Agilis Biotherapeutics Executives Deliver Keynote Speech at Foundation for Angelman Syndrome Therapeutics' Lite the Night on Fire Event

Event Supports Advancing Research for the Treatment of Angelman Syndrome

Cambridge, MA, September 30, 2016 -- 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 Agilis was a sponsor of the Foundation for Angelman Syndrome Therapeutics' (FAST) Lite the Night on Fire Event in Boston Massachusetts. Agilis executives Dr. Mark Pykett, Agilis President and CEO, Dr. Christopher Silber, Agilis Chief Medical Officer, and Dr. Jodi Cook, Agilis Chief Operating Officer, delivered the keynote address. They spoke about Agilis' close collaboration with FAST and the Company's progress on advancing gene therapy for Angelman Syndrome (AS) toward a first-in-man clinical trial.

"We have made significant progress in advancing our lead gene therapy candidate in collaboration with Dr. Edwin Weeber of the University of South Florida and in preparing for interactions with regulators," said Dr. Jodi Cook, Agilis COO, "Our work and commitment to AS have resulted in the first-ever orphan drug designations for an AS therapeutic in the US and Europe. Ongoing progress on our AS gene therapy has positioned the Company at the forefront of this exciting field, which holds great promise for treatment of this disease in which few options exist. We are pleased that Agilis was able to support this important FAST event, share our progress, and learn from the families and friends of those with Angelman syndrome."

Paula Evans, FAST Founder and Chair said, “I look forward to the day that our children can have simply ordinary lives. With the great work, support and collaboration provided to FAST by Agilis, that day is getting closer for all of us.”

About Angelman syndrome

Angelman syndrome is a rare genetic disorder caused by the deletion/mutation of the UBE3A gene. The UBE3A gene 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, resulting in chronic disability and the need for lifelong care. According to The Foundation for Angelman Syndrome Therapeutics, the disorder strikes an estimated 1 in 15,000 live births.

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

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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