August 16, 2016

Abeona Therapeutics Announces Second Quarter 2016 Financial Results and Recent Clinical Highlights

Investor Conference Call to be held Wednesday, August 17th at 10:00 am ET

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 08/16/16 -- Abeona Therapeutics Inc. (NASDAQ: ABEO) a clinical-stage biopharmaceutical company focused on delivering gene and plasma-based therapies for life-threatening rare diseases, today announced financial results for the second quarter. The Company will provide investors an update on recent and ongoing business activities and an overview of its 2Q16 financials on, Wednesday, August 17th, at 10:00 am (Eastern). Interested parties are invited to participate in the call by dialing 877-269-7756 (toll free domestic) or 201-689-7817 (international).

"The second fiscal quarter of 2016 brought significant advancements in our goal of building a strong leadership position in the development of innovative therapies for rare diseases," stated Steven H. Rouhandeh, Executive Chairman. "We are excited about the initial biopotency signals seen in our Sanfilippo Type A clinical trial, and are looking forward to advancing multiple important new therapeutic candidates for the treatment of epidermolysis bullosa (EB), as announced last week. We thank our collaborators, shareholders and staff for their ongoing commitment and support as we move these therapies further into clinical development."

Timothy J. Miller, Ph.D, stated, "Abeona achieved many important regulatory and clinical milestones in the second quarter of 2016. The milestones include the dosing of the first patient in our Phase 1/2 clinical trial with ABO-102 for patients with Sanfilippo syndrome type A, the FDA allowance of our Phase 1/2 clinical study in Sanfilippo syndrome type B (MPS IIIB), and, most recently, the European regulatory approval for our MPS IIIA Phase 1/2 clinical study to be conducted at Cruces University Hospital in Bilbao, Spain. Additionally, we are excited about our new collaboration with the EB Research Partnership, EB Medical Research Foundation and Stanford University to accelerate up to three new promising EB product candidates toward commercialization."

Recent Abeona Operating Highlights

- On May 17, 2016, Abeona announced that the first patient in its Phase 1/2 trial for ABO-102 (AAV-SGSH), a single treatment gene therapy strategy for patients with Sanfilippo syndrome type A (MPS IIIA), has been enrolled at Nationwide Children's Hospital in Columbus, Ohio.
- On May 24, 2016, Abeona announced the FDA Allowance of its Investigational New Drug (IND) for a Phase 1/2 clinical study with ABO-101 (AAV-NAGLU) for patients with

Sanfilippo syndrome type B (MPS IIIB).

- On August 2, 2016, Abeona provided an update on the initial subjects enrolled in this trial, stating that ABO-102 had been well tolerated with no safety or tolerability concerns identified through 30-days post-injection, and that encouraging signs of early biopotency had been observed in urinary and CSF GAG (heparan sulfate) measurements as well as potential disease-modifying effects in the liver and spleen.
- On August 4, 2016, the Company announced it had received European regulatory approval by the Agencia Espanola de Medicamentos y Productos Sanitarios for its Phase 1/2 trial for ABO-102 (AAV-SGSH) to be conducted at Cruces University Hospital (Bilbao, Spain).
- On August 9, 2016, Abeona announced a collaboration with the EB Research Partnership, EB Medical Research Foundation and Stanford University for the development of treatments for recessive dystrophic epidermolysis bullosa (RDEB). Clinical results for the lead EB program (EB-101) were recently presented at the opening Plenary Session of the Society for Investigative Dermatology in May 2016, and Investigators at Stanford are recruiting patients for a Phase 2 clinical trial of EB-101 in adolescents age 13 and older to determine the effect of type VII collagen gene corrective grafts on wound healing efficacy.

Second Quarter Summary Financial Results

- ***Cash Position*** : Cash, cash equivalents and marketable securities as of June 30, 2016 were $34.3 million, compared to $37.4 million as of March 31, 2016. Net cash used in operating activities in the Six Months Ended June 30, 2016 was $5.6 million as compared to $5.0 million in the same period in 2015, an increase of $0.6 million.
- ***Revenues*** : Revenues were $214 thousand for the second quarter of 2016, compared to $282 thousand in in the second 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 $0.20 for the second quarter of 2016, compared to a loss per share of $0.16 in comparable period in 2015.

About Abeona: Abeona Therapeutics Inc. is a clinical stage company developing gene therapy and plasma-based therapies for severe and 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. We are 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 is developing 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, that we 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 (heparan 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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