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# Abeona Therapeutics Announces Top-Line Data for ABO-102 Phase 1/2 MPS IIIA Gene Therapy Trial at ASGCT

- Positive dose response in central nervous system with 60.7% +/- 8.8% reduction of disease-causing heparan sulfate GAG observed in Cohort 2*
- Reduction of disease manifestation observed in decreased liver volume of 14.81% (+/- 1.2%)*
- ABO-102 well-tolerated in six subjects through 1100 days follow up with no Serious Adverse Events*
- Cohort 1 demonstrated stabilized or improved Leiter Nonverbal IQ scores at six months*
- Conference call today at 10:00am EDT; 877-269-7756 for domestic callers and 201-689-7817 for international callers*

NEW YORK and CLEVELAND, May 12, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene therapies for life-threatening rare diseases, announced updated data from the ongoing gene therapy clinical trial for Sanfilippo syndrome Type A (MPS IIIA) at the American Society Gene and Cell Therapy (ASGCT) 20<sup>th</sup> Annual Meeting. The ongoing Phase 1/2 trial for ABO-102 (AAV-SGSH) is a first-in-man clinical trial utilizing a single intravenous injection of AAV gene therapy for subjects with Sanfilippo syndrome (MPS IIIA), a rare autosomal-recessive lysosomal storage disease.

"Abeona continues to advance gene therapy for MPS IIIA patients and we are excited about the positive dose response in the CNS seen in Cohort 2. The observation of a dose response supports our clinical approach, and we are encouraged to observe further reductions in central nervous system (CNS) heparan sulfate, reduction in liver volume, and preliminary evidence of slowed neurocognitive decline, are very encouraging. We look forward to accelerating enrollment with the recently initiated global sites (Spain and Australia) and reporting additional clinical data in the ABO-102 global MPS IIIA trial later this year," stated Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics.

Per the design of the clinical trial, subjects received a single, intravenous injection of ABO-102 to deliver the AAV viral vector systematically throughout the body to introduce a corrective copy of the gene that underlies the cause of the MPS IIIA disease. Subjects are evaluated at multiple time points post-injection for safety assessments and initial signals of biopotency and clinical activity, which suggest that ABO-102 successfully reached target tissues throughout the body, including the central nervous system. Highlights reported data on five (n=3 Cohort 1, n=2 Cohort 2) out of the six patients treated to date in the gene therapy trial included:

*Biopotency: positive dose response observed in Cohort 2.*

--At 30 days post-injection, two patients in the Cohort 2 demonstrated 60.7%(+/- 8.8%) reduction in cerebral spinal fluid (CSF) heparan sulfate (HS).

*Hepatosplenomegaly: consistent reduction in liver volume observed.*

--At 30 days post-injection, Cohort 2 subjects demonstrated reductions in liver volumes of 14.81% (+/- 1.2%).

--The natural history study in 25 subjects with MPS III (Truxal et. al., 2016, Mol. Genet. Metab.) demonstrated that subjects had increased liver volumes averaging 116% at baseline that did not change over a year of follow-up.

*Cognitive Assessments: evidence of cognitive stabilization at six months in Cohort 1.*

--Cognitive assessments, taken at baseline, at the six-month timepoint for the Cohort 1 (n=3), subjects showed evidence of stabilization or improvement in the Leiter-R non-verbal IQ and Vineland (adaptive behavior) scales.

--Cognitive assessments are taken at six-month and twelve-month follow-up visits.

--Leiter Nonverbal IQ assessments in Cohort 1 subjects demonstrated stabilized or improved scores at six-months post-injection. Notably, one subject improved +10 (+/-6) points, while age-matched controls in the Natural History study would have predicted a decrease of -11.1 (+/-2.7) points over 6 months.

--Vineland assessments in Cohort 1 at six months post-injection suggest stabilization in adaptive behavior scores.

*Safety: well-tolerated in all subjects through 1100 days cumulative post-injection.*

--No serious adverse events (SAEs) reported in subjects in either cohort receiving ABO-102 (Cohort 1: 5E12 vg/kg and Cohort 2: 1E13 vg/kg).

“We remain encouraged by signs of tolerability and biological effects that we have observed in Cohort 1 and in the initial two subjects of Cohort 2,” stated Kevin M. Flanigan, M.D., principal investigator, Director of the Center for Gene Therapy at Nationwide Children’s Hospital and Professor of Pediatrics and Neurology at The Ohio State University College of Medicine. “We are pleased to see decreases in CSF HS compared to the Cohort 1 at 30 days post-injection, and we look forward to enrolling additional high-dose patients.”

The ongoing Phase 1/2 clinical trial, which has received FastTrack designation, Orphan Product Designation, and Rare Pediatric Disease designation by the FDA, is designed to evaluate safety and efficacy of ABO-102 in patients with MPS IIIA. The global clinical study is supported by a 25-subject MPS III Natural History Study, which included potential efficacy assessments consisting of neurocognitive evaluations, biochemical assays and MRI data generated over one year of follow-up assessments.

**Company Conference Call Details:** Abeona will host a live conference call briefing today at 10:00am EDT. Analysts and investors can participate in the conference call by dialing 877-269-7756 for domestic callers and 201-689-7817 for international callers.

**Sanfilippo syndromes** (or mucopolysaccharidosis (MPS) type III): a group of four inherited genetic diseases each caused by a single gene defect, described as type A, B, C or D, which cause enzyme deficiencies that result in the abnormal accumulation of glycosaminoglycans (GAGs, or sugars) in body tissues. MPS III is a lysosomal storage disease, a group of rare inborn errors of metabolism resulting from deficiency in normal lysosomal function. The incidence of MPS III (all four types combined) is estimated to be 1 in

70,000 births. Mucopolysaccharides are long chains of sugar molecule used in the building of connective tissues in the body. There is a continuous process in the body of replacing used materials and breaking them down for disposal. Children with MPS III are missing an enzyme which is essential in breaking down the used mucopolysaccharides called heparan sulfate. The partially broken down mucopolysaccharides remain stored in cells in the body causing progressive damage. In MPS III, the predominant symptoms occur due to accumulation within the central nervous system (CNS), including the brain and spinal cord, resulting in cognitive decline, motor dysfunction, and eventual death. Importantly, there is no cure for MPS III and treatments are largely supportive care.

**About Abeona:** Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB). Abeona is also developing ABO-101 (AAV-NAGLU) for Sanfilippo syndrome type B (MPS IIIB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) for treatment of infantile Batten disease (INCL), EB-201 for epidermolysis bullosa (EB), ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder and ABO-302 using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, Abeona has a plasma-based protein therapy pipeline, including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD, using its proprietary SDF™ (Salt Diafiltration) ethanol-free process. For more information, visit [www.abeonatherapeutics.com](http://www.abeonatherapeutics.com).

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*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 that involve risks and uncertainties. These statements include without limitation the statement that the addition of two additional global clinical site will accelerate our ability to enroll and evaluate ABO-102 as a potential treatment for patients with Sanfilippo syndrome type A, or MPS IIIA. Such statements are subject to 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 impact of competition; the ability to develop our products and technologies; 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; our belief that initial signals of biopotency*

*and clinical activity, which suggest that ABO-102 successfully reached target tissues throughout the body, including the central nervous system and the increased reductions in CNS GAG support our approach for intravenous delivery for subjects with Sanfilippo syndromes, 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 obligations to make any revisions to the forward-looking statements contained in this release or to update them to reflect events or circumstances occurring after the date of this release, whether as a result of new information, future developments or otherwise.*



Source: Abeona Therapeutics Inc