

January 31, 2019



Abeona Therapeutics to Present New Supportive Data for Novel Gene Therapies at WORLDSymposium™

NEW YORK and CLEVELAND, Jan. 31, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a leading clinical-stage biopharmaceutical company developing novel cell and gene therapies for serious diseases, today announced that data from ongoing clinical trials for lysosomal storage diseases and novel AIM™ chimeric AAV-based vector gene therapy programs will be presented at WORLDSymposium™, being held February 4-8, 2019 in Orlando, FL.

“The data to be presented at the WORLDSymposium™ will highlight study results on biodistribution and tissue tropism of the next-generation AIM™ AAV vector platform in Pompe and Fabry diseases, as well as data from our programs in Sanfilippo syndrome type A and CLN3 disease,” said João Siffert, M.D., Interim Chief Executive Officer of Abeona. “These new supportive data underscore the potential of our novel gene therapies for people living with lysosomal storage diseases.”

“Our AIM™ program continues to demonstrate how novel AAV capsids can target specific body tissues with high efficiency,” said Timothy J. Miller, Ph.D., President and Chief Scientific Officer.

Platform presentations:

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: Steven J. Gray, University of Texas Southwestern Medical Center, Dallas, TX

Date: Wednesday, February 6 at 7:45 a.m. ET

Phase 1/2 Clinical Trial of Systemic Gene Transfer of scAAV9.U1a.hSGSH for MPS IIIA Demonstrates 2 years of Safety, Tolerability, and Biopotency

Presenter: Kevin M. Flanigan, Center for Gene Therapy, Nationwide Children’s Hospital, Columbus, OH

Date: Wednesday, February 6 at 9:15 a.m. ET

Poster presentations:

A Novel AAV Capsid with Improved CNS Tropism for Treating Pompe Disease by Intravenous Administration

Poster #188

Date: Tuesday, February 5 at 4:30 p.m. ET

AAV Gene Therapy for the Treatment of Fabry Disease: A Novel Capsid with Improved Tropism to Heart, Kidney and CNS and Improved GLA Expression

Poster #187

Date: Tuesday, February 5 at 4:30 p.m. ET

An Improved, Novel, Systemically Administered AAV Gene Therapy For Treatment of CLN3 Juvenile Neuronal Ceroid Lipofuscinosis

Poster #186

Date: Tuesday, February 5 at 4:30 p.m. ET

Phase 1/2 Gene Transfer Clinical Trial of scAAV9.U1a.hSGSH for Mucopolysaccharidosis (MPS) IIIA Vector Shedding Results Over 6 Months Post-Gene Transfer

Poster #330

Date: Wednesday, February 6 at 4:30 p.m. ET

About Abeona Therapeutics

Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include EB-101, its gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa, and ABO-102, a novel AAV9 based gene therapy for Sanfilippo syndrome type A (MPS IIIA). The Company's portfolio of AAV9 based gene therapies also features ABO-101 for Sanfilippo syndrome type B (MPS IIIB), and ABO-201 and ABO-202 for CLN3 disease and CLN1 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). 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 and product portfolio, the clinical effect of ABO-102, the ability of the novel AIM™ vector platform in development to target specific tissues and address certain gene mutations and human disorders including Pompe disease and Fabry disease, the potential of investigational new therapies for the treatment of juvenile neuronal ceroid lipofuscinosis (CLN3) to improve outcomes, the market opportunities for the Company's products and product candidates, the ability to generate shareholder value, meet patient expectations, and achieve 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 submit protocols and protocol amendments to regulatory agencies, our ability to initiate

and enroll patients in clinical trials, the adequacy of manufacturing capabilities, the impact of competition, the ability to secure licenses or establish intellectual property rights for any technology that may be necessary to continue to develop and 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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