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Abeona Therapeutics Announces Positive Pre-BLA Meeting with FDA for EB-101 and Plans for BLA Submission

CLEVELAND, Aug. 30, 2023 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today announced the Company's intention to proceed with the submission of a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for EB-101, its investigational autologous, engineered cell therapy, for patients with recessive dystrophic epidermolysis bullosa (RDEB) after the recent completion of a pre-BLA meeting with the FDA.

At the meeting, Abeona reached alignment with the FDA that the EB-101 clinical efficacy and safety data appear adequate to support a BLA submission. The Agency also agreed that retroviral vector manufactured at Abeona and Indiana University appear comparable based on the data that Abeona provided in its briefing book. The FDA requested that Abeona include within its BLA submission additional background and data supporting the scientific rationale underlying its EB-101 potency and identity assays so that they can be fully evaluated by the Agency post-submission. The Agency also requested that supplemental data pertaining to certain chemistry, manufacturing, and controls and clinical topics be included in the BLA package. Abeona believes that it has the necessary supporting data in-hand to generate these additional reports, including those regarding potency and identity, to address the Agency's requests.

"We are pleased with the outcome of the pre-BLA meeting for EB-101 and believe that we have aligned with the FDA on what is needed for our upcoming BLA submission," said Vish Seshadri, Chief Executive Officer of Abeona. "We are focused on gathering and packaging the existing data over the coming weeks to meet the Agency's expectations. With the constructive feedback from the FDA now in-hand, we are proceeding on a clear regulatory path leading to the planned BLA submission for EB-101 early this Fall."

EB-101 has been granted Regenerative Medicine Advanced Therapy, Breakthrough Therapy, Orphan Drug and Rare Pediatric Disease designations 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," 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, 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.

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