Agilis Biotherapeutics Announces FDA Orphan Drug Designation for the Treatment of Friedreich's Ataxia (FA)

First Gene Therapy Candidate to Receive Designation for FA

Cambridge, MA, August 2, 2016 (BUSINESS WIRE)—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 the United States Food and Drug Administration (FDA) has granted Orphan Drug Designation to Agilis' gene therapy product candidate, AGIL-FA, being developed for the treatment of Friedreich's ataxia (FA), an inherited degenerative neuromuscular disorder resulting in loss of motor coordination and strength, hearing, vision, speech and often premature death. Agilis is the first company to receive orphan designation from the FDA for a gene therapy to treat FA and it is the fourth time this year that Agilis has been granted orphan designation in the US and Europe for its gene therapies. The Company's gene therapies for AADC deficiency and Angelman syndrome have previously received orphan status.

"Having the first gene therapy product candidate to receive orphan drug designation from the FDA for the treatment of FA is an honor," said Mark Pykett, President and CEO of Agilis. "The orphan designation is another step on our path to bringing this important new therapy to patients who currently lack treatment options." AGIL-FA is a gene-therapy product consisting of a unique gene construct developed in partnership with Intrexon Corporation (NYSE: XON) delivered with adeno-associated virus technology.

"We are extremely pleased to receive this landmark designation, as we move our FA

development program forward," said Christopher Silber, MD, Agilis Chief Medical Officer. "With each of our IND and clinical stage pipeline candidates having now received Orphan Designation, this achievement highlights our efforts to advance innovative therapeutics for patients with rare genetic diseases affecting the CNS."

Friedreich's ataxia (FA) is a rare and life-shortening neurodegenerative disease caused by a defect in the FXN gene that reduces production of the frataxin protein. Agilis' FA gene therapy program is focused on delivering corrective DNA to specific CNS cells to restore frataxin protein levels. Agilis has worked closely with the Friedreich's Ataxia Research Alliance (FARA) to focus the development program on patient needs. "FARA is delighted to continue our support of Agilis and their innovative approach to the treatment of FA," said Jennifer Farmer, MS, Executive Director of FARA. "We look forward to continuing our partnership to advance this important potential therapy, as well as supporting research to identify biomarkers and clinical outcome measures, which will advance the development of the product candidate into clinical trials."

FDA Orphan Drug Designation is evaluated for drugs from all classes (e.g. small molecules, proteins, and gene or cell therapies) that are intended for the treatment of rare diseases, defined as diseases affecting fewer than 200,000 people in the United States. The designation provides sponsors with development and commercial incentives, including seven years of market exclusivity in the US, prioritized consultation by FDA on clinical studies, and certain exemptions from or reductions in regulatory fees.

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

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