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Abeona Therapeutics Announces New MRI Data Showing Increased Brain Volume in Young Patients with Sanfilippo Syndrome Type A (MPS IIIA) After Treatment with ABO-102 Gene Therapy

New data from Phase 1/2 Transpher A study presented at 16th International Symposium on MPS and Related Diseases

NEW YORK and CLEVELAND, July 26, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene therapy, today announced magnetic resonance imaging (MRI) data from the Phase 1/2 Transpher A clinical study indicating that ABO-102 increased grey matter, corpus callosum and amygdala volumes in the brain in three young patients with Sanfilippo Syndrome Type A (MPS IIIA) at 24 months as compared to afflicted patients without treatment. The new data was presented during an oral presentation at the 16th International Symposium on MPS and Related Diseases.

“Brain volume loss is characteristic in children with MPS IIIA and is associated with long-term cognitive and physical disability. Specifically, grey matter is important for cognitive development, corpus callosum for motor function, and amygdala for fear learning as well as social/emotional development,” said Vishwas Seshadri, Ph.D., M.B.A., Head of Research & Clinical Development of Abeona. “The new MRI data shows the potential of ABO-102 to increase brain grey matter, corpus callosum and amygdala volumes and is consistent with previously reported results of preservation of neurocognitive development in these three young patients in the Transpher A study.”

The Transpher A study primary endpoints are neurodevelopment and safety. Secondary endpoints include brain volume, behavior evaluations, quality of life, enzyme activity in cerebrospinal fluid (CSF) and plasma, heparan sulfate levels in CSF, plasma and urine, and liver volume.

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 from birth to 2 years of age, or patients older than 2 years with a cognitive developmental quotient of 60% or above. ABO-102 gene therapy is delivered using AAV9 technology via a single-dose 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 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 a self-complementary 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, Rare Pediatric Disease, and Orphan Drug designations for the ABO-102 clinical program. In the EU, the Company holds PRIME and Orphan medicinal product designations.

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 CNS 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 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 in early childhood. MPS IIIA is caused by genetic mutations that lead to a deficiency in the SGSH enzyme responsible for breaking down glycosaminoglycans, which accumulate in cells throughout the body resulting in rapid health 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. Abeona's clinical programs include EB-101, its investigational autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, as well as ABO-102 and ABO-101, novel investigational AAV-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively, in Phase 1/2 development. The Company's development portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical need. Abeona's novel, next-generation AAV capsids are being evaluated to improve tropism profiles for a variety of devastating diseases. Abeona's fully integrated gene and cell therapy cGMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and planned commercial production of AAV-based gene therapies. 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. 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 the potential impacts of the COVID-19 pandemic on our business, operations, and financial condition, 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 disclosed in the Company's most recent Annual Report on Form 10-K and subsequent quarterly reports on Form 10-Q and other periodic reports filed 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 press release, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

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