March 31, 2017

Abeona Therapeutics Reports Q4 2016 Financial Results and Business Highlights

Management to Host Investor Conference Call on Monday, April 10th at 10:00 am ET to Provide Progress Report on Corporate Developments

NEW YORK and CLEVELAND, March 31, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene therapies for life-threatening rare genetic diseases, today announced financial results for the fourth quarter. The Company will host a call to update investors on recent clinical developments and year-end financial results on Monday, April 10th 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 past year has been characterized by advancement across all clinical programs within Abeona, having achieved many important regulatory and clinical milestones in 2016. Notably, we initiated dosing of our high-dose cohort in ABO-102 this January after demonstrating promising safety and biopotency in the completed low-dose cohort last year. The strength of our clinical programs was validated not only through critically reviewed clinical data, but also through the achievement of multiple EMA and FDA designations for our gene therapy programs in 2016. Another important highlight was the addition of our AIM™ vector platform, which provides Abeona the ability to leverage a proprietary AAV capsid portfolio for next generation gene therapies across multiple existing and potential new indications," stated Timothy J. Miller, Ph.D, President and CEO.

Abeona Recent Highlights:

• On March 8, 2017, Abeona announced the European Medicines Agency (EMA) Committee for Orphan Medicinal Products had granted Orphan Drug Designation for EB-101 in Epidermolysis Bullosa.

• On February 17, 2017, Abeona reported positive data from*the leading clinical gene therapy program for Sanfilippo syndrome type A patients, ABO-102, demonstrating central nervous system (CNS) and peripheral organ disease biopotency.*

- *ABO-102 was well-tolerated in 4 subjects (N=3 low dose, N=1 high dose) through 650 days follow up with no Serious Adverse Events*
- *63% +/- 0.5% central nervous system reduction of heparan sulfate GAG 6 months post-injection (N=2)*
- *Continued evidence of biopotency: reduced liver and spleen volumes and decreased urinary GAGs*
- *Two subjects assessed at the 6-month timepoint showed evidence for stabilization or improvement (average 60% over 2 subjects) in several Mullen subdomains*

- *Adaptive behavior ratings on the Vineland stabilized*
- *Subjects showed improved ability to complete individual items on the Leiter-R non-verbal IQ assessment resulting in improved raw scores*

• On February 1, 2017, Abeona enrolled the first high-dose subject in ABO-102 ongoing Phase 1/2 trial in MPS III. ABO-102 received Fast Track Designation and has been granted Orphan Product Designation in the USA and Europe, and has also received the Rare Pediatric Disease Designation in the United States. *Global ABO-102 enrollments in Europe and Australia are expected to commence later this year.*

• On January 19, 2017, the EMA Committee for Orphan Medicinal Products granted Orphan Drug Designation (EMA/OD/226/16) for Abeona's gene therapy program ABO-101 for children impacted by Sanfilippo syndrome type B (MPS IIIB). ABO-101 has previously been granted Orphan Product Designation in the United States and received the Rare Pediatric Disease Designation as a pre-requisite part of the FDA's Priority Review Voucher (PRV) process. The FDA has allowed the Investigational New Drug (IND) for a Phase 1/2 clinical trial, and enrollments are anticipated to begin later this year.

• On January 3, 2017, the EMA Committee for Orphan Medicinal Products granted Orphan Drug Designation for Abeona's ABO-201 program (AAV-CLN3), the AAV-based single intravenous gene therapy program for juvenile Batten disease, a fatal lysosomal storage disease of the nervous system caused by autosomal-recessive mutations in the *CLN3* gene.

• On November 11, 2016, Abeona announced the publication of positive Phase 1 clinical results for EB-101 in the Journal of the American Medical Association (JAMA). The paper highlighted that EB-101 therapy was well-tolerated in patients with recessive dystrophic epidermolysis bullosa (RDEB), and the trial demonstrated clinical efficacy of 67% healed wounds at 6 months post treatment, lasting through 12 months, including collage biomarker expression. The Company anticipates providing a clinical update at the upcoming annual meeting of the Society for Investigative Dermatology, in April, 2017.

"The fourth quarter of 2016 brought significant progress in our goal of building a strong leadership position in the development of novel therapies for rare diseases," stated Steven H. Rouhandeh, Executive Chairman. "With the positive biopotency data seen in our ABO-102 Phase 1/2 clinical trial in Sanfilippo syndrome Type A (MPS IIIA) and the continued enrollment of the high dose cohort, along with data seen in our ongoing EB-101 Phase 2 study in epidermolysis bullosa, the addition of our own proprietary vector platform, we remain focused on furthering our regulatory and clinical progress as we continue our work for 2017."

4th Quarter and Year-end Summary Financial Results:

• Cash position: Cash, cash equivalents and marketable securities as of December 31, 2016 were $69.1 million, compared to $31.2 million as of September 30, 2016. Net cash used in operating activities in the twelve months ended December 31, 2016 was $13 million as compared to $10.4 million in the same period in 2015, an increase of $2.6 million.

• Offering: During the fourth quarter, on November 1, 2016, Abeona closed an underwritten public offering of 6,000,000 shares of common stock, at a public offering price of $7.00 per share. The gross proceeds to the Company were $42,000,000 million, before deducting the

underwriting discounts and commissions and estimated offering expenses payable by the Company.

• Revenues: Revenues were $256 thousand for the fourth quarter of 2016, compared to $215 thousand in the fourth quarter of 2015. Revenues for twelve months ended December 31, 2016 were $889 thousand, compared to $1,040 thousand in the same period in 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 $0.19 for the fourth quarter of 2016, compared to a loss per share of $0.06 in the comparable period in 2015.

About Abeona: Abeona Therapeutics Inc. is a leading clinical-stage biopharmaceutical company developing gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include 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), EB-201 for epidermolysis bullosa (EB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) gene therapy for treatment of infantile Batten disease (INCL), and ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder and ABO-302 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 its proprietary SDF™ (Salt Diafiltration) ethanol-free process. For more information, visit www.abeonatherapeutics.com.

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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 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; our belief that initial signals of biopotency and clinical activity, which suggest that ABO-102 successfully reached target tissues throughout the body, including the central nervous system; our statement that

global ABO-102 enrollments in Europe and Australia are expected to commence later this year; our belief that the data demonstrate an early and robust systemic delivery of ABO-102, and the increased reductions in CNS GAG support our approach for intravenous delivery for subjects with Sanfilippo syndromes, 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.

Source: Abeona Therapeutics Inc