April 30, 2019

Abeona Therapeutics to Report New Preclinical Data Demonstrating Therapeutic Potential of ABO-401 for Treatment of Cystic Fibrosis at American Society of Gene and Cell Therapy Annual Meeting

NEW YORK and CLEVELAND, April 30, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today will report new preclinical data demonstrating that ABO-401, the Company's novel gene therapy for cystic fibrosis (CF), efficiently delivered a highly-expressed, functional copy of human mini-CFTR (hCFTR) to the lung of CF mice and restored CFTR function in human CF patient nasal and bronchial epithelial cells. The data will be presented this evening at the American Society of Gene and Cell Therapy 22nd Annual Meeting in Washington, D.C.

"These encouraging preclinical data add to the growing body of evidence suggesting ABO-401 may address the challenges in lung delivery and transgene expression that have limited the advancement of gene therapy for CF patients," said Timothy J. Miller, Ph.D., President and Chief Scientific Officer. "ABO-401 is a promising candidate from our AIM™ capsid library en route to IND-enabling studies that may ultimately change the landscape of CF treatment by introducing one-time gene therapy."

ABO-401 has a regulatable human mini-CFTR gene that is efficiently packaged into one of the Company's next-generation AIM™ library capsids, AAV204. In this and other preclinical studies, ABO-401 restored CFTR expression and chloride conductance in airway epithelia, the main cells of the lung that contribute to CF pathology in humans.

These new data demonstrated that ABO-401 efficiently delivered a highly-expressed, functional copy of hCFTR to the lungs of CF mice and restored CFTR function in nasal and bronchial epithelial cells of human donor cells with the delta-F508 mutation, the most common mutation of CF. ABO-401 transduced human CF nasal and bronchial epithelial cells, with CFTR-specific change in short-circuit current that was comparable or superior to existing modulator therapy in these same cells. Robust expression of AAV204 in the lungs of CF mice was observed and demonstrated that the AAV204 capsid was equally or more efficient at delivering gene expression cassettes to the lung compared to other naturally-occurring AAV capsids. Further, the data demonstrated that ABO-401 restored CFTR-specific nasal potential difference in CF mice, and that the ABO-401 gene expression cassette makes a fully-processed CFTR.

About ABO-401
ABO-401 is a novel gene therapy in development for the treatment of patients with cystic fibrosis (CF), a progressive genetic disease that results in persistent lung infections and limits the ability to breathe over time. Preclinical data demonstrate that ABO-401 is delivered to the lung using a next-generation AIM™ vector. ABO-401 targets airway cells and corrects the underlying CF chloride channel deficit and addresses all CF mutations, including the most common CF mutation, delta-F508.

About the AIM™ Vector Platform
Abeona is developing the AIM™ Vector Platform: a next generation of adeno-associated virus (AAV) capsids for use in gene therapies. The AIM™ capsid library can utilize AAV biology to selectively target delivery of genetic payloads to the central nervous system, lungs, eye, muscle, liver and other tissues. AIM™ vectors are non-virus-producing and have shown the potential to evade the immune responses generated by exposure to naturally-occurring AAV vectors. The Company's AIM™ library contains more than 100 capsids with tissue tropisms selected for their potential to target a wide range of organs and multiple routes of delivery.

About Abeona Therapeutics
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and cell therapies for serious diseases. The Company's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa, as well as ABO-102 and ABO-101, novel AAV9-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB). The Company's portfolio of AAV9-based gene therapies also features ABO-202 and ABO-201 for CLN1 disease and CLN3 disease, respectively. Its preclinical assets include ABO-401, which uses the novel AIM™ AAV vector platform to address all mutations of cystic fibrosis. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates and is the only company with Regenerative Medicine Advanced Therapy designation for two investigational therapies (EB-101 and ABO-102). For more information, visit www.abeonatherapeutics.com.

Forward Looking Statement
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 regarding our pipeline including the potential for the AIM™ vector platform in the treatment of cystic fibrosis, including the results of pre-clinical studies and initiation of clinical studies, and the company's goals and objectives. We have attempted to identify forward looking statements by such terminology as "may," will," "anticipate," "believe," "estimate," "expect," "intend," and similar expressions.

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: continued interest in our rare disease portfolio, our ability to initiate and enroll patients in clinical trials, 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 reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or 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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Source: Abeona Therapeutics Inc.