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Abeona Therapeutics Highlights New Preclinical Juvenile Neuronal Ceroid Lipofuscinosis (JNCL) Data at WORLDSymposium(TM) 2016

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- ***AB0-201 (AAV CLN3) demonstrates promising in vivo efficacy in preclinical models***
- ***IND enabling toxicology to commence in 2016***

Abeona Therapeutics, Inc. (NASDAQ: ABEO), a biopharmaceutical company focused on developing and delivering products for severe and life-threatening rare diseases, today announced the presentation of preclinical data on AB0-201 (AAV CLN3) for Juvenile neuronal ceroid lipofuscinosis (JNCL) (also known as juvenile Batten disease).

Title: ***Adeno-associated virus 9 gene therapy for Juvenile neuronal ceroid lipofuscinosis***

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Abstract: Juvenile Neuronal Ceroid Lipofuscinosis (JNCL) is a fatal pediatric lysosomal disease (LSD) caused by an autosomal recessive mutation in *CLN3*. Symptoms appear between 5-10 years, beginning with blindness and seizures, followed by progressive cognitive and motor decline, and premature death. We explored a gene therapy approach for JNCL by generating two self-complementary (sc) AAV (scAAV) constructs to address CLN3 dosage effects; one driving low CLN3 expression with the methyl-CpG-binding protein 2 (MeCP2) promoter that previously showed efficacy in a dose-dependent model of Rett syndrome, and a second driving high CLN3 levels via the β-actin promoter. The rationale for this approach was the expectation that low CLN3 levels are required for cellular homeostasis due to minimal CLN3 expression postnatally, although this had not yet been directly demonstrated in vivo. One month-old CLN3Δex7/8 mice received a dose of scAAV/MeCP2-hCLN3 or scAAV/β-actin-hCLN3 i.v., with GFP expressing viruses as controls. scAAV biodistribution was widespread in the central nervous system (CNS) following systemic i.v. injection, with $NeuN^+$ neurons and $GFAP^+$ astrocytes transduced, consistent with other reports using scAAV in models of MPS III and SMA. In addition to improving motor function at 5 months post-injection scAAV/MeCP2-hCLN3 reduced astrocyte activation and lysosomal storage material in the $CLN3^{\Delta ex7/8}$ brain. A promoter-dosage effect in $CLN3^{\Delta ex7/8}$ mice receiving scAAV/ MeCP2-hCLN3 vs. scAAV/β-actin-hCLN3 was confirmed in the eye and brain regions where neurons are destined to die in JNCL, including the thalamus, visual cortex, and hippocampus. Interestingly, we observed a difference between CLN3 levels and neuroprotection, with only the scAAV construct driving low CLN3 expression (scAAV/MeCP2-hCLN3) correcting motor deficits. No significant toxicity was observed in scAAV/MeCP2-hCLN3 treated mice through 1 year post-injection. Our results represent the first demonstration of a CLN3 dosage effect in vivo and support the use of scAAV/MeCP2-hCLN3 in clinical trials for treating JNCL.

"ABO-201 has demonstrated encouraging in vivo efficacy in preclinical Juvenile Neuronal Ceroid Lipofuscinosis (JNCL; also known as Juvenile Batten disease) model", stated Tim Miller, PhD, President & CEO, Abeona Therapeutics, "We look forward to moving our third lysosomal storage disease product towards clinical development for JNCL patients and their families."

About ABO-201 (AAV CLN3): ABO-201 (AAV CLN3) is an AAV-based gene therapy which has shown promising preclinical efficacy in delivery of a normal copy of the defective CLN3 gene to cells of the central nervous system with the aim of reversing the effects of the genetic errors that cause for Juvenile Neuronal Ceroid Lipofuscinosis (JNCL; also known as Juvenile Batten disease). JNCL is a rare, fatal, autosomal recessive (inherited) disorder of the nervous system that typically begins in children between 4 and 8 years of age. Often the first noticeable sign of JNCL is vision impairment, which tends to progress rapidly and eventually result in blindness. As the disease progresses, children experience the loss of previously acquired skills (developmental regression). This progression usually begins with the loss of the ability to speak in complete sentences. Children then lose motor skills, such as the ability to walk or sit. They also develop movement abnormalities that include rigidity or

stiffness, slow or diminished movements (hypokinesia), and stooped posture. Beginning in mid-to late childhood, affected children may have recurrent seizures (epilepsy), heart problems, behavioral problems, and difficulty sleeping. Life expectancy is greatly reduced. Most people with JNCL disease live into their twenties or thirties. As yet, no specific treatment is known that can halt or reverse the symptoms of JNCL.

About Abeona: Abeona Therapeutics, Inc. develops and delivers gene therapy and plasma-based products for severe and life-threatening rare diseases. Abeona's lead programs are ABO-101 (AAV NAGLU) and ABO-102 (AAV SGSH), adeno-associated virus (AAV)-based gene therapies for Sanfilippo syndrome (MPS IIIB and IIIA). We are also developing ABO-201 (AAV CLN3) gene therapy for Juvenile Neuronal Ceroid Lipofuscinosis (JNCL); and ABO-301 (AAV FANCC) for Fanconi anemia (FA) disorder using a novel CRISPR/Cas9-based gene editing approach to gene therapy program for rare blood diseases. In addition, we are also developing rare plasma protein therapies 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.

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, management plans for the Company, and general business outlook. 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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