



## **Agilis Biotherapeutics Co-Sponsors and Presents at the 2017 ASF Research Symposium and Family Conference**

### **Meeting Brings Together Clinicians, Scientists, Families and Industry Representatives in the Field of Angelman Syndrome Research and Therapeutic Development**

**Cambridge, MA**, July 20, 2017 -- Agilis Biotherapeutics, Inc. (Agilis), a biotechnology company advancing innovative DNA therapeutics for rare genetic diseases that affect the central nervous system (CNS), announced today that Agilis co-sponsored the 2017 ASF Research Symposium and Family Conference and participated in the 6-day event combining clinicians, scientists, families, and industry representatives in Phoenix, AZ. Jodi A. Cook, Ph.D., Chief Operating Officer of Agilis, and Edwin Weeber, Ph.D., Professor, Molecular Pharmacology and Physiology at University of South Florida, presented a talk entitled "AAV Mediated Strategies for the Treatment of Angelman syndrome", during the research symposium.

Dr. Weeber, Agilis' primary collaborator on Angelman syndrome said, "We continue to build an enabling data set in support of the use of gene therapy for the treatment of Angelman syndrome. Our on-going relationship with Agilis has progressed this science greatly as we continue to work toward a potential treatment for these families."

Agilis also supported the Angelman Biomarker and Outcome Measures (A-BOM) meeting, being instrumental in its development 18 months ago. Dr. Cook said, "As a patient-focused, clinical-stage Company, Agilis believes strongly that collaboration with clinicians, scientists, and families in the Angelman syndrome community will be instrumental to achieving our shared goal of developing new therapeutic approaches to Angelman syndrome, including the possibility of using gene therapy as a durable treatment option."

### **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, Inc.**

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. Agilis' gene therapies are engineered to impart sustainable clinical benefits by inducing persistent expression of a therapeutic gene through precise targeting and restoration of lost gene function to achieve long-term efficacy. Agilis' rare disease programs are focused on gene therapy for AADC deficiency, Friedreich's ataxia, and Angelman syndrome, all rare genetic diseases that include neurological deficits and result in physically debilitating conditions.

We invite you to visit our website at [www.agilisbio.com](http://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**

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