REGENXBIO Announces Presentations at the American Society of Gene and Cell Therapy's 23rd Annual Meeting

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ROCKVILLE, Md., April 28, 2020 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV® Technology Platform, today announced that three oral presentations, and several poster presentations, will be presented at the American Society of Gene and Cell Therapy's 23rd Annual Meeting taking place May 12-15, 2020 in virtual format. Presentations and posters will be available at www.asgct.org.

The oral presentations include:

Abstract Title: Quantitative PET/CT based pharmacokinetic study of AAV9 administered to the cerebrospinal fluid of non-human primates (abstract #135)
Session Title: AAV Vectors Preclinical and Proof-of-Concept Studies in Optimizing the Toolbox
Presenter: Mikhail Papisov, Ph.D., Massachusetts General Hospital, Shriners Hospitals for Children, Harvard Medical School
Date/Time: Tuesday, May 12, 2020 from 4:00 - 4:15 p.m. ET

Abstract Title: RGX-314 Ocular Gene Therapy: Overview of Phase I/IIa Ongoing Trial for Neovascular Age-related Macular Degeneration (nAMD) and Future Directions (abstract #1305)
Session Title: AAV Vectors - Clinical Studies
Presenter: Olivier Danos, Ph.D., Senior Vice President and Chief Scientific Officer, REGENXBIO
Date/Time: Friday, May 15, 2020 from 10:15-10:30 a.m. ET

Abstract Title: AAV gene therapy in mucopolysaccharidosis IVA murine models (abstract #1351)
Session Title: Gene Therapy for Inborn Errors of Metabolism: New Approaches
Presenter: Shunji Tomatsu, M.D., Ph.D., Nemours/Alfred I. duPont Hospital for Children
Date/Time: Friday, May 15, 2020 from 11:15-11:30 a.m. ET

The posters include:

Abstract Title: AAV-mediated antibody delivery for hereditary angioedema (abstract #190)
Presenter: Joseph T. Bruder, Ph.D., Senior Director, Target Discovery, REGENXBIO
Session Title: AAV Vectors - Preclinical and Proof-of-Concept Studies
Date/Time: Tuesday, May 12, 2020 from 5:30-6:30 p.m. ET

Abstract Title: Interim data from the first in human RGX-121 gene therapy trial for the treatment of severe MPS II (Hunter syndrome) (abstract #614)
Presenter: Marie-Laure Nevoret, M.D., Senior Clinical Development Lead, REGENXBIO
Session Title: AAV Vectors - Clinical Studies
Date/Time: Wednesday, May 13, 2020 from 5:30-6:30 p.m. ET

Abstract Title: Development of a vectorized antibody platform for liver and skeletal muscle gene transfer (abstract #556)
Presenter: Devin S. McDougald, Ph.D., Scientist II, Gene Transfer Technologies, REGENXBIO
Session Title: AAV Vectors – Virology & Vectorology
Date/Time: Wednesday, May 13, 2020 from 5:30-6:30 p.m. ET

Abstract Title: Use of Prophylactic Steroids to Mitigate Potential T-Cell Response in AAV8-Mediated *hLDLR* Gene Transfer in Subjects with Homozygous Familial Hypercholesterolemia (abstract #612)
Presenter: Marina Cuchel, M.D., Ph.D., University of Pennsylvania
Session Title: AAV Vectors - Clinical Studies
Date/Time: Wednesday, May 13, 2020 from 5:30-6:30 p.m. ET

Abstract Title: Process development and scaleup comparisons for transient Production of AAV (abstract #1266)
Presenter: Robert Stadelman, Senior Scientist, REGENXBIO
Session Title: Vector and Cell Engineering, Production or Manufacturing
Date/Time: Thursday, May 14, 2020 from 5:30-6:30 p.m. ET

Abstract Title: Structural and biochemical characterization of potentially under-utilized gene therapy vector AAV7 (abstract #1007)
Presenter: Samantha A. Yost, Ph.D., Scientist II, Gene Transfer Technologies, REGENXBIO
Session Title: AAV Vectors - Virology and Vectorology
Date/Time: Thursday, May 14, 2020 from 5:30-6:30 p.m. ET

Abstract Title: Development of a Sensitive and Robust Cell-Based Assay for Measuring Potency of the NAV AAV8 Vector-Derived RGX-314 Gene Therapy Product for the Treatment of Wet Age-Related Macular Degeneration (abstract #1005)
Presenter: Raza Zaidi, Senior Associate Scientist, REGENXBIO
Session Title: AAV Vectors - Virology and Vectorology
Date/Time: Thursday, May 14, 2020 from 5:30-6:30 p.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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