Agilis Biotherapeutics Announces Presentation of Data from Phase I/II Trial for Treatment of AADC Deficiency

Findings to Date in Second Study of Novel CNS Gene Therapy Demonstrate Functional Improvements in Treated Patients

Cambridge, MA, May 10, 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 is being presented at the Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) in Washington, D.C. The poster entitled "A Phase I/II Trial of Gene Therapy for an Inherited Disorder of Monoamine Neurotransmitter Deficiency" will be presented by Dr. Ni-Chung Lee.

The study is a 10-patient, open-label clinical trial performed under the direction of Paul Hwu, M.D., Ph.D., Professor of Pediatrics at National Taiwan University Hospital and study principal investigator. The gene therapy treatment candidate, AGIL-AADC, is an adeno-associated virus (AAV) vector containing the human gene for the AADC enzyme. Dr. Hwu and colleagues have treated 18 patients using a single administration of the AGIL-AADC gene therapy. Results to date in the present study reinforce findings Dr. Hwu and colleagues have observed in the first study of 8 patients previously treated with AGIL-AADC. One-year findings in the Phase I/II study reveal that treated patients exhibited gains in motor function and showed *de novo* dopamine production as visualized by F-DOPA PET imaging and the emergence of dopamine metabolites. In contrast, untreated patients with severe AADC deficiency typically do not gain motor function over time nor achieve critical developmental milestones, as observed in natural history cases. The University of Florida Powell Gene Therapy Center was instrumental in the manufacturing and toxicology work of the initial product.

Dr. Hwu stated, “We are pleased to present this important work highlighting the encouraging safety and efficacy observed to date in which AADC deficiency patients treated with the AGIL-AADC gene therapy have exhibited improvements across multiple functional scales, as well as biomarker and imaging measures.”

Dr. Kirsten Gruis, Chief Medical Officer of Agilis, commented, “Patients with AADC deficiency continue to face high unmet medical needs and devastating consequences of their disorder in the absence of approved treatment options. We are hopeful that continuing clinical and regulatory efforts with AGIL-AADC may provide an innovative intervention to address the profound symptoms of this rare disorder and enhance the quality of patients’ lives.”

“We are encouraged by the progress in advancing the AGIL-AADC gene therapy candidate,” said Mark Pykett, President and CEO of Agilis. “Clinical development of AGIL-AADC to date is rapidly advancing as we strive to position AGIL-AADC for registration and commercialization.”

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.

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