Agilis Biotherapeutics and the University of South Florida Enter Into Worldwide, Exclusive License Agreement for the Treatment of Cognitive Disorders using Reelin-Based Therapies

Agilis Expands Pipeline of DNA Therapeutics for Rare Central Nervous System Disorders

Cambridge, MA, September 17, 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 cognitive disorders using Reelin replacement technologies developed by Dr. Edwin Weeber, PhD, Director of the Neurobiology of Learning and Memory and Chief Scientific Officer at the USF Health Byrd Alzheimer's Institute at the University of South Florida. Reelin is an important extracellular signaling protein that has been shown in numerous studies to affect biochemical pathways involved with memory, learning and cognition. Dr. Weeber and colleagues have recently published evidence on the positive cognitive effects of Reelin protein replacement in a mouse model of Angelman Syndrome. The results support the hypothesis that the brain dysfunction and defects in learning and memory associated with Angelman Syndrome are potentially reversible. Furthermore, in addition to Angelman Syndrome, Reelin may be a critical factor in the treatment of other CNS disorders associated with cognitive declines such as Fragile X syndrome, schizophrenia and Alzheimer's disease (Hethorn et al, Eur. J. Neurosci., 2015). Under the license agreement with USF, Agilis will also sponsor research with Dr. Weeber to advance the clinical potential of Reelin in a range of CNS indications, including the rare disease Fragile X Syndrome.

"We are delighted to be working with Dr. Weeber in this important area of research," said Gregory Robinson, PhD, Agilis' Chief Scientific Officer. "Dr. Weeber is a leading expert in cognitive disorders, and his science is novel and compelling. This license provides Agilis with an excellent platform from which to develop Reelin-based therapies that have the potential to meaningfully improve cognitive dysfunction and quality of life for patients."

"Reelin represents a novel opportunity to treat CNS disorders associated with cognitive declines," Dr. Weeber added. "Our research has demonstrated that Reelin administration addresses many of the neurological symptoms of AS, and may play a role in the treatment of other rare disorders such as Fragile X syndrome and larger indications such as schizophrenia or Alzheimer's disease."

"Agilis is honored to expand our relationship with Dr. Weeber and USF and to build on his seminal insights into cognitive impairment," Dr. Mark Pykett, President and Chief Executive Officer of Agilis, commented. "Adding this technology to our portfolio 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 Reelin program, in collaboration with Dr. Weeber and USF, enhances our position in providing solutions to patients across of spectrum of CNS diseases."

About Reelin

Reelin is an important extracellular signaling protein that has been shown in numerous studies to affect biochemical pathways involved with memory, learning and cognition. Reelin interacts with the ApoE Receptor 2 and the VLDL Receptor resulting in dimerization and downstream signaling. It influences the maturation of NMDA receptors and the insertion of AMPA receptors on nerve terminals, promoting synaptic plasticity and facilitating neurite outgrowth. Reelin may also assist with maintaining proper function of tau proteins inside neurons by promoting their hypophosphorylation and may mitigate the effects of beta-amyloid aggregates outside of neurons, both of which have been shown to play an important role in cognitive function.

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 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 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 Friedreich's Ataxia, Angelman Syndrome and Fragile X Syndrome, rare genetic diseases that include severe neurological deficits and result in physically debilitating conditions. Friedreich's Ataxia is the most common hereditary ataxia with an estimated 5,000 to 10,000 patients in the U.S. There are an estimated 10,000 to 15,000 people living with Angelman Syndrome in the US. Fragile X Syndrome is the most common known cause of inherited intellectual disability with an estimated 64,000 patients living 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.

###

For more information contact:

Agilis Biotherapeutics

Dr. Jodi Cook

Vice President, Operations

Email: jcook@agilisbio.com

Phone: 510-673-7809