November 9, 2017

Abeona Enrolls First Subject in Spain in Ongoing Phase 1/2 Clinical Trial in MPS IIIA

- ***Company Opens European Subsidiary in Spain to Support Global Clinical Development***
- ***Nine subjects at global clinical sites enrolled to date with more than 2,000 days cumulative follow up assessed***

NEW YORK and CLEVELAND, Nov. 09, 2017 (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 diseases, announced today that the first patient was enrolled in the Company's ABO-102 Phase 1/2 clinical trial at the Hospital Clinico Universitario of Santiago de Compostela, Spain. In conjunction with the initiation of the Spain clinical site, Abeona has established a local subsidiary to manage clinical trial and regulatory development efforts in Europe.

"We are pleased to initiate enrollment at our Spain clinical site for ABO-102. We remain encouraged by the improvements observed in clinically relevant biomarkers post-dosing of the gene therapy in the patients in Cohort 3 and the ongoing safety profile ABO-102 demonstrates," stated Juan Ruiz, M.D., Ph.D., Chief Medical Officer of Abeona Therapeutics. "Developing a local company presence in Spain allows us to exercise closer supervision and further advance Abeona's gene therapy programs in Europe, as well as advance our relationships with the patient community. We are grateful to the many patient foundations and parents who have supported the research needed to advance a potential treatment for this devastating unmet medical need. We are also proud to collaborate with one of the leading clinical centers in Spain dealing with MPS IIIA patients."

Pursuant to the design of the ongoing clinical trial, subjects receive a single, intravenous injection of ABO-102 to deliver the AAV viral vector systemically 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 signals of biopotency and clinical activity, which to date have demonstrated that ABO-102 successfully reaches target tissues throughout the body, including the central nervous system.

"MPS IIIA is a profound and deadly lysosomal storage disease with no approved treatments available," stated Dr. Maria Luz Couce, Director of the Unit of Diagnosis and Treatment of Congenital Metabolic Diseases, Hospital Clinico Universitario of Santiago de Compostela in Spain, "We are excited to initiate enrollment and are encouraged by the durable and significant reduction in central nervous system heparan sulfate as a key biomarker of disease pathology and look forward to enrolling more patients in this gene therapy trial."

A total of nine subjects have been infused in the ongoing global MPS IIIA dose-escalation clinical trial, which has been well-tolerated through more than 2,000 cumulative follow-up days. Safety and efficacy data has been reviewed and enrolliment in Cohort 3 continues at all three active clinical sites (US, Spain, and Australia).

"We are grateful to see the fruition of our collaboration with Abeona, which has opened a promising new hope in Sanfilippo treatments. That the first patient in Spain has been treated with this therapy is great news for our community, and we look forward to supporting additional enrollments in this clinical trial," stated Emilio Lopez, President of Fundación Stop Sanfilippo.

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 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 a proprietary vector platform, AIM™, for next generation product candidates. For more information, visit www.abeonatherapeutics.com.

Investor Contact:
Christine Silverstein
Vice President, Investor Relations
Abeona Therapeutics Inc.
+1 (212)786-6212
csilverstein@abeonatherapeutics.com

Media Contact:
Lynn Granito
Berry & Company Public Relations
+1 (212) 253-8881
lgranito@berrypr.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 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.