Abeona Therapeutics and Taysha Gene Therapies Enter into Licensing and Inventory Purchase Agreements for ABO-202, a Clinical Stage, Novel, One-time Gene Therapy for CLN1 Disease

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NEW YORK and CLEVELAND and DALLAS, Aug. 17, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, and Taysha Gene Therapies, a patient-centric gene therapy company with a mission to eradicate monogenic CNS disease, today announced that they have entered into license and inventory purchase agreements for ABO-202, an adeno-associated virus (AAV) gene therapy for CLN1 disease (also known as infantile Batten disease). The agreements grant Taysha worldwide exclusive rights to intellectual property developed by scientists at the University of North Carolina at Chapel Hill (UNC) and Abeona, and Abeona know-how relating to the research, development, and manufacture of ABO-202. The therapy was originally developed in the lab of Steven Gray, Ph.D., Associate Professor at UT Southwestern Medical Center (formerly with UNC) and Chief Scientific Advisor for Taysha Gene Therapies. Abeona continued to progress the program, including development of the Phase 1/2 clinical trial protocol and manufacturing process, and received FDA clearance of its Investigational New Drug (IND) Application for a Phase 1/2 clinical trial that is anticipated to enter the clinic in 2021.

Under the terms of the agreement, Taysha will make initial cash payments to Abeona of $7 million, comprised of a $3 million upfront license fee and $4 million inventory purchase price, including GMP-sourced CLN1 plasmid from Abeona. In addition, Abeona is eligible to receive up to $56 million from Taysha upon the achievement of certain clinical, regulatory and sales milestones, plus high single-digit royalties on net sales of Taysha's CLN1 product.

"We are excited to partner with Taysha in their further development of a potential treatment for children living with Batten disease," said João Siffert, M.D., Chief Executive Officer of Abeona. "At the same time, these agreements allow Abeona to continue to focus its resources on advancing its key clinical programs in RDEB, MPS IIIA and MPS IIIB towards Biologics License Application submissions with the goal of providing safe and effective gene and cell therapies to patients who currently have no approved treatment options."

"CLN1 is a progressive monogenic CNS disease with significant unmet medical need, and we believe the ABO-202 data generated thus far demonstrate great translational potential and offer hope to children suffering from this devastating disorder," said RA Session II, President, Chief Executive Officer and Founder of Taysha. "We are excited to continue working closely with Dr. Gray to rapidly advance this promising gene therapy into the clinic."

ABO-202 is a one-time, self-complementary AAV (serotype 9) gene therapy designed to deliver a functional copy of the palmitoyl-protein thioesterase 1 (PPT1) gene. Preclinical studies in a CLN1 animal model demonstrated that ABO-202 normalized survival and led to improvement of neurological function in affected mice. The therapy has been granted Orphan Drug and Rare Pediatric Disease designations by the U.S. Food and Drug Administration (FDA) and has received Orphan Medicinal Product Designation from the European Medicines Agency.

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 delivers a functional copy of the PPT1 gene 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. Abeona's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, as well as ABO-102 and ABO-101, novel AAV-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively, in Phase 1/2 development. The Company's portfolio of AAV-based gene therapies also features ABO-201 for CLN3 disease. Abeona's novel, next-generation AIM™ capsids have shown potential to improve tropism profiles for a variety of devastating diseases. Abeona's fully functional, gene and cell therapy GMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and commercial production of AAV-based gene therapies. For more information, visit www.abeonatherapeutics.com.

About Taysha Gene Therapies
Taysha Gene Therapies is a patient-centric gene therapy company with a mission to eradicate monogenic CNS disease. We are focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the CNS in both rare and large patient populations. We were founded in partnership with The University of Texas Southwestern Medical Center, or UT Southwestern, to develop and commercialize transformative gene therapy treatments. Together with UT Southwestern, we are advancing a deep and sustainable product portfolio of 18 gene therapy product candidates, with exclusive options to acquire four additional development programs. By combining our management team's proven experience in gene therapy drug development and commercialization with UT Southwestern's world-class gene therapy research capabilities, we believe we have created a powerful engine to develop transformative therapies to dramatically improve patients' lives. More information is available at www.tayshagtx.com.

Forward-Looking Statements

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 about the Company's clinical trials and its products and product candidates, future regulatory interactions with regulatory authorities, as well as the Company's goals and objectives. We have attempted to identify forward looking statements by such terminology as "may," "will," "believe," "estimate," "expect," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. 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 the potential impacts of the COVID-19 pandemic on our business, operations, and financial condition, continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the outcome of any future meetings with the U.S. Food and Drug Administration or other regulatory agencies, 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 disclosed in the Company's most recent Annual Report on Form 10-K and subsequent quarterly reports on Form 10-Q and other periodic reports filed with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or to 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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