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Abeona Therapeutics Receives FDA Rare Pediatric Disease Designation for ABO-202 Gene Therapy Program in CLN1 Disease

Company's 4th Gene Therapy Program to Receive Rare Pediatric Disease Designation, enabling Priority Review Voucher

NEW YORK and CLEVELAND, March 15, 2018 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (NASDAQ:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel cell and gene therapies for life-threatening rare genetic diseases, announced today that the FDA has granted Rare Pediatric Disease Designation for the ABO-202 program (AAV-CLN1), an AAV-based gene therapy for the treatment of CLN1 disease (infantile and late infantile onset Batten disease). A fatal lysosomal storage disease of the nervous system caused by autosomal-recessive mutations in the CLN1 gene, also known as infantile neuronal ceroid lipofuscinosis, CLN1 disease is an inherited genetic disease that primarily affects the nervous system in newborns and progresses rapidly. In February 2018, the ABO-202 program was granted Orphan Drug Designation (ODD) by the FDA.

"This Rare Pediatric Disease designation for ABO-202 is a significant recognition of the strength of the data supporting a potential treatment for patients with CLN1, and is bolstered by the previous Orphan Drug designation from the FDA," stated Timothy J. Miller, Ph.D., President & CEO of Abeona Therapeutics Inc. "These regulatory designations highlight the urgent need for a treatment for this devastating rare disease, and we look forward to initiating human clinical trials later this year."

The rare pediatric disease designation indicates that the FDA may give the company a priority review voucher if the drug is approved for the rare pediatric indication. That voucher could then be used by the company for another drug—any drug—to be given a priority review. A priority review mandates that the FDA will review a BLA drug submission within 6 months instead of the standard 10 months. A priority review designation is only given to a drug candidate that has demonstrated the potential to be a significant improvement in safety and effectiveness for a serious, unmet disease condition. The priority review voucher may be used by the sponsor or transferred to accelerate the review timeline of another drug candidate.

About CLN1 Disease (infantile and late infantile onset): Infantile and late infantile neuronal ceroid lipofuscinosis is a severe lysosomal disease caused by mutations in the CLN1 gene, which encodes the soluble lysosomal enzyme Palmitoyl-Protein-Thioesterase-1 (PPT1) and result in osmiophilic granules accumulating in lysosomes and leading to neuroinflammation, neurodegeneration and death. CLN1 disease is a neurodegenerative, manifests shortly after birth, and is fatal in its classic form by 6 to 12 years of age. In the classic form, aggressive

clinical features appear, including rapid speech and motor deterioration, refractory epilepsy, ataxia, myoclonus, and visual failure between the ages of 6 and 24 months. By 5 years of age, CLN1 disease patients with the classic infantile form are typically poorly responsive and are no longer communicative. These patients subsequently die in the following few years.

About Abeona: Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and ABO-101 (AAV-NAGLU), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type B (MPS IIIB). Abeona is also developing ABO-201 (AAV-CLN3) gene therapy for CLN3 disease, ABO-202 (AAV-CLN1) for treatment of CLN1 disease, 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 is developing a proprietary vector platform, AIM™, for next generation product candidates. For more information, visit www.abeonatherapeutics.com.

Investor Contact:
Christine Silverstein
SVP, Investor Relations & Finance
Abeona Therapeutics Inc.
+1 (646) 813-4707
csilverstein@abeonatherapeutics.com

Media Contact:
Lynn Granito
Berry & Company Public Relations
+1 (212) 253-8881
lgranito@berrypr.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, FDA orphan drug designation for ABO-202 provides Abeona certain benefits and incentives, including marketing exclusivity, that are strategically important from a regulatory and commercial perspective, our preclinical work for ABO-202 and the recently published data supporting its clinical translation for patients with CLN1 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-202 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.

Source: Abeona Therapeutics Inc.