July 25, 2019

Abeona Therapeutics Announces Positive Interim Data from the ABO-102 Phase 1/2 Gene Therapy Clinical Trial in MPS IIIA

Neurocognitive development of youngest patients preserved 12-18 months post treatment; development scores remain within range of unaffected children

Robust and sustained biomarker improvement across all dose cohorts with 8 months to 2 years of follow up

RMAT meeting anticipated for 2H2019 to discuss next steps

NEW YORK and CLEVELAND, July 25, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced positive data from its ongoing Phase 1/2 clinical trial evaluating ABO-102, the Company's investigational one-time, adeno-associated virus 9 (AAV9) gene therapy for Sanfilippo syndrome type A (MPS IIIA). These new results showed that treatment of the youngest patients with ABO-102, all enrolled in the high-dose cohort 3, resulted in preservation of neurocognitive development 12-18 months post treatment. Robust and sustained improvement observed in biomarkers confers additional evidence of a clear biological effect following ABO-102 administration. In addition, longer-term safety remained favorable eight months to two years after treatment. Abeona is enrolling eligible patients at sites in the U.S., Spain, and Australia. Additional information about the trial, also known as The Transpher A Study (ABT-001), is available at AbeonaTrials.com and ClinicalTrials.gov (NCT02716246).

"We are very encouraged by these most recent results supporting the potential of a single intravenous administration of ABO-102 to treat children with MPS IIIA, a devastating lysosomal storage disease without an approved therapy. These data showed that treating younger children during early stages of their disease confers the greatest chance of neurocognitive benefits," said João Siffert, M.D., Chief Executive Officer. "The longer-term data continue to demonstrate that ABO-102 has a clear biological effect and a favorable safety profile."

Summary of Interim Data

- The three youngest patients enrolled in cohort 3 – ages 26 months, 19 months, and 14 months at dosing – continued to track within normal age equivalent development 12-18 months post treatment.
- Dose dependent and sustained reductions in CSF heparan sulfate were observed in all three cohorts; levels reached lower limit of quantitation in all eight patients treated with higher ABO-102 dose in cohort 3.

- No product-related serious adverse events were reported to date.

As part of the ABO-102 program's Regenerative Medicine Advanced Therapy (RMAT) designation, Abeona is pursuing a meeting with the U.S. Food and Drug Administration in the second half of 2019 to assess the next steps in the program's development pathway.

About ABO-102
ABO-102 is a novel gene therapy in Phase 1/2 development for Sanfilippo syndrome type A (MPS IIIA), a rare lysosomal storage disease with no approved treatment that primarily affects the central nervous system (CNS). ABO-102 is dosed in a one-time intravenous infusion using an AAV9 vector to deliver a functional copy of the SGSH gene to cells of the CNS and peripheral organs. The therapy is designed to address the underlying SGSH 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 Regenerative Medicine Advanced Therapy, Fast Track, and Rare Pediatric Disease designations for ABO-102 and Orphan Drug designation in both the U.S. and EU.

About The Transpher A Study
The Transpher A Study (NCT02716246) is an ongoing, two-year, open-label, dose-escalation, Phase 1/2 global clinical trial assessing ABO-102 for the treatment of patients with Sanfilippo syndrome type A (MPS IIIA). The study, also known as ABT-001, is intended for patients 6 months to 2 years of age, or patients older than 2 years with a cognitive Developmental Quotient of 60% or above. The study has enrolled 14 patients to date across three dose-escalating cohorts (N=3, N=3, N=8) and remains open for enrollment. The gene therapy ABO-102 is delivered using AAV9 technology via a one-time intravenous infusion. The study primary endpoints are neurodevelopment and safety, with secondary endpoints including behavior evaluations, quality of life, enzyme activity in cerebrospinal fluid (CSF) and plasma, heparan sulfate levels in CSF, plasma and urine, and brain and liver volume.

About Sanfilippo syndrome type A (MPS IIIA)
Sanfilippo syndrome type A (MPS IIIA) is a rare, fatal lysosomal storage disease with no approved treatment that primarily affects the central nervous system and is characterized by rapid neurodevelopmental and physical decline. Children with MPS IIIA 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 IIIA is a deficiency in the SGSH enzyme responsible for breaking down glycosaminoglycans, which accumulate throughout the body resulting in rapid decline associated with the disorder.

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 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 Statements

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 about the Company's clinical trials and its products and product candidates, future regulatory interactions with regulatory authorities, as well as the Company's goals and objectives. We have attempted to identify forward looking statements by such terminology as "may," "will," "believe," "estimate," "expect," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. 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 enroll patients in clinical trials, the outcome of any future meetings with the U.S. Food and Drug Administration or other regulatory agencies, 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 periodic reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or to 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.

Investor Contact:
Sofia Warner
Senior Director, Investor Relations
Abeona Therapeutics
+1 (646) 813-4710
swarner@abeonatherapeutics.com

Media Contact:
Scott Santiamo
Director, Corporate Communications
Abeona Therapeutics
+1 (718) 344-5843
ssantiamo@abeonatherapeutics.com

Source: Abeona Therapeutics Inc.