May 9, 2017

Abeona Therapeutics Receives Regulatory Approval to Initiate Clinical Trial in Australia with ABO-102 Gene Therapy For Patients with MPS IIIA

Clinical Trial Notification (CTN) Enables Initiation at 3rd Global Site and Accelerates Enrollment in the ABO-102 MPS IIIA Gene Therapy Program

NEW YORK and CLEVELAND, May 09, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene therapies for life-threatening rare diseases, today announced Australian regulatory approval to initiate a Phase 1/2 for the ABO-102 gene therapy program for patients with Sanfilippo syndrome type A (MPS IIIA). The clinical study was approved by the Australian Government Department of Health Therapeutic Goods Administration and the Company is conducting the Phase 1/2 clinical study at Adelaide Women's and Children's Hospital, Australia.

"Abeona has enrolled more MPS IIIA patients in a gene therapy trial than any other group in the world, and the addition of a third clinical site will accelerate our ability to evaluate ABO-102 as a potential treatment for patients with Sanfilippo syndrome type A, or MPS IIIA. We remain encouraged by the recently reported clinical data and look forward to enrolling patients in Australia and Spain shortly. Our work in Australia would not have been possible without The Sanfilippo Children's Foundation and families for helping advance these potentially life-changing therapies into global clinical trials," stated Timothy J. Miller, Ph.D., President & CEO.

"MPS IIIA is a profound degenerative disorder of childhood, which manifests progressive neuromotor and cognitive decline with associated systemic complications and premature death, typically before adulthood. The reported clinical data has proved most encouraging with a reduction in central nervous system (CNS) heparan sulphate, a reduction in liver volume, and preliminary evidence of slowed neurocognitive decline," indicated Nicholas Smith, M.D., Ph.D., Principal Investigator and Dept. Head, Neurology and Pediatric Neurologist at Women's and Children's Hospital. "With no approved treatments for this relentless disease, this investigational gene therapy approach, delivered as a single intravenous injection to treat the whole body, holds great potential for patients and their families."

The ongoing Phase 1/2 clinical trial, which has received FastTrack designation, Orphan Product Designation, and Rare Pediatric Disease designation by the FDA, is designed to evaluate safety and efficacy of ABO-102 in patients with MPS IIIA. Per the design of the trial, subjects receive 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, particularly the CNS. Subjects are evaluated at multiple time points over the initial six-months post-injection for safety assessments and initial signals of biopotency. The global clinical study is supported by a 25-subject MPS III 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.

“Abeona continues to demonstrate global leadership in advancing clinical and commercial development of promising gene therapies for children with Sanfilippo syndrome, and the additional clinical site in Australia will help advance global access to this potential treatment for families suffering from this devastating disease,” said Megan Donnell, Executive Director of The Sanfilippo Children’s Foundation.

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 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 has a plasma-based protein therapy pipeline, including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD, using its proprietary SDF™ (Salt Diafiltration) ethanol-free process. 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:
Andre'a Lucca
Vice President, Communications & Operations
Abeona Therapeutics Inc.
+1 (212)-786-6208
alucca@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 the statement that the addition of a third global clinical site will accelerate our ability to evaluate ABO-102 as a potential treatment for patients with Sanfilippo syndrome type A, or MPS IIIA. 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 develop our products and technologies; 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-102 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, 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