REGENXBIO Receives FDA Regenerative Medicine Advanced Therapy (RMAT) Designation for RGX-121 Gene Therapy for Hunter Syndrome

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- *RMAT recognizes that the preliminary clinical evidence from RGX-121, a potential one-time AAV Therapeutic, indicates the potential to address unmet medical needs for MPS II*
- *RMAT designation is for gene therapies intended to treat or cure serious condition in order to expedite the drug development and review processes*
- *CAMPSIITE™ trial is enrolling MPS II patients as part of a pivotal program that incorporates material from the NAVXpress™ platform process manufactured at the REGENXBIO Manufacturing Innovation Center and continues to support plan to file Biologics License Application in 2024 using the accelerated approval pathway*

ROCKVILLE, Md., May 23, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation for RGX-121, an investigational one-time AAV Therapeutic for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter syndrome. RMAT designation is designed to expedite the drug development and review processes for promising new treatments, including gene therapies, and recognizes that the preliminary clinical evidence from RGX-121 indicates its potential to address unmet medical needs for MPS II. RGX-121 is currently being studied in the CAMPSIITE™ trial that is enrolling MPS II patients as part of a pivotal program that incorporates material from the NAVXpress™ platform process manufactured at the REGENXBIO Manufacturing Innovation Center and continues to support plans to file Biologics License Application (BLA) in 2024 using the accelerated approval pathway.

"We are pleased that the FDA has granted RMAT designation for RGX-121 for its potential to address the neurological manifestations of MPS II and prevent or stabilize cognitive decline, which remains a significant unmet need for this community," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "This is an important regulatory milestone, and we remain on track to complete enrollment of CAMPSIITE in the first half of 2023 to support a BLA filing in 2024 using the accelerated approval pathway."

Established under the 21st Century Cures Act, a drug is eligible for RMAT designation if it is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug or therapy has the potential to address unmet medical needs for such disease or condition.

RMAT designation includes the benefits of early FDA interactions to discuss surrogate or intermediate endpoints, potential ways to support accelerated approval and satisfy post-approval requirements, potential priority review of the BLA and other opportunities to expedite development and review. In addition, sponsors of products that have been granted RMAT designation and which received accelerated approval may be able to fulfill the post-approval requirements from clinical evidence obtained from sources other than the traditional confirmatory clinical trials.

"Severe Hunter syndrome is a progressively debilitating disorder that affects a child's physical and mental development and leads to a shortened lifespan, and I applaud the FDA for granting RMAT designation to RGX-121," said Joseph Muenzer, M.D., Ph.D., Director, Muenzer MPS Research and Treatment Center, Bryson Distinguished Professor in Pediatrics Genetics, University of North Carolina at Chapel Hill. "I am encouraged by the FDA's recognition of the potential of a gene therapy like RGX-121 to improve the quality of life for boys with severe Hunter syndrome and the urgent need for new treatment options."

"We know that families are waiting for new treatment options for this serious disease and that every day matters," said Terri Klein, President and Chief Executive Officer, National MPS Society. "The expedited development of new treatment options like RGX-121 are critical to families facing this devastating diagnosis."

RGX-121 Program Highlights
The Phase I/II/III CAMPSIITE™ trial of RGX-121 for MPS II patients aged 4 months up to 5 years is ongoing and in February 2023, REGENXBIO announced additional interim data from the Phase I/II part of the trial, demonstrating that RGX-121 continued to be well-tolerated across 15 patients. Patients receiving the pivotal program dose level continued to demonstrate the largest reductions in CSF GAGs, including Heparin Sulfate (HS) and HS D2S6, which approached normal levels at 48 weeks. CSF GAGs have the potential to be considered a surrogate biomarker that is reasonably likely to predict clinical benefit in MPS II disease under the accelerated approval pathway, as buildup of GAGs in the CSF of MPS II patients correlates with clinical manifestations, including neurodevelopmental deficits. In addition, improvements in neurodevelopmental and daily activity skill acquisition were observed up to three years after RGX-121 administration.

A Phase I/II trial of RGX-121 for the treatment of pediatric patients with MPS II over the age of five years old is also ongoing.

About RGX-121
RGX-121 is designed to use the NAV® AAV9 vector to deliver the human iduronate-2-sulfatase gene (IDS) which encodes the iduronate-2-sulfatase (I2S) enzyme to the central nervous system (CNS). Delivery of the IDS gene 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. RGX-121 has received Orphan Drug Product, Rare Pediatric Disease, Fast Track and Regenerative Medicine Advanced Therapy designations from the U.S. Food and Drug Administration.

About Mucopolysaccharidosis Type II (MPS II)
MPS II, or Hunter Syndrome, is a rare, X-linked recessive disease caused by a deficiency in the lysosomal enzyme iduronate-2-sulfatase (I2S) leading to an accumulation of glycosaminoglycans (GAGs), including heparan sulfate (HS) in tissues which ultimately results in cell, tissue, and organ dysfunction, including in the central nervous system (CNS). MPS II is estimated to occur in 1 in 100,000 to 170,000 births. In severe forms of the disease, early developmental milestones may be met, but developmental delay is readily apparent by 18 to 24 months. Specific treatment to address the neurological manifestations of MPS II remains a significant unmet medical need. Key biomarkers of I2S enzymatic activity in MPS II patients include its substrate heparan sulfate (HS) D2S6, which has been shown to correlate with neurocognitive manifestations of the disorder.

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 and AAV9. 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.

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