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Abeona Therapeutics Announces Progress Update on Pz-cel Biologics License Application (BLA)

- On track for PDUFA target action date of May 25, 2024 -

CLEVELAND, Feb. 01, 2024 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today announced, as part of the review process by the U.S. Food and Drug Administration (FDA) for the Biologics License Application (BLA) for pz-cel (prademagene zamikeracel) for recessive dystrophic epidermolysis bullosa (RDEB), completion by the FDA of both a Bioresearch Monitoring (BIMO) inspection of Abeona and the BLA mid-cycle review meeting.

The BIMO inspection was conducted from January 22, 2024 through January 24, 2024 at Abeona's headquarters in Cleveland, Ohio, and reviewed the conduct and practices that pertain to the clinical studies of pz-cel. The FDA inspector did not issue any observations or FDA Form 483s during the inspection. The formal report from the FDA regarding the BIMO inspection will be received at a later date. FDA's BIMO program is a comprehensive program of on-site inspections, data audits, and remote regulatory assessments designed to monitor all aspects of the conduct and reporting of FDA regulated research. The BIMO program was established to assure the quality and integrity of data submitted to the agency in support of new product approvals and marketing applications.

Following the BIMO inspection, the BLA mid-cycle review meeting took place on January 25, 2024. The FDA reaffirmed its earlier indication that it does not currently plan to convene an Advisory Committee for pz-cel. In addition, the FDA advised that Risk Evaluation and Mitigation Strategies (REMS) are not anticipated for the pz-cel application at this time, though application review is ongoing, and reconfirmed the PDUFA target action date of May 25, 2024, on which an approval decision on the pz-cel BLA is expected.

"The Abeona team is committed to working with the FDA in its review of the pz-cel BLA, with the goal of bringing this therapy to patients as soon as possible," said Vish Seshadri, Chief Executive Officer of Abeona.

About Abeona Therapeutics
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for serious diseases. The U.S. FDA has accepted and granted Priority Review with a PDUFA target action date of May 25, 2024 for the Biologics License Application for pz-cel (prademagene zamikeracel), 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 our Biologics License Application submission to the FDA for pz-cel; the FDA's grant of a Priority Review Voucher; continued interest in our rare disease portfolio; our ability to enroll patients in clinical trials; the outcome of future meetings with and inspections from 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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