

February 12, 2020



# Abeona Therapeutics Announces Positive Interim Data from MPS III Gene Therapy Programs Presented at WORLDSymposium™

*Neurocognitive development of young MPS IIIA patients preserved up to two years post ABO-102 treatment*

*Dose-dependent and sustained reductions in disease-specific biomarkers denotes clear biologic effects of ABO-102 and ABO-101*

*First patient treated in cohort 3 of ABO-101 MPS IIIB trial; total enrollment eight patients*

*Favorable safety profile observed in both studies*

NEW YORK and CLEVELAND, Feb. 12, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that researchers from the Abigail Wexner Research Institute (AWRI) at Nationwide Children's Hospital presented positive interim data from two ongoing Phase I/II clinical trials evaluating ABO-102 and ABO-101, the Company's investigational gene therapies for MPS IIIA and MPS IIIB, respectively, at WORLDSymposium™. Results from the Transpher A study demonstrated that MPS IIIA patients younger than 30 months treated with ABO-102 in dose cohort 3 continue to show neurocognitive development 18 months to two years after treatment. Reductions in cerebrospinal fluid (CSF) heparan sulfate (HS), denoting enzyme activity in the central nervous system, and liver volume reductions remain stable two years after treatment. Results from the Transpher B study showed that ABO-101 also improved multiple disease biomarkers providing clear evidence of a biologic effect in patients with MPS IIIB. Dosing in cohort 2 is complete and the first patient in cohort 3 was treated in late January, with a total of 8 patients treated to date. Both therapies have been well-tolerated to date. Abeona licensed the AAV9-based gene therapy technology underpinning ABO-102 and ABO-101 from AWRI at Nationwide Children's where it was developed.

Today's presentations are available on [abeonatherapeutics.com](https://investors.abeonatherapeutics.com/news-events) by following this link: <https://investors.abeonatherapeutics.com/news-events>

"In total, the new results continue to show that early treatment with ABO-102 can help preserve neurodevelopment in children with MPS IIIA. These data will inform our ongoing discussions with the FDA and EMA, as we work towards providing a regulatory update in the second quarter," said João Siffert, M.D., Chief Executive Officer. "For ABO-101, the reductions in disease-specific biomarkers are encouraging and demonstrate a clear biologic effect, which parallels that seen in the MPS IIIA study. We look forward to enrolling the

Transpher B study as expeditiously as possible.”

Results from the Transpher A study, an ongoing Phase I/II clinical trial with ABO-102 showed that:

- Three young patients treated in high-dose cohort 3 (at ages 27 months, 19 months, and 12 months) continued to show improved neurocognitive skills 18 months to two years post treatment, compared with natural history.
- Across all cohorts (n=14), biomarker improvements included rapid and sustained, dose-related reductions in CSF-HS that reached lower limit of quantitation in Cohort 3 (n=2); a reduction in plasma HS levels; and a durable, dose-dependent reduction in liver volume with up to 2 years of follow up.
- ABO-102 has been well-tolerated, with long-term safety remaining favorable 15-45 months post treatment. There have been no treatment-related severe adverse events and no clinically-significant adverse events reported.

“The interim results presented today add to evidence suggesting a single intravenous dose of ABO-102 AAV9-based gene therapy has the potential to help MPS IIIA patients sustain neurocognitive development when they are treated at a young age,” said Kevin Flanigan, M.D., Director, Center for Gene Therapy at AWRI at Nationwide Children's and Transpher A study investigator. “These data showed that ABO-102 can deliver a functional copy of the SGSH gene to cells of the CNS and peripheral organs, as evidenced by the clinical benefits in neurocognition and biophysical measures and improvements in disease-specific biomarkers.”

Sites in the U.S., Spain, and Australia continue to enroll eligible patients into the Transpher A study. Additional information about the trial is available at [AbeonaTrials.com](http://AbeonaTrials.com) and [ClinicalTrials.gov](http://ClinicalTrials.gov).

Results from cohorts 1 and 2 (n=7) of the Transpher B study, an ongoing Phase I/II clinical trial showed that ABO-101 treatment demonstrated biologic effect in patients with MPS IIIB, as evidenced by initial improvements in multiple disease biomarkers associated with abnormal accumulation of glycosaminoglycans (GAGs) in the brain and throughout the body:

- Initial improvements in multiple disease-specific biomarkers include:
  - Decreased CSF-HS levels
  - Reduction in plasma and urine HS and GAGs
  - Reduction in liver volume
- ABO-101 has been well-tolerated to date, with no treatment-related severe adverse events and no clinically-significant adverse events reported (n=8)

“The Transpher B study provides hope that we may one day alter the course of this devastating disease,” said Kim McBride, M.D., Principal Investigator at AWRI at Nationwide Children's and co-investigator for the Transpher B study. “The impact on disease biomarkers in the early stages of follow up suggest the potential of ABO-101 gene therapy to break down the accumulation of glycosaminoglycans that underlie MPS IIIB pathology. I look forward to working with fellow investigators to gather more data from the study, including results from high-dose cohort 3.”

Dose cohort 2 has been completed and dosing is underway in cohort 3 (n=1). Sites in the U.S., Spain, and France continue to enroll eligible patients into the Transpher B study. Additional information about the trial is available at [abeonatherapeutics.com/clinical-trials](http://abeonatherapeutics.com/clinical-trials) and [ClinicalTrials.gov](http://ClinicalTrials.gov).

### **About The Transpher A Study**

The Transpher A Study (NCT02716246) is an ongoing, two-year, open-label, dose-escalation, Phase I/II 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 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 The Transpher B Study**

The Transpher B Study (NCT03315182) is an ongoing, two-year, open-label, dose-escalation, Phase I/II 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 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 8 patients to date across three dose-escalating cohorts (N=2, N=5, N=1) and remains open for enrollment. The gene therapy ABO-101 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 I/II 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 I/II 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. 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. 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). [www.abeonatherapeutics.com](http://www.abeonatherapeutics.com)

### **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 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.*

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Source: Abeona Therapeutics Inc.