Agilis Biotherapeutics Sponsors Inaugural Angelman Syndrome Biomarker and Outcome Measures Meeting

Meeting Draws Together Clinical, Research, and Industry Leaders with Families and Advocacy Groups to Advance Patient Centered Development of Therapeutics for Angelman Syndrome

Cambridge, MA, March 14, 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 the Company sponsored the first-ever Angelman Syndrome Biomarkers & Outcome Measures Consortium at the University of South Florida (USF), in Tampa, Florida. The one-day symposium, held on March 7, 2016 focused on the advent of reliable markers and clinical measures of Angelman syndrome (AS) for the development, evaluation and registration of innovative diagnostics and therapeutics. The meeting brought together leading researchers and clinicians from the Angelman syndrome community, AS families, representatives from the Angelman Syndrome Foundation (ASF) and the Foundation for Angelman Syndrome Therapeutics (FAST), and industry leaders from six companies.

Spearheaded by Dr. Jodi Cook (Agilis Vice President of Operations & Strategic Alliances) and Dr. Edwin Weeber (Chief Scientific Officer, USF Health Byrd Alzheimer's Institute, USF and a leading AS researcher), the open-forum event addressed topics important to patients, families and caregivers, such as communication and vocalization assessments, sleep and circadian rhythm measures, seizure assessments, movement and gait measures, and retrospective data analysis. The participants reviewed the current measures that are regularly used in clinical trials, previous controlled clinical trials and the challenges to developing effective therapeutics and diagnostics for AS going forward. A newly developed cloud-based international patient registry and data repository was reviewed by a representative from FAST Australia. The series of presentations and discussions culminated with agreement to develop a white paper summary prioritizing the areas of needed research and to identify experts and facilitators to lead research and development efforts.

"We are delighted to have participated in this important meeting of key stakeholders from the AS community to begin an important dialogue on biomarkers and outcome measures in Angelman syndrome research," said Dan Harvey PhD., Chief Scientific Officer of ASF. Paula Evans, Director of FAST, added, "The community recognizes the urgency of developing reliable measures needed for drug development and approval and for enhanced diagnostic accuracy in AS. The participation of a broad swath of the AS community at this meeting is representative of the growing support base across many diverse partners who are pulling together to make a difference in this very challenging disorder."

"Agilis is honored to have sponsored and organized the first-ever Angelman Syndrome Biomarkers & Outcome Measures Consortium," said Dr. Cook. "The identification and validation of biomarkers and outcome measures in patient-focused drug development is paramount to the successful approval of new therapeutics to help patients with AS. This open-forum meeting is reflective of the community's commitment and teamwork in finding solutions for the patients we serve."

About Angelman Syndrome

AS 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, 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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For more information contact:

Agilis Biotherapeutics

Dr. Jodi Cook

Vice President, Operations & Strategic Alliances

Email: jcook@agilisbio.com

Phone: 510-673-7809