May 2, 2017

Abeona Therapeutics Provides Update on EB-101 Gene Therapy for Severe Form of Epidermolysis Bullosa from the Society for Investigative Dermatology Conference

--EB-101 Demonstrates 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

--Clinical Endpoints Supported by Data from Natural History Study Observations from 1,436 Wounds in 128 patients with Recessive Dystrophic Epidermolysis Bullosa (RDEB)

NEW YORK and CLEVELAND, May 02, 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, announces updated clinical data from the ongoing Phase 1/2 clinical trial for the EB-101 gene therapy program for patients with Recessive Dystrophic Epidermolysis Bullosa (RDEB), a severe form of epidermolysis bullosa (EB), along with supportive natural history data for 128 patients with the fatal skin disease. These data were presented at the recently held Society for Investigative Dermatology (SID) conference by Abeona's scientific and clinical collaborators at Stanford University School of Medicine, a center of excellence for the treatment of patients with epidermolysis bullosa.

"Last week at the SID conference, our EB-101 team of clinical investigators and scientific collaborators presented data from the ongoing Phase 1/2 gene therapy clinical trial and a supportive natural history study of patients with RDEB that highlight the unprecedented wound healing and durable collagen C7 expression of four patients through two years post-treatment, including one patient that has continued to see EB-101 treated wounds remain healed three years post-treatment. The relevance of these benefits is highlighted when compared to non-treated control wounds evaluated from the 128-patient natural history study, which showed that RBEB patients suffer chronic and recurrent wounds that do not heal on their own and persist for several years," said Timothy J. Miller, Ph.D., President and CEO.

In the Phase 1/2 trial, EB-101 was administered to non-healing chronic wounds [mean length of time wounds were unhealed (unclosed) was 8.5 years prior to the gene therapy administration] on each subject and assessed for wound healing at predefined time points over years. The primary endpoint of the clinical trial is to assess safety and evaluate wound closure after EB-101 administration compared to control untreated wounds. Secondary endpoints include expression of full-length collagen C7 and restoration of anchoring fibrils at three and six months post-administration.

As reflected at the conference by Stanford collaborators, wounds were evaluated at three, six, 12, 24 and 36 months for appearance, durability, and resistance to blistering**:

Wound healing >50%: 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.

Wound healing >75%: defined as >75% closure after EB-101 administration was observed in:
- 83% (30/36 treated wounds, n=6 subjects) at 3 months;
- 61% (22/36 treated wounds, n=6 subjects) at 6 months;
- 50% (12/24 treated wounds, n=4 subjects) at 12 months;
- 71% (17/24 treated wounds, n=4 subjects) at 24 months;
- 83% (5/6 treated wounds, n=1 subject) at 36 months post-administration.

Collagen VII (C7) expression observed: C7 and morphologically normal NC2 reactive anchoring fibrils – the "zipper" that holds skin onto the underlying tissue and the primary deficit in RDEB patients – have been observed in EB-101 treatments up to two years post administration.

Data from a supportive natural history study of 1,436 wounds of 128 patients with RDEB, established by Stanford and EBCare Registry, were also presented at the conference. The natural history study characterized both chronic non-healing wounds, defined as an area that does not heal ≥12 weeks, and recurrent wounds, defined as an area that partially heals but then easily re-blisters. Results presented were characterized as 1041 recurrent wounds and 395 chronic open wounds. 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.

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. Investigators at Stanford University are enrolling patients for the ongoing Phase 2 portion of the Phase 1/2 clinical trial (NCT01263379). The EB-101 program has been granted orphan drug designation from the European Medicines Agency (EMA).

** Information presented in this press release updates and replaces data from earlier

abstracts included in the press release of April 25, 2017.
*** Apligraf® and Dermagraft® are registered trademarks of Organogenesis Inc.

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, 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 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