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Abeona Therapeutics Receives Orphan Drug Designation in The European Union for ABO-201 Gene Therapy Program in Juvenile Batten Disease

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 01/03/17 -- Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing therapies for life-threatening rare genetic diseases, announced today that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products has granted Orphan Drug Designation for Abeona's ABO-201 program (AAV-CLN3), the AAV-based single intravenous gene therapy program for juvenile Batten disease, a fatal lysosomal storage disease of the nervous system caused by autosomal-recessive mutations in the *CLN3* gene.

"Receiving European Union (EU) orphan drug designation for ABO-201 provides Abeona certain benefits and incentives, including marketing exclusivity, that are strategically important from a regulatory and commercial perspective," stated Timothy J. Miller, Ph.D., President & CEO of Abeona Therapeutics Inc. "The recently published ABO-201 preclinical data from Dr. Tammy Kielian's lab support the clinical translation for patients with juvenile Batten disease, and demonstrated the importance of selecting the right vector and delivery route for potential CNS benefit and to remove the underlying pathology associated with the disease. This designation helps advance the ABO-201 program and we look forward to initiating human clinical trials later this year."

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 recently published in the September 2016 issue of the Journal of Neuroscience (doi: 10.1523/JNEUROSCI.1635-16.2016). The publication article can be accessed by clicking on the following link: http://www.jneurosci.org/content/36/37/9669.short.

About European Union (EU) Orphan Drug Designation: The European Commission grants orphan drug designation status to provide incentives to develop medicinal products to treat, prevent or diagnose diseases or conditions that affect no more than five in 10,000 persons in the European Union. The orphan drug designation provides Abeona with incentives and benefits in the EU, including reduced fees and protection from market competition once ABO-201 is approved for the treatment of juvenile Batten disease patients.

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 biopharmaceutical company developing gene 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, European Union (EU) orphan drug designation for ABO-201 provides Abeona certain benefits and incentives, including marketing exclusivity, that are strategically important from a regulatory and commercial perspective, our preclinical work for ABO-201 and the recently published data supporting its clinical translation for patients with juvenile Batten disease demonstrated the importance of selecting the right vector and delivery route to target tissues in the CNS and treat the symptoms associated with the underlying disease pathology, we look forward to advancing the ABO-201 program and initiating human clinical trials later this year, 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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