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Abeona Therapeutics Announces Publication of Preclinical Data Supporting Clinical Translation of MPS IIIA Gene Therapy

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- *Preclinical efficacy data demonstrated significant benefits leading to restoration of SGSH activity and reduction of glycosaminoglycans (GAG) throughout the central nervous system (CNS) and peripheral organs*
- *Early biopotency signals seen in ongoing AB0-102 Phase 1/2 clinical trial directionally consistent with data seen in pre-clinical models of the MPS IIIA disease*

Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing products for life-threatening rare genetic diseases, announced today that preclinical data supporting clinical trials for ABO-102 (AAV-SGSH), the AAV-based single intravenous gene therapy program for MPS IIIA, (Sanfilippo Type A), were published in the June issue of Molecular Therapy Methods & Clinical Development (doi:10.1038/mtm.2016.36).

Researchers concluded that "… an intravenous injection of scAAV9-U1a-hSGSH vector, leading to restoration of SGSH activity and reduction of glycosaminoglycans (GAG) throughout the central nervous system (CNS) and somatic tissues at a dose…". Other benefits included improved learning ability, increased survival, and improved GAG storage pathology in the CNS, leading researchers to note that: "The study suggests that there is potential for gene therapy intervention in MPS IIIA at intermediate stages of the disease, and extends the clinical relevance of our systemic scAAV9-hSGSH gene delivery approach."

"The data support the clinical translation of ABO-102 for patients with Sanfilippo syndromes, and demonstrates AAV delivery to target tissues in the central nervous system as well as peripheral organs led to resolution of the underlying disease pathology," stated Timothy J. Miller, Ph.D., President & CEO. "This approach is also especially encouraging for potential treatment of patients with juvenile Batten disease, where patients are often initially diagnosed as a result of changes in vision, and the data demonstrate delivery of the AAV to the eye after an intravenous injection."

Abeona Therapeutics previously announced that ABO-102 preliminary measures of clinically relevant biomarkers in the ABO-102 Phase 1/2 clinical trial provided promising signals of potential systemic and CNS clinical benefits for patients suffering with MPS IIIA. The program has been granted Orphan Product Designation in the USA and received the Rare Pediatric Disease Designation. Abeona is currently working toward opening two additional

clinical sites to test ABO-102, one in Spain and one in Australia.

"These promising data continues to reinforce our conviction that our novel portfolio of gene therapies including ABO-102 for MPS IIIA have transformational potential to treat devastating monogenic diseases," stated Steven H. Rouhandeh, Executive Chairman. "We're excited about collaborating with our research partners and patient communities to expand ongoing clinical trials globally, as well as to leverage our insights to accelerate additional gene therapy candidates into clinical development."

The publication article can be accessed by clicking on the following link: (http://www.nature.com/articles/mtm201636).

About ABO-102 (AAV-SGSH): ABO-102 (AAV-SGSH) is a single treatment gene therapy strategy for patients with Sanfilippo syndrome type A (MPS IIIA), which is enrolling and treating patients in a Phase 1/2 clinical trial. MPS IIIA is a rare autosomal recessive disease that is caused by genetic mutations that result in a deficiency of SGSH enzyme activity, leading to abnormal accumulation of glycosaminoglycan (specifically, heparan sulfate, or "HS") in the CNS and systemic tissues and organs. This accumulation of HS results in neurocognitive decline, speech loss, loss of mobility, and premature death in children.

About Abeona: Abeona Therapeutics Inc. is a clinical stage company developing gene and plasma-based therapies for life-threatening rare genetic diseases. Abeona's lead programs are ABO-102 (AAV-SGSH) and ABO- 101 (AAV-NAGLU), adeno-associated virus (AAV) based gene therapies for Sanfilippo syndrome (MPS IIIA and IIIB), respectively. Abeona is also developing EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JBD); and ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder 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 our proprietary SDF™ (Salt Diafiltration) ethanol-free process. For more information, visit www.abeonatherapeutics.com.

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, our plans for continued development and internationalization of our clinical programs, that are looking forward to advancing multiple important new therapeutic candidates for the treatment of epidermolysis bullosa, that we plan to accelerate up to three new promising EB product candidates toward commercialization, that encouraging signs of early biopotency had been observed in urinary and CSF GAG (heparin sulfate) measurements as well as potential disease-modifying effects in the liver and spleen in our ABO-102 program, management plans for the Company, and general business outlook. These 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 achieve or obtain necessary regulatory approvals; the impact of changes in the financial markets and global economic conditions; and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K 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.

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