

June 6, 2018



# Abeona Therapeutics Announces Upcoming Presentation at Jefferies 2018 Global Healthcare Conference

**Company CEO to Present on Thursday, June 7th at 11:00 AM ET**

NEW YORK and CLEVELAND, June 06, 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 CEO, Carsten Thiel, Ph.D., will present at the at the Jefferies 2018 Global Healthcare Conference in New York City, NY.

**Event: Jefferies 2018 Global Healthcare Conference**

**Date:** Thursday, June 7th

**Presenter:** Carsten Thiel, Ph.D., CEO

**Presentation Time:** 11:00 AM – 11:25 AM ET

**Room Name:** Ballroom I

**Location:** New York City, NY

**Webcast:** <http://wsw.com/webcast/jeff113/abeo/index.aspx>

## **Abeona Recent Highlights**

### ***May 31<sup>st</sup>: Opening of Commercial Gene & Cell Therapy GMP Manufacturing Facility in Cleveland, Ohio***

- The Elisa Linton Center for Rare Disease Therapies to support development of advanced gene and cell therapies for treatment of serious rare diseases.
- The GMP facility will have the capability to manufacture clinical and commercial grade products over Abeona's multiple programs, including recessive dystrophic epidermolysis bullosa (RDEB) and Sanfilippo syndrome.

### ***May 18<sup>th</sup>: Clinical Update on MPS IIIA Gene Therapy Trial at the 21st Annual ASGCT Meeting***

- Ongoing ABO-102 (AAV-SGSH) trial results demonstrate robust and durable clinical effects achieved throughout various timepoints post-administration.
- 18-month efficacy and safety data continue to demonstrate time- and dose-dependent reductions in underlying disease pathology, including decreased CSF and urine GAGs and improved liver volumes.
- 11 subjects enrolled through > 4,200 days cumulative follow up.

### ***May 17<sup>th</sup>: Clinical Update from RDEB Gene & Cell Therapy Trial at the 21st Annual ASGCT Meeting***

- EB-101, the Company's gene-corrected skin graft cell therapy for patients suffering from RDEB is safe and well-tolerated, with durable efficacy.
- Trial results demonstrate robust and durable clinical effects achieved throughout various timepoints post-administration.
- Completed Phase 1/2 clinical trial included seven patients with 42 gene-corrected EB-101 grafts, with the first patient treated over three years ago with lasting effects and closed wounds to date.

**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](http://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 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 and licenses; 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.