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Abeona Therapeutics Initiates Pivotal Phase 3 Clinical Trial Evaluating EB-101 Gene Therapy for Recessive Dystrophic Epidermolysis Bullosa

Study open for enrollment and patient screening underway at Stanford University

Company expects first patient to be treated in the first quarter of 2020

EB-101 manufactured at Abeona facility in Cleveland

NEW YORK and CLEVELAND, Jan. 13, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that it has received Institutional Review Board (IRB) approval from Stanford University to commence the VIITAL™ study, the Company's pivotal Phase 3 clinical trial evaluating EB-101 for the treatment of recessive dystrophic epidermolysis bullosa (RDEB). The majority of patients targeted for enrollment have completed the pre-screening process at Stanford, and the Company expects the first patient in the VIITAL™ study to be treated in the first quarter of 2020.

"We expect 2020 to be a transformational year at Abeona, and we are proud to start it with the initiation of our pivotal Phase 3 study evaluating EB-101 in RDEB," said João Siffert, M.D., Chief Executive Officer. "We look forward to the first patient receiving EB-101 this quarter, setting in motion the final stages of this important program. We have devoted significant effort to establish and validate our independent, fully-functional GMP facility that will produce EB-101 for the VIITAL™ study and has capacity to support commercial launch. EB-101 has the potential to be the first approved therapy for RDEB and the only durable treatment to address large chronic wounds, which are the most painful and debilitating."

The VIITAL™ Phase 3 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 study endpoint is the proportion of wounds with greater than 50% healing at three months, comparing treated with untreated wound sites on the same patient. Secondary endpoints include the patient's global impression of change from baseline in pain as well as other patient reported outcomes assessing pain during dressing changes, pain impact and physical function. Investigators at Stanford University Medical Center are currently enrolling eligible patients into the VIITAL™ study, with additional study sites expected to be added in the coming months. Additional information about the trial is available at abeonatherapeutics.com/clinical-trials/rdeb.

Abeona will produce EB-101 for the pivotal 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 center is a 26,000 ft^2 facility housing large-scale cGMP capacity 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. The facility is also capable of clinical production of the Company's AAV gene therapies.

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 gene transfer to deliver COL7A1 genes into a patient's own skin cells (keratinocytes) 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 several 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. Its preclinical assets include ABO-401, which uses a novel vector from Abeona's AIM™ AAV capsid platform to address all mutations of cystic fibrosis. 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.

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