

March 13, 2017



# Abeona Therapeutics to Present at Multiple Upcoming Investor Conferences

## Company COO to Present on Tuesday, March 14th & Tuesday, March 21st

NEW YORK and CLEVELAND, March 13, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing therapies for life-threatening rare genetic diseases, today announced Chief Operating Officer, Jeffrey B. Davis, will present at the 29<sup>th</sup> Annual ROTH Conference and the Oppenheimer 27<sup>th</sup> Annual Healthcare Conference.

**Event:** The 29<sup>th</sup> Annual ROTH Conference

**Date:** Tuesday, March 14<sup>th</sup>

**Time:** 10:30 am PT / 1:30 pm ET

**Room:** Yellow - Salon 6

**Location:** The Ritz-Carlton, Laguna Niguel, CA

**Event:** The Oppenheimer 27<sup>th</sup> Annual Healthcare Conference

**Date:** Tuesday, March 21<sup>st</sup>

**Time:** 4:30 pm ET

**Room:** Track 3

**Location:** The Westin New York Grand Central, NY

**Webcast:** <https://www.veracast.com/webcasts/opco/healthcare2017/42114505926.cfm>

## Abeona Recent Highlights:

- March 8, 2017: Received Orphan Drug Designation in the EU for EB-101 in Epidermolysis Bullosa
- February 17, 2017: Provided update from ABO-102 Sanfilippo type A program (MPS IIIA) at *WORLD Symposium™* lysosomal storage disorders conference
  - 6 months post-injection (N=2): 63% +/- 0.5% reduction in the disease-causing sugar (heparan sulfate GAG) in the central nervous system
  - Continued evidence of biopotency: reduced liver and spleen volumes, decreased urinary GAGs
  - Evidence for stabilization or improvement (average 60%) in several Mullen subdomains
  - Adaptive behavior ratings on the Vineland stabilized
  - Improved raw scores on the Leiter-R non-verbal IQ assessments
  - Well-tolerated (N=4) through 650 days follow up with no Serious Adverse Events
- February 1, 2017: Enrolled first high-dose subject in ABO-102 ongoing Phase 1/2 trial in MPS IIIA

- January 19, 2017: Received Orphan Drug Designation in the EU for ABO-101 in MPS IIIB
- January 3, 2017: Received Orphan Drug Designation in the EU for ABO-201 in juvenile Batten disease

**About Abeona:** Abeona Therapeutics Inc. is a leading clinical-stage biopharmaceutical company developing gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include ABO-102 (AAV-SGSH) and ABO-101 (AAV-NAGLU), adeno-associated virus (AAV) based gene therapies for Sanfilippo syndrome (MPS IIIA and IIIB, respectively). Abeona is also developing EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), EB-201 for epidermolysis bullosa (EB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) gene therapy for treatment of infantile Batten disease (INCL), and 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](http://www.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, and that involve risks and uncertainties. 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 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; our belief that the data demonstrate an early and robust systemic delivery of ABO-102, 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 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.*

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