Agilis Biotherapeutics Sponsors and Presents at the AADC Research Trust's Third International Research Conference

Dr. Jodi Cook Presented Details of Agilis' Ongoing Gene Therapy Trials

Cambridge, MA May 19, 2016 - Agilis Biotherapeutics, LLC (Agilis), a biotechnology company advancing innovative gene therapies for rare genetic diseases that affect the central nervous system (CNS), announced today that the Company sponsored and participated in the AADC Research Trust International Research Conference held in Surrey, UK, May 13 and 14, 2016. In January, Agilis entered into an exclusive worldwide license agreement with National Taiwan University (NTU) for the treatment of Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency using gene therapy developed by Dr. Paul Hwu, Professor of Pediatrics at NTU Hospital. AADC Deficiency is a rare CNS disorder caused by mutations in the dopa decarboxylase (DDC) gene that leads to deficiency of the AADC enzyme and defects in the neurotransmitters dopamine, norepinephrine, epinephrine, serotonin and melatonin. In its profound forms, AADC Deficiency causes severe developmental delays, the inability to develop motor strength and control (global muscular hypotonia/dystonia) resulting in breathing, feeding, and swallowing problems, frequent hospitalizations, and the need for extensive, life-long care that ultimately culminates in premature death within the first decade of life.

The AADC Research Trust's Third International Research Conference was attended by families from 15 countries, and researchers and clinicians from 10 countries. The meeting was held in connection with the 10th anniversary of the annual TEDeBEAR fund raising gala ball.

Dr. Jodi Cook, Agilis Vice President of Operations and Strategic Alliances, provided an overview of the AADC gene therapy program that to date has treated 18 patients in two prospective clinical cohorts. Following a single administration of the gene therapy, treated subjects have exhibited substantial,

durable gains in motor and cognitive function over multiple years, shown *de novo* production of dopamine as visualized by F-DOPA PET imaging, and realized improvements in metabolic biomarkers. In contrast, natural history cases routinely fail to achieve developmental milestones and show continued clinical deterioration as the disease course progresses. In collaboration with NTU, the Company is now preparing for a Phase IIb clinical study.

In preparing the program for clinical trials, The University of Florida Powell Gene Therapy Center was instrumental in the development of the initial product manufacturing and toxicology work. "Dr. Hwu's pioneering studies in the treatment of AADC Deficiency using gene therapy have established a clinically relevant approach to treat this devastating disease," said Barry Byrne, MD, PhD, Director of University of Florida Powell Gene Therapy Center and Professor of Pediatrics. "The University of Florida is pleased to be contributing to the advancement of this much-needed therapy."

"To our knowledge, these studies represent the most advanced CNS gene therapy program in the world, with available long-term follow-up data over five years in some subjects. Agilis is excited to expand this program to patients in other parts of the world and will begin treating additional subjects with a higher dose later this year," said Dr. Cook.

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, long-lasting 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. Severe forms of the disease can arise from specific DNA mutations. Patients with severe forms usually die before the age of seven years due to extreme 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

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. Our therapies are engineered to impart sustainable clinical benefits, and potentially a functional cure, by inducing persistent expression of a therapeutic gene. The Company's technology is aimed at the precise targeting and restoration of a lost gene function, while avoiding unintended off-target effects. Our integrated strategy increases the efficiency of developing DNA therapeutics into safe, targeted gene therapies that achieve long-term efficacy and enable patients to remain asymptomatic without continuous invasive treatment. Agilis' rare disease programs are focused on gene therapy for AADC Deficiency, Friedreich's ataxia, Angelman syndrome, and Fragile X syndrome, rare genetic diseases that include severe 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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