May 4, 2022

Abeona Therapeutics Announces AAV204, a Novel AAV Capsid, Demonstrated Robust Macular Transduction Following Para-Retinal Administration in Non-Human Primates

NEW YORK and CLEVELAND, Ohio, May 04, 2022 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in cell and gene therapy, today announced the presentation of new preclinical data on AAV204 at the Association for Research and Vision in Ophthalmology (ARVO) Annual Meeting, taking place on May 1-4, 2022 in Denver, CO and virtually on May 11-12, 2022. The data was featured in a poster presentation entitled “AAV204, a Novel AAV Capsid, Demonstrates Superior Macular Transduction Following Para-Retinal Administration in Non-Human Primates.”

AAV204, a novel adeno-associated virus (AAV) capsid from Abeona’s in-licensed AIM™ capsid library, has previously been shown to facilitate transduction of both the inner and outer retina after intravitreal administration in mice and non-human primates. The purpose of the current study was to evaluate in non-human primates transduction levels in the macula and optic nerve following administration of AAV204 directly into the vitreous of the eye by para-retinal administration, a recently-developed method, which unlike subretinal administration does not create a retinal detachment. AAV204.GFP or AAV8.GFP were administered to four non-human primate eyes and green fluorescent protein (GFP) expression was monitored using scanning laser ophthalmoscopy (SLO), followed by immunohistochemistry analysis at 28 days post-injection.

Results from this study showed that AAV204 induced high GFP expression in the macula and optic nerve as measured by SLO imaging and immunohistochemistry analysis, while AAV8-injected animals showed little to no GFP expression in the macula or optic nerve.

“The results presented at ARVO show that AAV204 is able to achieve high macular and optic nerve transduction levels with an administration route that is less invasive and safer than subretinal injection,” said Brian Kevany, Ph.D., Chief Technical Officer and Head of Research at Abeona. “Of note, the AAV204 dose used in this study was lower than those typically used for intravitreal injections and resulted in no clinically-relevant inflammation in any treated animals.”

About the AIM™ capsid library
Capsids from the AIM™ capsid library exclusively licensed by Abeona are novel AAV serotypes that target delivery of genetic payloads to the central nervous system (including the retina), lungs, eye, muscle, liver and other tissues with potentially improved tropism

profiles key to enable treatment of a variety of devastating diseases. AIM™ vectors have shown the potential to evade the immune response generated by exposure to naturally-occurring AAV vectors. AAV204 is covered by U.S. Patent Nos. 10,532,110 and 10,561,743.

About Abeona Therapeutics

Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for serious diseases. Abeona's lead clinical program is EB-101, its investigational autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development. The Company's development portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical need. Abeona's novel, next-generation AAV capsids are being evaluated to improve tropism profiles for a variety of devastating diseases. Abeona's fully integrated cell and gene therapy cGMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and potential commercial production of AAV-based gene therapies. For more information, visit www.abeonatherapeutics.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. 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 potentially commercialize our EB-101 product candidate; obtaining a strategic partnership to take over development activities for ABO-102; 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 potentially commercialize our product candidates; 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; reducing our operating expenses and extending our cash runway; our ability to execute our operating plan and achieve important anticipated milestones; and other risks disclosed in the Company's most recent Annual Report on Form 10-K 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 press release, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

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