November 15, 2017

Abeona Reports Third Quarter 2017 Financial Results and Recent Business Highlights

- **Investor Conference Call to be held Monday, November 20th at 10:00 am ET**
- **Global enrollment continues in ABO-102 trial for MPS IIIA**
- **Screening initiated in ABO-101 Phase 1/2 trial for MPS IIIB**
- **Pivotal Phase 3 planning underway for EB-101 program in RDEB**

NEW YORK and CLEVELAND, Nov. 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 third quarter and recent business highlights. The Company will provide investors an update on recent and ongoing business activities and an overview of its 3Q17 financials on Monday, November 20th, 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 third quarter was marked with achievements across multiple clinical programs, including initiating enrollments at our global clinical sites for ABO-102 for MPS IIIA and reporting additional data that underscored the durability and clinical benefit of the gene therapy. Our Epidermolysis Bullosa program achieved FDA Breakthrough Therapy designation, completed its Phase 1/2 clinical trial and continues to advance as we finalize the clinical protocol before initiating the pivotal Phase 3 trial next year. We were pleased to have recently initiated screening in our MPS IIIB program and look forward to commencing enrollments shortly,” stated Timothy J. Miller, Ph.D., President and CEO. “In addition, work in optimizing our AIM™ vector platform demonstrated exciting progress, including enhanced tissue tropisms compared to naturally occurring AAV capsids.”

3rd Quarter Summary Financial Results:

- Cash position: Cash and cash equivalents as of September 30, 2017 were $56.5 million, compared to $58.3 million as of June 30, 2017. Net cash used in operating activities in the nine months ended September 30, 2017 was $17.6 million as compared to $9.6 million in the same period in 2016. Cash and cash equivalents includes approximately $5 million from exercised warrants in the third quarter. Subsequent to the end of the third quarter, the Company closed a public offering of common stock with gross proceeds of $92 million. Total cash as of October 31, 2017 was $142.6 million.
- Revenues: Revenues were $219 thousand for the third quarter of 2017, compared to $184 thousand in the third 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 $0.13 for the third quarter of 2017, compared to a loss per share of $0.08 in the comparable period in 2016.

Abeona Recent Highlights:

- November 9, 2017: Enrolled First Subject at Spain Clinical Site in Ongoing Phase 1/2 Clinical Trial in MPS IIIA
- October 19, 2017: Announced Closing of $92 Million Underwritten Public Offering and Full Exercise of Underwriters' Option to Purchase Additional Shares
- October 16, 2017: Announced a Grant of up to $13.85 Million from Leading Sanfilippo Syndrome Foundations for Clinical Development of MPS III Gene Therapies
- October 11, 2017: Hosted inaugural R&D day and announced enrollment of First Two Patients in Global Expansion of 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
    - Gene therapy demonstrated durable and significant reduction of underlying disease pathology across multiple clinical measures in Cohort 1 (n=3) compared to a natural history control group (n=8-12)
    - Systemic biopotency demonstrated time- and dose-dependent reductions of disease causing Heparan Sulfate in the Cerebrospinal fluid (CSF) and liver volumes
    - Preservation of deep brain architecture observed after intravenous administration
    - Stabilization of neurocognitive assessment scores at one year post-injection
- October 4, 2017: Announced Dedication of Commercial Gene Therapy Manufacturing Facility in Cleveland, Ohio
- September 28, 2017: Announced Collaboration with Brammer Bio for Commercial Translation of ABO-102
- August 29, 2017: Received FDA Breakthrough Therapy Designation for EB-101 Autologous Cell Therapy in Epidermolysis Bullosa
- July 25, 2017: Announced Appointment of Juan Ruiz, M.D., Ph.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

"We have made great progress in the quarter towards becoming a key player in the development of novel breakthrough gene and cell therapies for rare genetic diseases," stated Steven H. Rouhandeh, Executive Chairman. "The recent investment from high-quality investors and leading foundations is another achievement that demonstrates our internal capabilities and commitment to the advancement of our robust pipeline and next generation vector platform, including MPS III gene therapy products. We look forward to further strengthening our efforts with key hires, advancing clinical capabilities, and commercial expansion in the coming quarters."

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 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 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 screening in our MPS IIIB program and look forward to commencing enrollments by year-end; our expectation that we will continue to advance our gene therapy for MPS IIIA patients, our expectation of accelerating enrollment with our active global sites in Spain and Australia, and that we remain encouraged by signs of tolerability and biological effects observed in Cohort 1 post injection. 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.