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# **Abeona Therapeutics Receives European Medicines Agency PRIME Designation for ABO-102 Gene Therapy in MPS IIIA**

## **PRIME is sixth regulatory designation for the ABO-102 clinical program**

NEW YORK and CLEVELAND, Dec. 20, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that the European Medicines Agency (EMA) has granted Priority Medicines (PRIME) designation to the Company's ABO-102 program studying its adeno-associated virus 9 (AAV9) gene therapy for Sanfilippo syndrome type A (MPS IIIA). The PRIME designation is based on nonclinical data and clinical data from the Transpher A Study, a global Phase 1/2 clinical trial evaluating a single-dose of ABO-102 for the treatment of children with MPS IIIA.

"EMA's PRIME designation for the ABO-102 program recognizes the urgent need for a treatment option for children suffering from MPS IIIA, and underscores the potential of ABO-102 to modify the course of this devastating lysosomal storage disease," said João Siffert, M.D., Chief Executive Officer.

The Transpher A Study is enrolling patients at sites in the U.S., Spain, and Australia. Additional information about the trial is available at [AbeonaTrials.com](http://AbeonaTrials.com) and [ClinicalTrials.gov](http://ClinicalTrials.gov) (NCT02716246).

The PRIME initiative provides access to enhanced support for the development of medicines targeting an unmet medical need. The designation affords sponsors with enhanced interaction and early dialogue regarding promising medicines, as well as the possibility of accelerated assessment of medicines applications. PRIME is intended to optimize development plans and speed up evaluation so these medicines can help patients to benefit as early as possible from therapies that may significantly improve their quality of life.

### **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, 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 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 and 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 cohorts (N=3, N=3, N=8) and remains open for enrollment. The ABO-102 gene therapy is delivered using AAV9 technology via a single-dose intravenous infusion. The study's 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 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. 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 a novel vector from Abeona's AIM™ AAV capsid platform to address all mutations of cystic fibrosis. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates, including Regenerative Medicine Advanced Therapy designation for two candidates (EB-101 and ABO-102).

### **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 the potential of ABO-102 to modify the course of lysosomal storage disease; our pipeline including the therapeutic potential for ABO-202 in the treatment of CLN1; 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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