October 14, 2016

Abeona Therapeutics to Present Top-Line Data of Low-Dose Cohort for ABO-102 in Phase 1/2 Clinical Trial for MPS IIIA Patients at Upcoming Orphan Drugs and Rare Disease Congress October 19-20th in London, UK

Conference Call to Update Investors on ABO-102 Program During the Conference

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 10/14/16 -- Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing gene therapies for life-threatening rare diseases, announced today that top-line results of the low-dose cohort for ABO-102 in Phase 1/2 clinical trial for Sanfilippo type A (MPS IIIA) patients will be presented at the Orphan Drugs and Rare Disease Congress taking place in London, UK, from October 19th through October 20th. The company's ABO-102 program, enrolling at Nationwide Children's Hospital (Columbus, Ohio), is the first-in-man clinical trial utilizing a single intravenous injection of AAV gene therapy for MPS IIIA, a rare autosomal recessive disease that causes neurocognitive decline, speech loss, loss of mobility, and premature death in children. Abeona management will provide an update on the ABO-102 and other programs on a conference call during the conference.

Abeona Recent Highlights:

- October 5, 2016: announced Data Safety Monitoring Board Approved ABO-102 Dose Escalation for Second Cohort in a Phase 1/2 Clinical Trial for Sanfilippo Syndrome Type A
- September 26, 2016: enrolled First Patient in Phase 2 for EB-101 Gene Therapy Clinical Trial for Epidermolysis Bullosa
- September 21, 2016: announced the exclusive worldwide license of the AIM™ AAV capsid portfolio for next generation gene therapies from University of North Carolina at Chapel Hill
- September 8, 2016: enrolled 5th Patient in Phase 1/2 Gene Therapy Clinical Trial for Epidermolysis Bullosa
- August 29, 2016: completed enrollment of the Low-Dose Cohort for ABO-102 in Phase 1/2 Clinical Trial for MPS IIIA Patients (n = 3)
- August 9, 2016: announced a collaboration with the EB Research Partnership, EB Medical Research Foundation and Stanford University for the development of

treatments for recessive dystrophic epidermolysis bullosa (RDEB)
- August 4, 2016: announced it had received European regulatory approval by the Agencia Espanola de Medicamentos y Productos Sanitarios for a Phase 1/2 trial for ABO-102 (AAV-SGSH) to be conducted at Cruces University Hospital (Bilbao, Spain)
- August 2, 2016: provided an update on the initial subjects enrolled in the ABO-102 clinical trial, stating that ABO-102 had been well tolerated with no safety or tolerability concerns identified through 30-days post-injection, and 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

About ABO-102 (AAV-SGSH): ABO-102, the company's first-in-human, intravenously-administered AAV gene therapy, 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. 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 were evaluated for disease progression over one-year of follow up assessments. 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 version 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.

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 (GAGs) are long chains of sugar molecules used in building 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 of GAGs within the central nervous system (CNS), including the brain and spinal cord, and other tissues which result 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

Source: Abeona Therapeutics Inc.