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

NEW YORK and CLEVELAND, May 16, 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 first quarter and recent business highlights.

“We are very pleased with our progress during the first quarter and recent weeks, including the encouraging results reported from our lead programs, ABO-102 for MPS and EB-101 for RDEB IIIA patients. Along with the critically reviewed clinical data, the strength of our clinical programs was also underscored by the achievement of additional EMA and FDA designations for our gene therapy programs within the quarter,” stated Timothy J. Miller, Ph.D., President and CEO.

Abeona Recent Highlights:

May 12, 2017, Abeona Therapeutics 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%)
-- ABO-102 well-tolerated in six subjects through more than 1,100 days cumulative follow-up with no Serious Adverse Events
-- Cohort 1 demonstrated stabilized or improved Leiter Nonverbal IQ scores at six months

May 9, 2017, Abeona Therapeutics received regulatory approval to initiate clinical trial in Australia with ABO-102 Gene Therapy For Patients with MPS IIIA

May 2, 2017, Abeona Therapeutics provided update on EB-101 Phase 1/2a gene therapy for severe form of Epidermolysis Bullosa from the Society for Investigative Dermatology Conference
-- EB-101 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 data from Natural History Study observations from 1,436 wounds in 128 patients with Recessive Dystrophic Epidermolysis Bullosa (RDEB)

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.

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.

"We started 2017 making meaningful 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) recently and the initiation of our 2nd international clinical site, along with two year follow-up data seen in our ongoing EB-101 Phase 2 study in epidermolysis bullosa, we look forward to continuing our work for 2017."

1st Quarter Summary Financial Results:

- Cash position: Cash, cash equivalents and marketable securities as of March 31, 2017 were $63.2 million, compared to $69.1 million as of December 31, 2016. Net cash used in operating activities in the three months ended March 31, 2017 was $5.9 million as compared to $2.5 million in the same period in 2016.

- Revenues: Revenues were $186 thousand for the first quarter of 2017, compared to $235 thousand in the first 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 first quarter of 2017, compared to a loss per share of $0.17 in the comparable period in 2016.

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 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 include without limitation the statement that the ability to develop our products and technologies; 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