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Abeona Therapeutics Announces Fast Track Designation from FDA for ABO-102 in Sanfilippo Syndrome Type A

Fast Track Designation Added to Already Granted Orphan Drug Designation by the FDA and European Medicines Agency (EMA), and Rare Pediatric Disease Designation for ABO-102 in Sanfilippo Syndrome Type A (MPS IIIA)

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 10/25/16 --

Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing therapies for life-threatening rare genetic diseases, announced today that the U.S. Food and Drug Administration (FDA) granted Fast Track designation for ABO-102, a single intravenous injection of AAV gene therapy for subjects with MPS IIIA (Sanfilippo syndrome type A), a rare autosomal recessive disease affecting every cell and organ in the body causing neurocognitive decline, speech loss, loss of mobility, and premature death in children.

"Fast Track designation underscores the importance that the FDA places on developing new treatments for life-threatening disorders, such as MPS IIIA, and reinforces our mandate of accelerating the development of ABO-102 to market," stated Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics. "We look forward to providing additional updates for our ongoing Phase 1/2 clinical trial."

Fast Track designation is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions that address an unmet medical need. Advantages of Fast Track designation include opportunities for more frequent interactions with the FDA during all aspects of development, and eligibility for priority review and accelerated approval. This designation is in addition to ABO-102 being granted Orphan Drug designation by the FDA and the European Medicines Agency (EMA), as well as having already received the Rare Pediatric Disease Designation from the FDA.

"The Fast Track designation comes with an increase in interaction and feedback from the FDA during the development process of a drug and signifies that the FDA may be able to expedite the review and approval of the ABO-102 gene therapy product which, in preclinical and initial clinical work, has shown encouraging signals of biopotency," stated Steven H. Rouhandeh, Executive Chairman. "This designation also demonstrates to the children and families afflicted with MPS IIIA, the FDA's recognition of the severity and importance of addressing this rare orphan disease."

The ongoing Phase 1/2 study is designed to evaluate safety and preliminary indications of efficacy of ABO-102 in subjects suffering from MPS IIIA. Abeona recently announced initial 30 day post-injection data from subjects that received the low dose:

- ABO-102 was well-tolerated in subjects injected with the low dose of 5E12 vp/kg ABO-102 with no treatment related adverse events or serious adverse events (SAEs). Following favorable review of the safety data by the independent Data Safety Monitoring Board (DSMB), enrollment in the high dose cohort has commenced.

- In the natural history study evaluating MPS III subjects, it was shown that urine and cerebral spinal fluid (CSF) GAG (heparan sulfate or "HS") are significantly elevated in the subject population as a symptom of disease pathology.

- All subjects in the low-dose cohort experienced reductions from baseline in both urinary HS and CSF. At 30 days post-injection, urinary HS reduction was 57.6% +/- 8.2%. Reduction in CSF HS was 25.6% +/- 0.8%, suggesting that ABO-102 crossed the blood brain barrier after intravenous administration.

- 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 volume (17.7% +/- 1.9%), and spleen volume (17.6% +/- 7.1%) from baseline, as measured by MRI at 30 days post-injection.

Per the design of the clinical trial, subjects in the low-dose cohort 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 were evaluated at multiple time points over the initial 30 days post-injection for safety assessments and initial signals of biopotency, which suggest that ABO-102 successfully reached target tissues throughout the body, including the central nervous system, to reduce GAG content that underlies the lysosomal storage pathology central to MPS IIIA (Sanfilippo syndrome type A).

About ABO-102 (AAV-SGSH): ABO-102 is an adeno-associated viral (AAV)-based gene therapy for subjects with MPS IIIA (Sanfilippo syndrome), that is delivered as a one-time intravenous injection. ABO-102 delivers a functioning, corrective copy 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 of the disease. ABO-102 has been well tolerated through 30-day post-injection in subjects injected with the low-dose (n=3). 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 subjects continued through one-year of follow up assessments.

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 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, that ABO-102 is well-tolerated, that studies will continue to enroll in the US and internationally, having fast track designation and that early signs of potency are encouraging and will continue 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 subjects 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.

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