September 26, 2023

Abeona Therapeutics Submits Biologics License Application to U.S. FDA Seeking Priority Review and Approval of EB-101 for the Treatment of Patients with Recessive Dystrophic Epidermolysis Bullosa

CLEVELAND, Sept. 26, 2023 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today announced the Company has submitted a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) seeking approval of EB-101, its investigational autologous, engineered cell therapy, as a treatment for patients with recessive dystrophic epidermolysis bullosa (RDEB). As part of the submission, Abeona requested a Priority Review, which, if granted, would shorten the FDA's review period to six months from the filing acceptance of the BLA, instead of 10 months under standard review.

"The BLA submission for EB-101 is a historic milestone for Abeona and a critical step toward making our investigational EB-101 product an option for RDEB patients as the first individualized cell therapy with potential to provide years of wound healing and pain reduction following a one-time application," said Vish Seshadri, Chief Executive Officer of Abeona. "We appreciate the FDA's level of engagement and constructive guidance in the months leading up to the pre-BLA meeting. I am also grateful to the entire submission team for their tremendous dedication and effort in completing Abeona's first BLA submission."

The BLA submission for EB-101 followed ongoing discussions with the FDA and is supported by clinical efficacy and safety data from the pivotal Phase 3 VIITAL™ study (NCT04227106) and confirmatory evidence from a Phase 1/2a study (NCT01263379). Data from the VIITAL™ study were presented during the inaugural International Societies for Investigative Dermatology (ISID) Meeting in May 2023. Long-term follow up data up to eight years and quality of life data from the Phase 1/2a study were published in *Orphanet Journal of Rare Diseases*.

The FDA's decision on BLA acceptance is typically made during the 60-day window following submission. If accepted with Priority Review, Abeona expects potential BLA approval in the second quarter of 2024, at which time, Abeona believes that it would be eligible to receive a Priority Review Voucher. The voucher, if granted, could be used by the Company to accelerate the review of a future BLA or New Drug Application, or be sold to a third party. EB-101 has been granted Rare Pediatric Disease, Regenerative Medicine Advanced Therapy, Breakthrough Therapy and Orphan Drug designations.

About Recessive Dystrophic Epidermolysis Bullosa
Recessive dystrophic epidermolysis bullosa (RDEB), a rare connective tissue disorder

without a cure, is 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.

About EB-101

EB-101 is an autologous, engineered cell therapy currently being developed for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), a rare connective tissue disorder without a cure. The pivotal Phase 3 VIITAL™ study is a randomized clinical trial that evaluated the efficacy, safety and tolerability of EB-101 in 43 large chronic wound pairs in 11 subjects with RDEB. Treatment with EB-101 involves using gene transfer to deliver the functional 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. EB-101 has been granted Regenerative Medicine Advanced Therapy, Breakthrough Therapy, Orphan Drug and Rare Pediatric Disease designations by the U.S. FDA. 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 currently in development for recessive dystrophic epidermolysis bullosa. 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 produced 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," "anticipate," "expect," "intend," "potential," and similar words and 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, the timing and outcome of our Biologics License Application submission to the FDA for EB-101; continued interest in our rare disease portfolio; our ability to enroll patients in clinical trials; the outcome of future meetings with the FDA or other regulatory agencies, including those relating to preclinical programs; the ability to achieve or obtain necessary regulatory approvals; the impact of any changes in the financial markets and global economic conditions; risks associated with data analysis and reporting; 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.

Investor and Media Contact:
Greg Gin
VP, Investor Relations and Corporate Communications
Abeona Therapeutics
ir@abeonatherapeutics.com

Source: Abeona Therapeutics Inc.