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Abeona Therapeutics Activates Second Clinical Trial Site in EB-101 Pivotal Phase 3 VIITAL™ Study for Recessive Dystrophic Epidermolysis Bullosa

NEW YORK and CLEVELAND, July 23, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced the activation of a second clinical trial site in its pivotal Phase 3 VIITAL™ study of its investigational EB-101 treatment for recessive dystrophic epidermolysis bullosa (RDEB) at UMass Memorial Medical Center in Worcester, MA.

The EB-101 pivotal VIITAL™ study is currently ongoing at Stanford University Medical Center in Palo Alto, CA. Target enrollment is 10 to 15 RDEB patients with approximately 35 large, chronic wound sites to be treated in total. Treatment with EB-101 uses gene transfer to deliver a functional 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 facilitate wound healing by supplementing Type VII collagen expression in RDEB patients who lack a fully functional COL7A1 gene. The co-primary endpoints of the study are: 1) the proportion of RDEB wound sites with greater than or equal to 50% healing from baseline, comparing treated with untreated wound sites 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 treated and untreated wounds at Week 24 (Month 6).

"We are very pleased to have UMass Memorial Medical Center as one of our VIITAL™ study sites and look forward to collaborating with Dr. Karen Wiss, Director of Pediatric Dermatology at UMass Memorial to screen and enroll subjects as soon as possible," said Vishwas Seshadri, Ph.D., M.B.A., Head of Research & Clinical Development of Abeona. "With UMass and Stanford, we are able to provide convenient treatment locations on the East Coast and West Coast to make travel and logistics easier for patients and families, while also expanding physician experience with EB-101 as we plan for potential commercial launch."

"We are excited to participate in the EB-101 VIITAL™ study and to offer this potentially promising investigational therapy to RDEB patients who currently have no adequate treatment options. We look forward to participating in the Phase 3 trial to evaluate the efficacy and safety of this investigational treatment," said Karen Wiss, M.D., FAAD, Professor of Dermatology and Pediatrics at UMass Medical School and Principal Investigator of the study at UMass Memorial Medical Center.

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 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.

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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