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Abeona Therapeutics Announces Updated EB-101 Phase 1/2a Clinical Results in Recessive Dystrophic Epidermolysis Bullosa at the Society for Pediatric Dermatology 46th Annual Meeting

NEW YORK and CLEVELAND, July 07, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced updated Phase 1/2a clinical trial results up to six years following treatment with Abeona's investigational EB-101 for recessive dystrophic epidermolysis bullosa (RDEB). The data showed that investigator assessment of wound healing of 50% or greater from baseline was present in 69% of treated wounds (n=18/26) at 3 years, 93% (n=14/15) at 4 years, 80% (n=12/15) at 5 years, and 80% (n=4/5) at 6 years. The results were reported in a poster, titled "Long-Term Healing, Pain Reduction, and Patient-Reported Outcomes in Recessive Dystrophic Epidermolysis Bullosa Following EB-101 Treatment of Large, Chronic Wounds," at the Society for Pediatric Dermatology (SPD) 46th Annual Meeting.

The updated results also included investigator assessment of the presence of pain in treated wounds up to six years following treatment with EB-101. The data showed that pain was absent in all treated wounds that were evaluated at 3 years (n=26), 4 years (n=15), 5 years (n=15) and 6 years (n=5) of follow up, compared with presence of pain at baseline in 53% (n=20/38) of treated wounds. A separate survey of patient-reported pain at 3 years to 6 years following treatment with EB-101 was conducted and asked participants to rate change in pain compared with their pre-treatment state using a seven-point scale, ranging from 1 (very much improved) to 7 (very much worse). The survey data showed that 76% of the treated wounds with healing of 50% or greater were associated with improved pain scores, with 53% associated with "much/very much improved" pain scores.

"RDEB is a debilitating and life-threatening rare genetic disorder with high rates of morbidity and mortality, without an approved treatment option," said Vishwas Seshadri, Ph.D., M.B.A., Head of Research & Clinical Development of Abeona. "It is important that potential new treatments can durably address large, chronic wounds, which are the most severe wounds that cause substantial pain in patients with RDEB. The updated Phase 1/2a results showed safety and durable efficacy follow up, with EB-101 treated wounds continuing to show a considerable reduction in both wound burden and associated long-term pain for up to six years. We are excited about the data and look forward to further investigating EB-101's potential to provide durable benefit in our ongoing pivotal Phase 3 VIITAL™ study."

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 enrolling 10 to 15 RDEB patients with approximately 35 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 believed to enable normal Type VII collagen expression and facilitate wound healing. Abeona produces EB-101 for the VIITAL™ study at its fully integrated gene and cell therapy manufacturing facility in Cleveland, OH.

In a Phase 1/2a clinical trial of EB-101, participants with RDEB were ≥18 years old, had two COL7A1 genetic mutations and chronic open wounds ≥ 20 cm², for ≥ 12 weeks. Autologous keratinocytes were cultured from biopsies of intact skin and transduced with a retrovirus containing full-length COL7A1 to form gene-corrected epidermal sheets (EB-101) measuring 35 cm². EB-101 was transplanted onto 38 chronic wound sites in 7 participants from 2013 to 2017. Investigator assessment of wound healing and pain assessment from last available visit was recorded, followed by a survey 3 to 6 years after treatment asking participants to rate change in pain compared with their pre-treatment state using a seven-point scale, ranging from 1 (very much improved) to 7 (very much worse). 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 IIB), 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.

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, 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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