August 29, 2017

Abeona Therapeutics Receives FDA Breakthrough Therapy Designation for EB-101 Autologous Cell Therapy in Epidermolysis Bullosa

- *FDA recently guided Company to accelerate Phase 3 trial for EB-101 autologous cell therapy*
- *Breakthrough Therapy designation enables priority review and expedites approval process*
- *EB-101 has demonstrated significant efficacy in treated patients for over 2 years*
- *Accelerates Phase 3 clinical trial for EB-101, the leading gene therapy for patients with Recessive Dystrophic Epidermolysis Bullosa*

NEW YORK and CLEVELAND, Aug. 29, 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, announced today that the U.S. Food and Drug Administration has granted Breakthrough Therapy designation status to the Company's EB-101 gene therapy program for patients with Recessive Dystrophic Epidermolysis Bullosa (RDEB). The designation from the FDA enables collaborative discussions with senior FDA personnel, priority review and an expedited approval process to drug candidates where preliminary clinical trials indicate that a therapy may offer substantial treatment advantages over existing options for patients with serious or life-threatening diseases.

"EB-101 is an autologous gene-corrected cell therapeutic approach that utilizes a patient's own cells and genetically engineering them to produce the correct version of collagen, which helps hold skin on to the body, thereby reducing the number of painful blisters caused by injury and improving wound healing," stated Timothy J. Miller, Ph.D., Abeona's President and CEO. "We are grateful that the FDA has recognized the promising clinical data from the EB-101 program with Breakthrough Therapy designation and look forward to initiating our pivotal Phase 3 trial as we advance EB-101 for patients with this debilitating disease."

The Breakthrough Therapy designation is based on data from the Phase 1/2 EB-101 clinical trial, which demonstrated significant wound healing (greater than 50% healed) in treated wounds for over two years. Breakthrough Therapy designation is intended to expedite the development and review of drugs for serious or life-threatening conditions. The criteria for this particular designation require preliminary clinical evidence that demonstrates the drug may have substantial improvement on at least one clinically significant endpoint over available therapy. A Breakthrough Therapy designation conveys all fast track program features with more intensive FDA guidance on an efficient drug development program, an organizational commitment involving senior managers, and eligibility for rolling review and

priority review. This is the first Breakthrough Therapy designation for Abeona since the FDA initiated the program in 2013, highlighting the necessity to develop innovative therapies in diseases where there is a significantly unmet clinical need like RDEB.

The Company continues to engage the FDA on the final Phase 3 clinical trial design, planned to commence early 2018, and will provide an update on the program in the coming months. Abeona's EB-101 product is an autologous, *ex-vivo* gene-corrected cell therapy in which the COL7A1 gene is inserted into a patient's own skin cells (keratinocytes) for the treatment of the underlying disease in Recessive Dystrophic Epidermolysis Bullosa. The EB-101 program has been granted Orphan Drug and Rare Pediatric Disease Designations from the US Food and Drug Administration (FDA) and Orphan Drug Designation from the European Medicines Agency (EMA).

About EB-101 Phase 1/2 Clinical Trial:
In the recent Phase 1/2 clinical trial, EB-101 was administered to non-healing chronic wounds on each subject and assessed for wound healing at predefined time points. The trial met the primary endpoints safety and efficacy, where wound healing after EB-101 administration was compared to control untreated wounds from a supporting natural history study that evaluated 128 patients. Secondary endpoints included expression of collagen C7 and restoration of anchoring fibrils at three and six-months post-administration. Clinical data were presented at the Society of Investigative Dermatology (SID) conference by Stanford collaborators, and demonstrated that EB-101 treated wounds were significantly healed >50% for more than two years post-administration. The data included:

Wound healing, defined as >50% closure after EB-101 administration, was observed in:
--- *100% (36/36 treated wounds, n=6 subjects) at 3 months;*
--- *89% (32/36 treated wounds, n=6 subjects) at 6 months;*
--- *83% (20/24 treated wounds, n=4 subjects) at 12 months;*
--- *88% (21/24 treated wounds, n=4 subjects) at 24 months;*
--- *100% (6/6 treated wounds, n=1 subject) at 36 months post-administration.*

Collagen VII (C7) expression: C7 and morphologically normal NC2 reactive anchoring fibrils were observed in EB-101 treated wounds up to two years post-administration.

Importantly, data from a supportive natural history study of 1,436 wounds from 128 patients with RDEB, established by Stanford and EBCare Registry, were also presented at the conference and to the FDA. Notably, 13 RDEB patients with a total of 15 chronic wounds were treated with an allograft product, including Apligraf® and Dermagraft®. Of these wounds treated with allografts, only 7% (1/15 treated wounds) remained healed after 12 weeks, and 0% (0/15 treated wounds) remained healed after 24 weeks. This is a meaningful finding of the natural history study, as there are no approved therapies for RDEB patients that demonstrate significant wound closure after two months post-application.

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.

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

Media Contact:
Lynn Granito
Berry & Company Public Relations
+1 (212) 253-8881
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 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