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Abeona Therapeutics Announces Authorization to Commence Phase 1/2 Gene Therapy Clinical Study for Patients with MPS IIIB in Spain

- ***Clinical trial for ABO-101 (AAV-NAGLU) marks the 2nd MPS III program in Europe***
- ***Company plans to initiate additional clinical sites in European countries including France, Germany and the United Kingdom***

NEW YORK and CLEVELAND, Sept. 12, 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 announced authorization to move forward with a Phase 1/2 clinical trial in Spain for the Company's gene therapy product ABO-101 (AAV-NAGLU) for patients with MPS IIIB (Sanfilippo syndrome type B). The clinical study was approved by the Agencia Espanola de Medicamentos y Productos Sanitarios and is being conducted at Hospital Clinico Universitario of Santiago de Compostela, Spain. This will be the Company's second clinical trial conducted in Europe, alongside the ongoing Phase 1/2 clinical trial for patients with MPS IIIA (Sanfilippo syndrome type A). Abeona first initiated this trial in the United States. Abeona plans to add clinical sites for the trial in three European countries including France, Germany and the United Kingdom.

"The authorization of our ABO-101 trial in Spain is a significant milestone for children suffering from MPS IIIB, a devastating and deadly disease with no approved treatment options," stated Carsten Thiel, PhD, CEO. "We are encouraged by the preliminary results observed in our US trial to date, both in clinically relevant biomarkers and in the ongoing safety profile and are excited to bring this therapy to patients in Europe."

Subjects in the Phase 1/2 trial receive a single, intravenous infusion of ABO-101, which uses an AAV vector to introduce the functional NAGLU gene to treat patients with MPS IIIB disease. Subjects will be evaluated at multiple time points post-injection for safety assessments and efficacy parameters. The clinical program is supported by a Natural History Study which included potential efficacy assessments consisting of neurocognitive evaluations, biochemical assays and MRI data generated over one year of follow-up assessments.

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 Central Nervous System 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, ABO-202 (AAV-CLN1) for treatment of infantile batten disease, 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 Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties. Such statements include that we are encouraged by the results of the US trial, and that we plan to add clinical sites for the trial in three European countries. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, 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; 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.

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Source: Abeona Therapeutics Inc.