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Abeona Therapeutics Announces Publication of Positive Long-Term Data from Phase 1/2a Clinical Trial Evaluating EB-101 Gene Therapy for Recessive Dystrophic Epidermolysis Bullosa

Sustained wound healing and favorable safety profile observed at three years post-treatment

Durable wound healing in large, disabling, chronic wounds

EB-101 associated with long-term molecular expression of Type VII collagen protein

NEW YORK and CLEVELAND, Oct. 15, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced positive long term efficacy and safety results from its Phase 1/2a clinical trial evaluating EB-101, a gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa (RDEB). Treatment with EB-101 resulted in sustained wound healing with a favorable safety profile at three years post-treatment. These long-term follow-up data in seven RDEB participants with 42 treated wounds were published in the peer-reviewed journal *JCI Insight*. Full text of the publication can be accessed here: https://insight.jci.org/articles/view/130554

“These results bolster our belief that EB-101 is a safe and effective gene-corrected cell therapy capable of providing durable, long-lasting healing for the most disabling wounds in patients with RDEB,” said João Siffert, M.D., Chief Executive Officer of Abeona. “These results are particularly significant, as EB-101 treatment led to wound healing even in the most challenging to treat large and painful chronic wounds. Given the average RDEB chronic wound size is over 118 cm^2, it is essential that potential new treatments are capable of addressing these wounds to improve quality of life. We thank our collaborators at Stanford and the patients who volunteered to participate in this study and look forward to building upon this strong clinical foundation with the initiation of the pivotal Phase 3 VIITAL™ Study evaluating EB-101 for the treatment of RDEB.”

Key Study findings include:

- Wounds selected for treatment were present for a mean of 11.2 years (range 3-20 years)

- Three years after treatment with EB-101, a majority of RDEB patients had sustained wound healing, with 80% (16/20) of wounds achieving ³50% healing, and 70% (14/20)

achieving ≥75%

- Two years after treatment, only 1 of 6 untreated (17%), prospectively selected control wounds, had ≥50% healing

- 50% or greater wound healing was associated with no pain (0/16) and no itch (0/16) at treated sites three years post-treatment, compared with presence of pain in 53% (20/38) and itch in 61% (23/38) of wound sites at baseline

- EB-101 was associated with long-term molecular expression of type VII collagen protein, which plays an important role in anchoring the dermal and epidermal layers of the skin

- No serious treatment-related adverse events were observed during the three-year observation period

- No replication competent virus was present at any time point

Researchers from Stanford University School of Medicine conducted the Phase 1/2a single-center, open-label clinical trial to evaluate the long-term wound healing and safety of EB-101 in seven adult patients with severe generalized RDEB and to assess patient-reported outcomes following treatment. Chronic open wounds, defined as wounds present and unhealed for at least 12 weeks, with a total area of at least 100 cm^2, were required for enrollment. In the trial, gene-corrected EB-101 skin grafts (35 cm^2 each) were transplanted onto six wound sites in each of the seven adult participants (n= 42 sites total) and wounds selected for treatment had been present for a mean of 11.2 years (range: 3-20 years). Participants were followed for two to five years after transplantation of EB-101 and received standard of care therapies including iron supplementation and esophageal dilations during the study.

Abeona is currently continuing preparations for the pivotal Phase 3 VIITAL™ Study evaluating EB-101 for the treatment of RDEB pending the anticipated receipt of Chemical, Manufacturing and Controls (CMC) clearance from the U.S. Food and Drug Administration expected in Q4 2019.

About EB-101
EB-101 is an investigational, autologous, gene-corrected cell therapy poised to enter late-stage 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 facilitate wound healing. 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 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 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. Its preclinical assets include ABO-401, which uses the novel AIM™ 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 candidates (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 about the timing for CMC clearance for the VIITAL™ trial and the Company's beliefs relating thereto, the Company's ability to provide additional transport stability data points in response to the FDA clinical hold letter and the timing thereof, the Company's belief that completion of its CMC work and the durable safety and efficacy data will ultimately be critical to support a future Biologics License Application, the ability of its management team to lead the Company and deliver on key strategies, the market opportunities for the Company's products and product candidates, and the Company's goals and objectives. 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 FDA will lift the clinical hold respecting the Company's planned Phase 3 clinical trial for EB-101, 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.

Investor Contact:
Sofia Warner
Senior Director, Investor Relations
Abeona Therapeutics
+1 (646) 813-4710
swarner@abeonatherapeutics.com

Media Contact:
Scott Santiamo
Director, Corporate Communications
Abeona Therapeutics
+1 (718) 344-5843
ssantiamo@abeonatherapeutics.com

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