Correction of cardiovascular symptoms of MPS I in animal model

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REGENXBIO Inc. today announced that gene transfer mediated by REGENXBIO's NAV AAV8 vectors resulted in sustained serum α-L-iduronidase (IDUA) expression, as well as correction of systemic features of MPS I, or Hurler syndrome, a lysosomal storage disease (LSD) caused by the body's inability to produce the IDUA enzyme. Data from a study performed by researchers at the Perelman School of Medicine at the University of Pennsylvania ("Penn") show animals treated with a single intravenous injection of NAV AAV8 vectors expressing the IDUA gene not only demonstrated meaningful improvements in the biochemical features of MPS I in most tissues, but the majority also exhibited complete resolution of aortic valve lesions. This effect is significant since it has not been previously observed in MPS I patients treated with current therapies or animal models.

The study, which was funded in part by a grant from REGENXBIO, has been published online in *Proceedings of the National Academy of Sciences* (*PNAS*).

"This study by our collaborators at Penn demonstrates the potential of NAV Technology-based treatments to address cardiovascular symptoms of LSDs that are not well-managed with existing standards of care, which consists of hematopoietic stem cell transplantation or weekly enzyme replacement therapy," said Ken Mills, President and CEO of REGENXBIO. "While REGENXBIO's current development programs focus on a profile for treating the central nervous system manifestations of Hurler syndrome (MPS I) and Hunter syndrome (MPS II), our

research interests are rooted in a broad commitment to improve patient outcomes for all features of these and other LSDs."

James M. Wilson, MD, PhD, professor and director of the Gene Therapy Program in the Department of Pathology and Laboratory Medicine at Penn, added, "Our research underscores the prospect for AAV-mediated gene therapy to be further developed as a potentially safe and effective treatment for MPS I, as well as other LSDs that require lifelong systemic enzyme replacement."

More information: Christian Hinderer, Peter Bell, Brittney L. Gurda, Qiang Wang, Jean-Pierre Louboutin, Yanqing Zhu, Jessica Bagel, Patricia O'Donnell, Tracey Sikora, Therese Ruane, Ping Wang, Mark E. Haskins, and James M. Wilson "Liver-directed gene therapy corrects cardiovascular lesions in feline mucopolysaccharidosis type I." *PNAS* 2014 ; published ahead of print September 29, 2014, DOI: 10.1073/pnas.1413645111

Provided by REGENXBIO Inc.

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