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Abeona Receives FDA Regenerative Medicine Advanced Therapy Designation for EB-101 Gene Therapy in Epidermolysis Bullosa

- *First gene therapy RMAT designation for Epidermolysis Bullosa*
- *Enables accelerated approval path and real world data usage*

NEW YORK and CLEVELAND, Jan. 29, 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, announced today that the US Food and Drug Administration (FDA) has granted the Regenerative Medicine Advanced Therapy (RMAT) designation to EB-101, the Company's gene-corrected autologous cell therapy product for patients with recessive dystrophic epidermolysis bullosa (RDEB).

"EB-101 is an autologous gene-corrected cell therapy approach that utilizes a patient's own cells and genetically re-engineers them to produce the missing collagen protein, which helps hold skin on to the body. This reduces the number of painful blisters caused by injury and has demonstrated improved wound healing in our Phase 1/2 clinical trial for over 2 years," said Timothy J. Miller, Ph.D., President and CEO of Abeona. "The receipt of the RMAT and Breakthrough designations, both over the last six months, reaffirms the significance of the EB-101 clinical trial results and the need to advance promising therapies in areas of considerable unmet medical need. We are pleased that the FDA granted the RMAT designation, which will help accelerate the development of EB-101, and look forward to continuing our collaborative discussions in defining the pathway forward for the Phase 3 trial set to begin later this year."

Established under the 21st Century Cures Act, the RMAT designation is an expedited program for the advancement and approval of regenerative medicine products where preliminary clinical evidence indicates the potential to address unmet medical needs for life-threatening diseases or conditions. Similar to Breakthrough Therapy designation, the RMAT allows companies developing regenerative medicine therapies to work more closely and frequently with the FDA, and RMAT-designated products may be eligible for priority review and accelerated approval. In November 2017, the FDA expanded the RMAT designation for gene therapies. The sponsor of a RMAT therapy that is granted accelerated approval and is subject to post-approval requirements may, as appropriate, fulfill such requirements through submission of clinical evidence, clinical studies, patient registries, or other sources of real world data. For information on RMAT designation, visit the FDA website:

https://www.fda.gov/BiologicsBloodVaccines/CellularGeneTherapyProducts/ucm537670.htm

The Company continues to engage the FDA on its pivotal Phase 3 clinical trial design, 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 Breakthrough Therapy, 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: In the completed 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 for 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 and approximately 1500 chronic and recurring RDEB wounds. Secondary endpoints included expression of collagen C7 and restoration of anchoring fibrils at three and six-months post-administration. Clinical data 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% (42/42 treated wounds, n=7 subjects) at 3 months;
--- 90% (38/42 treated wounds, n=7 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 as early as 1 month in EB-101 treated wounds and have remained for at least two years post-administration. Importantly, data from a supportive natural history study of approximately 1,500 wounds from 128 patients with RDEB, established by Stanford and EBCare Registry, were also presented 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 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 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 is developing a proprietary vector platform, AIM™, for

next generation product candidates. For more information, visit www.abeonatherapeutics.com.

Investor Contact:
Christine Silverstein
SVP, Investor Relations & Finance
Abeona Therapeutics Inc.
+1 (646) 813-4707
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.