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Abeona Therapeutics Enrolls First Patient in ABO-101 Phase 1/2 Clinical Trial for MPS IIIB

NEW YORK and CLEVELAND, Dec. 20, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene and cell therapies for life-threatening rare diseases, announced today that the first patient in a Phase 1/2 clinical trial for ABO-101 (AAV-NAGLU), a single treatment gene therapy for patients with Sanfilippo syndrome type B (MPS IIIB), has been dosed at Nationwide Children's Hospital, Columbus, Ohio.

"With the dosing of the first patient with Sanfilippo syndrome type B, we are excited to begin enrolling our third gene therapy clinical trial targeting rare diseases," stated Timothy J. Miller, Ph.D., President and CEO of Abeona. "Having observed positive efficacy data and a strong safety profile in our MPS IIIA program, we look forward to further validating the clinical translation of this investigational gene therapy. This trial is the culmination of many years of research and development, and none of it would have been possible without continued support from the Sanfilippo patient advocacy community."

ABO-101 has been granted Orphan Product Designation by the U.S. Food and Drug Administration (FDA) and received Rare Pediatric Disease Designation as a pre-requisite component of the FDA's Priority Review Voucher process. The ABO-101 clinical study is supported by a 25-subject MPS III Natural History Study conducted at Nationwide Children's Hospital.

"MPS IIIB is a devastating and deadly lysosomal storage disease with no approved treatments available. Caused by a single gene defect, the disease afflicts children, many of whom do not reach adulthood. The ABO-101 gene therapy approach advances the potential of AAV gene therapy as a treatment paradigm for addressing this relentlessly progressive disease," noted Kevin M. Flanigan, M.D., Director and Principal Investigator with the Center for Gene Therapy at Nationwide Children's Hospital.

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 the underlying sugar (GAG) storage pathology to normal levels in cells. Preclinical in vivo efficacy studies in Sanfilippo syndrome animal model have 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 gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include ABO-102 (AAV-SGSH), an adeno-associated virus (AAV)-based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB). Abeona is also developing ABO-101 (AAV-NAGLU) for Sanfilippo syndrome type B (MPS IIIB), 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 its proprietary vector platform, AIM™, for next generation product candidates.

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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, including the statement that AAV treatment could potentially benefit patients with Sanfilippo syndrome. 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 obtain any necessary intellectual property to commercialize any of our products; the ability to achieve or obtain necessary regulatory

approvals and licenses; 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.

Source: Abeona Therapeutics Inc.