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

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- REGENXBIO grants Abeona new licenses to NAV AAV9 for the development and commercialization of treatments for MPS IIIA, MPS IIIB, CLN1 and CLN3 Batten Disease
- REGENXBIO could receive up to $180 million, including $40 million in guaranteed payments

ROCKVILLE, Md., Nov. 5, 2018 /PRNewswire/ -- REGENXBIO Inc. (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, and Abeona Therapeutics Inc. (Nasdaq: ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel cell and gene therapies for life-threatening rare genetic diseases, 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).

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."

"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."

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.

REGENXBIO Forward-Looking Statements
This press release includes "forward-looking statements," 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. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's future operations and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2017 and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (SEC) and are available on the SEC's website at www.sec.gov. All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

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