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Abeona Therapeutics Announces Successful Type B Meeting with U.S. FDA for ABO-102 AAV-based Gene Therapy in Sanfilippo Syndrome Type A (MPS IIIA)

Transpher A; pivotal study for intended registration of ABO-102 for MPS IIIA disease

Alignment with FDA on primary study endpoint

NEW YORK and CLEVELAND, July 28, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that the Company completed a successful Type B meeting with the U.S. Food and Drug Administration (FDA) regarding the pivotal trial to support filing and approval for its AAV-based gene therapy ABO-102 for the treatment of patients with Sanfilippo syndrome type A (MPS IIIA). ABO-102 is currently being evaluated in the single-arm Transpher A study in patients with MPS IIIA.

Based on the Type B meeting with the FDA, the ongoing Transpher A study will serve as the pivotal study for ABO-102 and could potentially support a Biologics License Application (BLA) submission depending on the data set. In addition, Abeona also aligned with the FDA on the definition of the primary endpoint for the study, neurocognitive assessment using the raw score from the Bayley Scales of Infant and Toddler Development (BSITD) and the Kauffman Assessment Battery for Children (KABC-2), which are already part of the assessment plan in the Transpher A protocol. Abeona intends to work closely with the FDA through the regenerative medicine advanced therapy (RMAT) mechanism to assemble the most robust pivotal data package possible for the registration of ABO-102.

“We are grateful to the FDA for their guidance and collaborative exchange regarding the pivotal trial to support bringing ABO-102 to MPS IIIA patients who currently have no approved treatment,” said Michael Amoroso, Chief Executive Officer of Abeona. “From 2016 to-date we have treated 21 patients in the Transpher A trial. We are excited about the safety and magnitude of benefit seen with our investigational ABO-102 therapy in the younger children from the higher dose cohort reported earlier this year. We remain hopeful that if the more recently dosed children in cohort 3 display a similar treatment effect, we could have an evaluable data set in 2022. The patients we serve have tremendous unmet need and we remain fully focused on operational excellence with the intent of now delivering potentially two pivotal data packages—one for ABO-102 and one for EB-101—in 2022.”

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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