December 21, 2020

Abeona Therapeutics Announces Acceptance of Late-Breaker Abstracts Highlighting New Clinical Data for Novel AAV-based Gene Therapies in MPS IIIA and MPS IIIB at WORLDSymposium™

NEW YORK and CLEVELAND, Dec. 21, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that abstracts detailing new interim results from its ABO-102 Phase 1/2 Transpher A study for MPS IIIA and ABO-101 Phase 1/2 Transpher B study for MPS IIIB have been accepted for platform oral presentations during the late-breaking abstract session at the 17th Annual WORLD*Symposium*™ being held February 8-12, 2021.

“Children born with MPS IIIA and MPS IIIB experience progressive neurodevelopmental decline and loss of motor function that is life-threatening,” said Michael Amoroso, Chief Operating Officer of Abeona. “We are excited to share new analyses from the Transpher A study that will add to the understanding of the potential for ABO-102 to help preserve neurocognitive development in patients with MPS IIIA when they are treated at a young age, and new results from the Transpher B study that will provide insights into ABO-101’s biologic effect in patients with MPS IIIB.”

Presentation Details

Title: Updated Results of Transpher A, a Multicenter, Single-Dose, Phase 1/2 Clinical Trial of ABO-102 Gene Therapy for Sanfilippo Syndrome Type A (Mucopolysaccharidosis IIIA)
Abstract Number: 390
Presenter: Kevin Flanigan, M.D., Center for Gene Therapy at Nationwide Children’s Hospital
Date/Time: Friday, February 12, 2021, time to be determined

Title: Updated Results of Transpher B, a Multicenter, Single-Dose, Phase 1/2 Clinical Trial of ABO-101 Gene Therapy for Sanfilippo Syndrome Type B (Mucopolysaccharidosis IIIB)
Abstract Number: 407
Presenter: Maria Jose de Castro, M.D., Hospital Clínico Universitario Santiago de Compostela
Date/Time: Friday, February 12, 2021, time to be determined

About the Annual WORLD*Symposium*™
The WORLD*Symposium*™ is designed for basic, translational and clinical researchers, patient advocacy groups, clinicians, and all others who are interested in learning more about

the latest discoveries related to lysosomal diseases and the clinical investigation of these advances. For additional information on the 17th Annual WORLD*Symposium*™, please visit https://worldsymposia.org/.

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 changes 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 the Transpher B Study
The Transpher B Study (NCT03315182) is an ongoing, two-year, open-label, dose-escalation, Phase 1/2 global clinical trial assessing ABO-101 for the treatment of patients with Sanfilippo syndrome type B (MPS IIIB). The study, also known as ABT-002, 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-101 gene therapy is delivered using AAV9 technology via a single-dose intravenous infusion. The study primary endpoints are neurodevelopment changes 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 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 a self-complementary 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 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 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 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 autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, as well as ABO-102 and ABO-101, novel 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 portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical needs. Abeona's novel, next-generation AIM™ capsids have shown potential to improve tropism profiles for a variety of devastating diseases. Abeona's fully functional, gene and cell therapy GMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and 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. These statements include statements about the Company exploring all strategic options, including the sale of some or all of its assets or sale of the Company. 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, the outcome of the strategic review, 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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Source: Abeona Therapeutics Inc.