

Dr. James M. Wilson Joins REGENXBIO as Chief Scientific Advisor

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Renowned gene therapy pioneer to take active role in accelerating development of groundbreaking treatments

WASHINGTON, DC November 18, 2014 – REGENXBIO Inc. today announced that James M. Wilson, M.D., Ph.D. has been named its Chief Scientific Advisor, effective immediately. Dr. Wilson is also the director of the Gene Therapy Program and professor of Pathology and Laboratory Medicine at the Perelman School of Medicine at the University of Pennsylvania (Penn).

REGENXBIO's NAV® Technology, which includes novel, recombinant AAV vectors AAV7, AAV8, AAV9 and AAVrh10, among others, was discovered through the pioneering research efforts of Dr. Wilson and his team at Penn. NAV Technology was the basis of REGENXBIO's founding in 2009. Today, the company is leveraging NAV Technology to develop gene therapy treatments for Hurler Syndrome (MPS I), Hunter Syndrome (MPS II), wet age-related macular degeneration and X-linked retinitis pigmentosa. In addition, REGENXBIO has licensed NAV Technology to eight gene therapy companies that are developing treatments for serious diseases with significant unmet needs.

"Since day one, Jim has helped shape REGENXBIO as our scientific founder and advisor," said Ken Mills, President and CEO of REGENXBIO. "With Jim now serving as our Chief Scientific Advisor, we look forward to leveraging his unparalleled gene therapy expertise to help build our R&D organization and advance the development of our NAV-based gene therapy treatments for lysosomal storage disorders and ocular diseases. Jim will also play an important role in progressing partnerships with our current and future NAV Technology Licensees."

"I'm delighted that this new role enables me to work more closely with REGENXBIO and our NAV Technology Licensees so we can advance important new treatments and realize our collective goal of bringing life-changing gene therapy treatments to patients," said Dr. Wilson. "This arrangement also strengthens the long-standing collaboration between Penn and REGENXBIO. Together, we'll continue to build upon the impressive progress we've made thus far in advancing potential gene therapy treatments into clinical development."

Dr. Wilson has dedicated his career to gene therapy and much of his research has focused on the development of AAV vectors as vehicles for gene delivery. Prior to joining Penn more than 20 years ago, Dr. Wilson held a faculty position at the University of Michigan, where he started his studies in gene therapy. Earlier, he completed a residency in Internal Medicine at the Harvard-affiliated Massachusetts General Hospital and a postdoctoral fellowship at MIT. Dr. Wilson holds an M.D. and a Ph.D. from the University of Michigan and a B.S. in Chemistry from Albion College.

Editor's Note

J.M. Wilson is an advisor to REGENXBIO, Dimension Therapeutics, and Solid Gene Therapy, and is a founder of, holds equity in, and receives sponsored research support from REGENXBIO and Dimension Therapeutics; in addition, he is a consultant to several biopharmaceutical companies and is an inventor on patents licensed to various biopharmaceutical companies.

About REGENXBIO

REGENXBIO Inc. is the leading next-generation AAV gene therapy company, developing a new class of personalized therapies based on its proprietary NAV® Technology platform for a range of severe diseases with serious unmet needs. NAV Technology includes novel AAV vectors AAV7, AAV8, AAV9, and AAVrh10. The company is developing gene therapy treatments to address lysosomal storage disorders and ocular diseases. REGENXBIO has enabled leading global partners including Baxter Healthcare, Fondazione Telethon, Audentes Therapeutics, Lysogene, Esteve, AveXis, AAVLife and Voyager Therapeutics to use its NAV Technology. In addition, together with Fidelity Biosciences, REGENXBIO formed Dimension Therapeutics, a company focused on the development and commercialization of NAV-based gene

therapies for rare diseases.

For more information about REGENXBIO, please visit www.regenxbio.com.

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