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Abeona Therapeutics Announces Successful Type B Meeting with FDA for Pivotal Phase 3 VIITAL™ Study of EB-101 in Recessive Dystrophic Epidermolysis Bullosa (RDEB)

Clinical trial amendment successfully completed for co-primary endpoints of partial wound closure and mean pain reduction

NEW YORK and CLEVELAND, Jan. 25, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that the company held a successful Type B meeting with the U.S. Food and Drug Administration (FDA) to align with the Agency on the company's proposal regarding co-primary endpoints for the pivotal Phase 3 VIITAL™ study of EB-101 in recessive dystrophic epidermolysis bullosa (RDEB). Following the meeting, Abeona is proceeding with its plan to enroll between 10 to 15 patients with RDEB, comprising approximately 35 large chronic wound sites treated in total.

"We appreciate the clarity provided by the FDA and we are pleased to be aligned with the Agency on the co-primary endpoints for the Phase 3 VIITAL™ study," said Michael Amoroso, Principal Executive and Chief Operating Officer of Abeona. "Following the successful completion of the FDA meeting, we continue with all necessary steps to enroll our next patient in the VIITAL™ study and aim to complete enrollment in 2021."

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).

As previously announced, data from a Phase 1/2a clinical trial presented at the 2020 Society for Pediatric Dermatology Annual Meeting showed that wound healing of 50% or greater following EB-101 treatment in patients with RDEB was associated with no pain at treated sites at three-, four- and five-years post-treatment, compared with presence of pain in 53% of wound sites at baseline.

Jodie Gillon, Vice President and Chief Patient Officer of Abeona commented, "We greatly appreciate the level of clarity we received from the FDA as we continue to work with our clinical study partners at Stanford University Medical Center and patient advocacy groups to

enroll additional patients in the VIITAL™ study."

Investigators at Stanford University Medical Center are currently enrolling eligible patients into the VIITAL™ study. Additional information about the trial, including eligibility criteria, is available at https://www.abeonatherapeutics.com/clinical-trials/rdeb and https://clinicaltrials.gov/ (Identifier: NCT04227106).

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 the 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 30 large, chronic wound sites treated in total. Treatment with EB-101 involves using gene transfer to deliver COL7A1 genes into a patient's own skin cells (keratinocytes and its progenitors) and transplanting them back to the patient to enable normal Type VII collagen expression and facilitate wound healing. Abeona produces EB-101 for the VIITAL™ study at its fully-functional gene and cell therapy manufacturing facility in Cleveland, OH. In a Phase 1/2a clinical trial, EB-101 provided durable wound healing for RDEB patients lasting 2+ to 5+ years, including for the largest, most challenging wounds that affect the majority of the RDEB population.

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 autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, as well as ABO-102 and ABO-101, novel 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 portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical needs. Abeona's novel, next-generation AIM™ capsids have shown potential to improve tropism profiles for a variety of devastating diseases. Abeona's fully functional, gene and cell therapy GMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and 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. These statements include statements about the Company's aim to complete enrollment of patients in our VIITAL study in 2021. 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, the outcome of our announced strategic review, 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.

Investor and Media Contact:
Greg Gin
VP, Investor Relations and Corporate Communications
Abeona Therapeutics
+1 (646) 813-4709
ggin@abeonatherapeutics.com

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