February 17, 2017

Abeona Therapeutics Provides Update from ABO-102 Phase 1/2 MPS IIIA Clinical Trial at the 13th Annual WORLDSymposium™ 2017

NEW YORK and CLEVELAND, Feb. 17, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq:ABEO):

- *ABO-102 gene therapy well-tolerated in 4 subjects (N=3 low dose, N=1 high dose) through 650 days follow up with no Serious Adverse Events*
- *63% +/- 0.5% central nervous system reduction of heparan sulfate GAG 6 months post-injection (N=2)*
- *Continued evidence of biopotency including reduced liver and spleen volumes and decreased urinary GAGs*
- *Two subjects assessed at the 6-month timepoint showed evidence for stabilization or improvement (average 60% over 2 subjects) in several Mullen subdomains*
- *Adaptive behavior ratings on the Vineland stabilized*
- *Subjects showed improved ability to complete individual items on the Leiter-R non-verbal IQ assessment resulting in improved raw scores*

Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing therapies for life-threatening rare genetic diseases, announced updated data from the ongoing gene therapy clinical trial for Sanfilippo syndrome Type A (MPS IIIA), at the 13th Annual WORLD*Symposium*™ 2017 lysosomal storage disorders conference in San Diego, CA. The ongoing Phase 1/2 trial for ABO-102 (AAV-SGSH) is a first-in-man clinical trial utilizing a single intravenous injection of AAV gene therapy for subjects with MPS IIIA, a rare autosomal recessive disease affecting every cell and organ in the body, which results in neurocognitive decline, speech loss, loss of mobility, and premature death in children.

“We remain encouraged by continued signs of tolerability and biopotency in the low-dose cohort, and enrollment of the high-dose cohort is underway,” stated Kevin M. Flanigan, M.D., principal investigator with the Center for Gene Therapy at Nationwide Children’s Hospital and Professor of Pediatrics and Neurology at The Ohio State University College of Medicine. “Additionally, we are pleased to see further decreases in CSF GAG measurements, as well as preliminary evidence for stabilization or improvement of some cognitive functions, at six months post-dosing.”

Per the design of the clinical trial, subjects received a single, intravenous injection of ABO-102 to deliver the AAV viral vector systematically throughout the body to introduce a corrective copy of the gene that underlies the cause of the MPS IIIA disease. Subjects are

evaluated at multiple time points post-injection for safety assessments and initial signals of biopotency and clinical activity, which suggest that ABO-102 successfully reached target tissues throughout the body, including the central nervous system. Observations reported at the WORLD*Symposium*™ conference included:

- *Safety*: ABO-102 is well-tolerated in subjects injected with the low dose of 5E13 vg/kg ABO-102, with no treatment related adverse events or serious adverse events (SAEs) through over 650 days cumulative post-injection. Enrollment in the high dose cohort has commenced with no Serious Adverse Events (SAEs) reported to date.

- *Biopotency*: As reflected in published natural history studies evaluating MPS III subjects, cerebral spinal fluid (CSF) and urine GAG (heparan sulfate or "HS") are significantly elevated in the subject population as a symptom of disease pathology. As announced previously, all subjects in the low-dose cohort experienced reductions from baseline in CSF HS of 25.6% +/- 0.8%, suggesting ABO-102 crossed the blood brain barrier after intravenous administration. At the six-month follow-up (n=2), CSF HS continued to decrease to 63.1% +/- 0.5% of baseline values, suggesting further improvement in the elimination of the storage pathology. Data presented showed reduction in urinary heparan sulfate and urinary total GAG fragments.

- *Hepatosplenomegaly*: The natural history study in 25 subjects with MPS III (*Truxal et. al., 2016, Mol. Genet. Metab*.) demonstrated that subjects had increased liver and spleen volumes averaging 116% and 88%, respectively at baseline that did not change over a year of follow-up. All three subjects demonstrated significant reductions in liver volumes at 30-days post injection (17.1% +/- 1.9%). At the six-month follow-up in low dose subjects (n=2), this effect was sustained, with a liver volume further decreased by 29.7 – 30.3% and spleen volume by 2.2 – 12.9% from baseline.

- *Cognitive Assessments*: The clinical trial utilizes three validated neurocognitive and behavioral assessment tools, including the Leiter International Performance Scale Third Edition (Leiter-3), the Vineland Adaptive Behavior Scale, Second Edition (Vineland-II) and the Mullen Scale of Early Learning. Cognitive assessments are taken at baseline, and have been taken at the six-month (n=2) and will be taken at the twelve-month follow-up visits. These assessments provide the opportunity to measure several sub-domains, such as fine motor, visual acuity, expressive language, receptive language, among others. Assessments at six-month for the first two lose-dose patients provided early evidence of cognitive stabilization. The two subjects assessed at the 6-month timepoint showed evidence for stabilization or improvement of scores (average of 60% across 2 subjects) in several Mullen subdomains. Adaptive behavior ratings on the Vineland also stabilized. Both subjects showed improved ability to complete individual items on the Leiter-R non-verbal IQ assessment resulting in improved raw scores.

"The data demonstrate an early and robust systemic delivery of ABO-102, and the increased reductions in CNS HS GAG support our approach for intravenous ABO-102 delivery for subjects with Sanfilippo syndromes," stated Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics. "We are excited about continued biomarker signals in this trial, as well as early positive signals in the neurocognitive assessments. While we are still very early in the trial, we are extremely encouraged by these early results and look forward to expanding

enrollment in this clinical trial with enrollments accelerating at two additional international clinical sites."

Abeona's MPS IIIA program, ABO-102, has been granted Orphan Product Designation in the USA and in the European Union, has received the Rare Pediatric Disease Designation in the US, and recently received Fast Track designation by the US FDA.

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 biopharmaceutical company developing gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include 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), EB-201 for epidermolysis bullosa (EB), 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 and ABO-302 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 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 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; our belief that initial signals of biopotency and clinical activity, which suggest that ABO-102 successfully reached target tissues throughout the body, including the central nervous system; our belief that the data demonstrate an early and robust systemic delivery of ABO-102, and the increased reductions in CNS GAG support our approach for intravenous delivery for subjects with Sanfilippo syndromes, 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