August 15, 2017

Abeona Therapeutics Reports Second Quarter 2017 Financial Results and Recent Business Highlights

Investor Conference Call to be held Tuesday, August 22nd at 10:00 am ET

NEW YORK and CLEVELAND, Aug. 15, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene therapies for life-threatening rare diseases, announced financial results for the second quarter and recent business highlights. The Company will provide investors an update on ongoing business activities and an overview of its 2Q17 financials on Tuesday, August 22nd, 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).

“We made a lot of progress during the second quarter, and the feedback received from the FDA to accelerate our EB-101 program to a pivotal Phase 3 trial supports the potential of this gene therapy program. The strengths of our clinical programs were also underscored by the achievement of additional regulatory designations for our gene therapy programs and the recent appointment of key executives within the Company,” stated Timothy J. Miller, Ph.D., President and CEO.

2nd Quarter Summary Financial Results:

Cash position: Cash, cash equivalents and marketable securities as of June 30, 2017 were $58.3 million, compared to $63.2 million as of March 31, 2017. Net cash used in operating activities in the six months ended June 30, 2017 was $10.8 million as compared to $5.6 million in the same period in 2016.

Revenues: Revenues were $217 thousand for the second quarter of 2017, compared to $214 thousand in the second quarter of 2016. 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 $0.21 for the second quarter of 2017, compared to a loss per share of $0.20 in the comparable period in 2016.

Abeona Recent Highlights:
July 25, 2017: Announced appointment of Juan Ruiz, Ph.D., M.D., as Chief Medical Officer

July 18, 2017: Received guidance from FDA to commence pivotal Phase 3 for EB-101

gene therapy for patients with Epidermolysis Bullosa (EB)

June 29, 2017: Received FDA Orphan Drug Designation for ABO-201 Juvenile Batten disease gene therapy program

May 30, 2017: Received Rare Pediatric Disease designation for EB-101 gene therapy in Epidermolysis Bullosa

May 25, 2017: Received FDA Orphan Drug Designation for EB-101 gene therapy in Epidermolysis Bullosa

May 12, 2017: Announced top-line data for ABO-102 Phase 1/2 MPS IIIA gene therapy trial at ASGCT
-- Positive dose response in central nervous system with 60.7% +/- 8.8% reduction of disease-causing heparan sulfate GAG observed in Cohort 2
-- Reduction of disease manifestation observed in decreased liver volume of14.81% (+/- 1.2%)
-- Cohort 1 demonstrated stabilized or improved Leiter Nonverbal IQ scores at six months

May 9, 2017: Received regulatory approval to initiate clinical trial in Australia with ABO-102 gene therapy for patients with MPS IIIA

May 2, 2017: Provided update on EB-101 Phase 1/2 gene therapy for severe form of Epidermolysis Bullosa from the Society for Investigative Dermatology (SID) Conference
-- Demonstrated significant wound healing (defined as greater than 50% healed) in 100% of treated wounds (36/36) at 3 months; 89% (32/36) at 6 months, 83% (20/24) at 12 months, 88% (21/24) at 24 months and 100% (6/6) at 36 months post-administration
-- Clinical endpoints supported by Natural History Study data observations from 1,436 wounds in 128 patients with Recessive Dystrophic Epidermolysis Bullosa (RDEB)

“In the second quarter, we continued making meaningful progress towards 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) recently, along with two-year follow-up data in our completed EB-101 Phase 1/2 study in EB, we look forward to accelerating each program over the coming months.”

About Abeona: Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB). Abeona is also developing ABO-101 (AAV-NAGLU) for Sanfilippo syndrome type B (MPS IIIB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) for treatment of infantile Batten disease (INCL), EB-201 for epidermolysis bullosa (EB), 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 proprietary vector platform, AIM™, for next generation product

candidates. 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 include, without limitation, our plans for continued development and internationalization of our clinical programs, that patients will continue to be identified, enrolled, treated and monitored in the EB-101 clinical trial, and that studies will continue to indicate that EB-101 is well-tolerated and may offer significant improvements in wound healing; the addition of two additional global clinical sites will accelerate our ability to enroll and evaluate ABO-102 as a potential treatment for patients with Sanfilippo syndrome type A, or MPS IIIA. Such 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 secure licenses for any technology that may be necessary to commercialize our products; 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 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 quarterly reports on Form 10-Q 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