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Abeona Therapeutics Announces Initial European Regulatory Approvals for Phase 1/2 Gene Therapy Clinical Studies for Patients With Sanfilippo Syndromes Type A (MPS IIIA) and Type B (MPS IIIB)

NEW YORK, NY, and CLEVELAND, OH -- (Marketwired) -- 01/11/16 --

- Approval of Genetically Modified Organism (GMO) and Ethical Committee (CEIC) regulatory filings for both ABO-101 (AAV-NAGLU) and ABO-102 (AAV-SGSH) in Spain
- Phase 1/2 dose escalation studies of ABO-101 and ABO-102 in Sanfilippo syndrome types A and B
- Natural history study in 25 patients established efficacy endpoints

Abeona Therapeutics, Inc. (NASDAQ: ABEO), a biopharmaceutical company focused on developing and delivering gene therapy and plasma-based products for severe and life-threatening rare diseases, today announced the Interministerial Council of Genetically Modified Organisms has approved the Genetically Modified Organism (GMO) Voluntary Release regulatory filings for both Phase 1/2 Gene Therapy Clinical Studies to treat patients with AB0-101 (AAV NAGLU) and ABO-102 (AAV SGSH) for patients with Sanfilippo syndrome type A (MPS IIIA) or type B (MPS IIIB). Additionally, the Comite Etico De Investigacion Clinica de Euskadi (CEIC-E) has approved the ethical committee regulatory filings for both ABO-101 and ABO-102. The Company plans to file CTAs for both programs shortly for the upcoming clinical studies to be conducted at Cruces University Hospital (Bilbao, Spain).

"The GMO and Ethical committee approvals -- which are similar to the Recombinant DNA Advisory Committee (RAC) and hospital Institutional Review Board processes in the United States -- support our planned path to clinical trials in the EU for the treatment of Sanfilippo syndromes type A and B. Pre-clinical studies have demonstrated that a single intravenous injection of ABO-101 or ABO-102 restored the enzyme activity in the cerebral spinal fluid and serum, and also corrected the lysosomal storage pathology throughout the CNS and body," noted Tim Miller, PhD, President & CEO. "We look forward to advancing these potentially life-changing therapies into global clinical trials and grateful for the support and guidance from regulators, clinicians, and patient community."

"Sanfilippo syndromes type A and B are devastating lysosomal storage diseases that affect children around the world. These gene therapies, delivered as a single, non-invasive intravenous injection, offer a new and promising treatment paradigm for patients with this relentless disease," commented Luis Aldámiz-Echevarría, M.D., PhD., Principal Investigator

of the clinical trials and Associate Professor in the Faculty of Medicine at the University of the Basque Country (Spain), Paediatrician in the Department of Paediatrics at Cruces University Hospital (Spain) and Principal Investigator of the Metabolic Inherited Disorders Group and the Metabolomics and Proteomics Platform at BioCruces Health Research Institute (Spain).

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) and ABO-102 (AAV SGSH) arenext generation adeno-associated viral (AAV)-based gene therapies for MPS IIIA and MPS IIIB, respectively. These gene therapies involve a one-time delivery of a genetically modified virus to deliver a normal copy of the defective gene to cells of the central nervous system and peripheral organs with the aim of reversing the effects of the genetic errors that cause the disease. After a single dose in preclinical animal models of Sanfilippo syndrome, ABO-101 and 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 animals with Sanfilippo syndrome have demonstrated functional benefits that remain for months after treatment. A single dose 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 and ABO-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 AB0-101 (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.

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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