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Abeona Therapeutics Announces FDA Clearance of Investigational New Drug Application for ABO-202 Gene Therapy in CLN1 Disease

NEW YORK and CLEVELAND, May 21, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that the Company is cleared to begin a Phase 1/2 clinical trial evaluating its novel, one-time gene therapy ABO-202 for the treatment of CLN1 disease, following acceptance of its Investigational New Drug (IND) application by the U.S. Food and Drug Administration (FDA). ABO-202 is designed to deliver a functional copy of the PPT1 gene to cells of the central nervous system and peripheral organs using a combined intravenous and intrathecal delivery via the AAV9 vector. The Company will provide guidance on the timing of the clinical trial later this year.

“This significant step brings hope to people impacted by this devastating disease and was achieved in partnership with Dr. Steven Gray and Taylor’s Tale,” said Timothy J. Miller, Ph.D., Co-Founder, President & Chief Scientific Officer. “We are very encouraged that ABO-202 was well-tolerated and demonstrated significant efficacy in preclinical studies. These results are consistent with findings in our other pre-clinical studies for AAV9-based programs for lysosomal storages diseases, MPS IIIA and MPS IIIB.”

In preclinical studies, ABO-202 effectively delivered a functional copy of the PPT1 gene to the central nervous system and peripheral organs. IND-enabling studies in a CLN1 animal model demonstrated that ABO-202 normalized survival and led to improvement of neurological function in affected mice. These studies also showed that combination intravenous and intrathecal dosing provided incremental efficacy over either delivery route alone and thus may enhance the therapeutic potential of this gene therapy.

ABO-202 is a one-time AAV gene therapy designed to enable cells to produce the normal PPT1 enzyme, which is critical for proper lysosomal function. Lack of this enzyme in patients with CLN1 disease results in neuroinflammation and neurodegeneration. ABO-202 has been granted Orphan Drug and Rare Pediatric Disease designations by the FDA and has received Orphan Medicinal Product designation in the EU. The global trial will be conducted at centers of excellence in CLN1 research, including the University of Rochester Medical Center in the U.S. and the University of Hamburg-Eppendorf in Germany.

“ABO-202 is a promising AAV9 gene therapy that extended survival and improved neurological function in the animal model of CLN1 disease. Importantly, the combined intravenous and intrathecal administration approach showed additional benefits compared to a single route of delivery, providing a new treatment paradigm for patients with devastating neurological diseases,” said Steven J. Gray, Ph.D., Batten disease researcher and Associate Professor, Pediatrics, UT Southwestern Medical Center.

About CLN1 disease (Infantile Batten disease)
CLN1 disease, also known as Infantile Neuronal Ceroid Lipofuscinosis or infantile Batten disease, is a rapidly-progressing rare lysosomal storage disease with no approved treatment. It primarily affects the central nervous system and typically manifests during the first year of life with vision impairment that can progress to blindness, progressive motor and cognitive decline, seizures and ultimately early death. The underlying cause of the disorder is a defect in the PPT1 gene that encodes the enzyme of the same name, resulting in the accumulation of lipopigments within cells, leading to neuroinflammation and neurodegeneration. Some patients with CLN1 disease develop symptoms later in childhood or in adulthood; these variants are called late-infantile, juvenile, or adult-onset CLN1.

About ABO-202
ABO-202 is a novel, one-time gene therapy for CLN1 disease, a rapidly-progressing rare lysosomal storage disease with no approved therapy. ABO-202 is dosed through intravenous and intrathecal infusions using the AAV9 vector to deliver a functional copy of the PPT1 gene to cells of the central nervous system and peripheral organs. Preclinical data demonstrated that combination intravenous and intrathecal dosing may enhance the therapeutic potential of ABO-202. The therapy is designed to address the underlying gene and enzyme deficiency that leads to abnormal buildup of lipopigments and results in neuroinflammation and neurodegeneration.

About Abeona Therapeutics
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and cell therapies for serious diseases. The Company's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa, as well as ABO-102 and ABO-101, novel AAV9-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively. The Company's portfolio of AAV9-based gene therapies also features ABO-202 and ABO-201 for CLN1 disease and CLN3 disease, respectively. Its preclinical assets include ABO-401, which uses the novel AIM™ AAV vector platform to address all mutations of cystic fibrosis. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates and is the only company with Regenerative Medicine Advanced Therapy designation for two candidates (EB-101 and ABO-102). For more information, visit www.abeonatherapeutics.com.

Forward Looking Statement
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 Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties. These statements include statements regarding our pipeline including the therapeutic potential for ABO-202 in the treatment of CLN1, including the ability to effectively treat CLN1 disease in human patients, the ability to obtain regulatory marketing approvals, and the company's goals and objectives. We have attempted to identify forward looking statements by such terminology as "may," will," "anticipate," "believe," "estimate," "expect," "intend," and similar expressions.

Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, numerous risks and uncertainties, including but not limited to: continued interest in our rare disease portfolio, our ability to initiate and enroll patients in clinical trials, the impact of competition, the ability to secure licenses for any technology that may be necessary to commercialize our products, the ability to achieve or obtain necessary regulatory approvals, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, and other risks as may be detailed from time to time in the Company's annual reports on Form 10-K and quarterly reports on Form 10-Q and other reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or update them to reflect events or circumstances occurring after the date of this presentation, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

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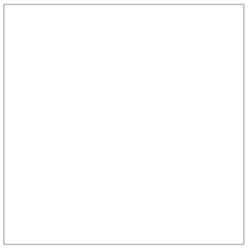

Source: Abeona Therapeutics Inc.