Agilis Biotherapeutics Presents Platform Paper on AADC Deficiency Gene Therapy at American Academy of Neurology Annual Meeting

Long-Term Follow-Up of Children Treated with Investigational Gene Therapy Shows Sustained Motor Improvements

**Lynnfield, MA, April 24, 2018** -- Agilis Biotherapeutics, Inc. (Agilis), a biotechnology company advancing innovative DNA therapeutics for rare genetic diseases that affect the central nervous system (CNS), announced that Agilis Chief Medical Officer, Kirsten Gruis, M.D., will present a platform paper entitled “Gene Therapy in Children with AADC Deficiency with AGIL-AADC Leads to De Novo Dopamine Production and Sustained Improvement in Motor Milestones over 5 Years,” at the American Academy of Neurology Annual Meeting in Los Angeles, CA. The presentation will be given on April 25, 2018, during the Child Neurology and Developmental Neurology session from 1:00-3:00 PM PST.

Eighteen children with severe AADC deficiency ranging in age from 21 months to 8.5 years were treated with the Company’s AAV gene therapy, designated AGIL-AADC, using a one-time treatment of a low dose of the gene therapy delivered by an established stereotactic procedure. At time of treatment, none of the children had developed any functional motor movement or achieved any motor development milestones, including head control, self-feeding, or sitting, consistent with the natural history of this devastating disease in which children never develop meaningful motor movement ability.

Eight of the treated children have been followed for at least 5 years, and 18 children for at least 2 years. All children have shown improved motor function as measured on validated, standardized scales. By 2 years and beyond, half of all patients have achieved at least one major motor milestone, approximately 40% sit unassisted allowing children to sit without support and use their hands for independent activities such as being able to feed themselves, and approximately 30% achieve standing with support,
enabling independent activities of locomotor activity with a wheeled walker or learning to walk. No serious adverse events attributable to the AGIL-AADC gene therapy or to the administration procedure were reported.

“We are pleased to have been selected for a platform presentation and are encouraged by the marked improvements in children treated with AGIL-AADC to date. These data highlight the long-term, clinically meaningful responses observed with AGIL-AADC administration thus far, and reinforce the premise that gene therapy may be able to provide durable benefits to patients with debilitating disorders that affect the central nervous system,” said Dr. Gruis.

Paul Hwu, M.D., Ph.D., Professor of Pediatrics at National Taiwan University Hospital, was the study principal investigator. Dr. Hwu and colleagues have treated 25 patients to date with severe AADC deficiency using a single administration of AGIL-AADC, an adeno-associated virus (AAV) vector containing the human gene for the AADC enzyme, the first of whom were treated in 2010. “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.” Pursuant to a successful end-of-phase 2 meeting on available data in the AADC program with the United States Food and Drug Administration (FDA) in 2017, Agilis is preparing a Biologics License Application for AGIL-AADC for submission to the FDA. If approved, AGIL-AADC may be the first gene therapy approved anywhere in the world for a disorder of the central nervous system.

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 for patients with AADC deficiency are limited and there are currently no approved therapies.
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 ataxia and Angelman syndrome, 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