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Abeona Therapeutics® Announces FDA Acceptance of BLA Resubmission of Pz-cel for the Treatment of Recessive Dystrophic Epidermolysis Bullosa

FDA assigns PDUFA target action date of April 29, 2025

CLEVELAND, Nov. 12, 2024 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today announced that the U.S. Food and Drug Administration (FDA) has accepted for review Abeona's resubmission of its Biologics License Application (BLA) for prademagene zamikeracel (pz-cel), its investigational autologous cell-based gene therapy, as a potential new treatment for recessive dystrophic epidermolysis bullosa (RDEB). The FDA has assigned a Prescription Drug User Fee Act (PDUFA) target action date of April 29, 2025.

"The FDA acceptance of our BLA resubmission moves us one step closer to providing pz-cel as a differentiated treatment option to address the persistent unmet needs of people with RDEB in the U.S.," said Vish Seshadri, Chief Executive Officer of Abeona. "We look forward to continuing to work with the FDA to finalize the review of the pz-cel application."

The BLA resubmission is supported by clinical efficacy and safety data after a one-time administration of pz-cel from the pivotal Phase 3 VIITAL™ study (NCT04227106) and a Phase 1/2a study (NCT01263379) with up to 8 years of follow-up. If approved, pz-cel would be the first autologous, cell-based gene therapy for RDEB, and the first RDEB treatment designed to provide collagen VII expression at wound sites via a stably integrated copy of the COL7A1 gene.

The Company's BLA for pz-cel was previously accepted for Priority Review by the FDA for patients with RDEB. Abeona may be eligible for a Priority Review Voucher (PRV) should pz-cel be approved.

About prademagene zamikeracel (pz-cel)
Prademagene zamikeracel (pz-cel), Abeona's investigational autologous, *COL7A1* gene therapy, is currently being developed for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), a rare genetic skin disease caused by a mutation in both copies of the *COL7A1* gene. As a result of this defect, cells are unable to express functional collagen VII protein which is needed to form anchoring fibrils that bond the epidermis to the dermis. Lack of anchoring fibrils leads to fragile skin that blisters easily and patients suffer from years of painful wounds, itch and increased risk of infection and squamous cell carcinoma. Pz-cel is made from patients' own skin cells that are genetically corrected with a functional *COL7A1* gene integrated into the skin cells' genome to express collagen VII. These gene-corrected

cells are expanded to form keratinocyte sheets to cover wound areas in a single surgical application. Pz-cel has been granted Regenerative Medicine Advanced Therapy, Breakthrough Therapy, Orphan Drug and Rare Pediatric Disease designations by the U.S. FDA.

About Abeona Therapeutics

Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for serious diseases. Prademagene zamikeracel (pz-cel) is Abeona's investigational autologous, COL7A1 gene-corrected epidermal sheets currently in development for recessive dystrophic epidermolysis bullosa. The Company's fully integrated cell and gene therapy cGMP manufacturing facility served as the manufacturing site for pz-cel used in its Phase 3 VIITAL™ trial, and is capable of supporting commercial production of pz-cel upon FDA approval. 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. 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," "potential," and similar words and 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 timing and outcome of the FDA's review of our BLA resubmission for pz-cel; the FDA's grant of a Priority Review Voucher upon pz-cel approval; continued interest in our rare disease portfolio; our ability to enroll patients in clinical trials; the outcome of future meetings with the FDA or other regulatory agencies, including those relating to preclinical programs; the ability to achieve or obtain necessary regulatory approvals; the impact of any 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 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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