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4D Molecular Therapeutics Announces First Patient Dosed in Phase 1/2 Clinical Trial of 4D-125 by Intravitreal Injection for the Treatment of X-Linked Retinitis Pigmentosa

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EMERYVILLE, Calif.--(BUSINESS WIRE)--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 1/2 clinical trial of 4D-125 for X-Linked Retinitis Pigmentosa (XLRP), a blinding and currently untreatable inherited retinal disease. 4D-125 is an AAV gene therapy with an optimized and proprietary vector designed to deliver a functional copy of the RPGR gene to photoreceptors in the retina. This vector enables both administration by intravitreal injection, a safe and routine clinical route, plus the potential to treat broader and earlier-stage patient populations as compared to subretinal approaches. 4D-125 is a 4DMT-declared ophthalmology therapeutic candidate under 4DMT's collaboration with Roche, where Roche has an option to take an exclusive world-wide license prior to initiation of a pivotal trial.

"4DMT's Therapeutic Vector Evolution platform harnesses the power of directed evolution to develop precision-guided AAV gene medicines. The result is products with characteristics optimized for specific diseases, including enhanced transduction, optimized delivery route, improved tolerability, lower doses, and antibody-resistance," said David Kirn, MD, co-founder, chairman and chief executive officer of 4DMT. "Dosing the first patient in the Phase 1/2 clinical trial of 4D-125 marks the second of three Therapeutic Vector Evolution pipeline candidates expected to enter the clinic in 2020. I am grateful for the XLRP patient community, their families and caregivers, and the clinical trial physicians and staff, who share in our vision of a next-generation gene therapy for XLRP patients."

"Presently there is no effective treatment for people suffering from this devastating disease, which leads to vision loss and ultimately blindness," said Dr. Marc Mathias, Assistant Professor, Ophthalmology, University of Colorado School of Medicine and a principal investigator on 4DMT's Phase 1/2 clinical trial of 4D-125. "4DMT's gene therapy approach for XLRP aims to address the underlying cause of XLRP through the therapeutic delivery of the RPGR gene into patients' retinal cells to produce functional protein. Furthermore, 4D-125 is promising for patients relative to other gene therapy approaches because it is delivered by intravitreal injection, a routine clinical route of administration."

The Phase 1/2 open-label, dose-exploration and dose-expansion study is expected to enroll approximately 18 male patients with X-Linked Retinitis Pigmentosa. The study is designed to assess the preliminary safety, tolerability and biological activity of a single intravitreal injection of 4D-125. In addition, the clinical trial will evaluate the effect of 4D-125 on the visual function and retinal degeneration.

About X-Linked Retinitis Pigmentosa and 4D-125

Affecting approximately 20,000 patients in the United States and European Union, XLRP is a rare inherited X-linked recessive genetic disorder, which causes progressive vision loss and blindness. It is characterized by dysfunction and degeneration of photoreceptors in the retina. Seventy percent of cases are caused by mutations in the retinitis pigmentosa GTPase regulator (RPGR) gene. Loss of RPGR function in retinal cells causes the progressive loss of rod and cone photoreceptors, leading to the loss of vision experienced by patients. Symptoms of XLRP are initially characterized by night blindness, followed by loss of peripheral visual field, decreasing visual acuity and eventually blindness. Males are usually the most affected with symptoms starting in the early years of life.

4DMT's precision-guided gene therapy approach holds promise for the treatment of XLRP by using a proprietary and optimized AAV vector to deliver a functional copy of the RPGR gene. 4D-125 is comprised of a RPGR 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 medicine 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 company to pursue previously untreatable patient populations and to address a broad range of rare and large market disease markets.

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