



## **Agilis Biotherapeutics Presents at World Orphan Drug Congress**

**Cambridge, MA**, April 24, 2017 -- Agilis Biotherapeutics (Agilis), a biotechnology company advancing innovative DNA therapeutics for rare genetic diseases that affect the central nervous system (CNS), announced today that Agilis presented in a Pitch and Partner session of the World Orphan Drug Congress 2017 in Washington D.C. Dr. Jodi Cook, Agilis' Chief Operating Officer presented, a talk entitled "Gene Therapy for CNS disorders: a promising treatment for AADC deficiency" on April 22, 2017. The session covered a range of topics in therapeutics development. Dr. Cook presented Agilis' recent progress in its clinical data analysis of the Company's lead clinical program in AADC Deficiency.

"The conference highlights the remarkable progress being realized in treating orphan disorders," said Dr. Cook. "We are pleased that Agilis was able participate this event. The number of presentations on gene therapy demonstrated the growing capabilities of this novel intervention strategy in bringing new treatment solutions to patients across a range of disorders. Agilis' progress in advancing its gene therapy programs positions the Company at the forefront of this exciting field, which holds great promise for a spectrum of serious disorders where few treatment options exist."

World Orphan Drug Congress USA brings together more than 150 elite speakers to educate and inspire its 1,000 attendees from big pharma, biotech, payers, patient groups, academics and government bodies. The congress focuses on building a vibrant community of leaders to help to drive innovation in the treatment of orphan indications.

### **About AADC Deficiency**

Aromatic L-amino acid decarboxylase (AADC) deficiency is a rare genetic condition resulting from deficits in the enzyme, AADC, which is responsible for the final step in the synthesis of the neurotransmitters dopamine (a precursor of norepinephrine and epinephrine) and serotonin (a precursor of melatonin).

AADC deficiency arises from mutations in the dopa decarboxylase (DDC) gene. In its profound forms, AADC deficiency results in severe developmental failures, global muscular hypotonia and dystonia, severe, seizure-like episodes known as oculo-gyric crises, frequent hospitalizations (including prolonged stays in intensive care), and the need for life-long care. Symptoms and severity vary depending on the type of underlying genetic mutation, which abrogates AADC enzyme function. Patients with severe forms usually die before the age of 7 years due to profound motor dysfunction, autonomic abnormalities, and secondary complications such as choking, hypoxia, and pneumonia. No treatment options other than palliative care currently exist for patients with severe AADC deficiency.

#### **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**

**Agilis Biotherapeutics, Inc.**

Dr. Jodi Cook  
Chief Operating Officer  
Email: [jcook@agilisbio.com](mailto:jcook@agilisbio.com)  
Phone: 510-673-7809