



REGENXBIO Announces Presentations of RGX-121 at the 21st Annual WORLDSymposium 2025

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ROCKVILLE, Md., Jan. 30, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced data from its RGX-121 (clevidsonine lanparvovec) program for the treatment of mucopolysaccharidosis type II (MPS II), also known as Hunter syndrome, will be shared at the 21st Annual WORLDSymposium™ 2025, taking place in San Diego, CA February 3-7, 2025.

The following presentations include an encore of the topline results from the pivotal phase of the Phase I/II/III CAMPSIITE® trial of RGX-121.

Abstract Title: Audiology assessment of participants in CAMPSIITE®, a phase I/II/III study of RGX-121 in neuropathic MPS II (poster 35)

Presenter: Nidal Boulos, Ph.D., Director, Clinical Science, REGENXBIO

Date/Time: Wednesday, February 5, 2025; 3:30 p.m. PT

Abstract Title: CAMPSIITE® Phase I/II/III: An interim clinical study update of RGX121, an investigational gene therapy for the treatment of neuropathic mucopolysaccharidosis type II (MPS II)

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

Date/Time: Thursday, February 6, 2025; 8:30 a.m. PT

ABOUT REGENXBIO Inc.

REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Since its founding in 2009, REGENXBIO has pioneered the development of AAV Therapeutics, an innovative class of gene therapy medicines. REGENXBIO is advancing a pipeline of AAV Therapeutics for rare and retinal diseases, including RGX-202 for the treatment of Duchenne, ABBV-RGX-314 for the treatment of wet AMD and diabetic retinopathy, being developed in collaboration with AbbVie, and RGX 121 for the treatment of MPS II. Thousands of patients have been treated with REGENXBIO's AAV Therapeutic platform, including Novartis' ZOLGENSMA for children with spinal muscular atrophy. Designed to be one-time treatments, AAV Therapeutics have the potential to change the way healthcare is delivered for millions of people. For more information, please visit www.regenxbio.com.

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