February 7, 2017

Abeona Therapeutics Announces Presentations and Posters at the 13th Annual WORLDSymposium™ 2017

Multiple Oral Platform Presentations and Poster Sessions Highlighting Gene Therapy Programs, Tuesday, February 14th through Thursday, February 16th

NEW YORK and CLEVELAND, Feb. 07, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing therapies for life-threatening rare genetic diseases, today announced that data on gene therapy programs for Sanfilippo syndrome Type A (MPS IIIA), Infantile Batten Disease (CLN1) and Juvenile Batten Disease will be highlighted at the upcoming 13th Annual WORLD*Symposium*™ 2017 lysosomal storage disorders conference, February 13-17, San Diego, CA. Details for the two oral presentations and three poster sessions are listed below.

Platform Presentations:

ABO-102 Phase 1/2 Clinical trial update - Sanfilippo syndrome Type A (MPS IIIA):

- *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
  - Thursday, February 16th, 2017 at 1:30 pm PST
  - Location: Seaport Ballroom E
  - Clinical data highlights include: CSF and urinary heparan sulfate GAG reduction, liver and spleen volume reduction, and neurological effects in pediatric subjects through six months post-injection

ABO-202 – CLN1, Infantile Batten Disease (INCL):

- *scAAV9 Gene Therapy is an Effective Treatment for CLN1-deficiency in the Preclinical INCL Mouse Model*
  - *Presenter:* Alejandra J. Rozenberg, Ph.D., University of North Carolina at Chapel Hill, Chapel Hill, NC.
  - Wednesday, February 15th, 2017 at 11:15 am PST
  - Location: Seaport Ballroom E

Poster Sessions:

ABO-102 Phase 1/2 Clinical trial update - Sanfilippo syndrome Type A:

- *Systemic gene transfer of scAAV9.U1a.hSGSH for MPS IIIA: tolerability and preliminary evidence for a biochemical effect* – Kevin M. Flanigan, M.D., Principal Investigator and Director, Research Institute of Nationwide Children's Hospital, Columbus, OH
  - Tuesday, February 14, 4:30 – 6:30 pm PST
  - Poster # 89, Harbor Ballroom

ABO-202 – CLN1, Infantile Batten Disease (INCL):

- *Gene therapy extends lifespan and improves quality of life in a mouse model for Infantile neuronal ceroid lipofuscinosis (INCL)* - Alejandra J. Rozenberg, Ph.D., Steven J. Gray, Ph.D., University of North Carolina at Chapel Hill, Chapel Hill, NC.
  - Wednesday, February 15, 4:30 – 6:30 pm PST
  - Poster # 292, Harbor Ballroom

ABO-201 – CLN3, Juvenile Batten Disease (JNCL):

- *Comparison of systemic routes of administration for CLN3 delivery via scAAV9*- S. Kaye Spratt, Ph.D., Abeona Therapeutics Inc.
  - Wednesday, February 15, 4:30 – 6:30 pm PST
  - Poster # 323, Harbor Ballroom

Abeona Recent ABO-102 Program Highlights:

- Enrolled and dosed the first patient in the high-dose cohort for ABO-102 in subjects with Sanfilippo syndrome type A
- Received FastTrack designation by the FDA
- Received Orphan Drug Designation in the European Union
- Reported top-line 30-day post-injection data of the low-dose cohort (n=3)
  - 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%
- Data to be presented at the WORLD*Symposium* will include CSF and urinary heparan sulfate GAG reduction, liver and spleen volume reduction, and neurological effects in pediatric subjects through six months post-injection with ABO-102

About WORLD*Symposium*™ : The goal of WORLD*Symposium*™ is to provide an interdisciplinary forum to explore and discuss specific areas of interest, research and clinical applicability related to lysosomal diseases. Each year, WORLD*Symposium*™ hosts a scientific meeting presenting the latest information from basic science, translational research, and clinical trials for lysosomal diseases. This symposium is designed to help researchers and clinicians to better manage and understand diagnostic options for patients with lysosomal diseases, identify areas requiring additional basic and clinical research, public policy and regulatory attention, and identify the latest findings in the natural history of lysosomal diseases. For more information, visit http://www.worldsymposia.org/

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) 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.

Investor Contact:
Christine Berni-Silverstein
Vice President, Investor Relations
Abeona Therapeutics Inc.
+1 (212)-786-6212
csilverstein@abeonatherapeutics.com

Media Contact:
Andre'a Lucca
Vice President, Communications & Operations
Abeona Therapeutics Inc.
+1 (212)-786-6208
alucca@abeonatherapeutics.com

Source: Abeona Therapeutics Inc