Agilis Biotherapeutics and the University of South Florida Enter Into Worldwide, Exclusive License Agreement for Angelman Syndrome Gene Therapy

Program expands Agilis' pipeline of DNA therapeutics for rare central nervous system disorders

Cambridge, MA, May 13, 2015 7:30 am EST --(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 Company has entered into an exclusive worldwide license agreement with the University of South Florida (USF) for the treatment of Angelman Syndrome (AS) using gene therapy technology developed by Edwin Weeber, PhD, Director of the Neurobiology of Learning and Memory and Chief Scientific Officer at the USF Health Byrd Alzheimer's Research Institute at the University of South Florida, and one of the world's foremost AS investigators. Dr. Weeber and colleagues have developed an innovative gene therapy approach for the treatment of AS and recently published evidence of therapeutic efficacy in the gold standard mouse model.

Agilis' collaboration with Dr. Weeber and USF will target the genetic malfunctions of AS using gene therapy to deliver a corrective UBE3a gene to rescue neurological deficits in patients suffering from this rare disease. Under the terms of the agreement, Agilis will be granted worldwide, exclusive rights to AS gene therapy candidates and related intellectual property.

"We are delighted to be working with Dr. Weeber in this exciting area of gene therapy," said Greg Robinson, PhD, Agilis' Chief Scientific Officer. "Dr. Weeber's science is compelling and provides an excellent platform from which to develop an effective UBE3a therapy for patients with AS, for whom no effective treatment options currently exist."

AS is a rare genetic disorder that primarily affects the central nervous system. Characteristic features of this condition include delayed development, intellectual disability, severe speech impairment, seizures and ataxia. According to The Foundation for Angelman Syndrome Therapeutics, the disorder strikes an estimated 1 in 15,000 live births.

“Angelman Syndrome continues to represent a rare CNS disorder with significant unmet medical need,” Dr. Weeber added. “Our research has demonstrated that restoration of UBE3a function has the potential to address many of the neurological symptoms of AS, and to positively impact the quality of life by addressing Angelman’s CNS manifestations.”

“Agilis is honored to be working with Dr. Weeber and USF,” Dr. Mark Pykett, President and Chief Executive Officer of Agilis, commented. “Adding this program to our pipeline enhances our corporate mission of developing functional cures for rare genetic diseases of the CNS. As we expand our programs, we are continually augmenting our differentiated technologies, skills and capabilities in addressing rare CNS disorders with innovative DNA therapeutic strategies. The AS gene therapy program, in collaboration with Dr. Weeber and USF, enhances our position in providing solutions to patients across of spectrum of CNS diseases.”

About Agilis Biotherapeutics, LLC

Agilis is advancing innovative DNA therapeutics 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 benefit, and potentially a functional cure, by inducing persistent expression of a therapeutic gene. The Company’s technology enables precise targeting and restoration of lost gene function, while avoiding unintended off-target effects. Our integrated strategy increases the efficiency of developing DNA therapeutics to more rapidly advance safe, targeted gene therapies that achieve long-term efficacy and enable patients to remain asymptomatic without continuous invasive treatment. Agilis’ lead program is focused on gene therapy for Friedreich’s ataxia (FA), a rare inherited disease that includes severe neurological deficits and results in a physically debilitating, life-shortening condition. FA is the most common hereditary ataxia, with an estimated 5,000 to 10,000 patients in the U.S.

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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