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Abeona Therapeutics Receives Rare Pediatric Disease Designation for EB-101 Gene Therapy Product for Patients with Epidermolysis Bullosa

Abeona's Third Gene Therapy Program to Receive Rare Pediatric Disease Designation, expected to enable Priority Review Voucher

NEW YORK and CLEVELAND, May 30, 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 FDA has granted Rare Pediatric Disease Designation for Abeona's EB-101 gene therapy program for patients with dystrophic epidermolysis bullosa (DEB), including recessive dystrophic epidermolysis bullosa (RDEB), which are life-threatening genetic skin disorders characterized by skin blisters and erosions that cover the body.

"These designations are granted to drugs with high promise that may address areas of unmet medical need for children with rare diseases. RDEB is a debilitating and life threatening inherited disorder with no approved treatment options available for patients today," stated Timothy J. Miller, Ph.D., President & CEO of Abeona Therapeutics Inc. "Building upon the already granted FDA and EMA Orphan Drug Disease Designations for the EB-101 gene therapy program, receiving the Rare Pediatric Disease Designation is another important validation of the science and clinical approach to developing a novel gene therapy for RDEB patients."

Typically, wounds on patients with RDEB, also known as "butterfly skin" syndrome, can remain unhealed for months to years due to the inability of the skin to stay attached to the underlying dermis and can cover a large percentage of the body. In the ongoing 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 over years. The primary endpoints of the clinical trial assess safety and evaluate wound healing after EB-101 administration compared to control untreated wounds. Secondary endpoints include expression of collagen C7 and restoration of anchoring fibrils at three and six months post-administration.

About Rare Pediatric Disease Designation: The rare pediatric disease designation indicates that the FDA may give the company a pediatric priority review voucher if the drug is approved for the pediatric indication. That voucher could then be used by the company for another drug—any drug—to be given a priority review. A priority review mandates that the FDA will review a BLA drug submission within six months instead of the standard 10 months. Normally, a priority review designation would only be given to a drug that is for a serious condition and has demonstrated the potential to be a significant improvement in safety and

effectiveness. The priority review voucher may be used by the sponsor, sold or transferred.

EB-101 Gene Therapy Program Highlights:

- Clinical data were recently presented at the Society of Investigative Dermatology (SID) conference by the Company's Stanford University collaborators, and demonstrated that EB-101 treated wounds were significantly healed >50% for more than 2 years post-administration.
    - *EB-101 demonstrated significant wound healing (defined as greater than 50% healed) in 100% of treated wounds (36/36) at 3 months; 89% (32/36) at 6 months, 83% (20/24) at 12 months, 88% (21/24) at 24 months and 100% (6/6) at 36 months post-administration.*
    - *Collagen VII (C7) expression: C7 and morphologically normal NC2 reactive anchoring fibrils – the "zipper" that holds skin onto the underlying tissue and the primary deficit in RDEB patients – were observed in EB-101 treated wounds up to two years post administration.*

- *Supportive Natural History Study:* A natural history study of 1,436 wounds from 128 patients with RDEB, established by Stanford and EBCare Registry, were also presented at the conference. Notably, in the natural history study, 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.

- The EB-101 program has also been granted orphan drug designation from FDA and the European Medicines Agency (EMA).

About EB-101: EB-101 is an autologous, *ex-vivo* gene therapy in which COL7A1 is transduced into autologous keratinocytes for the treatment of Recessive Dystrophic Epidermolysis Bullosa (RDEB). RDEB is a subtype of an inherited genetic skin disorder characterized by chronic skin blistering, open and painful wounds, joint contractures, esophageal strictures, pseudosyndactyly, corneal abrasions and a shortened life span. Patients with RDEB lack functional type VII collagen owing to mutations in the gene COL7A1 that encodes for C7 and is the main component of anchoring fibrils, which stabilize the dermal-epidermal basement membrane. Patients are being enrolled in the ongoing Phase 2 portion of the Phase 1/2 clinical trial (NCT01263379). The EB-101 program has also been granted orphan drug designation by the FDA and European Medicines Agency (EMA).

About Epidermolysis Bullosa (EB): EB is a group of devastating, life-threatening genetic skin disorders that is characterized by skin blisters and erosions all over the body. The most severe form, recessive dystrophic epidermolysis bullosa (RDEB), is characterized by chronic skin blistering, open and painful wounds, joint contractures, esophageal strictures, pseudosyndactyly, corneal abrasions and a shortened life span. Patients with RDEB lack functional type VII collagen (C7) owing to mutations in the gene COL7A1 that encodes for C7 and is the main component of anchoring fibrils that attach the dermis to the epidermis.

EB patients suffer through intense pain throughout their lives, with no effective treatments available to reduce the severity of their symptoms. Along with the life-threatening infectious complications associated with this disorder, many individuals often develop an aggressive form of squamous cell carcinoma (SCC).

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 plasma-based protein therapy pipeline, including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD, using its proprietary SDF™ (Salt Diafiltration) ethanol-free process. 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, the expected receipt of a Priority Review Voucher 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