4D Molecular Therapeutics Announces First Patient Dosed in Phase 1 Clinical Trial of 4D-110 by Intravitreal Injection for the Treatment of Choroideremia

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Emeryville, CA – July 27, 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 first patient has been dosed in the Phase 1 clinical trial of 4D-110, a Roche-licensed product candidate, delivered by a single intravitreal injection for Choroideremia (CHM). CHM is a blinding and currently untreatable X-linked inherited retinal disease.

“Dosing the first patient in the Phase 1 clinical trial of 4D-110 marks the first of three Therapeutic Vector Evolution pipeline candidates expected to enter the clinic in 2020,” said David Kirn, MD, co-founder, chairman and chief executive officer of 4DMT. “4DMT harnesses the power of directed evolution to develop precision-guided AAV gene medicines. In the case of 4D-110, this proprietary and optimized AAV vector is designed to provide targeted delivery to the retina of a functional copy of the CHM gene by intravitreal injection, a routine clinical route of administration. Ultimately, our aim is to alter the course of this debilitating disease and to treat patients with all stages of the disease. I would like to thank the Choroideremia patient community, their families and caregivers, the Choroideremia Research Foundation, and the clinical trial physicians and staff, without whom 4DMT would not have reached this stage.”

“With no currently approved therapies available for patients impacted by Choroideremia, gene therapy represents a promising therapeutic approach,” said David Birch, PhD, Scientific Director, Retina Foundation of the Southwest and a principal investigator for the study. “Due to its optimized vector, 4D-110 is a novel gene therapy approach that shows promise in safely treating a broad region of the retina and in a broad range of patients. The potential to slow-down or halt the debilitating visual field constriction seen in this disease is an exciting opportunity for patients.”

The Phase 1 open-label, dose-exploration and dose-expansion study is expected to enroll up to 15 patients with Choroideremia. The study is designed to assess the preliminary safety, tolerability and biological activity of a single intravitreal injection of 4D-110. In addition, the clinical trial will evaluate the effect of 4D-110 on the visual function and retinal degeneration.

“On behalf of the Choroideremia Research Foundation and Choroideremia patients internationally, I am extremely excited by the initiation of 4DMT’s clinical trial,” said Christopher Moen, MD, Chief Medical Officer of the Choroideremia Research Foundation. “This pivotal milestone brings us one step closer to a transformative treatment which has the potential to end blindness from Choroideremia.”

About Choroideremia and 4D-110

Affecting approximately 10,000 individuals in the United States and the European Union, Choroideremia is an X-linked, slowly-progressive, degenerative disease of the retina and choroid of the eye caused exclusively by deletions or mutations in the CHM gene, resulting in a missing or defective REP1 protein. Choroideremia initially manifests as night-blindness and peripheral visual field defects, usually starting in the first two decades of life. As the disease progresses, the visual field begins to constrict relatively early in the disease’s progression, which hinders patients’ ability to conduct daily activities and eventually leads to vision loss.

4DMT’s precision-guided gene therapy approach holds promise for the treatment of Choroideremia by using a proprietary and optimized AAV vector to deliver a functional copy of the CHM gene, resulting in the production of the REP1 protein. 4D-110, which is licensed to Roche, is comprised of a CHM transgene insert and 4DMT’s proprietary vector 4D-R100, a vector designed to provide targeted delivery via intravitreal administration and to efficiently transduce all layers of the retina.

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 AAV vectors. 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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