

May 8, 2017



Abeona Therapeutics Announces Gene Therapy Clinical Trial and Preclinical Program Presentations at ASGCT 20th Annual Meeting

Company to Host Live Conference Call Briefing on Friday, May 12th at 10:00am EDT

NEW YORK and CLEVELAND, May 08, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene therapies for life-threatening rare diseases, announced today that data from the Company's gene therapy clinical trial and preclinical research programs will be highlighted at the 20th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) being held in Washington, D.C. from May 10-13, 2017.

Abeona's collaborators will present two oral and one poster presentation during the conference. These presentations will detail data from clinical and research programs utilizing gene therapy as a potential treatment for patients with the lysosomal storage disorders Sanfilippo syndrome and Batten Disease, and the Company's next generation AAV AIMTM Vector platform. Details of the presentations and poster are listed below.

Oral Presentations:

[Session 243 - Clinical Trials for Neurologic and Neurosensory Disorders](#) 292. *A Phase 1/2 Clinical Trial of Systemic Gene Transfer of scAAV9.U1a.hSGSH for MPS IIIA: Safety, Tolerability, and Preliminary Evidence of Biopotency*

Presenter: Kevin M. Flanigan, M.D., Principal Investigator and Director, Research Institute of Nationwide Children's Hospital, Columbus, OH

Date/Time: Thursday, May 11th; 4:00pm–4:15pm EDT

Location: Marriott Salon 1

[Session 344 - Preclinical Progress Towards Therapies for Neurologic Disorders](#) 537. *Intrathecal scAAV9 Gene Therapy Is an Effective Treatment for PPT1-Deficiency in the Preclinical INCL Mouse Model*

Presenter: Alejandra J. Rozenberg, Ph.D. and Erik A. Lykken, Ph.D., - University of North Carolina at Chapel Hill, Chapel Hill, NC

Date/Time: Friday, May 12th; 4:45pm–5:00pm EDT

Location: Delaware AB

Poster Session:

[Session AAV Vectors II](#) 316. *Enhanced CNS Specificity of Adeno-Associated Viral*

Vectors Created by DNA Shuffling and Directed Evolution

Presenter: Daphne Chen, University of North Carolina at Chapel Hill, Chapel Hill, NC

Date/Time: Thursday, May 11th; 5:15pm–7:15pm EDT

Location: Exhibit Hall A & B South

Conference Call Details: Abeona will host a live conference call briefing on Friday, May 12th at 10:00am EDT. Analysts and investors can participate in the conference call by dialing 877-269-7756 for domestic callers and 201-689-7817 for international callers.

About Abeona: Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB). Abeona is also developing ABO-101 (AAV-NAGLU) for Sanfilippo syndrome type B (MPS IIIB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) for treatment of infantile Batten disease (INCL), EB-201 for epidermolysis bullosa (EB), ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder and ABO-302 using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, Abeona has a plasma-based protein therapy pipeline, including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD, using its proprietary SDF™ (Salt Diafiltration) ethanol-free process. For more information, visit www.abeonatherapeutics.com.

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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 that involve risks and uncertainties. These statements are subject to numerous risks and uncertainties, including but not limited to continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the impact of competition; the ability to develop our products and technologies; the ability to achieve or obtain necessary regulatory approvals; the impact of changes in the financial markets and global economic conditions; and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and other reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligations to make any revisions to the forward-looking statements contained in this release or to update them to reflect events or circumstances occurring after the date of this release, whether as a result of new information, future developments or

otherwise.



Source: Abeona Therapeutics Inc