

January 15, 2020



Abeona Therapeutics Announces Issuance of U.S. Patent for AIM™ Capsids

NEW YORK and CLEVELAND, Jan. 15, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that the United States Patent and Trademark Office (USPTO) issued a patent covering next-generation adeno-associated virus (AAV) capsids generated using the University of North Carolina's (UNC) AIM™ vector platform and that the USPTO allowed the claims of a companion patent application, which will issue in the next several weeks. Both patents are exclusively licensed by the Company from UNC.

"The issuance of these patents strengthens intellectual property around our library of AIM™ capsids that have shown potential to improve tropism profiles for a variety of devastating diseases," said João Siffert, M.D., Chief Executive Officer. "It is also a step forward in the development of next-generation AAV technology that could make re-treatment with gene therapy possible for patients who have been previously treated using certain AAV-based vectors."

On January 14, 2020, the USPTO issued UNC's U.S. Patent No. 10,532,110, entitled "AAV Vectors Targeted to the Central Nervous System." The patent provides protection for methods of delivering Abeona's AAV204-based gene therapy vectors to the central nervous system until November 2036.

On January 2, 2020, the USPTO allowed the claims of a companion UNC patent application covering Abeona's AAV204-based gene therapy vector compositions. A patent is expected to be issued for the application in the next several weeks, with an expected expiration date in November 2035.

Abeona continues to develop the AIM™ capsid library and to generate improved vectors for use in gene therapies. AIM™ capsids use AAV biology to selectively 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 are non-virus-producing and have shown the potential to evade the immune response 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), respectively. The Company's portfolio of AAV9-

based gene therapies also features ABO-202 and ABO-201 for CLN1 disease and CLN3 disease, respectively. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates, including Regenerative Medicine Advanced Therapy designation for two candidates (EB-101 and ABO-102). 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 about the Company's ability to secure patents for its technology, the Company's products, product candidates including AIM platform; and 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 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 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 periodic reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligation to revise these 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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