April 10, 2023

Abeona Therapeutics Announces Multiple Abstracts Accepted from its AAV Ophthalmology Program at the 26th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT)

CLEVELAND, April 10, 2023 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today announced the acceptance of three abstracts at the 26th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) taking place from May 16-20, 2023 in Los Angeles, CA. Accepted abstracts include new data on three internally developed investigational preclinical gene therapy product candidates from its adeno-associated virus (AAV) ophthalmology program, including ABO-504 for Stargardt disease, ABO-503 for X-linked retinoschisis (XLRS) and ABO-505 for autosomal dominant optic atrophy (ADOA).

“At ASGCT, the data we present will highlight the potential of our proprietary AAV capsids and gene constructs to express recombinant protein in target eye tissues and rescue mutant phenotypes,” said Brian Kevany, Ph.D., Chief Technical Officer and Head of Research at Abeona. “We have started to submit pre-IND meeting requests to the FDA for our gene therapy product candidates and anticipate meetings to take place in the second quarter of 2023. We are excited for the opportunity to share our new data with the scientific community at ASGCT.”

Details for the poster presentations are as follows:

Abstract Title: In Vivo Production of Full-Length ABCA4 Protein Following Cre-Mediated Recombination from Dual AAV Vectors in ABCA4-/- Mice
Presenter: Dr. Paul Wille, Director, Product Development, Abeona Therapeutics
Session Date/Time: Wednesday, May 17, 2023 at 12:00 PM PT
Abstract number: 715

Abstract Title: ABO-503, a Novel Gene Therapy for Treatment of X-Linked Retinoschisis
Presenter: Dr. Joseph Fogerty, Senior Scientist, Product Development, Abeona Therapeutics
Session Date/Time: Thursday, May 18, 2023 at 12:00 PM PT
Abstract number: 1131

Abstract Title: AAV Gene Therapy for Autosomal Dominant Optic Atrophy Caused by Mutation in the Opa1 Gene
Presenter: Dr. Rachel Stupay, Senior Scientist, Product Development, Abeona Therapeutics

Session Date/Time: Friday, May 19, 2023 at 12:00 PM PT
Abstract number: 1478

About Abeona's AAV Ophthalmology Program
Abeona's preclinical programs are investigating the use of novel adeno-associated virus (AAV) capsids in therapies for serious genetic eye diseases. The most common form of Stargardt disease is caused by mutations in the ABCA4 gene, which prevent removal of toxic compounds from photoreceptor cells that results in photoreceptor cell death and progressive vision loss. ABO-504 is designed to efficiently reconstitute the full-length ABCA4 gene by implementing a dual AAV vector strategy using the Cre-LoxP recombinase system.

XLRS is a rare, monogenic retinal disease that results in the irreversible loss of photoreceptor cells and severe visual impairment. XLRS is caused by mutations in the RS1 protein, which is normally secreted by retinal photoreceptors and bipolar neurons and functions to mediate cell-cell adhesion. ABO-503, composed of a functional human RS1 packaged in the novel AIM™ capsid AAV204, has shown preclinical efficacy following delivery to the retina in a mouse model of XLRS.

ADOA, a form of hereditary vision loss associated with RGC death, is predominantly caused by mutations in the Opa1 gene. ABO-505 is designed to express a functional copy of human Opa1 in the retina following para-retinal injection. AB0-505 aims to take advantage of the robust optic nerve and retinal ganglion cell (RGC) transduction ability of AAV204 to deliver its genetic payload to the cells most affected by ADOA.

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, engineered cell therapy currently in development for recessive dystrophic epidermolysis bullosa. 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," "anticipate," "expect," "intend," 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, our ability to continue as a going concern; the timing and outcome of our Biologics License Application submission to the FDA for EB-101; continued interest in our rare disease portfolio; our ability to enroll patients in clinical trials; the outcome of any future meetings with the FDA or other regulatory agencies; 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 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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Source: Abeona Therapeutics Inc.