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Abeona Therapeutics Treats First Patient in Second Cohort of Phase 1/2 Clinical Trial for ABO-101 in Sanfilippo Syndrome Type B (MPS IIIB)

Interim data expected in the second half of 2019

NEW YORK and CLEVELAND, May 14, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that the Company has completed dosing cohort 1 and treated the first patient in cohort 2 in an ongoing Phase 1/2 clinical trial evaluating ABO-101, its novel one-time gene therapy for Sanfilippo syndrome type B (MPS IIIB). The Company expects to report interim data from the trial in the second half of 2019.

“We believe that ABO-101 can help address the underlying lysosomal enzyme deficiency that ultimately results in progressive cell damage and neurodevelopmental and physical decline in children with MPS IIIB,” said João Siffert, M.D., Chief Executive Officer. “The product safety profile to date and clear evidence of biological activity provide support for advancing this clinical trial to the second cohort. This multicenter study is made possible by our close collaborations with expert academic investigators and the participants who enrolled to date. We look forward to presenting interim data in the second half of this year.”

The Phase 1/2 trial is a two-year, open-label, dose-escalation, global clinical trial in patients diagnosed with MPS IIIB who are older than six months of age and have a minimum cognitive Development Quotient of 60 or above. The study is expected to include up to nine patients and is designed to evaluate two doses of ABO-101 with cohort 1 receiving 2 X 10^{13} vg/kg and cohort 2 receiving 5 X 10^{13} vg/kg. ABO-101 is delivered using NAV® AAV9 technology via a one-time intravenous infusion. The primary endpoint of the study is to assess neurodevelopment and safety, with multiple secondary and exploratory endpoints including neurocognitive and behavior evaluations, quality of life, enzyme activity in cerebrospinal fluid (CSF) and plasma, biomarkers in CSF, plasma and urine, and brain and liver volume.

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. Additional information about the study is available at AbeonaTrials.com and ClinicalTrials.gov (NCT03315182).

About Sanfilippo syndrome type B (MPS IIIB)
Sanfilippo syndrome type B (MPS IIIB) is a rare and fatal lysosomal storage disease with no approved therapy that primarily affects the central nervous system and is characterized by rapid neurodevelopmental and physical decline. Children with MPS IIIB present with

progressive language and cognitive decline and behavioral abnormalities. Other symptoms include sleep problems and frequent ear infections. Additionally, distinctive signs such as facial features with thick eyebrows or a unibrow, full lips and excessive body hair for one's age and liver/spleen enlargement are also present. The underlying cause of MPS IIIB is a deficiency in the NAGLU enzyme responsible for breaking down glycosaminoglycans, which accumulate throughout the body resulting in rapid decline associated with the disorder.

About ABO-101
ABO-101 is a novel gene therapy in Phase 1/2 development for Sanfilippo syndrome type B (MPS IIIB), a rare lysosomal storage disease with no approved therapy that primarily affects the central nervous system (CNS). ABO-101 is dosed in a one-time intravenous infusion using the NAV® AAV9 vector to deliver a functional copy of the NAGLU gene to cells of the CNS and peripheral tissues. The therapy is designed to address the underlying NAGLU enzyme deficiency responsible for abnormal accumulation of glycosaminoglycans in the brain and throughout the body that results in progressive cell damage and neurodevelopmental and physical decline. In the U.S., Abeona holds Fast Track and Rare Pediatric Disease designations for ABO-101 and Orphan Drug designation in both the U.S. and EU.

About Abeona Therapeutics
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and cell therapies for serious diseases. The Company's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa, as well as ABO-102 and ABO-101, novel NAV® AAV9-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively. The Company's portfolio of AAV9-based gene therapies also features 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 candidates (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 including the potential for ABO-101 in the treatment of Sanfilippo syndrome type B, including the results of clinical studies, the ability to present interim data, the ability to continue to enroll patients in clinical studies, the ability to obtain regulatory marketing approvals, 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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