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Abeona Therapeutics Completes Enrollment of Low-Dose Cohort for ABO-102 in Phase 1/2 Clinical Trial for MPS IIIA Patients

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 08/29/16 -- Abeona Therapeutics Inc. (NASDAQ: ABEO):

- ***Low-dose cohort (n=3) enrollment completed for ongoing study to evaluate ABO-102 (AAV-SGSH), first-in-man AAV gene therapy by single intravenous injection in patients with MPS IIIA, a rare lysosomal storage disease***
- ***Encouraging safety profile and early biopotency observed in initial subjects***

Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing gene and plasma-based therapies for life-threatening rare genetic diseases, announced today that enrollment has been completed for the low-dose cohort (n=3) in its ongoing Phase 1/2 trial for ABO-102 (AAV-SGSH). The first-in-man clinical trial utilizes a single injection of AAV gene therapy for patients with MPS IIIA (Sanfilippo syndrome type A), a rare autosomal recessive disease that causes neurocognitive decline, speech loss, loss of mobility, and premature death.

"Completing enrollment of the low-dose cohort is an important milestone in the advancement of ABO-102 for patients with Sanfilippo syndrome type A," said Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics. "We look forward to providing an update of topline data from the completed cohort and the anticipated commencement of the high-dose cohort in the coming months."

The company recently announced that ABO-102 is well tolerated in initial subjects and preliminary biopotency appears promising. Abeona's MPS IIIA program, ABO-102, has been granted Orphan Product Designation in the USA and received the Rare Pediatric Disease Designation.

About ABO-102 (AAV-SGSH): ABO-102 is an 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. ABO-102, has been well tolerated in initial subjects of the low-dose cohort with no safety or tolerability concerns identified through 30 day post-injection in patients suffering from MPS IIIA, or Sanfilippo syndrome Type A, a rare autosomal recessive disease, is caused by genetic mutations that result in a deficiency of SGSH enzyme activity, leading to abnormal accumulation of GAG (specifically, heparan sulfate) in the CNS and systemic tissues and organs. This

accumulation of heparan sulfate results in neurocognitive decline, speech loss, loss of mobility, and premature death. 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 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 (JBD); 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, utilizing its 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 we are *looking forward to advancing multiple important new therapeutic candidates for the treatment of epidermolysis bullosa, that we plan to accelerate up to three new promising EB product candidates toward commercialization, that encouraging signs of early biopotency had been observed in urinary and CSF GAG (heparan sulfate) measurements as well as potential disease-modifying effects in the liver and spleen in our ABO-102 program, 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.*

Company Investor & Media Contacts:

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

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

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