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Abeona Therapeutics Announces Licensing of the AIM(TM) Next Generation AAV Gene Therapy Vector Platform From The University of North Carolina at Chapel Hill

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- *AIM ™ vector system is a novel AAV-based vector technology platform that may target CNS and other tissues with increased efficiency and tissue specificity*
- *Exclusive worldwide license adds ABO-202, an AAV gene therapy product, for the treatment of CLN1 patients with infantile neuronal ceroid lipofuscinosis (INCL, infantile Batten disease) to Abeona's Batten pipeline; clinical trials anticipated 2017*

Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical-stage biopharmaceutical company focused on developing therapies for life-threatening rare genetic diseases, announced today the exclusive worldwide license of a next generation gene therapy AAV capsid portfolio from University of North Carolina at Chapel Hill. The AIM™ vector system is a next generation platform of AAV capsids capable of widespread central nervous system gene transfer and can be used to confer high transduction efficiency for various therapeutic indications. Studies indicate that AIM vectors can efficiently and broadly target CNS tissue, and may provide a treatment for patients that have inhibitory antibodies to natural AAV serotypes. Importantly, the AIM vector system may provide second-generation treatment approaches for patients that have received a previous AAV injection.

"As we continue to build out our orphan and rare disease drug portfolio and move additional programs into the clinic, this agreement with UNC continues the execution of our strategy to combine our expertise in advancing gene therapy programs with the development of a next-generation proprietary AAV vector platform," stated Steven H. Rouhandeh, Executive Chairman. "We look forward to harnessing the clinical utility and therapeutic potential of the AIM vector system technology platform to address a broad range of rare genetic diseases."

In addition to the AAV capsid library, the license also adds ABO-202, an AAV-based CLN1 program, to Abeona's Batten pipeline. ABO-202, developed at UNC by Steven Gray, Ph.D. with the support of The Saoirse Foundation, Taylor's Tale, Hayden's Batten Disease Foundation, and the Batten Disease Support and Research Association, is anticipated to enter clinical trials in 2017 for patients with infantile neuronal ceroid lipofuscinosis (INCL, infantile Batten disease), an inherited fatal genetic disease that primarily affects the nervous system.

"ABO-202 has shown promising preclinical efficacy in INCL mice after delivery of a functioning copy of the CLN1 gene to cells of the central nervous system, by extending survival and preserving strength when administered early in the disease course," noted Steven J. Gray, Ph.D, Assistant Professor, Department of Ophthalmology, Gene Therapy Center, University of North Carolina at Chapel Hill. "Our work in developing these novel, next generation AAV gene therapy vectors has the potential to further advance the field of AAV-based technologies by efficiently and specifically targeting the CNS, with a likelihood of avoiding antibodies endogenously generated by natural AAV serotypes."

"The AIM vector system is a next generation AAV-based gene therapy technology platform that represents a transformational opportunity for Abeona. The AIM platform will allow us to leverage our current pipeline into second generation products for CNS and other tissue-specific delivery, and help provide an answer for patients that have existing inhibitory antibodies," stated Timothy J. Miller, Ph.D., President & CEO. "In addition, we add another AAV-based product ABO-202 (AAV-CLN1) for treatment of patients with infantile neuronal ceroid lipofuscinosis (INCL), which builds on our expertise in developing treatments for patients with forms of Batten disease."

About Abeona: Abeona Therapeutics, Inc. is a clinical stage company developing gene and plasma-based therapies for life-threatening rare genetic diseases. Abeona's lead programs are ABO-102 (AAV-SGSH) and ABO-101 (AAV-NAGLU), adeno-associated virus (AAV) based gene therapies for Sanfilippo syndrome (MPS IIIA and IIIB), respectively. Abeona is also developing EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL); ABO-202 (AAV-CLN1) gene therapy for treatment of infantile Batten disease (INCL), and ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, Abeona has a plasma-based protein therapy pipeline, including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD, using our proprietary SDF™ (Salt Diafiltration) ethanol-free process. For more information, visit www.abeonatherapeutics.com.

About Infantile neuronal ceroid lipofuscinosis (INCL): *CLN1*, also known as *PPT1*, encodes an enzyme called palmitoyl-protein thioesterase 1 that is insufficiently active in Infantile NCL. Infantile NCL (INCL or Santavuori-Haltia disease) begins between about ages 6 months and 2 years and progresses rapidly. Affected children fail to thrive and have microcephaly. Also typical are short, sharp muscle contractions called myoclonic jerks. These children usually do not reach age 5.

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 that involve risks and uncertainties. These statements include, without limitation, our plans for continued development and internationalization of our clinical programs, that the AIM ™ vector system is a next generation AAV-based vector technology platform that may target CNS and other tissues, with increased efficiency and tissue specificity, that studies indicate that AIM vectors can efficiently and broadly target CNS tissue, and may provide a treatment for patients that have inhibitory antibodies to natural AAV serotypes, that the AIM vector system may provide second-generation treatment approaches for patients that have received a previous AAV injection, that is anticipated to enter clinical trials in 2017 for patients with infantile neuronal ceroid lipofuscinosis.

These statements are subject to 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 impact of competition; the ability to develop our products and technologies; the ability to achieve or obtain necessary regulatory approvals; the impact of changes in the financial markets and global economic conditions; and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and other reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligations to make any revisions to the forward-looking statements contained in this release or to update them to reflect events or circumstances occurring after the date of this release, whether as a result of new information, future developments or otherwise.

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