June 29, 2017

Abeona Therapeutics Receives FDA Orphan Drug Designation for ABO-201 Juvenile Batten Disease Gene Therapy Program

Abeona's Fourth Gene Therapy Program to Receive FDA Orphan Drug Designation

NEW YORK and CLEVELAND, June 29, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene therapies for life-threatening rare diseases, announced today that the FDA has granted Orphan Drug Designation (ODD) 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 FDA orphan drug designation for ABO-201 augments Abeona's suite of regulatory achievements, providing 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 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 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 Orphan Drug Designation: Orphan drug designation is granted by the FDA to novel drugs or biologics that treat rare diseases or conditions affecting fewer than 200,000 patients in the U.S. The designation allows the drug developer to be eligible for a seven-year period of U.S. marketing exclusivity upon approval of the drug, as well as tax credits for clinical research costs, the ability to apply for annual grant funding, clinical trial design assistance, and the waiver of Prescription Drug User Fee Act (PDUFA) filing fees.

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 juvenile neuronal ceroid lipofuscinosis, also known as juvenile Batten disease (JBD). JBD 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 JBD is vision impairment, which tends to progress rapidly and eventually results 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 therapies for life-threatening rare genetic diseases. Abeona's lead programs include ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB). Abeona is also developing ABO-101 (AAV-NAGLU) for Sanfilippo syndrome type B (MPS IIIB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JBD), ABO-202 (AAV-CLN1) for treatment of infantile Batten disease (INCL), EB-201 for epidermolysis bullosa (EB), 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.

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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, FDA 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 ability to secure licenses for any technology that may be necessary to commercialize our products; 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.

Source: Abeona Therapeutics Inc