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Abeona Therapeutics Announces Publication of Preclinical Data Supporting Clinical Translation of Juvenile Batten Disease Gene Therapy

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 10/07/16 -- Abeona Therapeutics Inc. (NASDAQ: ABEO):

- *Preclinical efficacy data demonstrated corrected motor deficits, attenuated neuroinflammation and reduced lysosomal throughout the central nervous system (CNS) and peripheral organs*
- *A single, intravenous injection demonstrated dose-dependent benefits in the central nervous system that remained over one year post-injection*
- *Results from this study are the first demonstration of successful systemic administration to restore CLN3 in vivo using scAAV9*

Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing products for life-threatening rare genetic diseases, announced today that preclinical data supporting clinical trials for ABO-201 (AAV-CLN3), the AAV-based single intravenous gene therapy program for juvenile Batten disease, (juvenile neuronal ceroid lipofuscinosis, JNCL), were published in the September issue of the Journal of Neuroscience (doi: 10.1523/JNEUROSCI.1635-16.2016).

"This is the first demonstration of a systemic delivery route to restore CLN3 function, and highlights the importance of selecting the right vector, promoter and delivery route to treat the symptoms of this devastating disease," said Tammy Kielian, Ph.D., lead scientific investigator and Professor of the Department of Pathology and Microbiology, University of Nebraska Medical Center.

Researchers concluded that a single intravenous injection "led to widespread virus biodistribution in the brain, spinal cord, and eye" that was capable of "improving motor function, attenuating microglial and astrocyte activation, and reducing lysosomal pathology, all hallmarks of JNCL" at an age when significant lysosomal pathology had already manifested.

"The data support the clinical translation of ABO-201 for patients with juvenile Batten disease, and demonstrates AAV delivery to target tissues in the central nervous system as well as peripheral organs led to resolution of the underlying disease pathology," stated Timothy J. Miller, Ph.D., President & CEO. "Using a single, intravenous injection to treat the underlying lysosomal storage disease pathology mirrors our clinical trial approach for the treatment of patients with Sanfilippo syndromes type A and B."

The publication article can be accessed by clicking on the following link: (http://www.jneurosci.org/content/36/37/9669.short).

About ABO-201: ABO-201 (AAV-CLN3) is an AAV-based gene therapy which has shown promising preclinical efficacy in delivery of a normal copy of the defective CLN3 gene to cells of the central nervous system with the aim of reversing the effects of the genetic errors that cause for Juvenile neuronal ceroid lipofuscinosis (JNCL) (also known as Juvenile Batten disease). JNCL is a rare, fatal, autosomal recessive (inherited) disorder of the nervous system that typically begins in children between 4 and 8 years of age. Often the first noticeable sign of JNCL is vision impairment, which tends to progress rapidly and eventually result in blindness. As the disease progresses, children experience the loss of previously acquired skills (developmental regression). This progression usually begins with the loss of the ability to speak in complete sentences. Children then lose motor skills, such as the ability to walk or sit. They also develop movement abnormalities that include rigidity or stiffness, slow or diminished movements (hypokinesia), and stooped posture. Beginning in mid- to late-childhood, affected children may have recurrent seizures (epilepsy), heart problems, behavioral problems, and difficulty sleeping. Life expectancy is greatly reduced, and there are no approved treatments for JNCL.

About Abeona: Abeona Therapeutics Inc. is a clinical stage 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), 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 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 our 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 include, without limitation, our plans for continued development and internationalization of our clinical programs, that are looking forward to advancing multiple important new therapeutic candidates for the treatment of epidermolysis bullosa, that we plan to accelerate up to three new promising EB product candidates toward commercialization, that encouraging signs of early biopotency had been observed in urinary and CSF GAG (heparin sulfate) measurements as well as potential disease-modifying effects in the liver and spleen in our ABO-102 program, management plans for the Company, and general business outlook. 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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