4D Molecular Therapeutics and the Choroideremia Research Foundation Partner to Develop Gene Therapy Treatment for Choroideremia

February 23, 2016

Intravitreal delivery to the retina is critical to Choroideremia treatment

Emeryville, CA | February 23, 2016 — 4D Molecular Therapeutics (4DMT), a leader in Adeno-Associated Virus (AAV) gene therapy vector discovery and product development, and the Choroideremia Research Foundation (CRF), a non-profit dedicated to finding a cure for choroideremia, today announced a partnership to develop a gene therapy product optimized for intravitreal administration to treat Choroideremia.

Under the terms of the agreement, CRF will provide 4DMT funding to deploy 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 will be the basis of a 4DMT experimental gene therapeutic that will be evaluated and developed by 4DMT in close collaboration with the CRF.

"We believe that 4D's Therapeutic Vector Evolution approach to AAV vector design, which creates a vector with the ability to successfully penetrate the retina via intravitreal delivery, rather than subretinal injection, can potentially change the future of Choroideremia gene therapy," said Christopher Moen, MD, President of the CRF. "The outcomes of this collaboration may offer tremendous benefit to our patient community, and our unique partnership can serve as a model for other rare disease groups."

"CRF brings their crucial clinical expertise, network of doctors, trial investigators and patients together to inform the best possible product design, clinical trial design, and trial execution for this important program," said David Kirn, MD, co-founder and CEO of 4DMT. "Teaming up with CRF allows us to pursue our vision of providing the best gene therapy products to patients who truly need them."

Further terms of the agreement were not disclosed.

About Choroideremia

A rare, inherited form of blindness, Choroideremia is an x-linked retinal disease that begins as night blindness in childhood and progresses to complete blindness. It affects an estimated 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. 4D is creating a diverse and deep product pipeline through partnerships, while progressing internal 4D 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 with unmatched 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 in humans.

###

Contact:

Investors

Peter Rahmer, 646-378-2973

prahmer@troutgroup.com

Media

Sharon Tetlow, 415-515-3902

stetlow@4dmt.com

SHARE Linkedin Facebook