REGENXBIO Announces Presentations at the American Society of Gene & Cell Therapy (ASGCT) 25th Annual Meeting

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ROCKVILLE, Md., May 3, 2022 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced presentations at the American Society of Gene & Cell Therapy (ASGCT) 25th Annual Meeting, taking place virtually and in Washington, D.C. from May 16 through 19, 2022. The presentations highlight the Company's end-to-end capabilities across research and early development, clinical development and manufacturing.

The presentations will be presented as follows:

Presenter: Nina Hunter, Ph.D., VP, Regulatory and Science Policy at REGENXBIO, Pathway Development Consortium
Session: Accelerated Approval for Gene Therapies
Date/Time: Monday, May 16, 2022, 8:00 a.m. ET

Abstract Title: RGX-121 Gene Therapy for the Treatment of Severe Mucopolysaccharidosis Type II (MPS II): Interim Analysis of Data from the First in Human Study (abstract #52)
Presenter: Roberto Giugliani, M.D., Ph.D., Professor, Department of Genetics, UFRGS, Medical Genetics Service, HCPA, Porto Alegre, Brazil
Session: Gene and Cell Therapy Trials in Progress
Date/Time: Monday, May 16, 2022, 1:30 p.m. ET

Abstract Title: RGX-111 Gene Therapy for the Treatment of Severe Mucopolysaccharidosis Type I (MPS I): Interim Analysis of Data from the First in Human Study (abstract #802)
Presenter: Raymond Wang, M.D., Division of Metabolic Disorders, CHOC Children's Hospital, Department of Pediatrics, University of California, Irvine, CA
Session: Gene and Cell Therapy Trials in Progress
Date/Time: Tuesday, May 17, 2022, 5:30 p.m. ET

Abstract Title: VP1 Unique and VP1/2 Shared Region Serotype Swap Hybrids Enhance Desirable AAV Properties
Presenter: Samantha Yost, Ph.D., Senior Scientist, Gene Transfer Technologies at REGENXBIO & Randy Qian, Ph.D., Scientist I, Gene Transfer Technologies at REGENXBIO (abstract #500)
Session: AAV Vectors - Virology and Vectorology II
Date/Time: Tuesday, May 17, 2022, 5:30 p.m. ET

Abstract Title: A Novel Peptide Insertion into VR-IV or VR-VIII of AAV9 Improves Transduction Strength and Penetration Depth Upon Intravitreal Injection (abstract: #521)
Presenter: Wei-Hua Lee, Ph.D., Scientist II, Target Discovery at REGENXBIO & Samantha Yost, Ph.D., Senior Scientist, Gene Transfer Technologies at REGENXBIO (co-first authors)
Session: AAV Vectors - Preclinical and Proof-of-concept Studies II
Date/Time: Tuesday, May 17, 2022, 5:30 p.m. ET

Abstract Title: Adeno-Associated Virus Adsorption on Different Surfaces Relevant to Production of Pre-Clinical and Clinical Material (abstract #765)
Presenter: Amanda Zhang, Scientific Project Manager, Vector Core at REGENXBIO
Session: Vector Product Engineering, Development or Manufacturing II
Date/Time: Tuesday, May 17, 2022, 5:30 p.m. ET

Abstract Title: Gene Expression from AAV Vectors in the Liver-A Comparative Study Across Species, Promoters and AAV Serotypes (abstract #824)
Presenter: Subha Karumuthil-Melethil, Ph.D., Principal Scientist, Target Discovery at REGENXBIO
Session: AAV Developments in Liver, T-Cells and Toxicity
Date/Time: Wednesday May 18th, 2022, 5:00 p.m. ET

Abstract Title: Intraparenchymal Administration to the Striatum of a Barcoded AAV Library for the Characterization of Capsid Tropisms in Rodents and Non-human Primates (abstract #892)
Presenter: Jared Smith, Ph.D., Principal Scientist, Target Discovery at REGENXBIO
Session: AAV Vectors – Virology and Vectorology III
Date/Time: Wednesday, May 18, 2022, 5:30 p.m. ET

Abstract Title: A Longitudinal, Comparative Analysis of Transgene Expression Durability via Different Promoters in the Striatum of Mice Delivered by Intraparenchymal Injection of rAAV9 (abstract #894)
Presenter: Brad Hollidge, Ph.D., Scientist II, Target Discovery at REGENXBIO
Session: AAV Vectors - Virology and Vectorology III
Date/Time: Wednesday, May 18, 2022, 5:30 p.m. ET

Abstract Title: Stability of Microdystrophin Proteins Measured by Pulse-Chase Assays in Tissue Culture (abstract # 1065)
Presenter: Kirk Elliott, Scientist I, Gene Transfer Technology at REGENXBIO
Session: Musculo-skeletal Diseases
Date/Time: Wednesday, May 18, 2022, 5:30 p.m. ET

Abstract Title: AAV Vectors Consistently Display Higher Transcriptional Activity in MDX Mouse Muscle Versus Normal Mouse Skeletal Muscle (abstract #1063)

Presenter: Randy Qian, Ph.D., Scientist I, Gene Transfer Technologies at REGENXBIO
Session: Musculo-Skeletal Diseases
Date/Time: Wednesday, May 18, 2022, 5:30 p.m. ET

Abstract Title: Recruitment of nNOS and Other Dystrophin-Associated Protein Complex Members by Different Microdystrophin Constructs (abstract #1068)
Presenter: Steven Foltz, Ph.D., Scientist II, Target Discovery at REGENXBIO
Session: Musculo-Skeletal Diseases
Date/Time: Wednesday, May 18, 2022, 5:30 p.m. ET

Abstract Title: Evaluating the Impact of Transgene-Specific CpG Removal on AAV9-Mediated Gene Transfer and Immune Responses in the Balb/C Mouse Strain Provides Novel Insights of CpG Depletion (abstract #1671)
Presenter: Justin Glenn, Ph.D., Senior Scientist, Target Discovery at REGENXBIO
Session: Immunological Aspects of Gene Therapy and Vaccines II
Date/Time: Wednesday, May 18, 2022, 5:30 p.m. ET

Abstract Title: A Novel AAV8-Based Gene Therapy for Duchenne Muscular Dystrophy: Preclinical Studies in the Mdx Mouse (abstract #1067)
Presenter: SunJung Kim, Ph.D., DABT, Senior Scientist, Pharmacology and Toxicology at REGENXBIO
Session: Musculo-skeletal Diseases
Date/Time: Wednesday, May 18, 2022, 5:30 p.m. ET

Abstract Title: Identification and Characterization of an AAV9-Based Engineered Capsid Variant Capable of Mediating Enhanced Transcription in the Central Nervous System of Non-Human Primates and Rodents (abstract #1200)
Presenter: April Giles, Ph.D., Scientist II, Gene Transfer Technologies at REGENXBIO & Samantha Yost, Ph.D., Senior Scientist, Gene Transfer Technologies at REGENXBIO (co-first authors)
Session: Novel AAV Capsids for the Brain, Kidney and Eye
Date/Time: Thursday, May 19, 2022, 11:45 a.m. ET

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.

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