURE), AGTC, and Benitec.

About 4DMT’s Therapeutic Vector Evolution
Current clinical stage gene therapy products are based on AAV (Adeno-Associated Virus) 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 the desired 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 and selectively 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. 4DMT then applies proprietary 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 customized, novel, and proprietary pharmaceutical-grade vector uniquely designed for therapeutic gene delivery in humans.
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