Agilis Biotherapeutics Announces Presentation of Five Year Follow-Up Data of Gene Therapy Treatment for AADC Deficiency

Long-term Results Demonstrate Encouraging Durability for Novel CNS Gene Therapy

Cambridge, MA, May 11, 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 data from the Company's gene therapy, AGIL-AADC, for the treatment of Aromatic L-amino acid decarboxylase (AADC) deficiency are being presented at the Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) in Washington, D.C. The oral presentation "Gene Therapy for Aromatic L-amino Acid Decarboxylase Deficiency: 5 Years After AAV2-hAADC Transduction" will be presented by Paul Hwu, M.D., Ph.D., Professor of Pediatrics at National Taiwan University Hospital, and study principal investigator. The presentation is at 4:45PM during the session entitled Clinical Trials for Neurologic and Neurosensory Disorders.

Dr. Hwu and colleagues have treated 18 patients with severe AADC deficiency in two prospective clinical cohorts using a single administration of the gene therapy, AGIL-AADC, an adeno-associated virus (AAV) vector containing the human gene for the AADC enzyme. The presentation at the ASGCT conference presents, for the first time, multi-year data on the first cohort of treated patients demonstrating sustained motor function improvement and associated biomarker evidence of effect, representing one of the longest-term follow-up periods to date in gene therapy for a rare disease of the central nervous system.

Dr. Hwu stated, "We are pleased to have been selected to present our long-term follow-up data at the ASGCT meeting. Over the period of observation, we have seen important treatment benefits as well as a good safety and tolerability profile to date. AADC deficiency patients treated with AGIL-AADC have

exhibited improvements across multiple functional scales, developmental milestones, biomarkers and imaging measures."

Data to date indicate that treated subjects have exhibited substantial gains in motor function over multiple years following the gene therapy. Specifically, significant improvements have been observed in two established measures of motor function, the Peabody Development Motor Scale, Second Edition (PDMS-2) and the Alberta Infant Motor Scale (AIMS) and in the achievement of milestones of motor development. Patients have also shown *de novo* dopamine production as visualized by F-DOPA PET imaging and the emergence of dopamine metabolites. In contrast, untreated subjects typically do not achieve critical developmental milestones, as observed in natural history cases. The University of Florida Powell Gene Therapy Center contributed manufacturing and toxicology work of the initial product.

Kirsten Gruis, M.D., Agilis' Chief Medical Officer, said, "The emerging clinical data indicating motor function improvements over time following a single administration of the AADC gene therapy are encouraging. These data reinforce the premise that gene therapy may be able to provide durable benefits to patients with debilitating disorders that affect the central nervous system."

"We are delighted to have partnered with Dr. Hwu on this pioneering gene therapy effort in this devastating disease," said Mark Pykett, President and CEO of Agilis. "Ongoing development of AGIL-AADC remains promising, as we strive to position AGIL-AADC for registration and commercialization to potentially bring this important, innovative therapy to patients who currently lack treatment options."

About AADC Deficiency

Aromatic L-amino acid decarboxylase (AADC) deficiency is a rare genetic condition resulting in lack of functioning AADC enzyme responsible for the final step in the synthesis of key neurotransmitters dopamine (a precursor of norepinephrine and epinephrine) and serotonin (a precursor of melatonin). AADC deficiency results in developmental failure, global muscular hypotonia, severe, seizure-like episodes known as oculogyric crises, autonomic abnormalities, and the need for life-long care. Given this neurologically devastating illness, patients with severe AADC deficiency have a high risk for death

during childhood. Treatment options are limited and there are no approved therapies for patients with 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

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
Phone: 510-673-7809