

May 31, 2018



# Abeona Therapeutics Announces Opening of Commercial Gene & Cell Therapy Manufacturing Facility in Ohio

*-- The Elisa Linton Center for Rare Disease Therapies to support development of advanced gene and cell therapies for treatment of serious rare diseases*

*-- Ribbon-cutting ceremony of gene and cell therapy GMP production center in Ohio to take place May 31*

CLEVELAND and NEW YORK, May 31, 2018 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene and cell therapies for life-threatening rare diseases, announced today the opening of The Elisa Linton Center for Rare Disease Therapies, the commercial GMP manufacturing facility for gene and cell therapies in Cleveland, Ohio. 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. The ribbon-cutting ceremony and first facility walk-through will be held today, May 31, 2018.

"The opening of The Elisa Linton Center for Rare Disease Therapies is a momentous occasion and underscores Abeona's ongoing commitment to transforming patients' lives," said Carsten Thiel, Ph.D., Abeona's Chief Executive Officer. "Our development of internal manufacturing capabilities bolsters our position for commercial readiness as we continue to execute on our vision to bring these therapies to the patient communities that need them."

EB-101 is an autologous, ex-vivo gene-corrected cell therapy where the COL7A1 gene is inserted into a patient's own skin cells (keratinocytes) for the treatment of RDEB, a rare and devastating skin disorder. ABO-102 is an adeno-associated virus (AAV)-based gene therapy in development at Abeona for the treatment of Sanfilippo syndrome type A (MPS IIIA). Both programs were recently granted the Regenerative Medicine Advanced Therapy (RMAT) designation by the U.S. Food and Drug Administration (FDA), emphasizing the unmet need for patients with RDEB and MPS IIIA.

In addition to the production of the EB-101 and ABO-102 therapies and the AIM™ AAV vector lab, the 6,000 square foot facility will satisfy the necessary chemistry, manufacturing and controls (CMC) requirements for commercial development. The second stage of the Company's manufacturing strategy has been initiated with the construction of an additional 20,000 square foot facility that will be used to further meet the anticipated commercial demand for development programs in the longer term.

**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).

**Investor Contact:**

Christine Silverstein  
SVP, Finance & Investor Relations  
Abeona Therapeutics Inc.  
+1 (646) 813-4707  
[csilverstein@abeonatherapeutics.com](mailto:csilverstein@abeonatherapeutics.com)

**Media Contact:**

Lynn Granito  
Berry & Company Public Relations  
+1 (212) 253-8881  
[lgranito@berrypr.com](mailto:lgranito@berrypr.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 Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties. These statements include statements that the new facility will produce several gene and cell therapies from our pipeline aimed to treat life-threatening rare diseases; statements that the new facility will support the production of gene and cell therapies for our leading clinical programs, EB-101 and ABO-102; statements that the facility will have the capability to manufacture clinical and commercial grade products over multiple programs; our expectation that the facility will be used to produce our EB-101 and ABO-102 therapies and our proprietary AIM AAV vectors used to deliver the therapies and the necessary CMCs; and our expectation that our existing 6,000 sq. ft. facility and a new 20,000 sq. ft. facility will be used to meet the anticipated commercial demand for our development programs for the longer term. We have attempted to identify forward looking statements by such terminology as “may,” “will,” “anticipate,” “believe,” “estimate,” “expect,” “intend,” and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, 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 of our facility to adequately produce our therapies, that we will have a continued pipeline of gene and cell therapies, 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, 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, except as required by the federal securities laws.*



Source: Abeona Therapeutics Inc.