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Abeona Therapeutics Announces FDA Allowance of Investigational New Drug (IND) for Phase 1/2 Clinical Study With ABO-101 Gene Therapy for Patients With Sanfilippo Syndrome Type B (MPS IIIB)

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 05/24/16 -- Abeona Therapeutics Inc. (NASDAQ: ABEO)

- ABO-101 is Abeona's second AAV gene therapy to commence clinical trials in a rare CNS disease
- The clinical trial is a Phase 1/2 dose escalation study of ABO-101 in 6-9 patients
- A Natural History Study in 25 patients has established efficacy outcome measures
- FDA previously granted both Orphan Drug and Rare Pediatric Disease Designations

Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on delivering gene therapy and plasma-based products for severe and life-threatening rare diseases, today announced the FDA has allowed an Investigational New Drug (IND) Application for its Phase 1/2 Clinical Study with gene therapy candidate ABO-101 (AAV-NAGLU) for patients with Sanfilippo syndrome type B (MPS IIIB) to be conducted at Nationwide Children's Hospital (Columbus, OH). This is the second FDA allowance for a gene therapy trial from Abeona this year, following allowance of an IND in February for ABO-102, for patients with MPS IIIA which commenced with dosing of the first cohort of patients this month.

"We're very excited to bring decades of research into clinical trials for this unmet clinical need," noted Kevin M. Flanigan, M.D., principal investigator with the Center for Gene Therapy at Nationwide Children's and Professor of Pediatrics and Neurology at The Ohio State University College of Medicine. "As seen in other gene therapy trials, using AAV9 delivered by intravenous injection has strong potential to treat patients with Sanfilippo type B, a disease with profound central nervous system manifestations."

"Abeona is committed to building a leadership position in the development of innovative treatments for orphan diseases, and this second FDA IND allowance represents an important milestone in advancing our rare disease product pipeline to the clinic," stated Steven H. Rouhandeh, Executive Chairman of Abeona. "We remain active from a corporate development perspective in identifying complementary orphan drug programs as well as potential co-development partners, towards our goal of achieving value for our patients, their families, our academic partners and other stakeholders."

A Natural History Study for Sanfilippo, conducted by Nationwide Children's Hospital, has been completed in 25 patients to characterize how the rare disease progresses in its natural state. Information about a rare disease's natural history aids in clinical trial design, identifying study end points and developing and validating clinical outcome measures, and biomarkers.

"ABO-101 is Abeona's second AAV-based gene therapy program to advance to clinical trials. This first-in-man Phase 1/2 clinical trial is delivered by a single intravenous injection to treat the brain and peripheral manifestations of Sanfilippo. In pre-clinical models, an injection of ABO-101 restored the NAGLU enzyme activity in the cerebral spinal fluid and serum, and also corrected the lysosomal storage pathology throughout the CNS and in widespread somatic organs," noted Timothy J. Miller, Ph.D., President & CEO. "A single injection also led to the correction of astrocytosis and neurodegeneration, hallmarks of secondary damage in the central nervous system in MPS IIIB. Importantly, intravenous gene delivery improved both cognitive and motor functions, as well as extended survival in preclinical models. We are very encouraged by the results seen in other clinical trials and look forward to further building on the positive results seen in previous studies. Given the high level of need for therapies for Sanfilippo syndromes, we remain committed to advancing both our AAV-based gene therapy programs, ABO-101 and ABO-102, targeting two types of the inherited genetic disease, MPS IIIB and MPS IIIA."

"The collective efforts of multiple foundations have led us to this great achievement, and we are grateful for the groundbreaking research of Drs. McCarty and Fu. We thank Nationwide and Abeona for helping advance potential MPS IIIB gene therapies for our kids," said Susan Wilson, Director of The Children's Medical Research Foundation.

"This milestone is the culmination of more than a decade of research," said Haiyan Fu, Ph.D., the developer of the gene therapy with Doug McCarty, Ph.D. both principal investigators at Nationwide Children's. "It was achieved through the support of a translational research grant from the National Institutes of Neurologic Disease and Stroke. In addition to the NINDS, our entire team would like to thank many patient and family foundations for their longstanding support and financial commitment to advancing research and developing treatments for this heartbreaking disease."

About Sanfilippo syndromes: Sanfilippo syndromes (or mucopolysaccharidosis (MPS) type III) are a group of four inherited genetic diseases each caused by a single gene defect, described as type A, B, C or D, which cause enzyme deficiencies that result in the abnormal accumulation of glycosaminoglycans (sugars) in body tissues. MPS III is a lysosomal storage disease, a group of rare inborn errors of metabolism resulting from deficiency in normal lysosomal function. The incidence of MPS III (all four types combined) is estimated to be 1 in 70,000 births. Mucopolysaccharides are long chains of sugar molecule used in the building of connective tissues in the body. There is a continuous process in the body of replacing used materials and breaking them down for disposal. Children with MPS III are missing an enzyme which is essential in breaking down the used mucopolysaccharides called heparan sulfate. The partially broken down mucopolysaccharides remain stored in cells in the body causing progressive damage. Babies may show little sign of the disease, but as more and more cells become damaged, symptoms start to appear. In MPS III, the predominant symptoms occur due to accumulation within the central nervous system (CNS), including the brain and spinal cord, resulting in cognitive decline, motor dysfunction, and eventual death. Importantly, there is no cure for MPS III and treatments are largely

supportive care.

About ABO-101 (AAV-NAGLU): ABO-101 is next generation adeno-associated viral (AAV)-based gene therapy for MPS III (Sanfilippo syndrome), which involves a one-time delivery of a normal copy of the defective gene to cells of the central nervous system with the aim of reversing the effects of the genetic errors that cause the disease. After a single dose in Sanfilippo preclinical models, ABO-101 induced cells in the CNS and peripheral organs to produce the missing enzymes and help repair damage caused to the cells. Preclinical in-vivo efficacy studies in Sanfilippo syndrome have demonstrated functional benefits that remain for months after treatment. A single dose of ABO-101 significantly restored normal cell and organ function, corrected cognitive defects that remained months after drug administration, increased neuromuscular control and increased the lifespan of animals with MPS III over 100% one year after treatment compared to untreated control animals. These results are consistent with studies from several laboratories suggesting AAV treatment could potentially benefit patients with for Sanfilippo syndrome Type A and B, respectively. In addition, safety studies conducted in animal models of Sanfilippo syndromes have demonstrated that delivery of AB0-101 are well tolerated with minimal side effects.

About Abeona: Abeona Therapeutics Inc. delivers gene therapy and plasma-based products for severe and life-threatening rare diseases. Abeona's lead programs are ABO-101 (AAV-NAGLU) and ABO-102 (AAV-SGSH), adeno-associated virus (AAV)-based gene therapies for Sanfilippo syndrome (MPS IIIB and IIIA). The company is 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 its proprietary SDF™ (Salt Diafiltration) ethanol-free process. For more information, visit www.abeonatherapeutics.com

About The Research Institute at Nationwide Children's Hospital
Ranked 9th of only 12 children's hospitals on U.S. News & World Report's 2015-16 "America's Best Children's Hospitals Honor Roll," Nationwide Children's Hospital 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 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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