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# Abeona Therapeutics Reports on Initial Safety and Biopotency Signals in MPS IIIB Gene Therapy Clinical Trial

- *First-in-man AAV9-based gene therapy delivered by single intravenous injection targeting central nervous system and peripheral manifestations of MPS IIIB.*
- *ABO-101 is well tolerated and demonstrates early biopotency signals with significant disease-specific heparan sulfate (HS) reductions in cerebral spinal fluid, urine, and plasma and greater than 300-fold increase in NAGLU enzyme activity observed in first subject at 30 days post injection.*
- *Company plans for additional enrollments following pending review by independent Data Safety Monitoring Board (DSMB).*

NEW YORK and CLEVELAND, Feb. 07, 2018 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel cell and gene therapies for life-threatening rare genetic diseases, today reported preliminary 30-Day safety and biopotency signals from the first patient dosed in the company's ongoing Phase 1/2 trial for ABO-101, a gene therapy treatment for patients with MPS IIIB (Sanfilippo syndrome Type B), enrolling at Nationwide Children's Hospital in Columbus, Ohio. The ABO-101 therapy involves a single intravenous injection of AAV gene therapy for subjects with MPS IIIB, a rare autosomal recessive disease causing neurocognitive decline, speech and mobility loss, and premature death. Abeona plans to enroll a total of three patients in Cohort 1 (2E13 vg/kg) before dose-escalating to the Cohort 2 dose (5E13 vg/kg).

"MPS IIIB is a devastating and progressive lysosomal storage disease with no approved treatment options. The first patient treated with ABO-101 has demonstrated that the systemic AAV gene transfer is well-tolerated, and the preliminary evidence of biopotency in the CNS and periphery is very encouraging," stated Kevin M. Flanigan, MD, principal investigator and Director of the Center for Gene Therapy at Nationwide Children's Hospital and Professor of Pediatrics and Neurology at The Ohio State University College of Medicine. "We are especially pleased to see reductions in several key biopotency markers, including the reductions in cerebral spinal fluid, urine and plasma heparan sulfate and normalization of plasma NAGLU enzyme activity at days seven, 14 and 30 post-transfer."

The Phase 1/2 study is designed to evaluate safety and preliminary indications of efficacy of ABO-101 in subjects suffering from MPS IIIB. In the first patient treated in Cohort 1:

• ABO-101, at a systemic dose of 2E13 vg/kg, is well-tolerated, with no treatment related adverse events or serious adverse events (SAEs) through 30 days of follow up.

• Early biopotency signals include significant heparan sulfate (HS) reductions observed in

cerebral spinal fluid (50%), urine (69%), plasma (60%) and urinary total glycosaminoglycan (GAG) (67%).

- 50% decline in CSF heparan sulfate from baseline supports previous AAV9 clinical observations that ABO-101 crossed the blood brain barrier after intravenous administration.

• Normalized NAGLU enzyme activity observed represented by a greater than 300-fold increase over baseline at 30 days post administration.

“The reduction of heparan sulfate, the key biomarker of disease pathology in MPS IIIB, at 30 days post gene transfer indicates early and robust systemic delivery of ABO-101, and further supports our planned approach using intravenous delivery of ABO-101 in the treatment of Sanfilippo syndromes," stated Timothy J. Miller, PhD, President and CEO of Abeona Therapeutics. “Consistent with our ongoing ABO-102 clinical trial in MPS IIIA, these findings support our plans to continue enrollment in this trial pending review by the DSMB.”

Subjects in the Phase 1/2 trial receive a single, intravenous injection of ABO-101, which uses an AAV vector to introduce a corrective copy of the NAGLU gene associated with MPS IIIB disease. Subjects will be evaluated at multiple time points over the initial 30 days post-injection for safety assessments and initial signals of biopotency. Results in the first patient dosed with ABO-101 suggest strong CNS and broader systemic distribution, with the potential to reduce levels of glycosaminoglycans (GAGs) that represent the lysosomal storage pathology central to MPS IIIB disease progression.

ABO-101 has been granted Rare Pediatric Disease Designation in the U.S., and Orphan Product Designation in both the U.S. and the European Union.

**About ABO-101 (AAV-NAGLU):** ABO-101 is Abeona’s first-in-human, adeno-associated viral (AAV)-based gene therapy for MPS III (Sanfilippo syndrome). Treatment involves a one-time intravenous delivery of a functioning copy of the N-acetyl-α-D-glucosaminidase (NAGLU) gene to cells of the central nervous system (CNS) and peripheral organs, with the aim of correcting the effects that result from the genetic aberrations that are the root cause of the disease. Following administration of a single dose in Sanfilippo preclinical animal models, ABO-101 induced cells in the CNS and peripheral organs to produce the missing NAGLU enzyme, which then restored underlying sugar (glycosaminoglycan or GAG) storage pathology to normal levels in cells. In preclinical in vivo efficacy studies in Sanfilippo syndrome animal model, ABO-101 demonstrated functional benefits that continue for months after treatment. A single dose of ABO-101 significantly restored normal cell and organ function, corrected cognitive defects, increased neuromuscular function and normalized the lifespan of animals with MPS IIIB after treatment compared to untreated control animals. These results are consistent with studies from several laboratories suggesting AAV treatment could potentially benefit patients with Sanfilippo syndrome. Safety and efficacy studies of AAV gene therapy treatments for Sanfilippo syndrome have recently been published in several peer-reviewed scientific journals.

**About MPS IIIB:** (also known as Sanfilippo syndrome type B) is a genetic, progressive, and devastating rare lysosomal storage disease. In patients with MPS IIIB, genetic mutations result in a marked decrease in NAGLU enzyme activity, which leads to accumulation of heparan sulfate (HS) in the brain and other organs as well as progressive brain atrophy with

cortical gray matter volume loss. The accumulation of abnormal HS results in neurocognitive decline, behavioral disturbances, speech loss, increasing loss of mobility, and premature death. MPS IIIB typically presents in children during the first few years of life, and 70% of patients do not reach 18 years of age. There are no approved treatments for MPS IIIB.

About Abeona: Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and ABO-101 (AAV-NAGLU), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type B (MPS IIIB). Abeona is also developing ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) for treatment of infantile Batten disease (INCL), EB-201 for epidermolysis bullosa (EB), 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 is developing a proprietary vector platform, AIM™, for next generation product candidates. For more information, visit www.abeonatherapeutics.com.

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*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 expectation that we will continue to advance our gene therapy for MPS IIIB patients, and that we remain encouraged by signs of tolerability and biological effects observed in trial Cohorts post injection. Such 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 secure licenses for any technology that may be necessary to commercialize our products, 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-101 successfully reached target tissues throughout the body, including the central nervous system and the increased reductions in CNS GAG support our approach for intravenous delivery for subjects with Sanfilippo syndromes, risks associated with data analysis and reporting, and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and quarterly reports on Form 10-Q 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.*

Source: Abeona Therapeutics Inc.