

March 14, 2022



# **Abeona Therapeutics Achieves Target Enrollment in Pivotal Phase 3 VIITAL™ Study of EB-101 in RDEB**

## **Topline data from VIITAL™ expected in the third quarter of 2022**

NEW YORK and CLEVELAND, March 14, 2022 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that target enrollment has been achieved in its pivotal Phase 3 VIITAL™ study. The objective of VIITAL™ is to evaluate the safety and effectiveness of Abeona's investigational EB-101 product for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), 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. Large chronic wounds typically do not heal spontaneously and inflict the greatest pain and clinical burden on RDEB patients. Large chronic wounds treated in VIITAL™ measured greater than 20 cm<sup>2</sup> of surface area and had remained open for more than six months. In a phase 1/2a study conducted at Stanford University, large chronic wounds treated with EB-101 showed considerable wound healing and reduction in associated long-term pain for up to six years.

The EB-101 pivotal VIITAL™ study treated patients at Stanford University Medical Center in Palo Alto, CA and at UMass Memorial Medical Center in Worcester, MA and met the goal of randomizing a minimum of 36 wound pairs (i.e., each pair has a treated and untreated wound) in a minimum of 10 patients. The co-primary endpoints of the study are: 1) the proportion of randomized RDEB wound pairs with greater than or equal to 50% healing from baseline at Week 24 (Month 6), as determined by direct investigator assessment; and 2) pain reduction associated with wound dressing change assessed by the mean differences in scores of the Wong-Baker FACES scale between randomized wound pairs at Week 24 (Month 6). Given that the primary endpoint is measured at 24 weeks post treatment, the Company anticipates topline results in the third quarter of 2022.

"Meeting the target accrual of VIITAL™ marks an important milestone for Abeona and underscores the high unmet need in RDEB where there are currently no approved therapies," said Vish Seshadri, Ph.D., M.B.A., Chief Executive Officer of Abeona. "We thank the patients, families, and the clinical investigators at Stanford and UMass for participating in this study. We are especially grateful to our EB-101 program team for their relentless efforts in operationalizing the in-house production of both the retroviral vector and the autologous gene corrected epidermal sheets, and for ensuring that every patient biopsied to date in VIITAL has received EB-101 drug product. We look forward to sharing topline study results in the third quarter of 2022."

Additional information about the VIITAL™ study is available at <https://www.abeonatherapeutics.com/clinical-trials/rdeb> and <https://clinicaltrials.gov/> (Identifier: NCT04227106).

### **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, gene-corrected 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 gene and cell therapies for serious diseases. Abeona's clinical programs include EB-101, its investigational autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, as well as ABO-102 and ABO-101, novel investigational AAV-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively, in Phase 1/2 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 gene and cell therapy cGMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and planned commercial production of AAV-based gene therapies. For more information, visit [www.abeonatherapeutics.com](http://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," "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 the potential impacts of the COVID-19 pandemic on our business, operations, and financial condition, 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, our ability to execute our operating plan and achieve important anticipated milestones, and other risks disclosed in the Company's most recent Annual Report on Form 10-K and subsequent quarterly reports on Form 10-Q and other 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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Source: Abeona Therapeutics Inc.