



## 4D Molecular Therapeutics Announces Presentation of Preclinical Data at the 6th International Update on Fabry Disease and Provides Clinical Update

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– Novel vector, 4D-C102, demonstrates superior performance compared to commonly used AAV1, AAV8, and AAV9 –

– Fabry product candidate, 4D-310, is on track for anticipated clinical trial initiation in 2020 –

- Superior transduction of 4DMT's novel vector, 4D-C102, in human cardiomyocytes *in vitro* as compared to AAV1, AAV8 and AAV9
- Dose dependent GLA expression and function after administration of 4D-310, that incorporates the 4D-C102 vector, *in vitro* in Fabry patient fibroblasts
- Superior delivery of the 4D-C102 vector in non-human primate cardiac tissue *in vivo* as compared to AAV8 and AAV9

**Emeryville, CA – May 30, 2019** – 4D Molecular Therapeutics, Inc. (4DMT), a leader in the discovery and development of targeted, customized and proprietary next-generation adeno-associated virus (AAV) vectors and gene therapy products, announced new preclinical data with its novel vector, 4D-C102, broadly for neuromuscular diseases and specifically for the product candidate incorporating 4D-C102 for Fabry Disease, 4D-310. The data was highlighted in a poster presentation at the 6<sup>th</sup> International Update on Fabry Disease held May 26 to 28, 2019 in Prague, Czech Republic.

The data presented demonstrated:

- Superior transduction of 4DMT's novel vector, 4D-C102, in human cardiomyocytes *in vitro* as compared to AAV1, AAV8 and AAV9
- Dose dependent GLA expression and function after administration of 4D-310, that incorporates the 4D-C102 vector, *in vitro* in Fabry patient fibroblasts
- Superior delivery of the 4D-C102 vector in non-human primate cardiac tissue *in vivo* as compared to AAV8 and AAV9

Fabry disease is a rare, X-linked, monogenic disorder caused by mutations in the GLA gene that results in storage and accumulation of lipids and leads to debilitating effects on a wide range of organs and systems, including the heart. Current enzyme replacement therapy partially clears accumulated lipids from the endothelial cells of affected organs; however, clearance in other cell types, including cardiomyocytes, appears incomplete. As a result, heart disease is the most common cause of mortality in these patients. 4DMT's Fabry product candidate, 4D-310, aims to preferentially express the GLA enzyme in cardiomyocytes, in addition to other tissues including kidney and liver, to directly address the cause of Fabry-related heart disease.

"The data generated using the novel vector, 4D-C102, and the 4D-310 Fabry product candidate using that vector, helps validate 4DMT's Therapeutic Vector Evolution approach for *in vivo* cardiac tissue targeting. This data package also provides the basis of a preclinical data package that we believe will enable commencement of clinical development of our Fabry product candidate," said Dr. Gabriel Brooks, VP Clinical Research and Development, and Program Leader, Neuromuscular and LSD Therapeutic Areas at 4DMT.

"4DMT is building upon our Therapeutic Vector Evolution platform with the goal of ultimately bringing gene therapy products to patients with severe genetic diseases. We are pleased to present these data as an early demonstration of our platform's ability to potentially address neuromuscular diseases," said Dr. Peter Francis, Chief Medical Officer at 4DMT.

### About 4D Molecular Therapeutics

A copy of the poster presentation can be found in the "Investors & News" section of the company website, under "Events & Presentations": [www.4dmolecularterapeutics.com](http://www.4dmolecularterapeutics.com)

### About 4DMT's Therapeutic Vector Evolution

4DMT is advancing the field of targeted and optimized AAV vector technology by deploying principles of evolution and natural selection to create vectors that are designed to 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. 4DMT's Therapeutic Vector Evolution platform can deploy over 35 unique and proprietary AAV libraries comprised of an estimated 1 billion vector capsid sequences. After defining the target product profile, and the associated target vector profile, 4DMT then applies proprietary methods to identify lead vectors from within our AAV libraries. The result is a customized, novel, and proprietary pharmaceutical-grade product candidate designed for targeted therapeutic gene delivery and efficacy in humans.

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