REGENXBIO and Dimension Therapeutics Add Development of Treatments for Two Metabolic Diseases to Exclusive Licensing Agreement

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ROCKVILLE, MD April 23, 2015 – REGENXBIO Inc., a leading biotechnology company in gene therapy, today announced that it has added the indications of ornithine transcarbamylase deficiency (OTC) and Glycogen Storage Disease Type 1a (GSD1a) to its existing license agreement with Dimension Therapeutics. This will enable Dimension to develop and commercialize products to treat OTC and GSD1a – as well as hemophilia A and B, which Dimension licensed from REGENXBIO in 2013 – using REGENXBIO's proprietary NAV® Technology.

"The addition of OTC and GSD1a to our license agreement with Dimension is a natural extension of the founding intent of the company, as it enables the development of innovative liver-directed treatments using NAV Technology for patients suffering from these important, rare metabolic diseases," said Ken Mills, President and CEO of REGENXBIO. "In addition to our own programs, we have now granted 16 commercial licenses and remain committed to the development and commercialization of our NAV Technology in disease areas where patients need better treatment options."

About OTC Deficiency and GSD1a

OTC deficiency, the most common urea cycle disorder, is caused by a genetic defect in a liver enzyme responsible for detoxification of ammonia. Individuals with OTC deficiency can build-up excessive levels of ammonia in their blood, potentially resulting in neurological deficits and other toxicities. Neonatal onset disease in males is severe and can be fatal at an early age. The greatest percentage of patients experience late-onset disease, representing a clinical spectrum of disease severity. It is estimated that more than 10,000 patients are affected by OTC deficiency worldwide. Currently, the only curative approach is liver transplantation.

GSD1a, the most common glycogen storage disease, has significant unmet needs. Patients have a defective gene for the enzyme glucose-6-phosphatase, resulting in the inability to regulate blood sugar (glucose). If chronically untreated, patients develop severe lactic acidosis, progress to renal failure, and die in infancy or childhood. There are no approved pharmacological therapies, and no ongoing clinical development beyond dietary approaches. An estimated 6,000 or more patients are affected by GSD1a worldwide.

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. REGENXBIO has enabled leading global partners including Baxter Healthcare, Fondazione Telethon, Audentes Therapeutics, Lysogene, Esteve, AveXis, AAVLife, Voyager Therapeutics and Dimension Therapeutics to use its NAV Technology.

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

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