4D Molecular Therapeutics and Foundation Fighting Blindness Partner to Develop Gene Therapies for Retinal Diseases

July 24, 2017

Emeryville, CA and Columbia, MD | July 24, 2017 — 4D Molecular Therapeutics (4DMT), a leader in adeno-associated virus (AAV) gene therapy vector discovery and product development, and the Foundation Fighting Blindness (FFB), the world's largest non-governmental source of research funding for inherited retinal degenerations (IRD) and dry age-related macular degeneration (AMD), today announced a partnership to develop intravitreal gene therapeutics for patients with these blinding conditions using 4DMT-proprietary AAV vectors. Under the terms of the agreement, 4DMT will provide access to its vector technology, development expertise and manufacturing capabilities while FFB will identify potential academic and business collaborators, provide drug development expertise and fund approved projects to develop transformative gene therapy products. 4DMT retains all patent and commercial rights to its 4DMT proprietary AAV vector variants. FFB and 4DMT will jointly review and approve all programs initiated within this collaboration.

New vector technologies are critical to the successful use of gene therapies for IRDs in order to improve targeting to affected cells within the retina, and to maximize efficacy and safety. Vectors that can be delivered intravitreally would simplify the procedures used for treatment and reduce the costs of administration.

Affecting approximately 200,000 patients in the US, inherited retinal diseases (IRDs) are a major cause of adult and childhood blindness. Mutations in more than 260 genes are known to cause these rare, orphan conditions for which there are currently no approved therapies. Gene therapy holds tremendous promise for the treatment of these conditions by introducing genes to the retina that may be able to replace the lost or dysfunctional genes, correct underlying mutations, or deliver therapeutically-active genes that can prevent cell loss and degeneration.

"We are very impressed with 4D's vector evolution approach, the company's product pipeline and manufacturing expertise. The potential is great for developing a number of gene therapeutics that could treat those affected by retinitis pigmentosa and allied conditions using a simple intravitreal injection approach," said Patricia Zilliox PhD, Chief Drug Development Officer at the FFB.

"We are extremely excited by this collaboration with FFB, a globally-recognized leader in the effort to cure blindness due to inherited retinal degenerations. FFB has tremendous expertise identifying the best retinal research as well as an outstanding network of funded investigators and companies with whom we hope to collaborate to develop a portfolio of products that will benefit those affected with retinal degenerative diseases," said David Kirn, MD, co-Founder and CEO of 4DMT.

About Foundation Fighting Blindness (FFB)

Since FFB was established in 1971 it has raised more than $700 million toward its mission of preventing, treating and curing blindness caused by inherited retinal diseases. In excess of 10 million Americans, and millions more worldwide, experience vision loss due to retinal degenerations. Through its support of focused and innovative science, and by teaming with industry, FFB drives the research that has and will continue to provide treatments and cures for people affected by retinitis pigmentosa, macular degeneration, Usher syndrome and other inherited retinal diseases.

About 4D Molecular Therapeutics (4DMT)

4DMT is focused on the discovery and development of targeted and proprietary AAV gene therapy vectors and therapeutic 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 to any tissue or organ in the body, by optimal clinical routes of administration 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. 4DMT partners include: Pfizer (PFE), Roche (SIX: ROG; OTCQX: RHHBY), uniQure (QURE), AGTC, Benitec, Cystic Fibrosis Foundation and Choroideremia Research Foundation.

About 4DMT's Therapeutic Vector Evolution

Current clinical stage gene therapy products are generally based on one of 10 AAV vectors that are "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 natural selection to create vectors that efficiently and selectively target the desired cells within the diseased human organ via clinically optimal routes of administration with resistance to pre-existing antibodies in the population. Our Therapeutic Vector Evolution platform deploys approximately 100 million unique AAV variants from proprietary 4DMT AAV libraries with extensive diversity. 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 and efficacy in humans.

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