REGENXBIO Joins the Mucopolysaccharidosis Community to Advance Research and Innovation

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The company recognizes International MPS Awareness Day 2018 on May 15 and supports the 5th Annual Million Dollar Bike Ride for rare disease research

ROCKVILLE, Md., May 15, 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 recognizes International Mucopolysaccharidosis (MPS) Awareness Day 2018, which is dedicated to raising awareness of MPS, a group of inherited lysosomal storage disorders that have been estimated to affect one in 25,000 births in the United States.

"This week marks an important time for people living with MPS and their families, who have faced years of challenges and disappointment related to this devastating disease," said Terri Klein, President & CEO of the National MPS Society. "Gene therapy as emerging treatment in MPS offers hope to this community, and we're encouraged by the research conducted by REGENXBIO and other companies to make new treatment a reality for MPS families."

REGENXBIO recently announced it received Fast Track designation from the U.S. Food and Drug Administration (FDA) for RGX-121, the company's novel, one-time direct-to-CNS investigational treatment for MPS II, also known as Hunter syndrome, and is also developing RGX-111, a novel, one-time, direct-to-CNS investigational treatment for MPS I. The company expects to initiate dosing in clinical trials for RGX-111 and RGX-121 in mid-2018.

"At REGENXBIO, we are committed to collaborating with the entire MPS community, including patients, advocates and healthcare professionals, in search of treatments," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are dedicated to creating a brighter future for children with MPS I and II and their families by developing potentially life-changing gene therapies for these two rare and debilitating diseases with significant unmet needs."

On May 20, 2018, REGENXBIO employees and their families will join the National MPS Society in Philadelphia for the 5th Annual Million Dollar Bike Ride, a cycling event sponsored by the Orphan Disease Center of the University of Pennsylvania's School of Medicine. Proceeds from the event will support potentially lifesaving rare disease research.

About Mucopolysaccharidosis Type I (MPS I)

MPS I is a rare autosomal recessive genetic disease caused by deficiency of iduronidase (IDUA), an enzyme required for the breakdown of polysaccharides in the lysosomes. These polysaccharides, called glycosaminoglycans (GAGs), accumulate in tissues of MPS I patients, resulting in characteristic storage lesions and diverse clinical signs and symptoms including in the central nervous system (CNS), which can include excessive accumulation of fluid in the brain, spinal cord compression and cognitive impairment. MPS I is estimated to occur in 1 in 100,000 births. Current disease-modifying therapies for MPS I include bone marrow transplant (BMT) and enzyme replacement therapy with a recombinant form of human IDUA administered intravenously. However, intravenous enzyme therapy does not treat the CNS manifestations of MPS I, and BMT can be associated with clinically significant morbidity and mortality.

About Mucopolysaccharidosis Type II (MPS II)

MPS II is a rare X-linked recessive genetic disease caused by deficiency of I2S, an enzyme required for the breakdown of polysaccharides in the lysosomes. These polysaccharides, called GAGs, accumulate in tissues of MPS II patients, resulting in characteristic storage lesions and diverse clinical signs and symptoms including in the CNS, which can include neural cell death, excessive accumulation of fluid in the brain, spinal cord compression and cognitive impairment. MPS II is estimated to occur in 1 in 100,000 to 1 in 170,000 births. The current disease-modifying therapy for MPS II is enzyme replacement therapy with a recombinant form of human I2S administered intravenously. However, intravenous enzyme therapy does not treat the CNS manifestations of MPS II.

About RGX-111

RGX-111 is being developed as a novel, one-time, direct-to-CNS treatment for MPS I that includes the NAV AAV9 vector encoding a gene for human IDUA. Delivery of the enzyme that is deficient within cells in the CNS could provide a permanent source of secreted IDUA beyond the blood-brain barrier, allowing for long-term cross-correction of cells throughout the CNS. This strategy could also provide rapid IDUA delivery to the brain, potentially preventing the progression of cognitive deficits that otherwise occurs in MPS I patients.

About RGX-121

RGX-121 is being developed as a novel, one-time, direct-to-CNS treatment for MPS II that includes the NAV AAV9 vector encoding for human I2S. Delivery of the enzyme that is deficient within cells in the CNS could provide a permanent source of secreted I2S beyond the blood-brain barrier, allowing for long-term cross-correction of cells throughout the CNS. This strategy could also provide rapid I2S delivery to the brain, potentially preventing the progression of cognitive deficits that otherwise occurs in MPS II patients.

Treatment with RGX-121 has been shown to restore I2S enzyme activity in animal models of MPS II to levels equivalent to or greater than those in non-affected animals. The extent of CNS correction in animal studies suggests that RGX-121 has the potential to be an important and suitable therapeutic option for MPS II patients.

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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