REGENXBIO Announces Presentations at the 19th Annual WORLDSymposium™ 2023

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- *New interim data from Phase I/I/III trial of RGX-121 for the treatment of MPS II and Phase I/II trial of RGX-111 for the treatment of MPS I to be presented*

ROCKVILLE, Md., Feb. 17, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced that three oral and eight poster presentations will be presented at the 19th Annual WORLD*Symposium* ™ 2023, taking place in Orlando, Florida from February 22 through 26, 2023. The presentations include new interim results from the Phase I/II/III clinical trial of RGX-121 for the treatment of mucopolysaccharidosis type II (MPS II), also known as Hunter Syndrome, and the Phase I/II trial of RGX-111 for the treatment of severe mucopolysaccharidosis type I (MPS I).

REGENXBIO will also host a sponsored symposium session, titled "AAV Gene Therapy as a Potential Treatment Modality for Neuronopathic MPS II" on Friday, February 24, 2023, at 11:45 a.m. ET.

The oral presentations will be presented as follows:

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 (abstract #191)
Presenter: Raymond Wang, M.D., Division of Metabolic Disorders, CHOC Children's Hospital, Department of Pediatrics, University of California, Irvine, CA
Date/Time: Friday, February 24, 2023, 9:24 a.m. ET

Abstract Title: In vitro pharmacology study using retina organoids and retina-on-a-chip of CLN2 patient-derived induced pluripotent stem cells (abstract #311, also available as poster #201)
Presenter: Kwi Hye Kim, Ph.D., Principal Scientist, Preclinical Development at REGENXBIO Inc.
Date/Time: Saturday, February 25, 2023, 2:48 p.m. ET

Abstract Title: RGX-121 gene therapy for the treatment of neuronopathic mucopolysaccharidosis type II (MPS II): Interim analysis of data from the first in human study (abstract #LB-21)
Presenter: Can Ficicioglu, M.D., Ph.D., Professor of Pediatrics at Perelman School of Medicine at the University of Pennsylvania, Director of the Newborn Metabolic Screening Program and the Lysosomal Storage Diseases Program, Clinical Director of the Metabolic Disease Program Children's Hospital of Philadelphia
Date/Time: Sunday, February 26, 2023, 9 a.m. ET (also available as an ePoster Wednesday, February 22, 2023, 3 p.m. ET)

The poster presentations will be presented as follows:

Abstract Title: Spontaneous seizures associated with cortical interneuron loss in $Cln2^{R207X}$ mice are ameliorated via gene therapy (abstract #350)
Presenter: Keigo Takahashi, Ph.D. Candidate, Pediatric Storage Disease Lab, Washington University in St. Louis
Date/Time: Thursday, February 23, 2023, 3-4 p.m. ET

Abstract Title: RGX-381 Gene Therapy for the Treatment of Ocular Manifestations of Late-Infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2 Disease): Overview of Nonclinical Development Program (abstract #LB-17)
Presenter: Gary Chan, Ph.D., Scientist II, Preclinical Development at REGENXBIO Inc.
Date/Time: Thursday, February 23, 2023, 4-5 p.m. ET

Abstract Title: The relationship between the expanded neuronal ceroid lipofuscinosis 2 (CLN2) clinical rating scale for motor function (CLN2 CRS-MX) and GAITRite® parameters (abstract #152)
Presenter: Luca Hagenah, M.D., University Medical Center Hamburg-Eppendorf, Department of Pediatrics, Hamburg
Date/Time: Friday, February 24, 2023, 3-5 p.m.

Abstract Title: Daily living skills on the Vineland Adaptive Behavioral Scale Version 2 (VABS-II) following RGX-121 treatment in participants with neuronopathic Mucopolysaccharidosis Type II (MPS II) (poster #285)
Presenter: Dawn Phillips P.T., M.S., Ph.D., Senior Director of Clinical Outcomes Research at REGENXBIO Inc.
Date/Time: Saturday, February 25, 2023, 3-5 p.m.

Abstract Title: Quantifying and modelling disease progression trajectory for natural history of MPS II (abstract #73)
Presenter: Yoonjin Cho, Ph.D., Senior Director, Biostatistics at REGENXBIO Inc.
Date/Time: Saturday, February 25, 2023, 3-5 p.m.

Abstract Title: RGX-381: First-in-human clinical trial of an investigational AAV9 gene therapy encoding TPP1 for the treatment of ocular manifestations of CLN2 Batten disease (abstract #LB-45)
Presenter: Christina Ohnsman, M.D., Senior Clinical Development Lead at REGENXBIO Inc.
Date/Time: Saturday, February 25, 2023, 3-5 p.m.

Abstract Title: Characterization of retinal degeneration phenotype in classic CLN2 disease using OCT biomarkers and an in vitro retinal model (abstract #261)
Presenter: Christina Ohnsman, M.D., Senior Clinical Development Lead at REGENXBIO Inc.

Date/Time: Saturday, February 25, 2023, 3-5 p.m.

Abstract Title: Loss of visual function associated with photoreceptor degeneration in CLN2 disease (abstract #260)
Presenter: Christina Ohnsman, M.D., Senior Clinical Development Lead at REGENXBIO Inc.
Date/Time: Saturday, February 25, 2023, 3-5 p.m.

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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