THE LEADER IN AAV GENE THERAPY

REGENXBIO is a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy. Our proprietary AAV gene delivery platform (our NAV Technology Platform) consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. Our mission is to transform the lives of patients suffering from severe diseases with significant unmet medical needs by developing and commercializing in vivo gene therapy products based on our NAV Technology Platform. We seek to accomplish this mission through a combination of our internal development efforts and the efforts of our third-party licensees.

Our most advanced internally developed candidates include programs for the treatment of two severe and rare genetic diseases: Homozygous Familial Hypercholesterolemia (HoFH) and Mucopolysaccharidosis Type I (MPS I). We plan to build internal gene therapy franchises in the metabolic, neurodegenerative and retinal therapeutic areas, and develop multiple product candidates in these and other areas.

Our management team includes leaders who are experienced in building and operating innovative healthcare ventures and have expert knowledge in the development of AAV gene therapy.

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HoFH

Homozygous Familial Hypercholesterolemia

RGX-501 is our product candidate for the treatment of HoFH, which uses the AAV8 vector to deliver the human low-density lipoprotein receptor (LDLR) gene to liver cells. We believe that the liver is the preferred target organ for gene therapy of HoFH since LDLRs produced in the liver contribute to more than 90 percent of the capture and breakdown of low-density lipoprotein (LDL), making the liver the most important LDLR-producing organ. The liver is also the only organ capable of excreting cholesterol from the body, a function that is critical to the maintenance of cholesterol balance. Studies have shown that liver transplantation in HoFH patients corrects the disease, providing strong support that correction of hepatic LDL receptor activity by gene therapy is sufficient for metabolic correction of the disease.