November 5, 2018

Abeona Therapeutics and REGENXBIO Announce Worldwide Exclusive Licenses for the Treatment of Four Rare Lysosomal Storage Disorders Using NAV AAV9 Vector

- *Abeona granted new licenses to NAV AAV9 for the development and commercialization of treatments for MPS IIIA, MPS IIIB, CLN1 and CLN3 Batten Disease*
- *Abeona to pay REGENXBIO $20 million upfront, $10 million paid upon signing, $10 million by first anniversary of agreement; annual payments of $20 million guaranteed payable upon the second anniversary of agreement*

NEW YORK and CLEVELAND, Nov. 05, 2018 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc.(Nasdaq: ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene and cell therapies for life-threatening rare diseases, and REGENXBIO (Nasdaq: RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV® Technology Platform, today announced a license agreement to REGENXBIO's NAV AAV9 vector for the treatment of four diseases: Sanfilippo syndrome type A (MPS IIIA), Sanfilippo syndrome type B (MPS IIIB), Infantile Batten Disease, also known as neuronal ceroid lipofuscinosis type 1 (CLN1 Disease), and Juvenile Batten Disease, also known as neuronal ceroid lipofuscinosis type 3 (CLN3 Disease).

“This agreement is an important milestone that underpins the therapeutic potential we see in our Sanfilippo syndrome and Batten disease programs featuring the NAV AAV9 vector, which have the potential to transform the lives of patients,” said Carsten Thiel, Ph.D., Chief Executive Officer of Abeona. “Data from our clinical and preclinical programs and the success of the NAV AAV9 vector observed in other indications strongly positions the platform as a leading technology for investigational gene therapies for the systemic and CNS manifestations of lysosomal storage diseases.”

Under the terms of the agreement, REGENXBIO has granted Abeona an exclusive worldwide license (subject to certain non-exclusive rights previously granted for MPS IIIA), with rights to sublicense, to REGENXBIO’s NAV AAV9 vector for the development and commercialization of gene therapies for the treatment of MPS IIIA, MPS IIIB, CLN1 Disease and CLN3 Disease. In return for these rights, REGENXBIO will receive a guaranteed $20 million upfront payment, $10 million of which will be paid upon signing and $10 million of which will be paid within 12 months of the effective date. In addition, REGENXBIO will receive a total of $100 million in annual fees, payable upon the second through sixth anniversaries of the agreement, $20 million of which is guaranteed. REGENXBIO is also eligible to receive potential commercial milestone payments of up to $60 million. REGENXBIO will also receive low double-digit royalties on net sales of products incorporating the licensed intellectual property.

“This license agreement further validates the potential of NAV AAV9 for the treatment of systemic and CNS manifestations of lysosomal storage diseases, as well as the strength of our intellectual property portfolio,” said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. “We are pleased to initiate our partnership with Abeona as they continue to advance multiple programs using NAV AAV9 through and towards clinical trials in indications with significant unmet medical need.”

About Sanfilippo Syndrome
Sanfilippo syndrome, or MPS type III, is a group of rare genetic lysosomal storage diseases with no approved treatments. MPS III is characterized by aggressive behavior, seizures, loss of speech or vision, an inability to sleep, and premature death. An estimated 70% of children with MPS III do not reach age 18. The underlying cause of the syndrome is a missing enzyme that is essential to breaking down heparan sulfate. As a result, partially synthesized heparan sulfate accumulates in the central nervous system, including the brain and spinal cord, causing progressive damage. MPS III is categorized by the single gene defects associated with each type of the syndrome - A, B, C or D. The hallmark feature of MPS IIIA is a deficiency in the SGSH enzyme, while MPS IIIB is distinguished by a marked decrease in NAGLU enzyme activity.

About Batten Disease
Infantile and juvenile forms of Batten disease, known as CLN1 and CLN3, are rare autosomal recessive genetic disorders with no approved treatments. Batten disease is fatal, and most do not live past their twenties or thirties. The underlying cause of the disorder is a deficiency in proteins critical to lysosomal function that lead to abnormal buildup of lipopigments, and result in neuroinflammation and neurodegeneration. CLN1 and CLN3 are differentiated by mutations of their respective genes, yet the first noticeable sign of all forms of Batten disease is often vision impairment that can progress to blindness. Developmental regression is another hallmark of the disease, as children lose the ability to speak in complete sentences and to walk or sit, among other manifestations. Later in life, affected children may have recurrent seizures, heart problems, behavioral problems, and difficulty sleeping.

About REGENXBIO Inc.
REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates in multiple therapeutic areas.

About Abeona Therapeutics Inc.
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and ABO-101 (AAV-NAGLU), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type B (MPS IIIB). Abeona is also developing ABO-201 (AAV-CLN3) gene therapy for CLN3 disease, ABO-202 (AAV-CLN1) for treatment of CLN1 disease, EB-201 for epidermolysis bullosa (EB), ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder and ABO-302 using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, Abeona is developing a proprietary vector platform, AIM™, for next generation product candidates. For more information, visit www.abeonatherapeutics.com.

Abeona 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. These statements include statements that the NAV AAV9 vector may help advance our clinical programs in lysosomal storage diseases, the market opportunities for the company's products and product candidates, 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 (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 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 obligations to make any revisions to the forward-looking statements or to 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.

ABEONA CONTACT:

Investors
Christine Silverstein
SVP, Finance & Investor Relations
Abeona Therapeutics, Inc.
+1 (646) 813-4707
csilverstein@abeonatherapeutics.com

Media
Scott Santiamo
Director, Corporate Communications
Abeona Therapeutics, Inc.
+1 (646) 813-4719
ssantiamo@abeonatherapeutics.com

Source: Abeona Therapeutics Inc.