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Abeona Therapeutics Announces Data Safety Monitoring Board Approves ABO-102 Dose Escalation for Second Cohort in Phase 1/2 Clinical Trial for Sanfilippo Syndrome Type A

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 10/05/16 -- Abeona Therapeutics Inc. (NASDAQ: ABEO)

- *ABO-102 (AAV-SGSH) delivers first-in-man AAV-based gene therapy administered by single intravenous injection to treat CNS and peripheral manifestations of Sanfilippo syndrome type A*
- *Data Safety Monitoring Board (DSMB) approves ABO-102 dose escalation for the high-dose cohort*

Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing gene therapies for life-threatening rare diseases, announced today that the Data Safety Monitoring Board (DSMB), an independent group of medical experts closely monitoring the clinical trial, has reviewed the initial safety data from the low dose cohort (n=3) in the Phase 1/2 clinical trial of ABO-102 (AAV-SGSH) enrolling at Nationwide Children's Hospital in Columbus, Ohio. Following review of the safety data, the DSMB authorized that the clinical trial proceed with enrollment and dose escalation for the second cohort. The high-dose cohort will enroll up to six additional patients dosed at 1.0×10^{13} vg/kg, which is twice the amount of ABO-102 received by patients in the low-dose cohort.

"These early results support Abeona's unique approach to treating patients with Sanfilippo syndrome, where there are both profound CNS and whole body manifestations of the disease," stated Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics. "We look forward to reporting on future progress and potential for ABO-102 as we begin to enroll patients at the high dose and open additional clinical sites internationally."

Abeona's ABO-102 program has been granted both Orphan Product Designation and Rare Pediatric Disease Designation in the USA and plans to open two additional clinical sites, one in Spain and one in Australia, to test ABO-102. A Phase 1/2 clinical study of ABO-102 in Spain was recently approved by the Agencia Espanola de Medicamentos y Productos Sanitarios, and the Company is preparing to conduct this clinical study at Cruces University Hospital in Bilbao, Spain.

Sanfilippo Syndrome Type A, or MPS IIIA, is a rare lysosomal storage disease caused by genetic mutations that result in a deficiency of SGSH enzyme activity, leading to abnormal

accumulation of certain sugars (specifically, the glycosaminoglycan (GAG) heparan sulfate) in the central nervous system (CNS) and systemic tissues and organs. The accumulation of heparan sulfate results in neurocognitive decline, speech loss, loss of mobility, and premature death.

About ABO-102 (AAV-SGSH): ABO-102 is an adeno-associated viral (AAV)-based gene therapy for patients with MPS IIIA (Sanfilippo syndrome), that is delivered as a one-time intravenous injection. ABO-102 delivers a functioning, corrective copy of the SGSH gene to cells of the central nervous system (CNS) and other organs with the goal of correcting the underlying deficits caused by the inborn genetic errors that are the cause the disease. ABO-102 has been well tolerated through 30 day post-injection in subjects injected with the low-dose (n=3). 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 of the initial subjects enrolled and treated in the trial. 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.

About Sanfilippo syndromes (or mucopolysaccharidosis (MPS) type III): 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 (GAGs, or 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. 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 Abeona: Abeona Therapeutics Inc. is a clinical stage company developing gene and plasma-based therapies for 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. Abeona is also developing EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL); ABO-202 (AAV-CLN1) gene therapy for treatment of infantile Batten disease (INCL), and ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, Abeona has a plasma-based protein therapy pipeline, 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 in Spain and Australia; that early results support our unique approach to treating patients with Sanfilippo syndrome; that encouraging signs of early biopotency have been observed as well as potential disease-modifying effects in the liver and spleen of the initial subjects enrolled and treated in the trial; and that ABO-102 is well-tolerated through 30 day post-injection in subjects injected with the low dose; 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 ability to successfully continue 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.

Investor Contact:
Christine Silverstein
Vice President, Investor Relations
Abeona Therapeutics Inc.
+1 (212)-786-6212
csilverstein@abeonatherapeutics.com

Media Contact:
Andre'a Lucca
Vice President, Communications & Operations
Abeona Therapeutics Inc.
+1 (212)-786-6208
alucca@abeonatherapeutics.com

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