



Agilis Biotherapeutics Co-Sponsors Friedreich's Ataxia Research Alliance Annual Fundraising Gala and Research Symposium

Participates in Biomarker and Clinical Outcome Meeting

Cambridge, MA, September 19, 2016 -- Agilis Biotherapeutics, LLC (Agilis), a biotechnology company advancing innovative DNA therapeutics for rare genetic diseases that affect the central nervous system (CNS), announced today that it co-sponsored the Friedreich's Ataxia (FA) Research Alliance (FARA) annual gala and fund raiser in Tampa, Florida. Senior executives from Agilis participated in the FA research symposium, clinical expert roundtable, and biomarker meeting scheduled during the several day event. Christopher Silber, M.D., Chief Medical Officer of Agilis, moderated a clinical expert roundtable on gene therapy approaches to treating the CNS manifestations of FA, bringing together leaders from around the world to discuss emerging genetic treatments for the disease.

"The conference highlights the remarkable progress being realized in FA research and treatment," said Dr. Jodi Cook, Agilis Chief Operating Officer, "We are pleased that Agilis was able to co-sponsor this event. Agilis' progress in advancing its gene therapy program for Friedreich's ataxia to first-in-man studies positions the Company at the forefront of this exciting field, which has great promise for a treatment where few treatment options exist."

About Friedreich's ataxia

Friedreich's ataxia (FA) is an inherited neuromuscular disorder most commonly caused by a single genetic defect in the FXN gene that leads to reduced production of frataxin, a mitochondrial protein that is important for cellular metabolism and energy production. FA results in a physically debilitating, life-shortening condition and is the most common hereditary ataxia, with an estimated 5,000 to 10,000 patients in the US (i.e., one in every 50,000 people).

Both male and female children can inherit the disorder. Symptoms of FA include progressive loss of coordination and muscle strength, which lead to the full-time use of a wheelchair; scoliosis (which often requires surgical intervention); diabetes mellitus; hearing and vision impairment; serious heart conditions; and premature death. Current FA therapies are primarily focused on symptomatic relief, and there are no FDA-approved drugs to treat the cause of FA.

Visit www.curefa.org for more information.

About Agilis Biotherapeutics

Agilis is advancing innovative gene therapies designed to provide long-term efficacy for patients with debilitating, often fatal, rare genetic diseases that affect the central nervous system. Our therapies are engineered to impart sustainable clinical benefits, and potentially a functional cure, by inducing persistent expression of a therapeutic gene. The Company's technology is aimed at the precise targeting and restoration of a lost gene function, while avoiding unintended off-target effects. Our integrated strategy increases the efficiency of developing DNA therapeutics into safe, targeted gene therapies that achieve long-term efficacy and enable patients to remain asymptomatic without continuous invasive treatment. Agilis' rare disease programs are focused on gene therapy for AADC deficiency, Friedreich's ataxia, and Angelman syndrome, rare genetic diseases that include severe neurological deficits and result in physically debilitating conditions.

We invite you to visit our website at: www.agilisbio.com

Safe Harbor Statement

Some of the statements made in this press release are forward-looking statements. These forward-looking statements are based upon our current expectations and projections about future events and generally relate to our plans, objectives and expectations for the development of our business. Although management believes that the plans and objectives reflected in or suggested by these forward-looking statements are reasonable, all forward-looking statements involve risks and

uncertainties and actual future results may be materially different from the plans, objectives and expectations expressed in this press release.

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