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Abeona Therapeutics to Present at Gene, Cell and Molecular Therapies for Inherited Metabolic Diseases Meeting

NEW YORK, NY, and CLEVELAND, OH -- (Marketwired) -- 04/15/16 -- Abeona Therapeutics, Inc. (NASDAQ: ABEO), a clinical stage biopharmaceutical company focused on developing and delivering gene therapy and plasma-based products for severe and life-threatening rare diseases, today announced that President and CEO, Tim Miller, Ph.D will be presenting for the company at the Gene, Cell and Molecular Therapies for Inherited Metabolic Diseases Meeting in London, England, April 16th, 2016 at 10:00 AM.

On February 29 2016, Abeona announced FDA approval for the Company's Investigational New Drug Application for ABO-102 (AAV- SGSH), a single treatment strategy for Mucopolysaccharidosis Type IIIA (MPS IIIA). The ABO-102 IND application is now active and enables Nationwide Children's Hospital (Columbus, OH) to initiate a Phase 1/2 clinical study designed to assess the safety, tolerability and potential efficacy of ABO-102 in children with MPS IIII A.

About ABO-102 (AAV SGSH): ABO-102 is a next generation adeno-associated viral (AAV)-based gene therapy for MPS IIIA (Sanfilippo syndrome), which involves a one-time delivery of a normal copy of the defective gene to cells of the central nervous system (CNS) with the aim of reversing the effects of the genetic errors that cause the disease. After a single dose in Sanfilippo preclinical models, ABO-102 induced cells in the CNS and peripheral organs to produce the missing enzymes and help repair damage caused to the cells. Preclinical in-vivo efficacy studies in Sanfilippo syndrome have demonstrated functional benefits that remain for months after treatment. A single dose of ABO-102 significantly restored normal cell and organ function, corrected cognitive defects that remained months after drug administration, increased neuromuscular control and increased the lifespan of animals with MPS IIIA over 100% one year after treatment compared to untreated control animals. These results are consistent with studies from several laboratories suggesting AAV treatment could potentially benefit patients with for Sanfilippo syndrome Type A and B, respectively. In addition, safety studies conducted in animal models of Sanfilippo syndromes have demonstrated that delivery of AB0-102 are well tolerated with minimal side effects.

About the Inherited Metabolic Disease Meeting. This meeting aims to establish and encourage a closer interface between clinicians and scientists with the goal of translating gene therapy and novel technologies into clinical treatment of inherited metabolic disorders to UK patients at the earliest opportunity.

About Abeona: Abeona Therapeutics, Inc. develops and delivers gene therapy and plasma-based products for severe and life-threatening rare diseases. Abeona's lead programs are ABO-101 (AAV NAGLU) and ABO-102 (AAV SGSH), adeno-associated virus (AAV)-based

gene therapies for Sanfilippo syndrome (MPS IIIB and IIIA). We are also developing 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 program for rare blood diseases. In addition, we are also developing rare plasma protein therapies 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, 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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