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Abeona Announces FDA Grants RMAT Designation to ABO-102 Gene Therapy in MPS IIIA

First Gene Therapy Using AAV Approach Granted Regenerative Medicine Advanced Therapy Designation

NEW YORK and CLEVELAND, April 23, 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, announced today that the US Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to ABO-102, the Company's AAV-mediated gene therapy for the treatment of Sanfilippo syndrome Type A (MPS IIIA), a rare autosomal-recessive lysosomal storage disease.

"We are encouraged to have received the first gene therapy RMAT designation in MPS IIIA and look forward to further collaborating with the FDA to determine next steps in the development pathway for ABO-102," said Carsten Thiel, Ph.D., CEO of Abeona Therapeutics. "This action further reinforces the clinical significance in the data observed in the ongoing Phase 1/2 trial and the high unmet need for effective treatment options for patients suffering from MPS IIIA."

Established under the 21st Century Cures Act, RMAT designation is an expedited program for the advancement and approval of regenerative medicine products. A regenerative medicine is eligible for the designation if it is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such a disease or condition. RMAT allows companies developing regenerative medicine and gene therapies to work more closely and frequently with the FDA and grants all of the benefits of Breakthrough Therapy Designation.

The Company continues to engage the FDA on its ongoing Phase 1/2 trial. In the trial, subjects receive a single intravenous injection of ABO-102 to facilitate systemic delivery of a functional copy of the gene (SGSH) associated with onset and progression of MPS IIIA. Subjects are evaluated at multiple time points post-injection for safety, tolerability and efficacy. To date, 11 subjects have been enrolled. An update on results from the trial will be presented at the American Society for Gene and Cell Therapy (ASGCT) in May this year. ABO-102 has been granted Rare Pediatric Disease Designation, Fast Track Designation. and Orphan Product Designation in the U.S. and Orphan Drug Designation in the European Union.

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. 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 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 CLN3 disease, ABO-202 (AAV-CLN1) for treatment of CLN1 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 that involve risks and uncertainties. These statements include, without limitation, statements about our ability to develop our products and technologies; our plans for continued development of our products and technologies and internationalization of our clinical programs; that patients will continue to be identified, enrolled, treated and monitored in the ABO-102 clinical trial, and that studies will continue to indicate that ABO-102 is well-tolerated; our expectation that certain of our products may be eligible for priority review and accelerated approval by the FDA; and our expectation that we will continue to advance our gene therapy for MPS IIIA patients. 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 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, 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.

Source: Abeona Therapeutics Inc.