



REGENXBIO Announces Presentations at the 20th Annual WORLDSymposium 2024

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- *Pivotal topline results from the CAMPSIITE[®] trial of RGX-121 for the treatment of MPS II to be presented*
- *Conference call Wednesday, February 7, 4:30 p.m. ET*

ROCKVILLE, Md., Jan. 31, 2024 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced presentations on its program for the treatment of mucopolysaccharidosis type II (MPS II), also known as Hunter syndrome, will be shared at the 20th Annual WORLDSymposium[™] 2024, taking place in San Diego, CA February 3-9, 2024.

The presentations include the topline results from the pivotal phase of the Phase I/II/III CAMPSIITE[®] trial of RGX-121 for the treatment of MPS II.

The oral presentations will be presented as follows:

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: Wednesday, February 7, 2024; 8:36 a.m. PT

REGENXBIO will also present at the 3rd Annual Robert J. Gorlin Symposium on Tuesday, February 6, 2024 to discuss intracisternal administration of investigational AAV9 gene therapies to target the central nervous system in pediatric lysosomal disorders.

The poster presentations will be presented as follows:

Abstract Title: Audiology assessment of participants in CAMPSIITE[®], a phase I/II/III study of RGX-121 in neuropathic MPS II (abstract 037)

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

Date/Time: Thursday, February 8, 2024; 3 p.m. PT

Abstract Title: Caregiver Perspectives on their MPS II Journey (abstract 422)

Presenter: Vivian Fernandez, Executive Director, Patient Advocacy, REGENXBIO

Date/Time: Thursday, February 8, 2024; 3 p.m. PT

Conference Call

REGENXBIO will host a conference call Wednesday, February 7 at 4:30 p.m. ET with Dr. Harmatz and Raymond Wang, M.D., Children's Hospital of Orange County to discuss the CAMPSIITE trial pivotal phase topline results and the expedited plan for filing a Biologics License Application using the accelerated approval pathway in 2024.

Listeners can register for the webcast via this [link](#). Analysts wishing to participate in the question and answer session should use this [link](#). A copy of the slides being presented will be available via the Company's investor website. Those who plan on participating are advised to join 15 minutes prior to the start time. A replay of the webcast will also be available via the Company's investor website approximately two hours after the call's conclusion.

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 and AAV9. 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 "5x25" 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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