REGENXBIO Announces NAV® Technology Platform Will Support Bespoke Gene Therapy Consortium's First Rare Disease Clinical Portfolio

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- *Research in MPS IVA led by REGENXBIO scientists moving into clinic*
- *Donation of NAV® Technology licenses will support development of gene therapies for rare diseases*

ROCKVILLE, Md., May 16, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced that preclinical research in Mucopolysaccharidosis type IVA (MPS IVA), also known as Morquio syndrome, was selected for inclusion in the Foundation for the National Institutes of Health Accelerating Medicines Partnership® Bespoke Gene Therapy Consortium (AMP® BGTC), clinical trial portfolio. The Consortium brings together partners such as NIH and FDA, as well as partners from private and non-profit sectors.

Sponsored by Nemours Children's Hospital, MPS IVA is one of eight programs selected as part of AMP® BGTC's first clinical portfolio to help accelerate the development of bespoke gene therapies, with the goal of streamlining the regulatory approval process. MPS IVA is a metabolic condition that primarily affects the skeleton, and is estimated to impact 1 in 200,000 to 300,000 individuals.

"I am proud of our scientists who led this important clinical research work for REGENXBIO. Our mission to deliver the curative potential of gene therapy reflects not only patients that can be impacted by our clinical pipeline, but also all patients who should have the opportunity to be positively impacted by gene therapy," said Kenneth T. Mills, President and CEO REGENXBIO. "As a partner of the BGTC, REGENXBIO is pleased to see this program advancing important science into the clinic."

Criteria for selection included the adequacy of the gene for insertion into an adeno-associated virus (AAV) vector, sufficient proof of concept and natural history data, the existence of an established disease model, a lack of available treatment and an overall readiness for entering into a clinical trial. REGENXBIO will donate licenses to NAV® AAV8 and NAV® AAV9 from its NAV® Technology Platform to enable AMP® BGTC's development of these programs aimed at addressing ultra rare diseases. Use of the NAV® Technology Platform will help address the goal of making gene therapy more accessible by creating a platform approach with standardized processes to deliver novel therapies for many different genetic disorders. These programs together further validate the versatility of NAV® vectors and will provide additional data that collectively drive the advancement of the AAV gene therapy field.

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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