Initial Clinical Data of First Pediatric CLN2 Patient Dosed with RGX-181 Presented at SSIEM Annual Symposium

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- *Six-month data from single-patient, investigator-initiated trial showed that one-time administration of RGX-181 was well tolerated, achieved sustained gene expression and demonstrated clinically meaningful improvements across multiple measures including reduced (86%) seizure frequency.*
- *Investigators observed encouraging neurodevelopmental skill acquisition at 6 months*

ROCKVILLE, Md., Aug. 30, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced that initial interim data from a first-in-human single-patient, investigator-initiated trial of RGX-181 for the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, a form of Batten disease, were presented at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium in Jerusalem.

"CLN2 is a debilitating disease caused by mutations in the *CLN2* gene resulting in a deficiency of the TPP1 enzyme, which is needed to break down specific peptides associated with cellular waste. Symptoms include seizures; loss of motor, language, and cognitive skills; vision loss and premature death; and existing treatments do not stop or reverse most manifestations of the disease," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "We are encouraged by the initial results demonstrating that RGX-181 is well tolerated and dramatically reduced the number of seizures in the patient enrolled in this trial."

"As someone who treats patients with this devastating disease, I see the limitations of the current standard of care," said Carolina Fischinger de Souza, M.D., Ph.D., Hospital de Clínicas de Porto Alegre, Brazil and investigator of this trial. "The remarkable decrease in seizures, encouraging safety results and reduction in ERT frequency highlight the potential of this gene therapy to provide a meaningful treatment option to the CLN2 patient community."

Data Summary and Safety Data

Today, a physician investigator from the Hospital de Clinicas in Porto Alegre, Brazil reported initial results from a five-year-old child who received a one-time intracisternal dose of RGX-181. Time of post-administration follow up was six months.

As of June 30, 2023, RGX-181 was well tolerated with no serious adverse events. Key efficacy measures demonstrated sustained levels of TPP1 along with increased intervals between enzyme replacement therapy (ERT) infusions and an 86% reduction in seizure frequency through six months, leading to withdrawal of two anti-epileptic medications. Encouraging improvements in fine motor and expressive language skills were also observed.

"These results represent the third consecutive program in our clinical-stage pipeline for rare neurodegenerative conditions that have shown a potential one-time gene therapy is well tolerated and results in physiologically relevant levels of gene expression and clinically meaningful changes in young children. We have received important regulatory designations for each of these three pipeline programs," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "Our first BLA filing for a rare neurodegenerative disease, Hunter syndrome, is planned for 2024 using the Accelerated Approval pathway, and we look forward to advancing our additional investigational AAV therapeutics as quickly as possible."

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 administration of a single intracisternal injection, RGX-181 treatment is designed to provide a durable source of TPP1, potentially leading to 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 suggests that RGX-181 has the potential to be an important and suitable one-time 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. Deficiency in TPP1 enzymatic activity results in lysosomal accumulation of storage material and degeneration of nerve cells, particularly in the brain and retina. CLN2 disease is characterized by seizures, rapid deterioration of language and motor functions, cognitive decline, rapid 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 CNS enzyme replacement therapy, wherein recombinant TPP1 is administered into the lateral ventricles via a permanently implanted device on a biweekly basis, and palliative care. There are currently no approved treatments to treat ocular manifestations of CLN2 disease.

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, including late-stage and commercial programs, in multiple therapeutic areas. REGENXBIO is committed to a "5x'25" strategy to progress five AAV Therapeutics from our internal pipeline and licensed programs into pivotal-stage or commercial products by 2025.

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," "assume," "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, clinical trials, costs 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, 2022, 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. Except as required by law, 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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