March 17, 2020

Abeona Therapeutics Announces First Patient Treated in Pivotal Phase III Clinical Trial Evaluating EB-101 Gene Therapy for Recessive Dystrophic Epidermolysis Bullosa

Majority of potential study participants have been pre-screened

EB-101 successfully manufactured at Abeona and transplanted at Stanford University Medical Center

NEW YORK and CLEVELAND, March 17, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that investigators at Stanford University Medical Center have treated the first patient in the pivotal phase III VIITAL™ study evaluating EB-101, the Company's gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa (RDEB).

"Treating the first patient in our pivotal Phase III VIITAL™ study is an important achievement for the EB-101 program, now the most advanced gene therapy program in RDEB," said João Siffert, M.D., Chief Executive Officer. "This achievement confirms that Abeona can deliver EB-101 in a study setting that closely parallels its potential real-world application. We remain confident that VIITAL™ will replicate results from the Phase I/II trial demonstrating that EB-101 treatment resulted in sustained and durable wound healing with a favorable safety profile."

The VIITAL™ Phase III study is a multi-center, randomized clinical trial assessing EB-101 in up to 15 RDEB patients, with approximately 30 large, chronic wound sites treated in total. The primary outcome measure is wound healing, comparing treated with untreated wound sites on the same patient. Secondary endpoints include the assessments of pain, as well as other patient reported outcomes. Investigators at Stanford University Medical Center are currently enrolling eligible patients into the VIITAL™ study and preparations for an additional clinical site initiation are ongoing. Additional information about the trial is available at abeonatherapeutics.com/clinical-trials/rdeb.

Abeona is producing EB-101 for the VIITAL™ study at the Elisa Linton Center for Rare Disease Therapies, its fully-functional gene and cell therapy manufacturing facility centrally-located in Cleveland, OH. The 26,000 ft^2 center is housing large-scale cGMP capacity for AAV gene therapy and EB-101 cell therapy manufacturing, and state-of-the-art laboratories to support CMC development for process and analytics, all of which is validated and governed by comprehensive quality systems and overseen by experienced staff.

About EB-101
EB-101 is an autologous, gene-corrected cell therapy in late-stage clinical development for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), a rare connective tissue disorder without an approved therapy. Treatment with EB-101 involves using gene transfer to deliver COL7A1 genes into a patient's own skin cells (keratinocytes and its progenitors) and transplanting them back to the patient to enable normal Type VII collagen expression and facilitate wound healing. Data from a Phase I/IIa clinical trial conducted by Stanford University evaluating EB-101 showed that the gene-corrected cell therapy provided durable wound healing for RDEB patients lasting 2+ to 5+ years, including for the largest, most challenging wounds that affect the majority of the RDEB population. In the U.S., Abeona holds Regenerative Medicine Advanced Therapy, Breakthrough Therapy, and Rare Pediatric designations for EB-101 and Orphan Drug designation in both the U.S. and EU.

About Recessive Dystrophic Epidermolysis Bullosa
Recessive dystrophic epidermolysis bullosa (RDEB) is a rare connective tissue disorder characterized by severe skin wounds that cause pain and can lead to systemic complications impacting the length and quality of life. People with RDEB have a defect in the COL7A1 gene, leaving them unable to produce functioning Type VII collagen which is necessary to anchor the dermal and epidermal layers of the skin. There is currently no approved treatment for RDEB.

About Abeona Therapeutics
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and cell therapies for serious diseases. The Company's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa, as well as ABO-102 and ABO-101, novel AAV9-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively. The Company's portfolio of AAV9-based gene therapies also features ABO-202 and ABO-201 for CLN1 disease and CLN3 disease, respectively. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates, including Regenerative Medicine Advanced Therapy designation for two candidates (EB-101 and ABO-102). www.abeonatherapeutics.com

Forward Looking Statement
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 statements about the Company's clinical trials, including the timing and success thereof; the Company's products and product candidates; EB-101 can provide durable healing in large, chronic wounds that afflict many RDEB patients; future regulatory interactions with regulatory authorities; and the Company's goals and objectives. We have attempted to identify forward-looking statements by such terminology as "may," "will," "believe," "estimate," "expect," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, 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 outcome of any future meetings with the U.S. Food and Drug Administration or other regulatory agencies, the impact of competition, the ability to secure licenses for any technology that may be

necessary to commercialize our products, the ability to achieve or obtain necessary regulatory approvals, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and quarterly reports on Form 10-Q and other periodic reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligation to revise these forward-looking statements or update them to reflect events or circumstances occurring after the date of this presentation, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

Investor Contact:
Dan Ferry
LifeSci Advisors, LLC
+1 (617) 535-7746
daniel@lifesciadvisors.com

Media Contact:
Scott Santiamo
Director, Corporate Communications
Abeona Therapeutics
+1 (718) 344-5843
ssantiamo@abeonatherapeutics.com

Source: Abeona Therapeutics Inc.