



REGENXBIO Announces Presentations at the 18th Annual WORLDSymposium™ 2022

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- *Interim data from Phase I/II trials of RGX-121 for the treatment of MPS II and RGX-111 for the treatment of MPS I to be presented*

REGENXBIO Inc. (Nasdaq: RGNX) today announced that five oral and seven poster presentations will be presented at the 18th Annual WORLDSymposium™, taking place in San Diego, CA from February 7 through 11, 2022. The presentations include interim results from the Phase I/II clinical trials of RGX-121 for the treatment of mucopolysaccharidosis type II (MPS II), also known as Hunter Syndrome, and RGX-111 for the treatment of mucopolysaccharidosis type I (MPS I), also known as Hurler Syndrome.

The oral presentations will be presented as follows:

Abstract Title: Treatment of cardiac, neurologic, and skeletal manifestations of murine MPS I with AAV9-IDUA: Efficacy study of vector dose and route of administration

Presenter: Lalitha Belur, Ph.D., Research Assistant Professor Genetics, Cell Biology and Development at University of Minnesota

Date/Time: Tuesday, February 8, 2022, 12:12 p.m. ET

Abstract Title: RGX-121 gene therapy for the treatment of severe mucopolysaccharidosis type II (MPS II): Interim analysis of data from the first in-human study

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

Date/Time: Wednesday, February 9, 2022, 11:12 a.m. ET

Abstract 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: Wednesday, February 9, 2022, 11:36 a.m. ET

Abstract Title: Identification of a biomarker that differentiates neuronopathic forms of MPS I and MPS II

Presenter: Nidal Boulos, Ph.D., Director, Clinical Scientist at REGENXBIO Inc.

Date/Time: Thursday, February 10, 2022, 11:24 a.m. ET

Abstract Title: Establishment of in vitro model of CLN2 retinopathy using human induced pluripotent stem cells

Presenter: Kwi Hye Kim, Ph.D., Senior Scientist, Preclinical Development at REGENXBIO Inc.

Date/Time: Friday, February 11, 2022, 1:40 p.m. ET

The poster presentations will be presented as follows:

Abstract Title: Safety, Pharmacodynamics and Efficacy of AAV9.hCLN2 in Preclinical Studies (poster LB-10)

Presenter: Nicholas Buss, Ph.D., Senior Director, Preclinical Development at REGENXBIO Inc.

Date/Time: Monday, February 7, 2022, 6 p.m. – 8 p.m. ET

Abstract Title: Comparative effectiveness of intravenous and intrathecal AAV9.CB7.hIDS (RGX-121) in a murine model of mucopolysaccharidosis type II (poster #287)

Presenter: Miles C. Smith, University of Minnesota

Date/Time: Tuesday, February 8, 2022, 6 p.m. – 8 p.m. ET

Abstract Title: The Expanded Neuronal Ceroid Lipofuscinosis 2 (CLN2) Clinical Rating Scale for Motor and Language Function: Development and Inter-Rater Reliability (poster #239)

Presenter: Dawn Phillips, Ph.D., Director, Clinical Scientist, Outcomes Research at REGENXBIO Inc.

Date/Time: Thursday, February 10, 2022, 6 p.m. – 8 p.m. ET

Abstract Title: Natural History of Neurodevelopment in Neuronopathic Mucopolysaccharidosis Type II (MPS II): Mullen Scales of Early Learning (MSEL) Cognitive, Motor and Language Developmental Trajectories (poster #238)

Presenter: Dawn Phillips, Ph.D., Director, Clinical Scientist, Outcomes Research at REGENXBIO Inc.

Date/Time: Thursday, February 10, 2022, 6 p.m. – 8 p.m. ET

Abstract Title: Beyond the normative data: Understanding the Bayley Scales of Infant Development version 3 (BSID-III) (poster #49)

Presenter: Yoonjin Cho, Ph.D., Director, Biostatistics at REGENXBIO Inc.

Date/Time: Thursday, February 10, 2022, 6 p.m. – 8 p.m. ET

Abstract Title: Beyond the normative data: Understanding the Mullen Scales of Early Learning (MSEL)

Presenter: Yoonjin Cho, Ph.D., Director, Biostatistics at REGENXBIO Inc. (poster #50)

Date/Time: Thursday, February 10, 2022, 6 p.m. – 8 p.m. ET

Abstract Title: Pilot study of novel optokinetic nystagmus-based visual acuity test in children with CLN2 disease (poster #218)

Presenter: Christina Ohnsman, M.D., Senior Clinical Development Lead at REGENXBIO Inc.

Date/Time: Thursday, February 10, 2022, 6 p.m. – 8 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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