

June 2, 2017



Abeona Therapeutics to Present at the Jefferies 2017 Global Healthcare Conference

Company CEO to Present on Wednesday, June 7th at 3:30 PM ET

NEW YORK and CLEVELAND, June 02, 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, today announced President and CEO, Timothy J. Miller, Ph.D., will present at the at the Jefferies 2017 Global Healthcare Conference in New York City, NY.

Event: Jefferies 2017 Global Healthcare Conference

Date: Wednesday, June 7th

Presenter: Timothy J. Miller, Ph.D., President & CEO

Presentation Time: 3:30 PM – 3:55 PM ET

Room Name: 1 Ballroom

Location: New York City, NY

Webcast: <http://wsw.com/webcast/jeff105/abeo>

Abeona Recent Highlights

ABO-102 gene therapy program for Sanfilippo syndrome Type A:

- Abeona announced top-line data for 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 of 14.81% (+/- 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*
- Abeona received regulatory approval to initiate ABO-102 gene therapy clinical trial in Australia for patients with MPS IIIA

EB-101 gene therapy program for severe form of Epidermolysis Bullosa

- Abeona provided an update for Phase 1/2a gene therapy to 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)

- Abeona was granted Orphan Drug Designation for EB-101 in Epidermolysis Bullosa by the European Medicines Agency (EMA) Committee for Orphan Medicinal Products
- Abeona was granted Orphan Drug Designation and Rare Pediatric Designations for EB-101 in Epidermolysis Bullosa by the FDA, enabling Priority Review Voucher

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.

Investor Contact:

Christine Silverstein
Vice President, Investor Relations
Abeona Therapeutics Inc.
+1 (212)-786-6212
csilverstein@abeonatherapeutics.com

Media Contact:

Andre'a Lucca
Vice President, Communications & Operations
Abeona Therapeutics Inc.
+1 (212)-786-6208
alucca@abeonatherapeutics.com

This press release contains certain statements that are forward-looking within the meaning of Section 27a of the Securities Act of 1933, as amended, the expected receipt of a Priority Review Voucher 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. 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 ability to secure licenses for any technology that may be necessary to commercialize our products; the impact of changes in the financial markets and global economic conditions; 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