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Abeona Therapeutics Receives FDA Fast Track Designation for ABO-202 AAV9 Gene Therapy in CLN1 Disease

NEW YORK and CLEVELAND, June 18, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to its ABO-202 program. ABO-202, the Company's novel one-time AAV9 gene therapy for CLN1 disease, is designed to deliver a functional copy of the PPT1 gene to the central nervous system and peripheral organs using a combination of intravenous and intrathecal administrations. Abeona is preparing to initiate a Phase 1/2 clinical trial evaluating ABO-202 in patients with CLN1 disease and will provide guidance on the timing of the trial later this year.

"Receiving Fast Track designation acknowledges the urgency for developing a therapy for children suffering from this rapidly-progressing and fatal disease and highlights the significant potential of ABO-202 to address this unmet need," said João Siffert, M.D., Chief Executive Officer.

ABO-202 is administered as a one-time adeno-associated virus 9 (AAV9) gene therapy that delivers a functional copy of the *PPT1* gene to cells of the central nervous system and peripheral organs. This enables cells to produce a functioning PPT1 enzyme, which is critical for proper metabolism in lysosomes. The absence of this enzyme in patients with CLN1 disease results in malfunctioning cells, including brain cells, neuroinflammation, and neurodegeneration. In preclinical studies, ABO-202 normalized survival and improved neurological function in CLN1 mice. These studies also showed that a combination of intravenous and intrathecal administrations of ABO-202 improved efficacy over either delivery route alone, and that early treatment significantly improved outcomes.

Fast Track designation, granted by the FDA, is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. The purpose is to get important new drugs to the patient earlier through more frequent interactions with FDA, potential eligibility for accelerated approval, priority regulatory review, and rolling BLA review. ABO-202 has also received Orphan Drug designations in the U.S. and EU, and Rare Pediatric Disease designation from the FDA.

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 progresses to blindness, motor and cognitive decline, seizures and ultimately early death. The underlying cause of the disorder is

mutations in the *PPT1* gene that encodes the enzyme of the same name, resulting in lysosome dysfunction that leads to cellular dysfunction, 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 disease.

About ABO-202
ABO-202 is a novel, one-time AAV9 gene therapy for patients with CLN1 disease, a rapidly-progressing rare lysosomal storage disease with no approved therapy. ABO-202 is administered through intravenous and intrathecal infusions using an AAV9 vector to deliver a functional copy of the *PPT1* gene to cells of the central nervous system and peripheral organs. In preclinical studies, ABO-202 normalized survival and improved neurological function in CLN1 mice. These studies also showed that a combination of intravenous and intrathecal administrations of ABO-202 improved efficacy over either delivery route alone, and that early treatment significantly improved outcomes.

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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