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# **Abeona Therapeutics Announces Dedication of Commercial Gene Therapy Manufacturing Facility in Cleveland, Ohio**

*-- Facility to support development of advanced gene and cell therapies for treatment of life-threatening rare diseases*

*-- Leaders from local government, life sciences and medical technology to join on October 4 for dedication of first anticipated gene and cell therapy production center in Ohio*

CLEVELAND, Oct. 04, 2017 (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 ground-breaking of the first anticipated commercial gene therapy manufacturing facility in Ohio. The Cleveland-based facility, named The Elisa Linton Center for Rare Disease Therapies, will have the capacity to produce advanced gene and cell therapies to treat serious and debilitating rare diseases. The dedication and ground-breaking ceremony is being held today, October 4, 2017.

"We are very excited to announce the creation of The Elisa Linton Center for Rare Disease Therapies, which will be a global resource for production of gene therapies with the potential to bring new treatments to rare disease patients around the world," said Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics. "It is especially fitting that this center is named for Elisa Linton, who was born with Sanfilippo syndrome, a rare terminal disease. The memory of Elisa and courage of her family continue to be a great inspiration to all members of the rare disease community."

The Elisa Linton Center for Rare Disease Therapies will initially be used to produce ABO-101 and ABO-102, investigational gene therapies currently in development at Abeona for the treatment of patients with Sanfilippo syndrome, and EB-101, an investigational autologous cell therapy for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), a rare and devastating skin disorder. The Center will also house Abeona's expanded viral-vector lab, which will develop and produce unique and proprietary vectors used for the delivery of gene therapies. The 6,000 square foot Center will be built-out and validated over the next 12 months.

"The Epidermolysis Bullosa Medical Research Foundation, along with the EB Research Partnership, is a longtime supporter of research that may help patients living with EB including the development of EB-101. The development of EB-101 is a great example of the progress we are making in EB research today," said Paul Joseph, Chief Financial Officer of the Epidermolysis Bullosa Medical Research Foundation. "We are very pleased to support Abeona taking steps to establish a world-class gene therapy production facility that will bring

new hope to people living with EB and other serious diseases and conditions.”

Several leaders from local government and life sciences are scheduled to attend the dedication ceremony of the Center, including representatives from United States Senator Sherrod Brown’s office, Case Western Reserve University, BioEnterprise, MidTown Cleveland, Inc. and JumpStart Inc.

“Today’s groundbreaking celebration represents another successful investment in Ohio innovation and manufacturing,” said Senator Brown. “It’s exciting to see investments in gene therapy treatments for rare diseases happening right here in Cleveland, helping to cement Ohio’s leadership in the healthcare and technology industries. Today’s ceremony is another step forward for the health of rare disease patients and for the strength of the region’s economy.”

“We applaud Abeona’s decision to open a state-of-the-art gene therapy facility in Cleveland’s Health-Tech Corridor,” said Aram Nerpouni, President and CEO of BioEnterprise. “The Cleveland bioscience industry has grown remarkably in the past decade and Abeona’s investment is a potent example of our region’s momentum. We look forward to our ongoing relationship with Abeona and other emerging bioscience companies that continue to grow and strengthen our local economy.”

**About EB Medical Research Foundation:** As the leader in research funding, the Epidermolysis Bullosa Medical Research Foundation is an all-volunteer, non-profit 501(c) Foundation dedicated to funding research for Epidermolysis Bullosa to determine its causes, develop successful treatments, and ultimately find a cure. EB is a rare, debilitating and often fatal genetic disorder which causes painful scarring and blistering of the skin and internal organs. Our continued goal is to raise awareness through special events, the media and fundraising programs. [www.ebmrf.org](http://www.ebmrf.org)

**About BioEnterprise:** BioEnterprise is a business formation, recruitment, and acceleration effort designed to support the growth of bioscience companies. Located in Cleveland, BioEnterprise provides management counsel and support services to health IT, medical device, and biopharmaceutical companies. BioEnterprise founders are Case Western Reserve University, Cleveland Clinic, and University Hospitals. Additional technology partners include the NASA Glenn Research Center, Cleveland State University, and BioOhio. The initiative comprises the collective activities of BioEnterprise and its partners’ commercialization offices: The Case Office of Technology Transfer, Cleveland Clinic Innovations, and University Hospitals Cleveland – Center for Clinical Research. The combined efforts of these groups has created, recruited, and accelerated more than 300 companies in 15 years. For more information: [www.BioEnterprise.com](http://www.BioEnterprise.com).

**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 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 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; 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