

January 21, 2020



# Abeona Therapeutics Announces Participation in Inaugural World Congress on Epidermolysis Bullosa

NEW YORK and CLEVELAND, Jan. 21, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced its participation in the first World Congress on Epidermolysis Bullosa (EB2020), which is convening the world's leading experts on epidermolysis bullosa (EB) research and clinical management in London, January 19-23, 2020. Organized by The Global EB Alliance, EB2020 is a forum to share state-of-the art knowledge on this devastating disease and to debate the direction of future research.

Dr. Jean Tang of Stanford University Medical Center will present updated recessive dystrophic epidermolysis bullosa (RDEB) natural history data on large wounds and highlights from a completed Phase I/II clinical trial of Abeona's investigational EB-101 gene therapy for RDEB. The presentation is part of the Clinical Trials and Research Program track at the congress. Details of the presentation are as follows:

*Large wounds: an update on natural history data and EB-101*

Jean Tang, M.D., Ph.D.

Professor of Dermatology, Stanford University Medical Center

Tuesday, January 21

14:25 GMT

## 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 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 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. Data from a Phase I/IIa clinical trial conducted by Stanford University evaluating EB-101 showed that the gene-corrected cell therapy provided durable wound healing for RDEB patients lasting several 2+ to 5+ years, including for the largest, most challenging wounds that affect the majority of the RDEB population. 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 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. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates, including Regenerative Medicine Advanced Therapy designation for two candidates (EB-101 and ABO-102). [www.abeonatherapeutics.com](http://www.abeonatherapeutics.com)

### **Forward Looking Statement**

*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 regarding our pipeline including the potential use of investigational product EB-101 in the treatment of recessive dystrophic epidermolysis bullosa (RDEB). We have attempted to identify forward-looking statements by such terminology as "may," "will," "anticipate," "believe," "estimate," "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 continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, 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 risk of whether or when the Company will complete its Phase 3 clinical trial for EB-101 and any potential results thereof, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, and other risks as maybe detailed from time to time in the Company's Annual Reports on Form 10-K and quarterly reports on Form 10-Q and other reports filed by the Company 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 presentation, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.*

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