April 22, 2024

Abeona Therapeutics Provides Regulatory Update on Pz-cel

Receives FDA Complete Response Letter (CRL) based on need for additional CMC information

CRL did not identify deficiencies related to clinical efficacy or clinical safety data in BLA, and no new clinical studies requested by FDA to support approval

Anticipates completing and submitting requested CMC information in 3Q 2024

Conference call and webcast on Tuesday, April 23, 2024 at 8:30 a.m. ET to provide details on the requested CMC information

CLEVELAND, April 22, 2024 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today announced a regulatory update for prademagene zamikeracel (pz-cel). The U.S. Food and Drug Administration (FDA) has issued a Complete Response Letter (CRL) in response to the Company's Biologics License Application (BLA) for pz-cel for the treatment of patients with recessive dystrophic epidermolysis bullosa (RDEB). The CRL follows the completion of Abeona's Late Cycle Review Meeting with the FDA in March 2024. At the Late Cycle Review Meeting and in a subsequent information request, the FDA noted that certain additional information needed to satisfy Chemistry Manufacturing and Controls (CMC) requirements must be satisfactorily resolved before the application can be approved. In response, the Company submitted plans to the FDA with the commitment to provide CMC data prior to BLA approval, and full validation reports after approval in mid-2024. In addition, the Company discussed these plans with the FDA in a subsequent informal meeting. In the CRL, the FDA indicated that the proposed timing of the data submission by Abeona would not allow sufficient time for the FDA to complete its review by the May 25, 2024 PDUFA date.

The information needed to satisfy the CMC requests in the CRL pertains to validation requirements for certain manufacturing and release testing methods, including some that were captured in the observations during the FDA's pre-license inspection (PLI). The CRL did not identify any deficiencies related to the clinical efficacy or clinical safety data in the BLA, and the FDA did not request any new clinical trials or clinical data to support the approval of pz-cel.

"While we are surprised and disappointed by this CRL, we are committed to providing the CMC information necessary to respond to the agency's asks, with the goal of bringing pz-cel to patients with RDEB as quickly as possible," said Vish Seshadri, Chief Executive Officer of Abeona. "We are already hard at work generating the additional CMC information, and we expect that all of FDA's requests will be addressable in a reasonable timeframe. We anticipate completing the BLA resubmission in the third quarter of 2024 with necessary

updates to fully satisfy all the deficiencies outlined in the CRL."

The BLA for pz-cel was accepted for filing and granted priority review designation by the FDA in November 2023. The application is supported by clinical efficacy and safety data from the pivotal Phase 3 VIITAL™ study (NCT04227106) and a Phase 1/2a study (NCT01263379). Abeona believes that both studies demonstrate that a single application of pz-cel on large and chronic wounds will deliver sustained wound healing and pain reduction.

Conference Call Details
Abeona Therapeutics will host a conference call and webcast to provide details on the requested CMC information on Tuesday, April 23, 2024, at 8:30 a.m. ET. To access the call, dial 888-506-0062 (U.S. toll-free) or 973-528-0011 (international) and Entry Code: 857208 five minutes prior to the start of the call. A live, listen-only webcast and archived replay of the call can be accessed at https://investors.abeonatherapeutics.com/events. The archived webcast replay will be available for 30 days following the call.

About prademagene zamikeracel (pz-cel)
Prademagene zamikeracel (pz-cel), Abeona's investigational autologous, *COL7A1* gene-corrected epidermal sheets, is currently being developed for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), a rare connective tissue disorder caused by a defect in the *COL7A1* gene that results in the inability to produce Type VII collagen. Pz-cel is designed to incorporate the functional collagen-producing *COL7A1* gene into a patient's own skin cells and enable long-term gene expression by using a retroviral vector to stably integrate into the dividing target cell genome. Pz-cel is being investigated for its ability to enable normal Type VII collagen expression and to facilitate wound healing and pain reduction in even the toughest-to-treat RDEB wounds after a one-time application procedure. The pivotal Phase 3 VIITAL™ study is a randomized clinical trial that evaluated the efficacy, safety and tolerability of pz-cel in 43 large chronic wound pairs in 11 subjects with RDEB. Pz-cel has been granted Regenerative Medicine Advanced Therapy, Breakthrough Therapy, Orphan Drug and Rare Pediatric Disease designations by the U.S. FDA. Abeona produces pz-cel for the VIITAL™ study at its fully integrated gene and cell therapy manufacturing facility in Cleveland, Ohio.

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 results of ongoing testing and other corrective actions being performed in response to the FDA’s identified deficiencies, which could delay the Company’s BLA resubmission; the timing and outcome of the FDA’s review of our resubmission; the FDA’s grant of a Priority Review Voucher upon 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.

Investor and Media Contact:
Greg Gin
VP, Investor Relations and Corporate Communications
Abeona Therapeutics
ir@abeonatherapeutics.com

Source: Abeona Therapeutics Inc.