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Abeona Therapeutics Provides Update on Initial Subjects in Sanfilippo Type A Gene Therapy Trial, Demonstrating Encouraging Early Biopotency Signals

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 08/02/16 -- Abeona Therapeutics Inc. (NASDAQ: ABEO)

- ***ABO-102 (AAV-SGSH) delivers first-in-man AAV-based gene therapy delivered by single intravenous injection to treat central nervous system and peripheral manifestations of MPS IIIA***
- ***Encouraging safety profile and early biopotency levels enables additional patient accrual***
- ***Additional global studies to commence in Europe and Australasia in 2016***

Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing therapies for severe and life-threatening rare genetic diseases, provided today an update on its ongoing Phase 1/2 trial for ABO-102 (AAV-SGSH), a single treatment gene therapy strategy for patients with MPS IIIA (Sanfilippo syndrome type A), enrolling at Nationwide Children's Hospital (Columbus, Ohio). Following a review of safety data by independent Data Safety and Monitoring Board (DSMB) on the initial patient enrolled, a second patient has been enrolled and treated. Additionally, preliminary measures of clinically relevant biomarkers provide promising signals of potential systemic and CNS clinical benefits for patients suffering with MPS IIIA.

MPS IIIA, or Sanfilippo Syndrome Type A, a rare autosomal recessive disease, is caused by genetic mutations that result in a deficiency of SGSH enzyme activity, leading to abnormal accumulation of GAG (specifically, heparan sulfate) in the CNS and systemic tissues and organs. This accumulation of heparan sulfate results in neurocognitive decline, speech loss, loss of mobility, and premature death.

ABO-102, the company's first-in-human, intravenously-administered AAV gene therapy to date, has been well tolerated in both subjects with no safety or tolerability concerns identified through 30 day post-injection. Encouraging signs of early biopotency have been observed in urinary and CSF GAG (glycosaminoglycan, specifically, heparan sulfate) measurements, as well as potential disease-modifying effects in the liver and spleen. The clinical study is supported by neurocognitive evaluations, biochemical assessments and MRI data generated in a 25-subject MPS III Natural History Study, also conducted at Nationwide Children's Hospital, where patients continued through one-year of follow up assessments.

"MPS IIIA is a devastating and progressive lysosomal storage disease with no approved

treatment options. We are encouraged that initial data suggest that the ABO-102 is well tolerated thus far," stated 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. "Early biopotency signals suggest a reduction of urinary and CSF GAG, and if confirmed will suggest that intravenous administration of ABO-102 may ultimately provide both CNS and systemic benefits to patients."

Abeona previously announced that its MPS IIIA program, ABO-102, has been granted Orphan Product Designation in the USA and received the Rare Pediatric Disease Designation. Abeona plans to open two additional clinical sites to test ABO-102, one in Spain and one in Australia in 2016.

"These early results continue to support Abeona's unique approach to treating patients with lysosomal storage diseases, where there are profound whole-body manifestations of the disease," stated Tim Miller, PhD., President & CEO. "We are very encouraged that ABO-102 appears well-tolerated in patients, and that early signals of GAG (HS) reduction in the urine and CSF demonstrate penetration through the blood-brain barrier. Importantly, these early signals are directionally consistent with data seen in pre-clinical models of the MPS IIIA disease."

"While data from the ABO-102 study are still very early, we are pleased that the gene therapy appears to be well tolerated, and that initial biopotency measures show signals of clinically relevant and important activity," stated Steven H. Rouhandeh, Executive Chairman. "As we continue to build out our orphan drug portfolio and move additional programs into the clinic, we look forward to gaining further clarity on the therapeutic potential for ABO-102 as we continue enrolling patients and obtaining potency and clinical measures over the coming months."

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 therapies 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:
Named to the Top 10 Honor Roll on *U.S. News & World* Report's 2016-17 list of "America's Best Children's Hospitals," Nationwide Children's Hospital is one of America's largest not-for-profit freestanding pediatric healthcare systems providing wellness, preventive, diagnostic, treatment and rehabilitative 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 Hospital 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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