March 16, 2018

Abeona Therapeutics Reports Fourth Quarter 2017 Financial Results and Business Highlights

Investor Conference Call on Tuesday, March 27th at 10:00 am ET

NEW YORK and CLEVELAND, March 16, 2018 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel cell and 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 Tuesday, March 27th at 10:00 am (Eastern). Interested parties are invited to participate in the call by dialing 877-407-9210 (toll free domestic) or 201-689-8049 (International).

“The past year was marked by several defining events in the company’s history, having advanced our two lead clinical programs, EB-101 in Epidermolysis Bullosa and ABO-102 in MPS IIIA, and initiated our third clinical program, ABO-101 in MPS IIIB. The strong safety and biopotency data observed in our three active clinical trials and the strategic initiative of building an in-house commercial GMP manufacturing facility further strengthens our position in developing gene and cell therapies to treat these devastating and rare pediatric diseases,” stated Timothy J. Miller, Ph.D., President and CEO.

4th Quarter and Year-end Summary Financial Results:

- Cash position: Cash, cash equivalents and marketable securities as of December 31, 2017 were $137.8 million, compared to $56.5 million as of September 30, 2017. Net cash used in operating activities in the twelve months ended December 31, 2017 was $22.9 million as compared to $13.0 million in the same period in 2016, an increase of $9.9 million.

- Offering: During the fourth quarter, on October 19, 2017, Abeona closed an underwritten public offering of 5,750,000 shares of common stock, at a public offering price of $16.00 per share. The gross proceeds to the Company were $92 million, before deducting the underwriting discounts and commissions and estimated offering expenses payable by the Company.

- Revenues: Revenues were $215 thousand for the fourth quarter of 2017, compared to $256 thousand in the fourth quarter of 2016. Revenues for twelve months ended December 31, 2017 were $837 thousand, compared to $889 thousand in the same period in 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 $0.19 for the fourth quarter of 2017, compared to a loss per share of $0.18 in the comparable period in 2016. Loss per share was $0.66 for the twelve months ended December 31, 2017, compared to a loss per share of $0.64 in the same period in 2016.

Abeona Recent Highlights:

- March 15, 2018: Abeona received FDA Rare Pediatric Disease Designation for ABO-202 gene therapy program in CLN1 disease
- February 12, 2018: Received FDA Orphan Drug Designation for ABO-202 program in CLN1 disease
- February 8, 2018: Reported top-line data from Phase 1/2 gene therapy trial in MPS IIIA
  – ABO-102 results presented at WORLDSymposium for Lysosomal Diseases showed significant time- and dose-dependent reduction of underlying disease pathology, including decreased CSF and urine GAGs (HS fragments) and diminished liver volumes
  – Evidence of cognitive benefit at six months post treatment in Cohort 2 and at one year in Cohort 1
  – Company receives FDA allowance to lower enrollment age to six months
- February 7, 2018: Reported on initial safety and biopotency signals in MPS IIIB gene therapy clinical trial
  – ABO-101 is well tolerated and demonstrates early biopotency signals with significant disease-specific heparan sulfate (HS) reductions in cerebral spinal fluid, urine, and plasma and greater than 300-fold increase in NAGLU enzyme activity observed in first subject at 30 days post injection
- January 29, 2018: Received FDA Regenerative Medicine Advanced Therapy designation for EB-101 in Epidermolysis Bullosa
- December 20, 2017: Enrolled first patient in ABO-101 Phase 1/2 clinical trial for MPS IIIB
- November 9, 2017: Enrolled first subject in Spain in ongoing Phase 1/2 clinical trial in MPS IIIA
- October 16, 2017: Announced $13.85M grant from leading Sanfilippo syndrome foundations for clinical development of MPS III gene therapies
- October 11, 2017: Enrolled first two patients in the Cohort 3 expansion of the Phase 1/2 clinical trial in MPS IIIA
- October 6, 2017: Announced top-line one year data from ABO-102 MPS IIIA Trial at ARM's Cell & Gene Meeting on the Mesa
- October 4, 2017: Abeona broke ground on GMP commercial manufacturing facility for cell and gene therapies

"2017 was transformative for Abeona, with significant progress made towards our goal of building a strong leadership position in rare disease gene therapy development, and manufacturing technology and capability," stated Steven H. Rouhandeh, Executive Chairman. "The momentum will continue in 2018, with the continued enrollment in the high-dose cohort in our ABO-102 Phase 1/2 clinical trial in Sanfilippo syndrome Type A (MPS IIIA), alongside the clinical and regulatory progress in moving our EB program to a pivotal Phase 3 study and the additional work on our own proprietary AIM™ vector

platform."

About Abeona: Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and ABO-101 (AAV-NAGLU), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type B (MPS IIIB). Abeona is also developing ABO-201 (AAV-CLN3) gene therapy for CLN3 disease, ABO-202 (AAV-CLN1) for treatment of CLN1 disease, 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 is developing 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, statements about our ability to develop our products and technologies; 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 and we plan to initiate a pivotal Phase III trial early next year; we have recently initiated enrollment in our MPS IIIB program; our expectation that we will continue to advance our gene therapy for MPS IIIA patients. 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, risks associated with data analysis and reporting, 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.