

October 21, 2019



# Abeona Therapeutics Announces Presentations at the 27th European Society of Gene and Cell Therapy (ESGCT) Congress

NEW YORK and CLEVELAND, Oct. 21, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced the presentations of data from the Transpher A Study, the Company's ongoing Phase 1/2 clinical trial evaluating ABO-102 for the treatment of Sanfilippo syndrome type A (MPS IIIA), and research updates from its library of novel AIM™ adeno-associated virus (AAV) capsids at the 27<sup>th</sup> European Society of Gene and Cell Therapy (ESGCT) Congress, to be held October 22-25, 2019 in Barcelona, Spain.

The data will be presented as follows:

*Safety, Tolerability, Biopotency and Neurocognitive Data of ABO-102 in Transpher A, an Open-Label, Multicenter, Single-Dose, Dose-Escalation, Phase 1/2 Clinical Trial in Sanfilippo Syndrome type A (Mucopolysaccharidosis IIIA)*

Oral Presentation #039

Presenter: Maria Jose de Castro, M.D., Hospital Universitario Santiago de Compostela

Session 4c: Metabolic and Genetic Diseases

Date/Time: Friday, October 25, 2019, 9:00 a.m. to 11:00 a.m. CEST

Location: Room 112

*Novel AAV Capsids Show Increased Evasion to Neutralizing Antibodies Against Natural Serotypes*

Poster #P347

Session Title: Poster Session I

Date/Time: Wednesday, October 23, 2019, 1:00 p.m. to 3:00 p.m. CEST

Location: Multipurpose Hall

*Development of an Improved Novel AAV Capsids for Intramuscular Delivery*

Poster #P027

Session Title: Poster Session I

Date/Time: Wednesday, October 23, 2019, 1:00 p.m. to 3:00 p.m. CEST

Location: Multipurpose Hall

*Novel AAV Capsids for Delivery to the Retina by Intravitreal Administration*

Poster #P009

Session Title: Poster Session I

Date/Time: Wednesday, October 23, 2019, 1:00 p.m. to 3:00 p.m. CEST

Location: Multipurpose Hall

*Development of a Novel AAV Capsid with Improved PNS Tropism for Treating Pompe Disease by Intravenous Administration*

Poster #P007

Session Title: Poster Session I

Date/Time: Wednesday, October 23, 2019, 1:00 p.m. to 3:00 p.m. CEST

Location: Multipurpose Hall

### **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), respectively. 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 twenty regulatory designations from the FDA and EMA for its pipeline candidates. For more information, visit [www.abeonatherapeutics.com](http://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. These statements include statements about the Company's clinical trials and its products and product candidates, future regulatory interactions with regulatory authorities, as well as the Company's goals and objectives. We have attempted to identify forward looking statements by such terminology as "may," "will," "believe," "estimate," "expect," and similar 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 continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the outcome of any future meetings with the U.S. Food and Drug Administration or other regulatory agencies, 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 periodic reports filed by the Company 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 presentation, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.*

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