May 16, 2016

Abeona Therapeutics Announces First Quarter 2016 Summary Financial Results and Recent Operational Highlights

Investor Conference Call to Be Held on Tuesday, May 17th at 10 am ET

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 05/16/16 -- 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 summary financial results for the first quarter. The Company will provide a business update for investors and other stakeholders on a conference call, Tuesday, May 17th, at 10 am (Eastern). Tim Miller, Ph.D., President and Chief Executive Officer 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 1Q16 financials, and a discussion of business highlights.

"The first quarter of this year has led to significant advancements in our goal of building a leadership position in the field of gene therapy and plasma protein therapies for rare diseases," stated Steven H. Rouhandeh, Executive Chairman. "We thank our collaborators, shareholders and staff as we prepare to launch human clinical trials in up to four different rare diseases over the next 12 months."

Tim Miller, PhD, stated, "In the first quarter, Abeona hit important regulatory milestones with the FDA allowance of our Phase 1/2 clinical study in Sanfilippo syndrome type A (MPS IIIA) and the European approvals of the Genetically Modified Organisms (GMO) and Ethical Committee (CEIC) regulatory filings. Additionally, Abeona and its academic collaborators presented meaningful pre-clinical data at the World Symposium of Lysosomal Storage Diseases in San Diego, and on its proprietary CRISPR/Cas9 platform at the 2nd Annual CRISPR Precision Gene Editing Congress in Boston, MA."

Recent Abeona Operating Highlights

- The Interministerial Council of Genetically Modified Organisms has approved the Genetically Modified Organism (GMO) Voluntary Release and Ethical Committee (CEIC) regulatory filings for both Phase 1/2 Gene Therapy Clinical Studies to treat patients with AB0-101 (AAV NAGLU) and ABO-102 (AAV SGSH) for patients with Sanfilippo syndrome type A (MPS IIIA) or type B (MPS IIIB)

- FDA allowed 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)

- Abeona highlighted new preclinical Juvenile Neuronal Ceroid Lipofuscinosis (JNCL) data at WORLDSymposium(™) 2016 which demonstrated encouraging in vivo efficacy in preclinical JNCL (also known as Juvenile Batten disease) model

- Abeona partnered with Therapure Biopharma to continue its efforts in developing rare plasma proteins in SDF Alpha for inherited COPD

- Abeona presented compelling data at the 2nd Annual CRISPR Precision Gene Editing Congress in Boston, MA which showed that CRISPR/Cas9 gene repair resulted in normalization of the FANCC gene in Fanconi anemia (FA)

First Quarter Summary Financial Results

- ***Cash Position***: Cash, cash equivalents and marketable securities as of March 31, 2016 were $37.4 million, compared to $40.1 million as of December 31, 2015. Net cash used in operating activities in 1Q16 was $2.5 million as compared to $3.2 million in the same period in 2015, a decrease of $647 thousand.

- ***Revenues***: Revenues were $235 thousand for the first quarter of 2016, compared to $258 thousand in in the first quarter of 2015. 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 $0.17 for the first quarter of 2016, compared to a loss per share of $0.10 in comparable period in 2015.

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). The company is 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, Abeona is developing plasma protein therapies 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.

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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