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# Abeona Therapeutics Announces European Regulatory Approval for Phase 1/2 Gene Therapy Clinical Study for Patients With Sanfilippo Syndrome Type A (MPS IIIA)

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Abeona Therapeutics, Inc. (NASDAQ: ABEO)

- Approval of clinical trial for ABO-102 (AAV-SGSH) in European Union marks 3rd regulatory approval for the company, 2nd for ABO-102
- Approval following encouraging safety profile and early biopotency signals observed in 2 low dose patients through 30 Days post-injection in ongoing US trial

Abeona Therapeutics, Inc. (NASDAQ: ABEO), a leading clinical-stage biopharmaceutical company focused on delivering gene and plasma-therapies for life-threatening rare diseases, today announced European regulatory approval for a Phase 1/2 Gene Therapy Clinical Trial utilizing ABO-102 (AAV-SGSH) for patients with Sanfilippo syndrome type A (MPS IIIA). The clinical study was approved by the Agencia Espanola de Medicamentos y Productos Sanitarios, and the Company is conducting the Phase 1/2 clinical study at Cruces University Hospital (Bilbao, Spain).

"We are encouraged by the recently reported early clinical data suggesting ABO-102 is well tolerated, with early biopotency signals showing reduced urinary and CSF GAG (heparan sulfate). This is the second clinical trial approval supporting the advancement of ABO-102 as a potential treatment for patients with Sanfilippo syndrome type A, or MPS IIIA. We thank the foundations and regulatory agencies for helping advance these potentially life-changing therapies into global clinical trials," stated Timothy J. Miller, Ph.D, President & CEO.

The clinical study is supported by a 25-subject MPS III Natural History Study, which included potential efficacy assessments consisting of neurocognitive evaluations, biochemical assays and MRI data generated over 1 year of follow up assessments.

"Sanfilippo syndromes are devastating and progressive 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).

"Today we consider that a period of hard work ends and a period full of hope for other children affected by Sanfilippo begins. None of this would have been possible without the help of thousands of people who rely on us, so we want to thank the volunteers, all the people that have supported us financially, our scientific committee, the AEMPS, Abeona, the research team that developed this program, and all parents of children affected for their integrity and dedication," said Emilio Lopez Alvarez, President, Stop Sanfilippo Foundation, 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-102 (AAV-SGSH)*** ABO-102 is an adeno-associated viral (AAV)-based gene therapy for patients diagnosed with MPS IIIA (Sanfilippo syndrome). ABO-102 is delivered as a one-time intravenous injection of a normal copy of the defective gene (SGSH) for delivery to cells of the central nervous system (CNS) and peripheral organs with the aim of correcting the underlying genetic errors that cause the disease. In Sanfilippo preclinical models, a single dose of ABO-102 induced cells in the CNS and peripheral organs to produce the missing enzyme, and repaired the underlying cell pathology that causes the disease. 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 SGSH 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. Clinical studies are ongoing and have demonstrated that ABO-102 has a strong safety profile through 30 Days post-injection and early biopotency signals are being observed.

***About Abeona:*** Abeona Therapeutics Inc. is a leading 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.

*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, that AAV treatment to replace the defective SGSH gene could potentially benefit patients with Sanfilippo syndrome, that early clinical data suggest that ABO-102 is well tolerated with early biopotency signals showing reduced urinary and CSF GAG (heparan sulphate), 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 success of our 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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