REGENXBIO™

THE LEADER IN AAV GENE THERAPY

REGENXBIO (RGNX) 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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MPS I

Mucopolysaccharidosis Type I

RGX-111 is REGENXBIO's product candidate for the treatment of MPS I, which uses the AAV9 vector, intended to deliver the human α-l-iduronidase (IDUA) gene to the central nervous system (CNS). Delivery of the enzyme that is deficient within cells in the CNS could provide a permanent source of secreted IDUA beyond the blood-brain barrier, allowing for long-term cross-correction of cells throughout the CNS. This strategy could also provide rapid IDUA delivery to the brain, potentially preventing the progression of cognitive deficits that otherwise occurs in MPS I patients.

MPS II

Mucopolysaccharidosis Type II

RGX-121 is REGENXBIO's product candidate for the treatment of MPS II, which uses the AAV9 vector, intended to deliver the human iduronate-2-sulfatase (IDS) gene to the CNS. Delivery of the gene encoding the enzyme that is deficient within cells in the CNS could provide a permanent source of secreted IDS beyond the blood-brain barrier, allowing for long-term cross-correction of cells throughout the CNS. We believe this strategy could also provide rapid IDS delivery to the brain, potentially preventing the progression of cognitive deficits that otherwise occurs in Hunter syndrome patients.