REGENXBIO Announces Presentations at the American Society of Gene & Cell Therapy 28th Annual Meeting

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ROCKVILLE, Md., May 8, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced presentations at the American Society of Gene & Cell Therapy (ASGCT) 28th Annual Meeting, taking place in New Orleans, May 13 through 17, 2025. The presentations highlight REGENXBIO's leading end-to-end capabilities across research and early development, late-stage clinical development, and gene therapy manufacturing.

Presentations include encore data from late-stage clinical trials of RGX-121 (clemidsogene lanparvovec) for the treatment of MPS II and RGX-202 for the treatment of Duchenne muscular dystrophy, as well as preclinical research supporting the novel construct of RGX-202 with the C-Terminal domain, capsid discovery research, and RGX-202 manufacturing process development enabling industry-leading purity levels in Duchenne gene therapy.

Oral Presentations:
Abstract Title: Development of a Commercial Manufacturing Process for RGX-202, a Systemically Delivered AAV for the Treatment of Duchenne Muscular Dystrophy
Presenter: Donald Startt, Executive Director, Process Development, REGENXBIO
Session: AAV Vector Manufacturing: Process Development
Date/Time: May 13, 1:30 p.m. CT

Abstract Title: RGX-121 (clemidsogene lanparvovec): an Investigational AAV Gene Therapy for the Treatment of Neuronopathic Mucopolysaccharidosis Type II
Presenter: Olivier Danos, Ph.D., Executive Vice President, Chief Scientific Officer, REGENXBIO
Session: CNS Gene Delivery for Metabolic Diseases: History and Challenges
Date/Time: May 14, 8:00 a.m. CT

Abstract Title: RGX-202, an Investigational Gene Therapy for the Treatment of Duchenne Muscular Dystrophy: Interim Clinical Data
Presenter: Olivier Danos, Ph.D., Executive Vice President, Chief Scientific Officer, REGENXBIO
Session: Gene Therapy Trials: In-Vivo Gene Therapy Modification
Date/Time: May 16, 3:45 p.m. CT

Poster Presentations:
Abstract Title: Impact of Sample Collection Container Material, Hold Time, and Storage Temperature on Adeno-Associated Virus Endotoxin Testing
Presenter: Amanda Zhang, Associate Director Vectore Core, REGENXBIO
Session: Tuesday Poster Session
Date/Time: May 13, 6:00 p.m. CT

Abstract Title: AAV-Expressed Microdystrophin Containing Extended C-Terminus Improves Muscle Function and Protects Against Injury in a Mouse Model of Duchenne Muscular Dystrophy
Presenter: Benjamin Heithoff, Ph.D., Scientist II, REGENXBIO
Session: Wednesday Poster Session
Date/Time: May 14, 5:30 p.m. CT

Abstract Title: Development of In Vitro Methods for the Analysis of TLR9 Stimulation by AAV Vector Genomes
Presenter: Justin Glenn, Ph.D., Senior Scientist, REGENXBIO
Session: Wednesday Poster Session
Date/Time: May 14, 5:30 p.m. CT

Abstract Title: In Vitro and In Vivo Characterization of Oversized AAV Vectors with High Genome Integrity that Encode Microdystrophins with Extended C-Terminal Sequences
Presenter: Randolph Qian, Ph.D., Senior Scientist, REGENXBIO
Session: Wednesday Poster Session
Date/Time: May 14, 5:30 p.m. CT

Abstract Title: Blood-Brain Barrier Crossing AAV Vectors Targeting the Transferrin Receptor Engineered Using Two Different Approaches
Presenter: Elad Firnberg, Ph.D., Principal Scientist, REGENXBIO
Session: Thursday Poster Session
Date/Time: May 15, 5:30 p.m. CT

ABOUT REGENXBIO Inc.
REGENXBIO is a biotechnology company on a mission to improve lives through the curative potential of gene therapy. Since its founding in 2009, REGENXBIO has pioneered the field of AAV gene therapy. REGENXBIO is advancing a late-stage pipeline of one-time treatments for rare and retinal diseases, including RGX-202 for the treatment of Duchenne; clemidsogene lanparvovec (RGX-121) for the treatment of MPS II and RGX-111 for the treatment of MPS I, both in partnership with Nippon Shinyaku; and surabgene lomparvovec (ABBV-RGX-314) for the treatment of wet AMD and diabetic retinopathy, in collaboration with AbbVie. Thousands of patients have been treated with REGENXBIO's AAV platform, including those receiving Novartis' ZOLGENSMA®. REGENXBIO's investigational gene therapies have the potential to change the way healthcare is delivered for millions of people. For more information, please visit www.REGENXBIO.com.

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 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 timing or likelihood of payments from AbbVie or Nippon Shinyaku, the monetization of any priority review voucher, 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, 2024, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the 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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