



## 4D Molecular Therapeutics Announces Upcoming Oral and Poster Presentations at the 23rd Annual Meeting of the American Society of Gene and Cell Therapy

April 29, 2020

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– Oral presentations to include data on 4D-310 Product Candidate for Fabry Disease and 4D-A101 Aerosol Vector for Cystic Fibrosis and other Lung Diseases

– Additional poster presentations to include data on 4D-C102 IV Low Dose Vector for Lysosomal Storage Diseases and Neuromuscular Diseases

EMERYVILLE, CA. – April 29, 2020 – 4D Molecular Therapeutics Inc. (4DMT), a leader in precision-guided AAV gene medicines based on directed evolution, today announced the company will present two oral and two poster presentations at the American Society of Gene and Cell Therapy's 23rd Annual Meeting, to be held virtually May 12-15, 2020.

These data will highlight the following preclinical evidence: safety and efficacy of the company's product candidate 4D-310 in both human cell (in vitro) and animal (in vivo) models of Fabry disease; diffuse payload delivery and gene expression throughout the primate lung with the proprietary aerosol-delivered vector 4D-A101; diffuse payload delivery and gene expression throughout primate cardiac and skeletal muscle tissue with the proprietary low dose IV vector 4D-C102; improved targeting of cardiac and skeletal muscle tissue versus the liver in primates with low dose 4D-C102 as compared to conventional AAV vectors; improved transduction of human cardiomyocytes and skeletal muscle cells with 4D-C102 versus conventional AAV vectors.

"We are looking forward to sharing these important advances for our differentiated product candidates and industrialized directed evolution-based vector discovery platform. These data add to the growing body of evidence demonstrating our proprietary vectors solve the key challenges facing conventional AAV vectors that are in clinical and pre-clinical development today," said David Kirn, MD, CEO and Chairman of 4DMT.

### Oral Presentations

#### **A Highly-Evolved Novel AAV Gene Therapy Directly Addresses Fabry Disease Pathology In Vivo by Cell Autonomous Expression in the Heart and Other Target Organs**

*Date & Time:* Tuesday May 12, 2020 5:15 PM – 5:30 PM ET

*Abstract number:* 140

*Session title:* AAV Vectors Preclinical and Proof-of-Concept Studies in Optimizing the Toolbox

#### **Directed Evolution of AAV Targeting Lung Epithelia Using Aerosol Delivery Identifies 4D-A101, a Variant Demonstrating Robust Gene Delivery in Non-Human Primates**

*Date & Time:* Friday May 15, 2020 11:00 AM – 11:15 AM ET

*Abstract number:* 1336

*Session title:* Cardiovascular and Pulmonary Diseases

### Poster Presentations:

#### **In Vitro Fabry Disease Correction in Patient iPSC-Derived Cardiomyocytes and Endothelial Cells Using an Evolved and Optimized AAV Gene Therapeutic (4D-310)**

*Date & Time:* Wednesday May 13, 2020 5:30 PM – 6:30 PM

*Abstract number:* 594

*Session Title:* AAV Vectors – Preclinical and Proof-of-Concept Studies

#### **4D-C102, a Novel Muscle-Tropic AAV Variant Demonstrates Superior Gene Delivery in Cardiac and Skeletal Muscle Tissues Versus Wild-Type AAV in Human Cells and Non-Human Primates**

*Date & Time:* Tuesday May 12, 2020 5:30 PM – 6:30 PM

*Abstract number:* 143

*Session Title:* AAV Vectors – Virology and Vectorology

Abstracts with more details may now be accessed by visiting the ASGCT Annual Meeting's website (<https://annualmeeting.asgct.org>). Shortly after the conference, the presented materials will also be published to the 4DMT website (<https://www.4dmoleculartherapeutics.com>).

### About 4DMT

4DMT is a precision gene medicines 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 allowing customization of AAV vectors to target specific tissue types associated with the underlying disease. These proprietary AAV vectors are designed to provide targeted delivery by routine clinical routes, 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 AAVs. 4DMT vectors are designed to exhibit improved therapeutic profiles that enable the company to pursue previously untreatable diseases and to address a broad range of rare and large market diseases.

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