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Abeona Therapeutics Announces FDA Allowance of Investigational New Drug (IND) for Systemic AAV Phase 1/2 Clinical Study With ABO-102 Gene Therapy for Patients With Sanfilippo Syndrome Type A (MPS IIIA)

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- Phase 1/2 dose escalation study of ABO-102 in 6-9 patients
- Natural history study in 25 patients establishes baseline for efficacy endpoints
- FDA previously granted both Orphan Drug Designation and Rare Pediatric Disease Designation

Abeona Therapeutics, Inc. (NASDAQ: ABEO), a biopharmaceutical company focused on developing and delivering products for severe and life-threatening rare diseases, today announced the FDA cleared the Company's Investigational New Drug Application for ABO-102 (AAV- SGSH), a single treatment strategy for Mucopolysaccharidosis Type IIIA (MPS IIIA). The ABO-102 IND application is now active and enables Nationwide Children's Hospital (Columbus, OH) to initiate a Phase 1/2 clinical study designed to assess the safety, tolerability and potential efficacy of ABO-102 in children with MPS IIII A.

"MPS IIIA is a devastating lysosomal storage disease that is caused by a single gene defect. The AAV SGSH gene therapy approach developed by Drs. Douglas McCarty and Haiyan Fu at Nationwide Children's, represents a new treatment paradigm for addressing this relentlessly progressing disease with a single, intravenous administration," noted Kevin M. Flanigan, MD, 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. "We would like to thank these dedicated researchers, as well as the many patient foundations, for their dedicated support and commitment to advancing new treatment options forward for this devastating unmet medical need."

"ABO-102 is well positioned to advance gene therapy trials for patients with Sanfilippo syndrome type A, who have profound neurological deficits, as well as numerous peripheral organ manifestations. In pre-clinical studies, a single intravenous injection of ABO-102 restored the SGSH enzyme activity, and corrected the lysosomal storage pathology throughout the CNS and body," noted Timothy Miller, PhD, President & CEO. "The treatment also led to the correction of astrocytosis and neurodegeneration, hallmarks of secondary neuropathology of MPS IIIA. Importantly, intravenous gene delivery of ABO-102 improved

cognitive and motor functions, as well as extended survival in preclinical animal models."

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 molecules 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.

"The progress of ABO-102 into human clinical trials represents a significant milestone in advancing our rare disease product pipeline towards achieving value for patients and their families, as well as shareholders," stated Steven Rouhandeh, Executive Chairman.

About ABO-102 (AAV SGSH): ABO-102 is a next generation 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 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-102 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 IIIA 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 AB0-102 are well tolerated with minimal side effects.

About Abeona: Abeona Therapeutics, Inc. develops and 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). 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, we are also developing rare 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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