Regenx Biosciences and Voyager Therapeutics Announce License Agreement

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- Voyager acquires rights to REGENX's proprietary NAV® vectors in multiple CNS disorders
- Eighth third-party commercial license of REGENX's NAV vectors since 2010
- REGENX to receive undisclosed upfront payment, milestones and royalties in exchange for non-exclusive worldwide license

WASHINGTON, DC and CAMBRIDGE, Mass. June 2, 2014 – REGENX Biosciences, LLC and Voyager Therapeutics today announced that they have entered into a license agreement for use of REGENX's proprietary NAV® vectors for the development and commercialization of gene therapies to treat Amyotrophic Lateral Sclerosis (ALS), Friedreich's ataxia (FA) and Huntington's disease (HD).

Under the terms of the agreement, REGENX has granted Voyager a non-exclusive worldwide license, as well as sublicensing rights, to REGENX's NAV vectors for the treatment of ALS, FA and HD. In exchange for these rights, REGENX will receive an undisclosed upfront payment, ongoing fees, milestone payments, and royalties on net sales of products incorporating NAV vectors. REGENX will also receive a share of certain sublicensing revenues.

"This license agreement serves as further validation of our proprietary NAV vector technology platform, and is an important step towards the successful development of NAV-based gene delivery treatments for patients afflicted with the serious and debilitating rare diseases to which Voyager is committed," said Ken Mills, President and CEO of REGENX. "As the leader in next-generation AAV gene therapy, REGENX is pleased to be collaborating with Voyager, which is well-positioned to develop innovative treatments through the application of our NAV technology."

Mark Levin, Interim CEO of Voyager, commented, "Voyager is the leading AAV gene therapy company focused on developing life-changing treatments for patients with devastating CNS disorders. We are committed to advancing the AAV gene therapy field via broad-based investment in a number of key technological areas. In addition to providing a valuable addition to Voyager's intellectual property portfolio, the rights to use REGENX's NAV vectors will position us to rapidly advance the development of breakthrough CNS gene therapies."

About Amyotrophic Lateral Sclerosis

Amyotrophic Lateral Sclerosis (ALS), also known as Lou Gehrig's disease, is a progressive, fatal neurodegenerative disease that leads to muscle weakness, loss of mobility, impaired speech, and difficulty breathing and swallowing. Most ALS patients only live three to five years after initial symptoms appear, and it is estimated that as many as 30,000 patients in the United States and 450,000 worldwide are living with the disease. Familial ALS accounts for 5 to 10 percent of ALS cases, including an estimated 20 percent of familial ALS cases caused by toxic gain of function mutations in the SOD1 gene.

About Friedreich's Ataxia

Friedreich's ataxia (FA) is the most common hereditary ataxia, with approximately 8,000 patients living with the disease in the United States and Europe. FA patients have a genetic mutation in the FXN gene, which limits the production of the protein frataxin, causing a variety of debilitating symptoms and complications, loss of coordination and balance, muscle weakness, impaired vision, hearing and speech, scoliosis, diabetes, and cardiomyopathy.

About Huntington's Disease

Huntington's disease (HD) is an inherited neurodegenerative disorder where symptoms typically become noticeable between 30 and 50 years of age. HD is caused by a genetic mutation in the huntingtin gene, which leads to the production of a mutated huntingtin protein, resulting in symptoms such as chorea, rigidity, abnormal posturing, cognitive impairment

and psychiatric symptoms, and difficulty with speech and swallowing. It is estimated that 1 in every 10,000 Americans has HD and more than 250,000 others are at-risk of having inherited the HD genetic mutation.

About REGENX Biosciences

ReGenX Biosciences is the leading next-generation AAV gene therapy company, developing a new class of personalized therapies based on its proprietary NAV® vector technology platform for a range of severe diseases with serious unmet needs. NAV vector technology includes novel AAV vectors rAAV7, rAAV8, rAAV9, and rAAVrh10. The company's treatments in development include programs addressing lysosomal storage disorders and ocular diseases. ReGenX's leadership in AAV gene therapy and corresponding intellectual property has enabled it to establish collaborations with leading global partners including Baxter Healthcare, Fondazione Telethon, Audentes Therapeutics, Lysogene, Esteve, AveXis and AAVLife. In addition, together with Fidelity Biosciences, ReGenX formed Dimension Therapeutics, a company focused on the development and commercialization of AAV gene therapies for rare diseases.

For more information regarding ReGenX, please visit www.regenxbio.com.

About Voyager Therapeutics

Voyager Therapeutics is a gene therapy company developing life-changing treatments for fatal and debilitating diseases of the central nervous system (CNS). Voyager is committed to advancing the field of AAV (adeno-associated virus) gene therapy through innovation and investment in vector optimization and engineering, dosing techniques, as well as process development and production. The company's initial pipeline is focused on CNS diseases in dire need of effective new therapies, including Parkinson's disease, a monogenic form of amyotrophic lateral sclerosis (ALS), and Friedreich's ataxia. Founded by scientific and clinical leaders in the fields of AAV gene therapy, expressed RNA interference and neuroscience, Voyager Therapeutics was launched in 2014 with funding from leading life sciences investor Third Rock Ventures and is headquartered in Cambridge, Mass. For more information, please visit www.voyagertherapeutics.com.

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