March 8, 2016

# Abeona Therapeutics Announces Fourth Quarter and Full Year 2015 Summary Financial Results and Recent Operational Highlights

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 03/08/16 -- Abeona Therapeutics, Inc. (NASDAQ: ABEO), a biopharmaceutical company focused on developing and delivering gene therapy and plasma-based products for severe and life-threatening rare diseases, today announced summary financial results for the fourth quarter and full fiscal year ended December 31, 2015. The Company will provide a business update for investors and other stakeholders on a conference call, Wednesday, March 9th, at 4:45 pm (Eastern). Tim Miller, Ph.D., President and CEO and Jeffrey Davis, Chief Operating Officer, together with other executives, will conduct the call. Interested parties are invited to participate in the call by dialing 877-269-7756 (toll free domestic) or 201-689-7817 (international). The call will consist of an overview of the Company's 4Q15 financials, and a discussion of business highlights.

"The past year has led to significant advancements in our goal of building a leadership position in the field of gene therapy and plasma protein therapies towards transforming the lives of patients with rare diseases," stated Steven H. Rouhandeh, Executive Chairman. "In 2015, we expanded our pipeline with two clinical stage AAV gene therapies for Sanfilippo syndrome types A and B, added a third AAV gene therapy product in Juvenile Neuronal Ceroid Lipofuscinosis (JNCL) (also known as juvenile Batten disease), signed a license to an innovative CRISPR-Cas9 gene editing platform in rare blood disorders, with an initial focus in Fanconi anemia, strengthened our team, and added substantial financial resources to our balance sheet. In 2016, our priorities include driving our AAV gene therapy and alpha-1 protease inhibitor programs into the clinic, and advancing our gene editing programs including defining of regulatory pathways to bring our CRISPR product candidates to patients."

Tim Miller, Ph.D., President and CEO, stated, "2016 will be an exciting, transformative year for Abeona Therapeutics as we position ourselves to enter multiple human clinical trials with our pipeline of innovative product candidates. As recently announced, the FDA allowance of the IND for the Phase 1/2 clinical study of ABO-102 for patients with Sanfilippo syndrome type A (MPS IIIA) moves our programs into the clinic here in the US, and we look forward to working with our collaborators to expand this program into Europe and Australia later this year. We believe that our gene therapy programs in Sanfilippo syndrome type B (ABO-101) and Juvenile Neuronal Ceroid Lipofuscinosis (ABO-201) will follow shortly. Lastly, we would like to thank our dedicated researchers and clinical collaborators, as well as the many dedicated patient foundations, for their tireless efforts

and commitment to advancing new treatment options for these devastating unmet medical needs."

### *Recent Abeona Highlights*

- ***Sanfilippo syndrome gene therapy programs***: On February 29, 2016, Abeona announced the FDA allowance of an Investigational New Drug (IND) for systemic AAV Phase 1/2 clinical study with ABO-102 gene therapy for patients with Sanfilippo syndrome type A (MPS IIIA). On January 11, 2016, we announced that initial regulatory approvals from European bodies -- the Genetically Modified Organism (GMO) Voluntary Release regulatory filings, and the ethical committee regulatory filings -- for both the ABO-101 and ABO-102 programs in Spain. Abeona plans to commence both programs for the upcoming human clinical trials to be conducted at Cruces University Hospital in Bilbao, Spain. Both the ABO-101 and ABO-102 programs have received Orphan Drug and Pediatric Rare Disease designations from the FDA.
- ***SDF-Alpha plasma protein program***: Abeona has completed optimization of the downstream chromatography steps for our SDF-Alpha™ (alpha-1 protease inhibitor) for inherited COPD. Additional provisional patent applications have been filed to provide the Company with expanded intellectual property protection. The Company has expanded its CMO relationships to ensure it has the ability to manufacture clinical material for future trials. The Company confirms that its proprietary SDF platform provides significantly enhanced yields of alpha-1 protease inhibitor, at levels up to 10 times of that achievable with the industry standard Cohn processes, and with purity levels consistent with that achieved by other commercial processes.
- ***Advanced pipeline programs***: Together with its academic collaborators, the Company continued to progress its pre-clinical programs in juvenile Batten disease and its CRISPR-Cas9 program in Fanconi anemia (FA) and other rare blood disorders. Juvenile Batten disease is the most common form of a group of disorders known as neuronal ceriod lipofuscinosis (NCL), a lysosomal storage disease that affects the nervous system in children and for which there are no approved treatment options. Fanconi anemia is a rare pediatric blood disease characterized by multiple physical abnormalities, bone marrow failure and a higher than normal risk of cancer.

### *Fourth Quarter and Full 2015 Summary Financial Results*

- ***Cash Position***: Cash, cash equivalents and marketable securities as of December 31, 2015 were $40.1 million, compared to $11.6 million as of December 31, 2014. The increase was primarily driven by net proceeds from multiple equity financings partially offset by cash used to fund operations. Cash used in operations in 2015 was $10.4 million.
- ***Revenues***: Revenue was $215 thousand and $1.0 million for the fourth quarter of 2015 and the full year 2015, respectively, compared to $234 thousand and $925 thousand in comparable periods in 2014. Revenues consisted of a combination of royalties from marketed products, primarily MuGard®, and recognition of deferred revenues related to upfront payments from early license agreements.
- ***Loss per share***: Loss per share was $0.07 and $0.53 for the fourth quarter of 2015 and the full year 2015, respectively, compared to a loss per share of $2.05 and

$15.26 in comparable periods in 2014.

***About ABO-101 (AAV NAGLU) and ABO-102 (AAV SGSH) are***next generation adeno-associated viral (AAV)-based gene therapies for MPS IIIA and MPS IIIB, respectively. These gene therapies involve a one-time delivery of a genetically modified virus to deliver a normal copy of the defective gene to cells of the central nervous system (CNS) and peripheral organs with the aim of reversing the effects of the genetic errors that cause the disease. After a single dose in preclinical animal models of Sanfilippo syndrome, ABO-101 and 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 animals with Sanfilippo syndrome have demonstrated functional benefits that remain for months after treatment. A single dose significantly restored normal cell and organ function, corrected cognitive defects that remained for months after drug administration, increased neuromuscular control and increased the lifespan of animals with MPS III 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 Sanfilippo syndrome Type A and B,. In addition, safety studies conducted in animal models of Sanfilippo syndromes have demonstrated that delivery of ABO-101 and ABO-102 are well tolerated with minimal side effects.

***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 Neuronal Ceroid Lipofuscinosis (also known as juvenile Batten disease); 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 using our proprietary SDF™ (Salt Diafiltration) ethanol-free process, including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD. 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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