April 20, 2021

Abeona Therapeutics Announces Presentation on New Preclinical Data Supporting the Potential of Cre-Mediated Dual AAV Vector Technology to Enable Delivery of Large Genes Targeted for Treatment of Stargardt Disease at ARVO 2021 Annual Meeting

NEW YORK and CLEVELAND, April 20, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that new data from its preclinical research will be highlighted during an oral presentation at the Association for Research in Vision and Ophthalmology (ARVO) 2021 Annual Meeting, being held virtually from May 1-7, 2021.

“Autosomal recessive Stargardt disease is caused by mutations in the ABCA4 gene, preventing removal of toxic substances from photoreceptor cells that result in photoreceptor death and progressive vision loss,” said Linas Padegimas, Ph.D., Senior Director, Product Development at Abeona. “The preclinical data being presented at ARVO's Annual Meeting provides compelling evidence that two independent AAV vectors utilizing Cre recombinase can efficiently reconstitute the ABCA4 gene, leading to full-size ABCA4 protein expression. Future studies that explore Cre-mediated ABCA4 gene reconstitution and lipofuscin clearance *in vivo* could provide additional findings to inform clinical development plans for a dual AAV vector approach as a potential treatment for Stargardt disease. In addition, our Cre-mediated dual AAV vector technology holds potential benefit for other indications that require delivery of larger genes that exceed the encapsidation capacity of AAV vectors.”

Details of the oral presentation are as follows:

Title: *Dual AAV Vector Strategy for Expression of Large Genes Targeted for Stargardt Disease Gene Therapy Development*
Presenter: Dr. Brianna Barrett, Abeona Therapeutics Inc.
Session Title: Gene therapy in ocular diseases
Session Date and Time: May 4, 2021 from 2:15 PM to 3:45 PM EDT

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 also features AAV-based gene therapies for ophthalmic diseases with high unmet medical needs. 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.

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 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 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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