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Abeona Therapeutics Reports Preclinical Data Demonstrating Broad Therapeutic Potential of AIM™ Gene Therapy in Retinal Diseases at Association for Research in Vision and Ophthalmology Annual Meeting

NEW YORK and CLEVELAND, May 01, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that intravitreal administration of the Company's novel AIM™ AAV204 capsid to non-human primates led to robust transgene expression in the inner and outer retina. These new preclinical data also support the potential use of intravitreal administration to deliver gene therapy in an out-patient setting for a wide range of inherited and acquired retinal diseases. Findings were presented at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting in Vancouver.

"The broad retinal tropism of the AIM™ AAV204 capsid in non-human primates underscores the potential of our platform to deliver gene therapy beyond inherited diseases, including treatment of acquired retinal disorders that may be currently underserved," said Timothy J. Miller, Ph.D., President and Chief Scientific Officer. "Intravitreal administration of AAV gene therapy, which does not require surgery, could potentially be performed in an out-patient setting and may be a safer and less invasive approach."

In the preclinical study, intravitreal administration of the novel AIM™ AAV204 capsid in non-human primates resulted in broad transgene expression in the peripheral retina as well as intense expression in the fovea 25 days post-administration. AAV204 also transduced photoreceptor cells in retinal explants and transduced the outer retina, with positive green fluorescent protein (GFP) expression.

The non-human primate data were complemented by findings from mice models, which identified AAV204 as one of three lead candidate AIM™ capsids that demonstrate robust transduction of retinal cells. The data in mice demonstrated that intravitreal administration resulted in broad retinal expression of AAV204 that penetrated to the photoreceptor and retinal pigmented epithelium (RPE) layers.

About the AIM™ Vector Platform
Abeona is developing the AIM™ Vector Platform: a next generation of adeno-associated virus (AAV) capsids for use in gene therapies. The AIM™ capsid library can utilize AAV biology to selectively target delivery of genetic payloads to the central nervous system, lungs, eye, muscle, liver and other tissues. AIM™ vectors are non-virus-producing and have shown the potential to evade the immune responses generated by exposure to naturally-

occurring AAV vectors. The Company's AIM™ library contains more than 100 capsids with tissue tropisms selected for their potential to target a wide range of organs and multiple routes of delivery.

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). 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 investigational therapies (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 potential for the AIM™ vector platform in the treatment of retinal diseases based upon pre-clinical data, 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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