Agilis Biotherapeutics Announces FDA Orphan Drug Designation for AGIL-AS for the Treatment of Angelman Syndrome

Cambridge, MA, November 4, 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 U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to AGIL-AS, the Company's gene therapy product candidate being developed for the treatment of Angelman syndrome (AS), a neuro-genetic disorder characterized by severe intellectual and developmental disability. AGIL-AS is being investigated as a new therapeutic to treat AS by delivering a corrective UBE3A gene to rescue neurological deficits in patients suffering from this rare disease. This is the first drug to be granted an Orphan Drug Designation status by the U.S. FDA for the indication of Angelman syndrome.

AS is a rare genetic disorder caused by the deletion/mutation of the UBE3A gene. UBE3A encodes the ubiquitin ligase E6-AP, a protein which plays a critical role in the function of the central nervous system. Characteristic features of the condition include delayed development, intellectual disability, severe speech impairment, seizures and ataxia. According to The Foundation for Angelman Syndrome Therapeutics (FAST), the disorder strikes an estimated 1 in 15,000 live births.

"Receiving orphan drug designation from the FDA for AGIL-AS is another step on our path to bringing this important new medicine to patients in dire need of an effective, durable treatment. Moreover, it signifies a landmark event, as it marks the first Angelman syndrome drug to achieve this regulatory milestone," stated Mark Pykett, President and CEO of Agilis.

”We're extremely pleased that we continue to make important clinical and regulatory progress, positioning us to advance this new treatment to address such a devastating disease.”

In May, the Company entered into an exclusive worldwide license agreement with the University of South Florida (USF) for the treatment of AS using gene therapy technology developed by Edwin Weeber, PhD, Professor, Molecular Pharmacology and Physiology, Director of the Neurobiology of Learning and Memory, and Chief Scientific Officer at the USF Health Byrd Alzheimer's Research Institute, and one of the world’s foremost AS investigators. Dr. Weeber and colleagues have published proof-of-concept of UBE3A gene therapy and established efficacy of gene therapy in the central nervous system in models of AS.

“Agilis’ Orphan Drug Designation for AGIL-AS is an important step forward for the technology and for the Angelman syndrome community,” Dr. Weeber stated. “Restoration of UBE3A function using gene therapy has the potential to address many of the neurological symptoms of AS and to positively impact the quality of life of AS patients.”

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 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.

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