Agilis Biotherapeutics Announces Orphan Designation Approval in Europe for the Treatment of Angelman Syndrome

Cambridge, MA, May 4, 2016 7:30 am EST -- (BUSINESS WIRE)—Agilis Biotherapeutics, (Agilis), a biotechnology company advancing innovative DNA therapeutics for rare genetic diseases that affect the central nervous system (CNS), announced today that the European Commission (EC) has granted Orphan Medicinal Product (OMP) designation in the European Union (EU) to the Company's gene therapy product candidate, AGIL-AS. The EC's approval follows a positive opinion in March from the European Medicine Agency's (EMA) Committee for Orphan Medicinal Products (COMP). AGIL-AS is being developed for the treatment of Angelman syndrome (AS), a neuro-genetic disorder characterized by severe intellectual and developmental disability. AGIL-AS is the first therapeutic to receive orphan designation for Angelman syndrome in Europe.

The orphan designation provides 10 years of market exclusivity for the product upon approval, and potential incentives for Agilis including Protocol Assistance (scientific advice for orphan products), and reduced filing and review fees with the EMA.

"Receiving orphan status from the EC, in conjunction with the previous orphan drug designation from the US FDA, is another step on our path to bringing this important new medicine to patients in need of an effective, durable treatment," said Mark Pykett, President and CEO of Agilis. "The orphan designation in Europe provides important benefits during development and commercialization, and represents important progress as we seek to bring this novel treatment for AS to the market."

Patricia Williams, Head of Regulatory Affairs at Agilis, said, “Receiving orphan designation for Angelman syndrome in Europe, represents an important regulatory milestone aimed at facilitating development of novel treatments for this difficult to treat disease.”

AGIL-AS is an innovative gene therapy candidate being investigated to treat AS by using an AAV vector to deliver a corrective UBE3A gene to rescue neurological deficits in patients suffering from this disease. AS is a rare genetic disorder caused by the deletion or mutation of the UBE3A gene. UBE3A encodes the ubiquitin ligase E6-AP, a protein that plays a critical role in the function of the central nervous system. Characteristic features of AS 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 one in 15,000 live births.

“Agilis’ receiving designation as an Orphan Medicinal Product is another exciting milestone achieved for the Angelman syndrome community,” said Paula Evans, Director of FAST. “We are thrilled with the news and are looking forward to participating in clinical trials when the therapeutic is available for evaluation.”

EC Orphan Designation is granted to drugs that are intended for the treatment of life threatening or chronically debilitating rare diseases where no therapeutic options either exist or are satisfactory. Rare diseases are those defined as having a prevalence of less than five in 10,000 in Europe. The designation provides sponsors with development and commercial incentives, including 10 years of market exclusivity, prioritized consultation by EMA on the development of the drug, including clinical studies, and certain exemptions from or reductions in regulatory fees.

About Agilis Biotherapeutics, LLC

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 technologies are

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 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, Angelman syndrome, and Fragile X 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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