July 18, 2017

Abeona Therapeutics Receives Guidance from FDA to Commence Pivotal Phase 3 for EB-101 Gene Therapy for Patients with Epidermolysis Bullosa

Pivotal Phase 3 clinical trial is planned to commence in early 2018

EB-101 gene therapy for patients with Recessive Dystrophic Epidermolysis Bullosa (RDEB) has demonstrated promising efficacy and safety in the Phase 1/2 clinical trial

EB-101 has received Orphan Drug and Rare Pediatric Disease Designations in the USA and Orphan Drug Designation in the EU

NEW YORK and CLEVELAND, July 18, 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 guidance from a recent Type-C meeting with the FDA which has recommended accelerating the EB-101 program into a pivotal Phase 3 trial. 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.

“The FDA guidance is an important milestone in our clinical development plan for EB-101, and we are pleased to be moving forward into a registrational Phase 3 clinical study in 2018. Abeona is committed to advancing innovative gene therapies that address the unmet needs of patients suffering with dystrophic epidermolysis bullosa, a devastating rare skin disease. We are grateful that the FDA has recognized EB-101 as a rare disease product that addresses the underlying disease pathology to offer significant therapeutic benefit for RDEB patients, and we look forward to the collective work ahead in advancing this therapy,” stated Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics Inc.

Abeona’s EB-101 product is an autologous, *ex-vivo* gene 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).

“EB Research Partnership (EBRP), along with EB Medical Research Foundation, are honored to have helped support EB-101 development by the dedicated researchers at Stanford University. Their tireless efforts in combination with Abeona’s leadership is driving

real progress in the RDEB patient community. EBRP is encouraged by clinical results to date and looks forward to realizing the promise of EB-101 in addressing the devastating effects on RDEB patients' quality of life and disease burden," stated Alexander Silver, co-founder and chairman EB Research Partnership.

About EB-101 Phase 1/2 Clinical Trial:
In the Phase 1/2 clinical trial, EB-101 was administered to non-healing chronic wounds on each subject which were assessed for wound healing at predefined time points up to several years to date. The primary endpoints of the clinical trial assessed safety and evaluated wound healing after EB-101 administration compared to control untreated wounds. 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 Epidermolysis Bullosa (EB) and Recessive Dystrophic Epidermolysis Bullosa (RDEB): EB is a group of devastating, life-threatening genetic skin disorders 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, pseudosyndactyly and a shortened life span. 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. Patients with RDEB lack functional type VII collagen owing to mutations in the gene COL7A1 that produces C7 collagen and is the main component of anchoring fibrils, the "velcro" that helps stabilize the skin on the basement membrane.

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 EB Research Partnership (EBRP): EBRP is the largest 501(c)(3) nonprofit dedicated to funding research aimed at treating and ultimately curing Epidermolysis Bullosa, a group of devastating and life-threatening skin disorders that affect children from birth. EBRP uses a sustainable philanthropic model via venture philanthropy for all of its research commitments. To learn more, please visit www.ebresearch.org.

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.

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

Media Contact:
Andre'a Lucca
Vice President, Communications & Operations
Abeona Therapeutics Inc.
+1 (212)-786-6208
alucca@abeonatherapeutics.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