December 9, 2019

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

FDA removes clinical hold; Company may proceed with VIITAL™ study

Company expects to initiate study in the first quarter of 2020

Primary endpoint confirmed as proportion of wounds with greater than 50% healing at 3 months vs control wounds

Majority of potential subjects have been pre-screened for the study

NEW YORK and CLEVELAND, Dec. 09, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that the U.S. Food and Drug Administration (FDA) has removed its clinical hold and provided clearance to proceed with the VIITAL™ study, the Company's pivotal Phase 3 clinical trial evaluating EB-101 for the treatment of recessive dystrophic epidermolysis bullosa (RDEB). The FDA removed the clinical hold following the Company's submission of additional data points on transport stability of EB-101 to clinical sites. Abeona expects to initiate the VIITAL™ study in first quarter of 2020.

"The Abeona team has worked diligently to provide a prompt and thorough response to the FDA, enabling us to proceed with our pivotal Phase 3 trial for EB-101," said João Siffert, M.D., Chief Executive Officer of Abeona. "Recently published long-term follow up data from our Phase 1/2 trial leaves us increasingly confident that EB-101 can provide durable healing for large, chronic wounds that afflict many RDEB patients. We are now focused on initiating the VIITAL™ study in the first quarter of 2020. The success in building and qualifying a state-of-the-art GMP manufacturing facility also represents a critical step toward bringing this novel product to patients in dire need of effective treatment."

With two to five years of follow-up, data from a Phase 1/2 clinical trial conducted by Stanford University evaluating EB-101 showed that the gene-corrected cell therapy provided durable wound healing for RDEB patients, including for the largest, most challenging wounds that constitute the majority of wounds in this population.

About The VIITAL™ Study
The VIITAL™ Phase 3 study will be a multi-center, randomized clinical trial assessing EB-101 in 10 to 15 RDEB patients, with approximately 30 chronic wound sites treated in total. The primary study endpoint will be 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 in pain from baseline as well as other patient reported outcomes assessing pain during dressing changes, pain impact and physical function.

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 transplanting them back to the patient to enable normal Type VII collagen expression and facilitate wound healing. 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 the novel AIM™ AAV vector platform to address all mutations of cystic fibrosis. Abeona has received twenty regulatory designations from the FDA and EMA for its pipeline candidates. For more information, visit www.abeonatherapeutics.com.

Forward-Looking Statements
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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