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Abeona Therapeutics Announces Database Lock for Pivotal Phase 3 VIITAL™ Study of EB-101 in Patients with Recessive Dystrophic Epidermolysis Bullosa (RDEB)

NEW YORK and CLEVELAND, Oct. 19, 2022 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today announced the database lock on October 18, 2022 for the pivotal Phase 3 VIITAL™ study of its investigational autologous, engineered cell therapy, EB-101, in patients with recessive dystrophic epidermolysis bullosa (RDEB). With the database now locked, the Company remains on track to report topline results in the next two to three weeks, consistent with guidance provided in early-October.

"I want to thank the clinical team at Abeona and our study sites Stanford and UMass for their collaborative effort and speed in achieving this milestone in about two weeks following completion of the last patient visit," said Vish Seshadri, Chief Executive Officer of Abeona.

Abeona expects the VIITAL™ study, if positive, to serve as a basis for seeking approval by the U.S. Food and Drug Administration (FDA) of EB-101 for the treatment of patients with RDEB.

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 EB-101

EB-101 is an autologous, engineered cell therapy currently being investigated in Abeona's pivotal Phase 3 VIITAL™ study for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), a rare connective tissue disorder without an approved therapy. The EB-101 VIITAL™ study is a randomized clinical trial with target enrollment of at least 10 to 15 RDEB patients with approximately 36 large, chronic wound sites treated in total. Treatment with EB-101 involves using gene transfer to deliver the COL7A1 gene into a patient's own skin cells (keratinocytes and its progenitors) and transplanting those cells back to the patient. EB-101 is being investigated for its ability to enable normal Type VII collagen expression and to facilitate wound healing. The U.S. FDA has granted Rare Pediatric Disease Designation for EB-101. Abeona produces EB-101 for the VIITAL™ study at its fully integrated gene and cell

therapy manufacturing facility in Cleveland, Ohio. EB-101 is an investigational product not yet approved by the FDA.

About Abeona Therapeutics

Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for serious diseases. Abeona's lead clinical program is EB-101, its investigational autologous, engineered cell therapy for recessive dystrophic epidermolysis bullosa is in Phase 3 development. The Company's development portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical need. Abeona's novel, next-generation AAV capsids are being evaluated to improve tropism profiles for a variety of devastating diseases. Abeona's fully integrated cell and gene therapy cGMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and potential commercial production of AAV-based gene therapies. 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. We have attempted to identify forward-looking statements by such terminology as “may,” “will,” “believe,” “plan to,” “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, our ability to report topline results from our Phase 3 VIITAL™ study and the timing thereof, our ability to continue as a going concern; the outcome of any future meetings with the U.S. Food and Drug Administration or other regulatory agencies; the ability to achieve or obtain necessary regulatory approvals; and other risks disclosed in the Company's most recent Annual Report on Form 10-K and subsequent periodic reports filed with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or to update them to reflect events or circumstances occurring after the date of this press release, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

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