FDA Grants Orphan Drug Designation To RGX-181 Gene Therapy For The Treatment Of CLN2 Form Of Batten Disease

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- Novel, one-time investigational treatment for CLN2 disease designed to halt progression of neurological decline
- Company continues to advance toward IND submission in 2019

ROCKVILLE, Md., Nov. 14, 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, today announced the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation to RGX-181, a one-time treatment candidate for late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, one of the most common forms of Batten disease caused by mutations in the tripeptidyl peptidase 1 (TPP1) gene.

"We believe RGX-181 has the potential to correct the underlying genetic condition, halt progression and address many of the serious and life-threatening symptoms of CLN2 disease," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "CLN2 disease is an extremely debilitating disease in children with no cure and limited treatment options. Receiving Orphan Drug Designation from the FDA signifies our continued progress and commitment to develop RGX-181 as a potential one-time treatment for children with CLN2 disease."

FDA Orphan Drug Designation is granted to investigational therapies addressing rare medical diseases or conditions that affect fewer than 200,000 people in the United States. Orphan drug status provides benefits to drug developers including assistance in the drug development process, tax credits for clinical costs, exemptions from certain FDA fees and seven years of marketing exclusivity.

RGX-181 is designed to use REGENXBIO's NAV AAV9 vector to deliver the TPP1 gene directly to the central nervous system (CNS), which may induce sustained levels of TPP1, the enzyme deficient in children with CLN2 disease. REGENXBIO plans to submit an Investigational New Drug (IND) application for RGX-181 to the FDA in 2019 to enable initiation of a first-in-human clinical trial.

About RGX-181
RGX-181 is being developed as a novel, one-time treatment for CLN2 disease utilizing the NAV AAV9 vector to deliver the gene encoding for TPP1, the enzyme deficient in children with CLN2 disease. Following a single administration given by intracisternal injection, RGX-181 treatment is designed to modify cells in the CNS, thereby providing a durable source of TPP1 and allowing for long-term correction of cells throughout the CNS. In an animal model for CLN2 disease, treatment with RGX-181 has been shown to restore TPP1 activity to levels greater than those in non-affected animals, and to improve neurobehavioral function and survival. The extent of CNS correction observed in animal studies suggests that RGX-181 has the potential to be an important and suitable therapeutic option for patients with CLN2 disease.

About CLN2 Disease
Late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, a form of Batten disease, is a rare, pediatric-onset, autosomal recessive, neurodegenerative lysosomal storage disorder caused by mutations in the TPP1 gene. Mutations in the TPP1 gene, and subsequent deficiency in TPP1 enzymatic activity, result in lysosomal accumulation of storage material and degeneration of tissues including the brain and retina. CLN2 disease is characterized by seizures, rapid deterioration of language and motor functions, cognitive decline, loss of vision and blindness, and premature death by mid-childhood. Onset of symptoms is generally between two to four years of age with initial features of recurrent seizures (epilepsy), language delay, and difficulty coordinating movements (ataxia). There is currently no cure for CLN2 disease. Current treatment options include palliative care or enzyme replacement therapy, wherein recombinant TPP1 is administered into the lateral ventricles via a permanently implanted device on a biweekly basis.

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.

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 clinical trials. 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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