

NEWS RELEASE

Dimension Therapeutics to Develop Gene Therapy Treatments for Patients with Ornithine Transcarbamylase (OTC) Deficiency and Glycogen Storage Disease Type Ia (GSDIa)

Severe Forms of These Diseases Have Devastating Consequences for Patients and Families, with Few or No Effective Treatment Options

CAMBRIDGE, MASS., April 23, 2015 – Dimension Therapeutics, Inc. (“Dimension” or the “Company”), a rare disease company advancing novel, liver-directed treatments for diverse genetic disorders, today announced that the company is advancing programs to treat two rare diseases with often devastating outcomes for patients and their families - Ornithine Transcarbamylase (OTC) deficiency and Glycogen Storage Disease Type Ia (GSDIa). Both diseases, due to genetically inherited inborn errors of metabolism, usually present in the neonatal period or infancy, and can have grave effects on patients of all ages. For severe forms, there are few or no effective treatment options other than liver transplantation, leaving patients with significant unmet needs. Dimension is developing novel gene therapy approaches to replace the missing or faulty gene, correcting the underlying defect to restore metabolic function.

“In an important step for both patients and for Dimension, we are launching our first two liver-associated, metabolic disease programs to address rare disorders with the greatest needs and fewest treatment options, other than liver transplant. Transplants are often associated with significant risks both during the procedure and over the long term,” said Dimension’s CEO Annalisa Jenkins, MBBS, MRCP. “Our mission – working in collaboration with patient advocacy communities and with the world’s leading clinical investigators and advisors – is to rapidly and responsibly develop new therapies that can offer hope and better health to those patients in the greatest need.”

Dimension looks forward to continuing the work previously championed by advocacy organizations, including The Children’s Fund for GSD Research, which has funded much of the existing preclinical work in GSDIa. Eric Crombez, MD, Dimension’s Chief Medical Officer, commented, “Dimension is developing and strengthening partnerships with the patient communities, families, and physicians focused on improving the lives of patients affected by OTC deficiency and GSDIa. We will ensure that patient organizations, as key partners, will play an active role in developing these clinical programs.” The company anticipates commencing clinical trials in both patient populations in 2016.

Mark L. Batshaw, MD, Principal Investigator of the Urea Cycle Disorders Consortium (UCDC) and Physician-in-Chief of Children’s National Health System, stated, “In order to solve the most pressing issues in pediatric medicine, we encourage diverse constituencies to come together in a true spirit of collaboration. This approach has been critical to the success of programs at the UCDC, and we believe it will be paramount in addressing the needs of patients and families affected by OTC deficiency.”

David A. Weinstein, MD, MMSc, Director, Glycogen Storage Disease Program at the University of Florida, noted, “Our research program, the largest of its kind pursuing treatments for GSDIa, has dedicated substantial resources to enabling gene therapy, which we ultimately believe will be the most promising approach to long-term care of

patients with GSDIa. New therapies are critically needed, and I look forward to Dimension's progress in advancing its gene therapy program into the clinic."

Dimension's Adeno-Associated Virus (AAV) Vector Platform

Dimension is advancing therapies based on a next-generation AAV platform that has demonstrated early validation and compelling preclinical *in vivo* proof of concept across multiple disease states in leading academic research centers. Dimension's current efforts focus on AAV8 and AAVrh10, two forms of AAV vectors that selectively target liver cells and have been optimized to deliver missing intact genes in diseases associated with the liver. Initial indications of positive net benefit of this approach in patients living with hemophilia B, plus demonstration of robust gene expression in multiple preclinical *in vivo* models and commercially viable novel CMC approaches, provide a strong foundation for Dimension's proprietary programs.

Background on OTC Deficiency and GSDIa

OTC deficiency, the most common urea cycle disorder, is caused by a genetic defect in a liver enzyme responsible for detoxification of ammonia. Individuals with OTC deficiency can build-up excessive levels of ammonia in their blood, potentially resulting in neurological deficits and other toxicities. Neonatal onset disease in males is severe and can be fatal at an early age. The greatest percentage of patients experience late-onset disease, representing a clinical spectrum of disease severity. It is estimated that more than 10,000 patients are affected by OTC deficiency worldwide. Currently, the only curative approach is liver transplantation.

GSDIa, the most common glycogen storage disease, has significant unmet needs. Patients have a defective gene for the enzyme glucose-6-phosphatase, resulting in the inability to regulate blood sugar (glucose). If chronically untreated, patients develop severe lactic acidosis, progress to renal failure, and die in infancy or childhood. There are no approved pharmacological therapies, and no ongoing clinical development beyond dietary approaches. An estimated 6,000 or more patients are affected by GSDIa worldwide.

About Dimension Therapeutics

Dimension Therapeutics, Inc., is a rare disease company focused on developing novel treatments for devastating disorders associated with the liver and based on an industry-leading, extensively validated adeno-associated virus (AAV) platform. The company is advancing multiple programs toward clinical development, including: programs addressing unmet needs for patients suffering from OTC deficiency and GSDIa; a collaboration with Bayer HealthCare in hemophilia A, and a wholly owned program in hemophilia B, which is expected to enter clinical testing in the second half of 2015. Dimension has preferred access to multiple best-in-class AAV vectors from REGENXBIO, which founded Dimension with Fidelity Biosciences in October 2013. The Dimension team and senior advisors include biotech industry veterans and renowned thought leaders in gene therapy and rare diseases. For more information, please visit www.dimensiontx.com.

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