May 17, 2018

Abeona Reports Update from EB-101 Gene Therapy in Epidermolysis Bullosa at 21st Annual ASGCT Meeting

Phase 1/2 Study Update Confirms EB-101 is Safe and Well-tolerated, with Durable Efficacy

NEW YORK and CLEVELAND, May 17, 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 updated clinical data from the Phase 1/2 trial for EB-101, the company's gene-corrected skin graft cell therapy for patients suffering from recessive dystrophic epidermolysis bullosa (RDEB). The results demonstrate robust and durable clinical effects achieved throughout various timepoints post-administration. Results were reported today during the ASGCT (American Society for Gene and Cell Therapy) 21st Annual Meeting in Chicago, IL.

"RDEB patients suffer throughout their lives from intense pain, life-threatening complications, and face a shortened life expectancy. Currently there are no effective treatments available to reduce the severity of their symptoms," stated Carsten Thiel, Ph.D., Chief Executive Officer of Abeona. "The advancements made through this trial are clinically meaningful, showing significant and durable wound healing results and improved quality of life in these patients. Together with the FDA, we are working to finalize the design of our pivotal Phase 3 trial for this program and are defining the pathway forward for the study to begin later this year."

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.

Conducted at Stanford University School of Medicine, the 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. In the 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, tolerability and preliminary efficacy, where wound healing after EB-101 administration was compared to untreated wounds from a supportive natural history study that evaluated 128 patients with approximately 1,500 chronic and recurring RDEB wounds. Secondary endpoints included expression of collagen C7 and restoration of anchoring fibrils at three- and six-months post-administration respectively. The wound healing effects of EB-101 were associated with meaningful reductions in pain and itch, as reported through patient recorded outcomes. The data updated at the ASGCT conference 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;
--- 67% (24/36 treated wounds, n=6 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 one month in EB-101 treated wounds and have remained up to three years post-administration. Importantly, data from a supportive natural history study of 128 patients with RDEB with approximately 1,500 wounds, 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 an important 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.

The EB-101 program has been granted Regenerative Medicine Advanced Therapy, 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 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

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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 Section 21E of the Securities Exchange Act of 1934, 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 EB-101 clinical trials, 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, except as required by the federal securities laws.

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