



# REGENXBIO®

## **REGENXBIO Announces Pipeline Expansion with RGX-181 Gene Therapy for CLN2 Form of Batten Disease**

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**Program aims to develop a one-time treatment to halt progression of this rare, pediatric, neurodegenerative disease**

**Company intends to advance RGX-181 to clinical trials with IND submission expected in 2019**

ROCKVILLE, Md., Aug. 30, 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<sup>®</sup> Technology Platform, today announced it is developing a new product candidate, RGX-181, for the treatment of 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.

"Our NAV technology platform holds tremendous promise for advancing this new product candidate in our neurodegenerative disease pipeline," said Olivier Danos, Ph.D., Senior Vice President and Chief Scientific Officer of REGENXBIO. "CLN2 is a rapidly progressing, fatal disease with limited treatment options and no cure. Children with CLN2 disease experience an array of serious symptoms such as seizures, deterioration of language and motor skills, blindness, cognitive decline and premature death. The goal of our RGX-181 clinical program is to develop a single-dose treatment to halt progression of neurological decline and improve a broad range of these devastating symptoms experienced by children with CLN2 disease."

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. Pharmacology studies conducted in an animal model of CLN2 disease demonstrated that a single administration of RGX-181 resulted in widespread distribution and sustained expression of the TPP1 enzyme in the CNS with significant improvements in neurobehavioral function and survival of the animals.

"For children living with Batten disease and their families, the promise of effective gene therapy gives incredible hope," said Margie Frazier, Ph.D., the Executive Director at the Batten Disease Support and Research Association. "A diagnosis of Batten disease is devastating, yet children with the condition, and their families, face the disease with courage. More and widely available treatment options are desperately needed to reduce the cognitive and physical effects of the disease."

REGENXBIO plans to submit an investigational new drug (IND) application for RGX-181 to the U.S. Food and Drug Administration in 2019 to enable initiation of a global first-in-human clinical trial with clinical centers planned in the United States and Europe. REGENXBIO has two other CNS products, RGX-111 and RGX-121, in clinical trials for the treatment of Mucopolysaccharidosis Type I (MPS I) and Mucopolysaccharidosis Type II (MPS II) diseases, respectively.

"Given the unmet need and the potential for NAV gene therapy, we believe CLN2 disease is a natural addition to our neurodegenerative disease pipeline," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "As we prepare to initiate dosing in our clinical trials of RGX-111 for MPS I and RGX-121 for MPS II, adding RGX-181 to our research pipeline furthers our commitment to finding potential cures for children affected by these extremely serious and life-threatening neurodegenerative conditions."

### **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](http://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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