May 8, 2019



# Abeona Therapeutics Announces Presentation of EB-101 Data at Society for Investigative Dermatology Annual Meeting

NEW YORK and CLEVELAND, May 08, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that long-term follow up data from a completed Phase 1/2 study evaluating EB-101 for the treatment of recessive dystrophic epidermolysis bullosa (RDEB) will be presented at the 77<sup>th</sup> Annual Meeting of the Society for Investigative Dermatology (SID), being held May 8-11 in Chicago. The data will be presented by Stanford University researcher Shaundra Eichstadt, M.D. in two forums at the meeting, as detailed below.

As previously reported, these data showed that three years after treatment with EB-101, a majority of RDEB patients had durable wound healing. Notable improvements were also reported in outcomes associated with wound healing, including patient reported reductions in pain and itching. No serious treatment-related adverse events were observed at three years, and no replication competent virus was present at any time point.

Genetically corrected autologous keratinocyte epidermal grafts improve wound healing and patient reported outcomes in patients with recessive dystrophic epidermolysis bullosa (RDEB)

Selected e-Poster Discussion I Thursday, May 9, 11:15 a.m. – 12:15 p.m. CT Poster Session III Saturday, May 11, 12:45 – 2:45 p.m. CT

#### About EB-101

EB-101 is an investigational, autologous, gene-corrected cell therapy poised to enter latestage development for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), a rare connective tissue disorder without an approved therapy. Treatment with EB-101 involves using 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 skin function. 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 Recessive Dystrophic Epidermolysis Bullosa

Recessive dystrophic epidermolysis bullosa, or RDEB, is a rare connective tissue disorder without an approved therapy in which patients suffer with severe epidermal wounds that bring pain, itching, and widespread complications impacting the length and quality of their lives. People with RDEB have a defect in the COL7A1 gene, leaving them unable to produce Type VII collagen that helps anchor the dermal and epidermal layers of the skin.

### 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). The Company's portfolio of AAV9-based gene therapies also features ABO-202 and ABO-201 for CLN1 disease and CLN3 disease, respectively. Its preclinical assets include ABO-401, which uses the novel AIM<sup>™</sup> AAV vector platform to address all mutations of cystic fibrosis. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates and is the only company with Regenerative Medicine Advanced Therapy designation for two investigational therapies (EB-101 and ABO-102). For more information, visit 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) based upon pre-clinical data. We have attempted to identify forward looking statements by such terminology as "may," will," "anticipate," "believe," "estimate," "expect," "intend," and similar expressions.

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 initiate and 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 impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, and other risks as may be 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 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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