REGENXBIO Announces Presentations at the American Society of Gene & Cell Therapy 26th Annual Meeting

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ROCKVILLE, Md., May 2, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced presentations at the American Society of Gene & Cell Therapy (ASGCT) 26th Annual Meeting, taking place in Los Angeles, California from May 16 through 20, 2023. The presentations highlight the Company's end-to-end capabilities across clinical development and research and early development.

The presentations will be presented as follows:

Oral Presentations:

Abstract Title: AUF1 Gene Therapy for Duchenne Muscular Dystrophy Increases Durable Endogenous Utrophin Expression, Muscle Regeneration and Muscle Function Performance in Pre-clinical Animal Studies (abstract #135)
Presenter: Dounia Abbadi, Postdoctoral Fellow, NYU Langone Medical Center
Session: Gene Tx Approaches for Muscle & Skeletal Diseases
Date/Time: Thursday, May 18, 2023, 2:38-2:55 p.m. PST

Abstract Title: Mechanistic Evaluation of Liver-specific Transgene Repression from AAV Vectors in Non-Human Primates and Minipigs (abstract #249)
Presenter: Zhuo Wang, Ph.D., Scientist II, Research & Early Development at REGENXBIO
Session: AAV Vector Genome Biology & Engineering II
Date/Time: Friday, May 19 3:45-4:00 p.m. PST

Poster Presentations:

Abstract Title: RGX-121 gene therapy for the treatment of neuronopathic mucopolysaccharidosis type II (MPS II): Interim analysis of data from the first in human study (abstract #807)
Presenter: Paul Harmatz, M.D., Professor in Residence at University of California, San Francisco
Session: Wednesday Poster Session
Date/Time: Wednesday, May 17, 2023 12:00-2:00 p.m. PST & 5:30-7:00 p.m. PST

Abstract Title: In Silico Prediction and In Vivo Testing of Promoters Targeting GABAergic Inhibitory Neurons (abstract #385)
Presenter: Robert Duba-Kiss, Ph.D. Candidate, Pharmacology/Neuroscience at University of Toronto University of Toronto
Session: Wednesday Poster Session
Date/Time: Wednesday, May 17, 2023 12:00-2:00 p.m. PST & 5:30-7:00 p.m. PST

Abstract Title: Development of AAV-Expressed C5 Inhibitor to Locally Suppress Complement Pathway Activation in the Eye as a Potential Treatment for Dry Age-related Macular Degeneration (abstract #364)
Presenter: Wei-Hua Lee, Ph.D., Senior Scientist, Research & Early Development at REGENXBIO
Session: Wednesday Poster Session
Date/Time: Wednesday, May 17, 2023 12:00-2:00 p.m. PST & 5:30-7:00 p.m. PST

Abstract Title: Addition of a Protein Domain from the Dystrophin C-Terminus Improves Functional and Biochemical Properties of AAV-Encoded Microdystrophin (abstract #668)
Presenter: Steven Foltz, Ph.D., Senior Scientist, Research & Early Development at REGENXBIO
Session: Wednesday Poster Session
Date/Time: Wednesday, May 17, 2023 12:00-2:00 p.m. PST & 5:30-7:00 p.m. PST

Abstract Title: Optimization of AAV-DARPin Fusions to Redirect Capsid Tropism (abstract #447)
Presenter: Elad Firnberg, Ph.D., Senior Scientist, Research & Early Development at REGENXBIO
Session: Wednesday Poster Session
Date/Time: Wednesday, May 17, 2023 12:00-2:00 p.m. PST & 5:30-7:00 p.m. PST

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 #932)
Presenter: Laura Pisani-Betancourt, MD, MBA, FACMG, Senior Medical Director, Clinical Development Lead at REGENXBIO
Session: Thursday Poster Session
Date/Time: Thursday May 18, 2023 12:00-2:00 p.m. PST & 5:30-7:00 p.m. PST

Abstract Title: Local Delivery of Vectorized Therapeutics Targeting a Pro-inflammatory Cytokine Effectively Reduces Disease Severity in a Mouse Model of Non-Infectious Uveitis (abstract #860)
Presenter: Jessica Gumerson, Ph.D., Scientist II, Research & Early Development at REGENXBIO
Session: Thursday Poster Session
Date/Time: Thursday May 18, 2023 12:00-2:00 p.m. PST & 5:30-7:00 p.m. PST

Abstract Title: High Resolution Biodistribution Analysis Following Suprachoroidal Administration of a Pool of AAV3B, AAV8, and AAV9 Vectors to Non-Human Primates Reveals Spatial and Cell-Based Tropism Differences (abstract #876)
Presenter: April R. Giles, Ph.D., Senior Scientist, Research & Early Development at REGENXBIO

Session: Thursday Poster Session
Date/Time: Thursday, May 18, 2023 12:00-2:00 p.m. PST & 5:30-7:00 p.m. PST

Abstract Title: Utility of Cryofluorescence Tomography (CFT) as an Unbiased Method to Describe AAV Biodistribution (abstract #1278)
Presenter: Samantha A. Yost, Ph.D., Senior Scientist, Research & Early Development at REGENXBIO
Session: Friday Poster Session
Date/Time: Friday, May 19, 2023 12:00-2:00 p.m. PST & 5:30-7:00 p.m. PST

Abstract Title: Development of a Self-Complementary AAV.U7snRNA Vector for Efficient Dystrophin Exon 53 Skipping (abstract #1565)
Presenter: Randolph Qian, Ph.D., Scientist II, Research & Early Development at REGENXBIO
Session: Friday Poster Session
Date/Time: Friday, May 19, 2023 12:00-2:00 p.m. PST & 5:30-7:00 p.m. PST

Abstract Title: Kinetic Analyses Reveal Very Early CpG-specific Antagonism to AAV-Mediated Transgene Expression: Implications for TLR9 Stimulatory Effects Prior to Effector T Cell Activation Following Gene Transfer (abstract #1487)
Presenter: Justin D. Glenn, Ph.D., Senior Scientist, Research & Early Development at REGENXBIO
Session: Friday Poster Session
Date/Time: Friday, May 19, 2023 12:00-2:00 p.m. PST & 5:30-7:00 p.m. PST

NAV® Technology Licensees:

Abstract Title: AAV Vector DNA Carrying Two Concatenated miRNA Stem-Loops is Highly Homogenous and Stable in GLP-Grade AAV Product
Presenter: Chenxia HE, Ph.D., Director of Gene Therapy at uniQure, in collaboration with REGENXBIO
Session: AAV Vectors – Product Development Manufacturing: Analytics & Stability Studies
Date/Time: Thursday, May 18, 2023, 1:45-2:00 p.m. PST

Abstract Title: Preclinical Proof-of-Concept of AMT-260, a Novel AAV9-Dual microRNA-Based Vector Targeting GRIK2 for the Treatment of Temporal Lobe Epilepsy (abstract #1159)
Presenter: Nick Pearson, Head of Toxicology & Translational Safety at uniQure, in collaboration with REGENXBIO
Session: Thursday Poster Session
Date/Time: Thursday, May 18, 2023 12:00-2:00 p.m. PST & 5:30-7:00 p.m. PST

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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