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Abeona Therapeutics Receives FDA Orphan Drug Designation for ABO-202 Gene Therapy Program in Infantile Batten Disease

NEW YORK and CLEVELAND, Feb. 12, 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 Orphan Drug Designation (ODD) to its ABO-202 program (AAV-CLN1), an AAV-based gene therapy for the treatment of infantile 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 (INCL), infantile Batten disease is an inherited fatal genetic disease that primarily affects the nervous system in newborns and progresses rapidly.

“This designation builds on our clinical portfolio of AAV gene therapies that have received FDA and EMA orphan drug designations, which is an important validation of the scientific and clinical translation of these products for the severely underserved CLN1 patient population,” stated Timothy J. Miller, Ph.D., President & CEO of Abeona Therapeutics Inc. “The ABO-202 preclinical data from Dr. Steven Gray’s lab support the clinical translation for patients with infantile Batten disease, and provide valuable insight for potentially improving efficacy using a combination of delivery routes for CNS and whole-body benefit to remove the underlying pathology associated with the disease. This designation helps advance the ABO-202 program and we look forward to initiating human clinical trials later this year.”

ABO-202, developed with Steven Gray, Ph.D. and the support of The Saoirse Foundation, Taylor's Tale, Garrett the Grand Batten Fighter, Hayden's Batten Disease Foundation, and the Batten Disease Support and Research Association, is anticipated to enter clinical trials in 2018.

"ABO-202 is an AAV gene therapy that has shown promising preclinical efficacy in the INCL animal model of disease by extending survival and improving muscle function when administered early in the disease course," noted Steven J. Gray, Ph.D., Assistant Professor, Pediatrics and Neurology, UT Southwestern.

The preclinical data were presented last week at the WORLDSymposium held in San Diego. Key findings included:

- *CLN1 mice recapitulate the major features of the human disease manifestations;*
    - *The data demonstrate that a single intrathecal (IT) injection of self-complementary adeno-associated virus 9 (scAAV9) encoding the*

human CLN1 gene to CLN1 mice at 1 week and 1 month (pre-symptomatic) significantly increased their survival, improved behavior and reduced motor deficits.

- *Higher IT doses further improved these observations, suggesting that methods increasing CNS exposure may be beneficial and provided some survival and behavioral benefit to symptomatic INCL mice.*
- *A combination approach delivering ABO-202 by both intravenous and intrathecal routes of administration further increased survival efficacy 50% and improved potential treatment options for older animals with advanced disease manifestations.*

About Infantile Batten Disease: Infantile neuronal ceroid lipofuscinosis (INCL) 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 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 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 juvenile Batten disease (JNCL), 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 is developing a proprietary vector platform, AIM™, for next generation product candidates. 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-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 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-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.