November 3, 2022

Abeona Therapeutics Announces Positive Topline Results with Both Co-Primary Endpoints Met in Pivotal Phase 3 VIITAL™ Study of EB-101

Co-primary endpoint measuring >50% wound healing, other endpoints measuring >75% and complete wound healing at six months all met

Co-primary endpoint measuring pain reduction at six months met; greater magnitude of pain reduction benefit was observed in post-hoc analysis of EB-101 treated wounds with severe baseline pain

EB-101 was well-tolerated with no serious treatment-related adverse events, consistent with past clinical experience

Plans to submit Biologics License Application (BLA) to U.S. FDA

Conference call on November 3, 2022 at 8:30 a.m. EDT

NEW YORK and CLEVELAND, Nov. 03, 2022 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today announced positive topline data from its pivotal Phase 3 VIITAL study assessing the safety and efficacy of EB-101 for the treatment of patients with recessive dystrophic epidermolysis bullosa (RDEB). The VIITAL study met its two co-primary efficacy endpoints demonstrating statistically significant, clinically meaningful improvements in wound healing and pain reduction in large chronic RDEB wounds.

“We are very pleased with the topline results from our pivotal VIITAL study, which reinforce the strong value proposition of EB-101 as a potential one-time therapy to both significantly improve wound healing and reduce pain for the most disabling, challenging to treat wounds in patients with RDEB,” said Vish Seshadri, Chief Executive Officer of Abeona. “The VIITAL study is differentiated from any other pivotal study in RDEB by the co-primary endpoint measuring patient-reported pain. We believe the significant result in this endpoint supports EB-101’s potential for improving the daily life of RDEB patients. Based on the efficacy and safety profile of EB-101 in VIITAL, we are looking forward to sharing the VIITAL study results with the FDA and progressing toward submission of a BLA. We are grateful to the patients, their families, caregivers, and the patient advocacy groups for their support of this study, and are also thankful for the clinical investigators, study site personnel, and the entire Abeona team who collectively contributed to this milestone achievement.”

Summary of Topline Results: All Evaluated Endpoints Successfully Achieved

The pivotal Phase 3 VIITAL study evaluated the efficacy, safety and tolerability of EB-101 in

43 large chronic wound pairs in 11 subjects with RDEB. The large chronic wounds randomized and treated in VIITAL measured greater than 20 cm^2 of surface area and had remained open for a minimum of six months and a maximum of 21 years (mean 6.2 years). The co-primary endpoints of the study were: 1) the proportion of RDEB wound sites with greater than or equal to 50% healing from baseline, comparing randomized treated with matched untreated (control) wound sites at the six-month timepoint, 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 randomized treated and matched untreated (control) wounds at the six-month timepoint. The study allowed for wounds not included in the randomized primary efficacy analysis to receive EB-101 treatment (n=14 non-randomized wounds). The tables below summarize the topline primary efficacy results:

- **Wound Healing Endpoints:** EB-101 significantly improved wound healing vs. control at six months

Wound Healing Level from Baseline (investigator assessed)	**% Randomized Treated Wounds (n=43)**	**% Randomized Untreated Control Wounds (n=43)**	**p-value***
50% or greater (co-primary endpoint)	81.4%	16.3%	<0.0001

* Two-sided p-value calculated from permutation test using randomized wound pairs (n=43 pairs).

Other VIITAL study endpoints measuring proportion of wounds achieving 75% or greater wound healing
and complete wound healing at six months also achieved statistical significance.

- **Pain Endpoint:** EB-101 showed significant pain reduction associated with wound dressing changes vs. control at six months

	Randomized Treated Wounds (n=43)	**Randomized Untreated Control Wounds (n=42)**	**p-value****
Mean pain reduction from baseline* (co-primary endpoint)	3.07	0.90	0.0002

* Based on patient reported outcomes assessing pain severity on 0-10 scale in increments of 2 (i.e., 0, 2, 4, 6, 8, 10).
** Two-sided p-value calculated from permutation test using randomized wound pairs (n=42 pairs).

- **Post-Hoc Analysis of Pain Data**

In addition to meeting the co-primary pain endpoint, in a post-hoc analysis of the EB-101 treated severe wounds (baseline pain score of 6 or greater), including randomized and non-

randomized (n=27), a mean pain reduction from baseline at six months of 5.70 was observed, as compared to a mean pain reduction of 3.51 for all treated randomized and non-randomized wounds for which pain was evaluated (n=53).

- **Safety Results**

EB-101 was shown to be well-tolerated with no serious treatment-related adverse events observed, consistent with past clinical experience. There were no deaths or instances of positive replication-competent retrovirus (RCR) results, and no systemic immunologic responses were reported during the study, as well as no squamous cell carcinoma (SCC) at treatment sites after application of EB-101. Two subjects reported at least one serious adverse event (SAE) unrelated to EB-101. Four subjects reported related treatment emergent adverse events (TEAEs), including procedural pain, muscle spasms and pruritis. Infections unrelated to EB-101 were observed in eight patients.

Abeona anticipates submitting results from this study, including further details with additional exploratory endpoints and the Week 12 results, for presentation at future medical meetings and for publication in a peer-reviewed journal. The Week 12 results are similar to Week 24 results, achieving statistical significance for pain reduction and wound healing at all levels.

Jean Tang, M.D., Ph.D., Professor of Dermatology, Stanford University School of Medicine and Principal Investigator of the EB-101 pivotal Phase 3 VIITAL study said, “Large chronic RDEB wounds are the toughest to treat and often associated with intense chronic pain that significantly impacts the quality of life of RDEB patients, necessitating frequent use of opioids. In the Phase 3 VIITAL study, EB-101 has been shown to both heal such large chronic wounds and significantly reduce pain. And we continue to see durable clinical benefit of EB-101 with up to eight years of follow-up in our Phase 1/2a study.”

Brett Kopelan, Executive Director, debra of America, and father to Rafi, a 15-year-old with RDEB, said, “I am incredibly enthused to see new clinical evidence of EB-101’s potential to treat the more difficult chronic and large wounds. Our patient community needs options to address not only the healing of wounds but also the chronic pain and the acute treatment related pain of daily wound care associated with these wounds. Today’s standard of care comprises hours of brutal and painful wound care, and EB-101’s promise to be a transformational option for RDEB patients is truly exciting.”

Next Steps

Based on the positive topline results, Abeona intends to submit a Biologics License Application (BLA) for EB-101 to the U.S. Food and Drug Administration (FDA) in the second quarter of 2023. EB-101 has been granted Orphan Drug and Rare Pediatric Disease (RPD) designations by the FDA. Among the benefits of Orphan Drug designation are seven years of market exclusivity following FDA approval, potentially preventing FDA approval of another product deemed to be the same as the approved product for the same indication, waiver of application fees, and tax credits for clinical testing expenses conducted after orphan designation is received. A sponsor who receives an approval for a BLA with RPD designation may qualify for a Priority Review Voucher (PRV), subject to final determination by the FDA. The PRV can be used to receive an expedited review process of a subsequent marketing application for a different product or sold to another company.

Conference Call Details

Abeona Therapeutics will host a conference call and webcast on Thursday, November 3, 2022, at 8:30 a.m. EDT, to discuss the positive topline results from the VIITAL study. To access the call, dial 888-506-0062 (U.S. toll-free) or 973-528-0011 (international) and Entry Code: 844393 five minutes prior to the start of the call. A live, listen-only webcast and archived replay of the call can be accessed on the Investors & Media section of Abeona's website at www.abeonatherapeutics.com. The archived webcast replay will be available for 30 days following the call.

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, engineered cell therapy currently being developed 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 with target enrollment of at least 10 to 15 RDEB patients with approximately 36 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 being investigated for its ability to enable normal Type VII collagen expression and to facilitate wound healing. The U.S. FDA has granted Rare Pediatric Disease designation for EB-101. Abeona produces EB-101 for the VIITAL study at its fully integrated gene and cell therapy manufacturing facility in Cleveland, Ohio. 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 cell and gene therapies for serious diseases. Abeona's lead clinical program is EB-101, its investigational autologous, engineered cell therapy currently in development for recessive dystrophic epidermolysis bullosa. 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 cell and gene therapy cGMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and potential 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," "anticipate," "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, our ability to continue as a going concern; the timing and outcome of our Biologics License Application submission to the FDA for EB-101; continued interest in our rare disease portfolio; our ability to enroll patients in clinical trials; the outcome of any future meetings with the FDA or other regulatory agencies; 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 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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