



REGENXBIO Announces Presentations at the 17th Annual WORLDSymposium™ 2021

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ROCKVILLE, Md., Feb. 1, 2021 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced that three oral and nine poster presentations will be presented at the 17th Annual WORLDSymposium™, taking place virtually from February 8 through 12, 2021. The oral presentations include interim results from the Phase I/III clinical trial of RGX-121 for the treatment of mucopolysaccharidosis type II (MPS II).

The oral presentations will be presented as follows:

Abstract Title: Comparative systemic and neurologic effectiveness of intravenous and intrathecal AAV9 delivered individually or combined in a murine model of mucopolysaccharidosis type I

Presenter: Laliitha Belur, Ph.D., Assistant Professor Genetics, Cell Bio at University of Minnesota

Date/Time: Monday, February 8, 2021, 10:06 a.m. ET

Abstract Title: Intracisternal administration of AAV9 gene therapies to target the central nervous system

Presenter: Bryan A. Pukenas, M.D., Assistant Professor of Radiology at Hospital of the University of Pennsylvania

Date/Time: Tuesday, February 9, 2021, 11:36 a.m. ET

Abstract Title: RGX-121 gene therapy for severe mucopolysaccharidosis type II (MPS II): Interim results of an ongoing first in human trial

Presenter: Marie-Laure Nevoret, M.D., Senior Clinical Development Lead at REGENXBIO

Date/Time: Thursday, February 11, 2021, 9:42 a.m. ET

The poster presentations will be presented as follows:

Abstract Title: Subretinal injection of RGX-381 to cynomolgus monkeys leads to supraphysiological levels of TPP1 in the eye (poster #24)

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

Date/Time: Tuesday, February 9, 2021, from 2:30 to 3:30 p.m. ET

Abstract Title: Intracisternal administration of AAV9 gene therapies to target the central nervous system (poster #207)

Presenter: Bryan A. Pukenas, M.D., Assistant Professor of Radiology at Hospital of the University of Pennsylvania

Date/Time: Tuesday, February 9, 2021, from 2:30 to 3:30 p.m. ET

Abstract Title: First in-human intracisternal dosing of RGX-111 (adeno-associated virus 9/human α -Liduronidase) for a 20-month-old child with mucopolysaccharidosis type I (MPS I): 1 year follow-up (poster #264)

Presenter: Raymond Wang, M.D., Biochemical Genetics Specialist at CHOC Children's Hospital

Date/Time: Tuesday, February 9, 2021, from 2:30 to 3:30 p.m. ET

Abstract Title: RGX-121 gene therapy for severe mucopolysaccharidosis type II (MPS II): A clinical program to address central nervous system manifestations (poster #45)

Presenter: Kirsten Cowley, Director, Clinical Operations at REGENXBIO Inc.

Date/Time: Wednesday, February 10, 2021, from 2:30 to 3:30 p.m. ET

Abstract Title: Clinical and numerical presentation of neurocognitive assessments for MPS II patients using the Bayley Scales of Infant Development - version 3 (BSID-III) (poster #34)

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

Date/Time: Thursday, February 11, 2021, from 2:30 to 3:30 p.m. ET

Abstract Title: RGX-121 gene therapy for severe mucopolysaccharidosis type II (MPS II): Interim results of an ongoing first in human trial (poster #171)

Presenter: Marie-Laure Nevoret, M.D., Senior Clinical Development Lead at REGENXBIO Inc.

Date/Time: Thursday, February 11, 2021, from 2:30 to 3:30 p.m. ET

Abstract Title: Characterizing expressive language skills in children with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2): The caregiver perspective (poster #198)

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

Date/Time: Thursday, February 11, 2021, from 2:30 to 3:30 p.m. ET

Abstract Title: Characterizing visual function in children with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2): The caregiver perspective (poster #199)

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

Date/Time: Thursday, February 11, 2021, from 2:30 to 3:30 p.m. ET

Abstract Title: New research examines the evolution of data sharing practices in natural history studies and patient registries among patient advocate leaders, industry and academia (poster #LB-12)

Presenter: Vivian Fernandez, Senior Director of Patient Advocacy at REGENXBIO Inc.

Date/Time: Friday, February 12, 2021, from 2:30 to 3:30 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.

Contacts:

Tricia Truehart
Investor Relations and Corporate Communications
347-926-7709
ttruehart@regenxbio.com

Investors:
Brendan Burns, 212-600-1902
brendan@argotpartners.com

Media:
David Rosen, 212-600-1902
david.rosen@argotpartners.com



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