



REGENXBIO Announces Presentations at the Society for the Study of Inborn Errors of Metabolism Annual Symposium

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ROCKVILLE, Md., Aug. 23, 2022 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced presentations at the Society for the Study of Inborn Errors of Metabolism Annual Symposium, taking place from August 30 through September 2, 2022, in Freiburg, Germany. The presentations will highlight new data from the Phase I/II/III CAMPSITE™ trial of RGX-121, an investigational one-time AAV Therapeutic for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter Syndrome, and an encore data presentation for RGX-111, an investigational one-time AAV Therapeutic for the treatment of severe Mucopolysaccharidosis Type I (MPS I), or Hurler syndrome.

Presentations include:

Title: RGX-121 gene therapy for the treatment of severe mucopolysaccharidosis type II (MPS II): Interim analysis of data from a Phase 1/2 study
Presenter: Roberto Giugliani, M.D., Ph.D., Professor, Department of Genetics, UFRGS, Medical Genetics Service, HCPA, Porto Alegre, Brazil
Date/Time: Wednesday, August 31, 2022, 10:00 – 10:15 a.m. CEDT

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
Presenter: Raymond Wang, M.D., Division of Metabolic Disorders, CHOC Children's Hospital, Department of Pediatrics, University of California, Irvine, CA
Date/Time: Thursday, September 1, 2022, 11:30 – 11:45 a.m. CEDT

Title: Natural History of Neurodevelopment in Neuronopathic Mucopolysaccharidosis Type II (MPS II): Mullen Scales of Early Learning (MSEL) Cognitive, Motor, and Language Developmental Trajectories

Presenter: Maria Escolar, M.D., M.S., Professor of Pediatrics, Director, Program for the Study of Neurodevelopment in Rare Disorders, UPMC Children's Hospital of Pittsburgh
Date/Time: Wednesday, August 31, 2022, 6:45 – 8:15 p.m. CEDT

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, 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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