April 4, 2019

Abeona Therapeutics Receives FDA Fast Track Designation for ABO-101 for Treatment of Sanfilippo Syndrome Type B (MPS IIIB)

Ongoing Phase 1/2 study of ABO-101 enrolling eligible patients with MPS IIIB

NEW YORK and CLEVELAND, April 04, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in cell and gene therapy development, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to ABO-101, the Company's novel one-time gene therapy for Sanfilippo syndrome type B (MPS IIIB). ABO-101 is designed to deliver a functional copy of the NAGLU gene to the central nervous system and peripheral tissues. Abeona is enrolling eligible patients with MPS IIIB at sites in the U.S. and Spain and expects to activate additional sites globally this year to accelerate enrollment. Information about the study is available at AbeonaTrials.com and ClinicalTrials.gov (NCT03315182).

"Receiving Fast Track designation is recognition by FDA that ABO-101 shows promise in treating a serious life-threatening disease and may address the significant unmet medical need of children with MPS IIIB," said João Siffert, M.D., Chief Executive Officer. "We will continue to interact with global regulatory authorities about the ABO-101 clinical program to facilitate development of this one-time AAV9 mediated gene therapy."

Fast Track designation is granted by the FDA to facilitate the development and expedite review of investigational therapies intended to treat serious or life-threatening diseases that show potential for addressing significant unmet medical needs. Clinical development of drug products receiving the designation benefit from frequent FDA interactions during all aspects of development and if specific criteria are met, they are eligible for priority review and accelerated approval. ABO-101 has also received Orphan Drug designations in the U.S. and EU, and Rare Pediatric Disease designation from the FDA.

ABO-101 is part of the Company's portfolio of novel, one-time, AAV9-based gene therapies for rare lysosomal storage diseases. Pre-clinical data demonstrated that ABO-101 delivers a functional copy of the NAGLU gene to the central nervous system and peripheral tissues through a single intravenous infusion. The therapy is designed to address the underlying lysosomal enzyme deficiency responsible for abnormal accumulation of glycosaminoglycans in the brain and throughout the body that result in progressive cell damage and neurodevelopmental and physical decline.

About Sanfilippo Syndrome

Sanfilippo syndrome, or MPS type III, is a group of devastating rare genetic lysosomal storage diseases with no approved treatments. MPS III is characterized by developmental decline, including loss of ability to communicate, cognitive impairment, hearing loss, behavioral disturbances, seizures, as well as difficulties with sleep. Systemic manifestations include coarsening of facial features and enlargement of organs such as the liver. An estimated 70% of children with MPS III do not reach age 18. The underlying cause of the syndrome is a missing enzyme in the lysosome that is essential to breaking down heparan sulfate. As a result, partially degraded heparan sulfate accumulates inside the cells, including the brain and spinal cord, causing progressive damage. MPS III is caused by single gene defects unique to each type of the syndrome - A, B, C or D. The cause of MPS IIIA is a deficiency in the SGSH enzyme, while MPS IIIB is distinguished by a marked decrease in NAGLU enzyme activity.

About Abeona Therapeutics

Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. The Company's lead programs include EB-101, its gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa, and ABO-102, a novel AAV9-based gene therapy for Sanfilippo syndrome type A (MPS IIIA). The Company's portfolio of AAV9-based gene therapies also features ABO-101 for Sanfilippo syndrome type B (MPS IIIB), and ABO-202 and ABO-201 for CLN1 disease and CLN3 disease, respectively. Its preclinical assets include ABO-401, which uses the novel AIM™ AAV vector platform to address all mutations of cystic fibrosis. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates and is the only company with Regenerative Medicine Advanced Therapy designation for two investigational therapies (EB-101 and ABO-102). For more information, visit www.abeonatherapeutics.com.

Forward Looking Statement

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. These statements include statements regarding our pipeline and the potential for ABO-101 for the treatment of Sanfilippo syndrome, status of the clinical program including ongoing Phase 1/2 studies, pending INDs and feedback from regulatory agencies, timelines for initiation of further clinical studies, and the company's goals and objectives. We have attempted to identify forward looking statements by such terminology as "may," will," "anticipate," "believe," "estimate," "expect," "intend," and similar expressions.

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 initiate and 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 obligation to revise the forward-looking statements or update them to reflect events or circumstances occurring after

the date of this presentation, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

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Source: Abeona Therapeutics Inc.