



## REGENXBIO Announces Presentations at the Society for the Study of Inborn Errors of Metabolism Annual Symposium 2023

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ROCKVILLE, Md., Aug. 23, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced presentations at the Society for the Study of Inborn Errors of Metabolism Annual Symposium 2023 in Jerusalem, Israel (August 29 – September 1). Investigators will deliver encore presentations of interim clinical trial data from REGENXBIO's pipeline of one-time investigational AAV Therapeutics for progressive, neurodegenerative lysosomal storage disorders, as well as initial interim data from the single-patient, investigator-initiated study of RGX-181 for the treatment of CLN2 disease.

### Presentations:

**Title:** RGX-121: An investigational gene therapy for the treatment of neuronopathic mucopolysaccharidosis type II (MPS II), an interim analysis of data from the first-in-human study

**Presenter:** Paul Harmatz, M.D., UCSF Benioff Children's Hospital, USA

**Date/Time:** Wednesday, August 30, 2023, 2:00 pm Israeli Daylight Times (IDT)

**Title:** RGX-111: An investigational gene therapy for the treatment of severe mucopolysaccharidosis type I (MPS I): Interim analysis data from first-in-human study

**Presenter:** Raymond Wang, M.D., Division of Metabolic Disorders, CHOC Children's Hospital, USA

**Date/Time:** Wednesday, August 30, 2023, 9:15 am IDT

### Posters:

**Title:** First-in-human intracisternal dosing of RGX-181 (adeno-associated virus 9 / human tripeptidyl peptidase 1) for a 5-year-old child with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2): 6-month follow up

**Authors:** Carolina Fischinger De Souza, M.D., Ph.D., Hospital de Clinicas de Porto Alegre, Brazil

**Date/Time:** Wednesday, August 30, 2023, 6:35 pm - 8:30 pm IDT

**Title:** Daily living skills on the Vineland Adaptive Behavioral Scale version 2 (VABS-II) in neuronopathic mucopolysaccharidosis type II (MPS II)

**Authors:** Michelle Wood, Great Ormond Street NHS Foundation Trust; Dawn Phillips, Ph.D., Yoonjin Cho, Ph.D., Caroline Mulatya, Catherine Wilson, D.P.T., Joe Hagood,

Paulo Falabella, M.D., Ph.D., REGENXBIO Inc.

**Date/Time:** Wednesday, August 30, 2023, 6:35 pm - 8:30 pm IDT

### 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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