

## Abeona Therapeutics to Present at 2016 Genetic Rx Boston Biotech Conference at Harvard Medical School

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 12/06/16 --

Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing therapies for life-threatening rare genetic diseases, today announced President and CEO, Timothy J. Miller, Ph.D., will participate in a panel discussion at the 2016 Genetic Rx Conference at the Harvard Medical School in Boston, MA.

President and CEO of Abeona Therapeutics, Timothy J. Miller, PhD, among several industry leaders, will speak in the panel discussion entitled "Gene Therapy: New Therapeutics Modalities."

## Genetic Rx, Gene Therapy: New Therapeutics Modalities Panel

Participating: Timothy J. Miller, PhD

Date: Thursday, December 8th, 2016

Time: 9:00 am EST

## Abeona Recent Highlights:

- November 2, 2016: Abeona announced JAMA publication of Positive Phase 1 study results for EB-101 Gene Therapy clinical trial for Epidermolysis Bullosa
- October 25, 2016: Abeona announced receipt of Fast Track designation from the FDA for ABO-102
- October 18, 2016: Abeona received Orphan Drug Designation in the European Union for ABO-102 Gene Therapy in Sanfilippo syndrome Type A
- October 14, 2016: Abeona presented top-line data of low-dose cohort for ABO-102 in Phase ½ clinical trial for MPS IIIA patients at Orphan Drugs and Rare Disease Congress October 19-20<sup>th</sup> in London, UK
  - ABO-102 reduced GAG (heparan sulfate) in urine 57.6% +/- 8.2%
  - ABO-102 reduced GAG (heparan sulfate) in the CSF 25.6 +/- 0.8%
  - Reduction in liver volume of 17.7% +/- 1.9%
  - Reduction in spleen volume of 17.6% +/- 7.1%
- October 7, 2016: Abeona announced publication of preclinical data supporting clinical translation of Juvenile Batten Disease gene therapy
- October 5, 2016: Abeona announced Data Safety Monitoring Board approved ABO-102 dose escalation for second cohort in Phase 1/2 Clinical Trial for Sanfilippo

## syndrome Type A

About Abeona: Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and plasma-based therapies for life-threatening rare genetic diseases. Abeona's lead programs are ABO- 102 (AAV- SGSH) and ABO-101 (AAV-NAGLU), adeno-associated virus (AAV) based gene therapies for Sanfilippo syndrome (MPS IIIA and IIIB, respectively). Abeona is also developing EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), EB-201 for epidermolysis bullosa (EB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) gene therapy for treatment of infantile Batten disease (INCL), and 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.

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.

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