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Abeona Therapeutics Announces Two Presentations Related to Its RDEB Clinical Program at the Society for Pediatric Dermatology 45th Annual Meeting

- *EB-101 treatment of large, chronic wounds is associated with durable healing and pain relief in patients with RDEB*
- *Literature review of 65 studies confirms and expands understanding of substantial disease burden of RDEB with considerable clinical, economic and humanistic impact on patients and their families*

NEW YORK and CLEVELAND, July 10, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that two poster presentations related to its clinical program for recessive dystrophic epidermolysis bullosa (RDEB) were featured at the Society for Pediatric Dermatology (SPD) 45th Annual Meeting. The first poster includes a detailed analysis of patients with RDEB in the EB-101 Phase 1/2a trial showing that wound healing following EB-101 treatment was associated with improved long-term pain relief. A separate poster provides insights on the significant disease burden associated with RDEB, highlighting data from a literature review on the clinical characteristics, humanistic consequences and economic impact of living with RDEB on patients and their families.

“The large wounds of RDEB cause substantial pain, and only palliative treatments are currently available,” said João Siffert, M.D., Chief Executive Officer of Abeona. “The data presented at SPD showed that EB-101 treatment of large, chronic wounds resulted in considerable and durable reduction in wound burden, which was associated with long-term pain relief for up to five years. The second poster at SPD helps to characterize the disease burden and management of RDEB, providing an important reminder of the extraordinary toll RDEB takes on quality of life, and underscores the need for therapies that reduce wound burden and the associated humanistic and economic impact.”

EB-101 Treatment of Large, Chronic Wounds Is Associated with Durable Healing and Pain Reduction in Patients with Recessive Dystrophic Epidermolysis Bullosa (RDEB)

Jean Tang, M.D., Ph.D., Professor of Dermatology, Stanford University Medical Center and Principal Investigator of the EB-101 pivotal Phase 3 VIITAL^{TM} study, presented long-term outcomes following EB-101 treatment for large, chronic wounds in patients with RDEB. EB-101 treatment resulted in considerable and durable reduction in wound burden in the range of three to five years in a Phase 1/2a study. Wound healing of 50% or greater following EB-101 treatment was associated with no pain at treated sites at three years, four years and five

years post-treatment, compared with presence of pain in 53% of wound sites at baseline. The ongoing VIITAL™ study will further characterize the relationship between reduction of wound burden and pain relief following EB-101 treatment.

The Full Burden of Recessive Dystrophic Epidermolysis Bullosa (RDEB)

M. Peter Marinkovich, M.D., Bullous Disease Clinic Director, Stanford University Medical Center, and Investigator in the VIITAL™ study, presented findings from a literature review of 65 studies that provide new insights on the disease burden from the perspective of patients with RDEB and their families. Key observations of the clinical, humanistic and economic burden of RDEB include:

- Large, chronic wounds comprise a major clinical burden of RDEB and are correlated with pain.
- Many patients experience anxiety and depression.
- Parents of children with RDEB reported negative effects on their relationship, choosing to not have more children.
- 50% of U.S. families characterized the economic impact of managing RDEB as "high" or "severe."

Abeona's posters from the SPD 45th Annual Meeting are available on the "News/Events" page under the "Investors & Media" section of Abeona's website at www.abeonatherapeutics.com.

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 multi-center, 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 their 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. More information on the clinical trials of EB-101 can be found at https://www.abeonatherapeutics.com/clinical-trials/rdeb and ClinicalTrials.gov (Identifier: NCT04227106).

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 of AAV-based gene therapies also features ABO-202 and ABO-201 for CLN1 disease and CLN3 disease, respectively. 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 clinical trials and its products and product candidates, future regulatory interactions with regulatory authorities, as well as the Company's goals and objectives. 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 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 periodic 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.

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