

April 15, 2019



Abeona Therapeutics to Present Data for ABO-401 in Cystic Fibrosis and Retinal Disorders at the American Society of Gene and Cell Therapy Annual Meeting

New IND-enabling data for ABO-202 in CLN1 disease also to be highlighted

NEW YORK and CLEVELAND, April 15, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that new data demonstrating the capabilities of the AIM™ capsid library in cystic fibrosis and retinal disorders will be presented at the American Society of Gene and Cell Therapy 22nd Annual Meeting, being held April 29 – May 2, 2019 in Washington, D.C. Additional preclinical data for ABO-202, the Company's AAV9-based gene therapy for the treatment of patients with CLN1 disease, will also be presented.

"The data to be presented at the ASGCT meeting will highlight study results on the delivery and expression of hCFTR gene in cystic fibrosis and the potential of the AIM™ AAV vector platform in the treatment of multiple retinal disorders. New data from IND-enabling studies of our program in CLN1 disease, expected to enter the clinic later this year, will also be presented," said João Siffert, M.D., Chief Executive Officer.

"The AIM capsid library has demonstrated significant versatility with the ability to deliver genes to target tissues with enhanced specificity," added Timothy J. Miller, Ph.D., President and Chief Scientific Officer. "This next-generation AAV capsid platform has the potential to transform how we can target pulmonary, retinal and neurological disorders with gene therapy."

Identification of AAV Developed for Cystic Fibrosis (CF) Gene Therapy That Restores CFTR Function in Human CF Patient Cells

Presenter: Paul T. Wille, Ph.D., Case Western Reserve University, Cleveland, OH; Abeona Therapeutics Inc.

Date/Time: Tuesday April 30, 5:00 – 6:00 p.m. ET

Poster Board Number: 92

Session title: Cardiovascular and Pulmonary Diseases

Room: Columbia Hall

Abstract number: 528

Novel AAV Capsids Demonstrate Strong Retinal Expression in Non-Human Primates After Intravitreal Administration

Presenter: Brian Kevany, Ph.D., Abeona Therapeutics Inc.

Date/Time: Monday April 29, 5:00 – 6:00 p.m. ET
Session title: Neurologic Diseases
Room: Columbia Hall
Abstract number: 225

Intrathecal and Intravenous Combination Gene Therapy in the Mouse Model of Infantile Neuronal Ceroid Lipofuscinosis Extends Lifespan and Improves Behavioral Outcomes in Moderately Affected Mice

Presenter: Erik A. Lykken, Ph.D., University of Texas Southwestern Medical Center, Dallas, TX

Date/Time: Monday April 29, 11:45 a.m. – 12:00 p.m. ET
Session title: Tools, Delivery and Neuro Capsids
Room: Monroe

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 multiple disorders including cystic fibrosis and retinal diseases, and the potential for ABO-202 for CLN-1, including the initiation of clinical studies, the nature of feedback from regulatory agencies, 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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