REGENX Biosciences enters into agreement with AAVLife for Friedreich's ataxia using NAV vectors

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WASHINGTON, DC and Paris, France April 2014 – REGENX Biosciences, LLC announces that the company has entered into an agreement with AAVLife for the development and commercialization of products to treat Friedreich's ataxia (FA) using **NAV** technology.

Under the terms of the Agreement, REGENX granted AAVLife an exclusive worldwide license, with rights to sublicense, to deliver REGENX's **NAV** rAAVrh10 vector via non CNS routes to treat FA in humans. In addition, AAVLife was granted an option to obtain a non‑exclusive worldwide license to additional **NAV** vectors for CNS delivery for the treatment of FA in humans. In return for these rights, REGENX receives payments in the form of up‑front and on‑going fees, certain milestone fees and royalties on net sales of products incorporating **NAV** vectors. REGENX would also receive a share of any sublicensing revenues.

"REGENX has been engaged with the team at AAVLife, including its stakeholders like the Friedreich's Ataxia Research Alliance (FARA), since first becoming aware of their gene therapy research results and during the company's process of formation. We are pleased to formally continue our collaboration with a team who has the leadership, expertise, resources, and commitment to patients that is required in order to develop innovative treatments for patients with FA through the application of **NAV** technology," said Ken Mills, President and CEO of REGENX. "We believe this license agreement will be a key component to the successful development of treatments for patients suffering with FA."

Amber Salzman, Ph.D., Chief Executive Officer and a co‑founder of AAVLife, commented: "The right to the REGENX vector is a critical part of our program to advance into clinical trials a gene‑therapy approach to treating Friedreich's ataxia."

Jennifer Farmer, Executive Director of FARA, added: "Heart disease accounts for most early deaths due to Friedreich's ataxia. We believe that **NAV** technology will enable successful clinical studies that are urgently needed for patients with Friedreich's ataxia."

About Friedreich's Ataxia (FA)

Friedreich's ataxia is a rare, degenerative, life‑shortening neuro‑muscular disorder that affects children and adults, and involves the loss of strength and coordination usually leading to wheelchair use. Other symptoms may include diminished vision, hearing and speech; scoliosis (curvature of the spine); and increased risk of diabetes. Also associated with the disorder is a progressive decline in cardiac function which is the most common cause of death. There are no FDA‑approved treatments.

About REGENX Biosciences

REGENX Biosciences (www.regenxbio.com) is the leading AAV gene therapy company that is 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 such as rAAV7, rAAV8, rAAV9, and rAAVrh10. Our treatments in development include programs for hypercholesterolemia, mucopolysaccharidoses, and retinitis pigmentosa. REGENX's leadership in AAV gene therapy and corresponding intellectual property has enabled it to establish collaborations with leading global partners including Chatham Therapeutics, Fondazione Telethon, Audentes Therapeutics, Lysogene, Esteve, and AveXis. In addition, together with Fidelity Biosciences, REGENX has formed Dimension Therapeutics, a company focused on the developmentand commercialization of AAV gene therapies for rare diseases. For more information regarding REGENX, please visit www.regenxbio.com.

About AAVLife

AAVLife, registered in Paris, is a privately held company dedicated to advancing gene therapy for rare diseases. Further information is available at www.aavlife.com.

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