



REGENXBIO Announces Presentations at the American Society of Gene and Cell Therapy's 24th Annual Meeting

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ROCKVILLE, Md., April 27, 2021 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced that four oral and nine poster presentations will be presented at the American Society of Gene and Cell Therapy's 24th Annual Meeting taking place from May 11 to 14, 2021, in virtual format. Presentations and posters will be available at <https://www.asgct.org/>.

Oral presentations include:

Abstract Title: Effects of Sexual Dimorphism and Genetic Background on AAV Tissue Transduction in Mice Following Intravenous Administration of a Diverse Capsid Pool (abstract #94)

Presenter: Elad Firnberg, Ph.D., Scientist II, Gene Transfer Technologies, REGENXBIO

Session Title: Novel Factors in AAV Transduction and AAV Genomes

Date/Time: Wednesday, May 12, 2021 from 6:00 to 6:15 p.m. ET

Abstract Title: Intravenous Administration of a Barcoded and Pooled AAV Library for the Comprehensive Characterization and Comparison of Capsid Tropisms (abstract #189)

Presenter: April Giles, Ph.D., Scientist II, Gene Transfer Technologies, REGENXBIO

Session Title: Novel AAV Biology and Platform Technologies

Date/Time: Thursday, May 13, 2021 from 6:00 to 6:15 p.m. ET

Abstract Title: High-Throughput Screening of AAV Productivity to Enable Rapid Capsid Characterization (abstract #192)

Presenter: Jenny Egley, Scientist, Gene Transfer Technologies, REGENXBIO

Session Title: Novel AAV Biology and Platform Technologies

Date/Time: Thursday, May 13, 2021 from 6:45 to 7:00 p.m. ET

Abstract Title: RGX-121 Gene Therapy for the Treatment of Severe Mucopolysaccharidosis Type II: Interim Analysis of the First in Human Study (abstract #215)

Presenter: Roberto Giugliani, M.D., Ph.D., Professor, Department of Genetics, UFRGS, Medical Genetics Service, HCPA, *Porto Alegre, Brazil*

Session Title: Gene Therapy for Lysosomal Storage Disorders

Date/Time: Friday, May 14, 2021 from 12:15 to 12:30 p.m. ET

Poster presentations include:

Abstract Title: Identification of Transcriptional Start Sites in a Tandem Promoter by 5'-RACE and NGS (abstract #317)

Presenter: Kirk Elliot, Senior Associate Scientist, REGENXBIO

Session Title: AAV Vectors - Virology and Vectorology

Date/Time: Tuesday, May 11, 2021 from 8:00 to 10:00 a.m. ET

Abstract Title: Comparative Cardiac, Neurologic, and Skeletal Effectiveness of Intravenous and Intrathecal AAV9-IDUA Delivered Individually or Combined in a Murine Model of Mucopolysaccharidosis Type I (abstract #490)

Presenter: Lalitha Belur, Ph.D., Assistant Professor Genetics, Cell Bio at University of Minnesota

Session Title: Metabolic, Storage, Endocrine, Liver and Gastrointestinal Diseases

Date/Time: Tuesday, May 11, 2021 from 8:00 to 10:00 a.m. ET

Abstract Title: Comprehensive Evaluation of an AAV Library in the Mice and Nonhuman Primate Ocular Tissues Following Intravitreal Delivery (abstract #568)

Presenter: Wei-Hua Lee, Ph.D., Scientist II, Research and Early Development, Target Discovery, REGENXBIO

Session Title: Neurologic Diseases

Date/Time: Tuesday, May 11, 2021 from 8:00 to 10:00 a.m. ET

Abstract Title: RGX-202, an AAV8 Vector Encoding an Extended Microdystrophin Efficiently Restores Dystrophin Associated Protein Complex and Corrects Satellite Cells in a Dystrophic Mouse Model (abstract #597)

Presenter: Chunping Qiao, Ph.D., Principal Scientist, REGENXBIO

Session Title: Musculo-Skeletal Diseases

Date/Time: Tuesday, May 11, 2021 from 8:00 to 10:00 a.m. ET

Abstract Title: A Novel AAV8 Vector for Microdystrophin Gene Therapy of Duchenne Muscular Dystrophy: Preclinical Studies in the MDX Mouse (abstract #614)

Presenter: SunJung Kim, Ph.D., Senior Scientist, Pharmacology and Toxicology, Preclinical Development, REGENXBIO

Session Title: Musculo-Skeletal Diseases

Date/Time: Tuesday, May 11, 2021 from 8:00 to 10:00 a.m. ET

Abstract Title: A Case Study on Viral Clearance for a Downstream AAV Process using a Model Virus Panel and Non-Infectious MVM Surrogate (abstract #877)

Presenter: Shaojie Weng, Scientist II

Session Title: Pharmacology/Toxicology Studies or Assay Development

Date/Time: Tuesday, May 11, 2021 from 8:00 to 10:00 a.m. ET

Abstract Title: Assessment of Inflammatory and Neurodegenerative Biomarkers in NHPs with DRG Toxicity (abstract #889)

Presenter: Ryan Reddinger, Ph.D., Scientist I, Preclinical Development, REGENXBIO

Session Title: Pharmacology/Toxicology Studies or Assay Development

Date/Time: Tuesday, May 11, 2021 from 8:00 to 10:00 a.m. ET

Abstract Title: Quantification of Double-Stranded RNAs Produced by AAV Vectors: A Methodological Reassessment (abstract #891)

Presenter: Chunping Qiao, Ph.D., Principal Scientist, REGENXBIO

Session Title: Pharmacology/Toxicology Studies or Assay Development

Date/Time: Tuesday, May 11, 2021 from 8:00 to 10:00 a.m. ET

Abstract Title: Identification of a Kidney Specific AAV Following Insertion of Tissue Homing Peptides in Variable Region IV (abstract #292)

Presenter: Samantha A. Yost, Ph.D., Scientist II, Gene Transfer Technologies, REGENXBIO

Session Title: AAV Vectors - Virology and Vectorology

Date/Time: Tuesday, May 11, 2021 from 8:00 to 10:00 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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