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Abeona Therapeutics Doses First Patient in Phase 1/2 Trial With ABO-102 Gene Therapy for Patients With Sanfilippo Syndrome Type A (MPS IIIA)

First-In-Man Gene Therapy Delivering AAV by Single Intravenous Injection to Treat Central Nervous System and Peripheral Manifestations of Disease

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 05/17/16 -- Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing products for severe and life-threatening rare genetic diseases, announced today that the first patient in a Phase 1/2 trial for ABO-102 (AAV-SGSH), a single treatment gene therapy strategy for patients with Sanfilippo syndrome type A (a.k.a Mucopolysaccharidosis Type IIIA or MPS IIIA) has been enrolled at Nationwide Children's Hospital (Columbus, Ohio).

"MPS IIIA is a profound and deadly lysosomal storage disease with no approved treatments available. Caused by a single gene defect, most of the children with this disease do not reach adulthood. This investigational gene therapy approach, delivered as a single intravenous injection to treat the whole body, represents a new treatment paradigm for addressing this relentlessly progressing disease," noted Kevin M. Flanigan, MD, principal investigator with the Center for Gene Therapy at Nationwide Children's Hospital and Professor of Pediatrics and Neurology at The Ohio State University College of Medicine. "We are grateful to the many patient foundations and parents who have supported the research needed to advance a potential treatment for this devastating unmet medical need."

"This treatment trial is the culmination of more than eighteen years of research and development, and none of it would have been possible without support from the Sanfilippo foundations," say Drs. Haiyan Fu and Doug McCarty, who lead the lysosomal disease basic research program in the Center for Gene Therapy.

The company previously announced that ABO-102 has been granted Orphan Product Designation in the USA and received the Rare Pediatric Disease Designation.

The clinical study is supported by neurocognitive testing data generated in a 25-subject MPS III Natural History Study, where all patients are through 1 year of follow up assessments.

"Abeona has demonstrated global leadership in supporting clinical and commercial development of promising gene therapies for children with Sanfilippo syndrome, and we applaud their efforts to bring these potential treatments to clinical trials," said Kathleen Buckley, President of Team Sanfilippo.

"This first-in-man gene therapy, which uses an AAV delivered by a single intravenous injection to treat the central nervous system and the peripheral disease manifestations in Sanfilippo patients, helps advance the field of gene therapy with new treatment options for these devastating diseases," noted Timothy J. Miller, PhD, Abeona's President and CEO.

About ABO-102 (AAV-SGSH): ABO-102 is an adeno-associated viral (AAV)-based gene therapy for MPS IIIA (Sanfilippo syndrome), which involves a one-time delivery of a normal copy of the defective gene to cells of the central nervous system (CNS) with the aim of reversing the effects of the genetic errors that cause the disease. After a single dose in Sanfilippo preclinical models, ABO-102 induced cells in the CNS and peripheral organs to produce the missing enzyme repaired the underlying cell pathology that is the cause of the disease. Preclinical in vivo efficacy studies in Sanfilippo syndrome have demonstrated functional benefits that are sustained for months to years after treatment. A single dose of ABO-102 significantly restored normal cell and organ function, corrected cognitive deficits, increased neuromuscular function and normalized the lifespan of animals with MPS IIIA over 100% for more than one year after treatment compared to untreated control animals. These results are consistent with studies from several laboratories suggesting AAV treatment to replace the defective gene could potentially benefit patients with Sanfilippo syndrome. In addition, safety studies conducted in animal models of Sanfilippo syndrome have demonstrated that delivery of ABO-102 is well tolerated with minimal side effects.

About Abeona: Abeona Therapeutics Inc. is a clinical stage company developing gene therapy and plasma-based products for severe and life-threatening rare genetic diseases. Abeona's lead programs are ABO-102 (AAV-SGSH) and ABO-101 (AAV-NAGLU), adeno-associated virus (AAV) based gene therapies for Sanfilippo syndrome (MPS IIIA and IIIB), respectively. We are also developing ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JBD); and ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder using a novel CRISPR/Cas9-based gene editing approach to gene therapy program for rare blood diseases. In addition, Abeona is developing plasma protein therapies, including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD, using our proprietary SDF™ (Salt Diafiltration) ethanol-free process. For more information, visit www.abeonatherapeutics.com.

About Nationwide Children's Hospital: Ranked 7th of only 10 children's hospitals on U.S. News & World Report's 2014-15 "America's Best Children's Hospitals Honor Roll" and among the Top 10 on Parents magazine's 2013 "Best Children's Hospitals" list, Nationwide Children's is one of the nation's largest not-for-profit freestanding pediatric healthcare networks providing care for infants, children and adolescents as well as adult patients with congenital disease. As home to the Department of Pediatrics of The Ohio State University College of Medicine, Nationwide Children's faculty train the next generation of pediatricians, scientists and pediatric specialists. The Research Institute at Nationwide Children's is one of the Top 10 National Institutes of Health-funded free-standing pediatric research facilities in the U.S., supporting basic, clinical, translational and health services research at Nationwide Children's. The Research Institute encompasses three research facilities totaling 525,000 square feet dedicated to research. More information is available at NationwideChildrens.org/Research.

This press release contains certain statements that are forward-looking within the meaning of Section 27a of the Securities Act of 1933, as amended, and that involve risks and uncertainties. These statements include, without limitation, our plans for continued

development and internationalization of our clinical programs, management plans for the Company, and general business outlook. These statements are subject to numerous risks and uncertainties, including but not limited to continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the impact of competition; the ability to develop our products and technologies; the ability to achieve or obtain necessary regulatory approvals; the impact of changes in the financial markets and global economic conditions; and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and other reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligations to make any revisions to the forward-looking statements contained in this release or to update them to reflect events or circumstances occurring after the date of this release, whether as a result of new information, future developments or otherwise.

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