



## 4DMT and ROCHE Expand Ophthalmology Partnership to Develop and Commercialize Multiple AAV Gene Therapy Products

April 30, 2018

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**Emeryville, CA, April 30, 2018** — 4D Molecular Therapeutics (4DMT), a leader in *Therapeutic Vector Evolution* for adeno-associated virus (AAV) gene therapy vector discovery and product development, today announced the expansion of its 2015 research agreement with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc. into a broad long-term partnership to develop and commercialize multiple ophthalmology products.

The expanded agreement will allow each company to leverage its primary strengths to bring highly-optimized gene therapeutics to ophthalmology patients as expeditiously as possible. 4DMT will be responsible for vector discovery and optimization, product design and engineering, pre-clinical and early-stage clinical development, including manufacturing activities, while Roche will conduct pivotal clinical trials and commercialize the new therapeutics globally.

4DMT's intravitreally-delivered choroideremia clinical candidate, 4D-110, is the first collaboration program; IND-enabling studies and activities are underway. Additional clinical candidate development programs are underway to treat retinal diseases with high unmet need.

"Together we elected to expand our partnership after 4DMT completed proprietary intravitreal vector discovery and characterization. We have created clear synergies between 4D's vector discovery, gene therapy development and manufacturing capabilities and Roche's expertise in late-stage clinical development and global biologics commercialization. The decision to expand our partnership represents validation of our ophthalmology platform, clinical candidates and team," said David Kim MD, CEO and co-founder of 4DMT.

"This expanded partnership could prove a major catalyst to bring new therapies for blinding retinal disorders. 4D's gene therapy technology and Roche's expertise in biologics and should be a powerful combination," says Jeffrey S. Heier, MD, Co-President and Medical Director, Ophthalmic Consultants of Boston.

"The expansion of this partnership holds promise for accelerating treatments for our patients with retinal diseases," says Dr. Stephen Rose, Chief Scientific Officer of Foundation Fighting Blindness, positioned as the largest non-governmental/non-commercial supporter of R&D for inherited retinal degenerations.

"The prospect of an intravitreally-delivered gene therapy for choroideremia could potentially benefit the whole population of patients with this condition," explains Ian MacDonald MD, Professor in the Department of Ophthalmology and Visual Sciences, University of Alberta.

Affecting approximately 200,000 patients in the US and similar numbers in Europe, inherited retinal diseases are, collectively, a major cause of adult and childhood blindness. Mutations in more than 200 genes are known to cause these rare, orphan conditions for which there are currently few approved therapies. More common diseases of the retina affect several million patients in the US and EU.

### About 4D Molecular Therapeutics (4DMT)

4DMT is focused on the discovery and development of targeted and proprietary AAV gene therapy 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 specifically to any tissue or organ in the body, by optimal clinical routes of administration, at manageable doses and with resistance to pre-existing antibodies. These proprietary and targeted products may be developed to treat both rare genetic diseases and large market complex diseases. 4DMT is creating a diverse and deep product pipeline through its own internal 4D products, as well as through partnered programs.

4DMT patient advocacy organization partners in ophthalmology include Foundation Fighting Blindness and Choroideremia Research Foundation.

### About 4DMT's Therapeutic Vector Evolution

Gene therapy has shown promise for the treatment of rare diseases, yet current clinical stage gene therapy products are not targeted and are generally based on one of a few AAV vectors that are "naturally-occurring" or "wild-type", meaning they were found in nature (e.g. as laboratory contaminants or as monkey infections). These first-generation AAV vectors, while generally safe and well-tolerated in patients, do not have targeted or optimized delivery properties and often require aggressive and/or invasive injection at high doses to attempt the desired transduction of target cells in the body. 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, at manageable doses and with resistance to pre-existing antibodies in the population. Our *Therapeutic Vector Evolution* platform deploys an estimated 100 million unique AAV variants with extensive diversity, from over 35 unique and proprietary 4DMT AAV vector libraries. After defining the Target Product Profile, and the associated Target Vector Profile, 4DMT then applies proprietary methods to identify lead vectors for the specific Target Vector Profile from within our AAV libraries. The result is a customized, novel, and proprietary pharmaceutical-grade product uniquely designed for targeted therapeutic gene delivery and efficacy in humans.

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