FARA Friedreich's Ataxia Research Alliance

PRESS RELEASE

Voyager Therapeutics, a new gene therapy company focused on CNS diseases, including Friedreich's Ataxia (FA), is launched

Downingtown, PA- February 12, 2014- FARA is pleased to recognize today's launch of Voyager Therapeutics and its commitment to developing gene therapies for central nervous system disorders, including FA. Voyager is backed by leading life sciences investor Third Rock Ventures, and the company has assembled leaders in adeno-associated virus (AAV) gene therapy to develop life-changing treatments with the goal of dramatically improving patients' lives.

See press release from Voyager Therapeutics:
http://www.voyagertherapeutics.com/pdfs/Voyager_02.11.14.pdf

FA is a candidate for gene therapy because it is caused by a mutation in a single gene, and this mutation is in a non-coding region of the gene. This means that individuals with FA are frataxin protein deficient not frataxin null, and they don't have to battle an abnormal protein, just make more of the existing protein. If the native gene can be supplemented with a new, correct gene that produces additional frataxin, the body should recognize the needed frataxin and not mount an immune reaction.

FARA's Scientific Advisory Board and Board of Directors have recognized that gene therapy approaches would have the opportunity for profound therapeutic benefit, and this area of research is a priority for the organization. FARA is funding different approaches to gene therapy in academic labs around the world and partnering with companies to advance gene therapy strategies to clinic.

Jennifer Farmer, Executive Director of FARA states, "Shortly after the frataxin gene was identified in 1996, I can remember individuals and families diagnosed with FA asking me about gene therapy as a treatment option. It is very exciting that what we conceptualized as a therapy over a decade ago has advanced in the lab, especially in terms of new vectors and delivery mechanisms, such that we have new companies, like Voyager, taking aim at FA with this research."

About 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; diminished vision, hearing and speech; scoliosis (curvature of the spine); increased risk of diabetes; and a life-threatening heart condition. There are no FDA-approved treatments.

About FARA

The Friedreich's Ataxia Research Alliance (FARA) is a 501(c)(3), non-profit, charitable organization dedicated to accelerating research leading to treatments and a cure for Friedreich's ataxia. www.CureFA.org☐

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