Agilis Biotherapeutics, First Gene Therapy Company Selected for NIH Therapeutics for Rare and Neglected Diseases Program

Collaboration with the National Center for Accelerating Translational Sciences Focuses on Advancing Agilis' Gene Therapy Treatment for AADC Deficiency

Cambridge, MA, June 27, 2016--Agilis Biotherapeutics, LLC (Agilis), a biotechnology company advancing innovative DNA therapeutics for rare genetic diseases that affect the central nervous system (CNS), announced today that the Company has been selected by the National Center for Accelerating Translational Sciences (NCATS) as an awardee of a Cooperative Research and Development Agreement (CRADA) under the National Institutes of Health's (NIH) Therapeutics for Rare and Neglected Diseases (TRND) program. NCATS and the Company have signed a letter of intent for the CRADA to facilitate development activities in support of registration of Agilis' gene-therapy candidate for the treatment of Aromatic L-Amino Acid Decarboxylase (AADC) deficiency, a rare-genetic disease that can result in profound developmental failure, frequent hospitalizations, life-long care, and premature death.

The mission of the TRND program is to use NIH intramural resources to encourage and speed the development of new treatments for diseases with unmet medical need. As the first gene therapy company selected for this collaborative program, the partnership merges Agilis' expertise with the NIH's scientific team, development experience, and overall intramural resources to expedite this much needed treatment for patients with AADC deficiency. The partnership could potentially be worth up to several million dollars to Agilis in in-kind services and activities from NIH.

Agilis' AADC deficiency gene therapy program was licensed from National Taiwan University (NTU) based on breakthrough work performed by Dr. Paul Hwu at NTU Hospital, where 18 patients have been treated. Following a single administration of the gene therapy, treated

patients have shown substantial, durable gains in motor and cognitive function over multiple years, exhibited *de novo* production of dopamine as visualized by F-DOPA PET imaging, and realized improvements in metabolic biomarkers. 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. The Agilis-TRND partnership will conduct toxicology, process development, and manufacturing work necessary for overall development of this therapeutic, and registration in the US and abroad.

"We are honored to be the first gene therapy company to receive this significant CRADA which combines the extraordinary expertise of the NIH with Agilis' commitment to solving this rare, devastating pediatric condition," said Mark Pykett, President and CEO of Agilis. "The competitive application and review process for TRND was rigorous, requiring multiple expert evaluations. We are pleased to have been selected from among numerous, strong applications and look forward to working with NCATS and the NIH to develop this important therapeutic and further advance our mission to improve the lives of children with no other options."

"TRND has established productive partnerships with companies and researchers to identify the most promising therapeutics and to advance its mission in developing new treatments for rare and neglected diseases," said Nora Yang, Ph.D. Director of TRND. "It is important for society that TRND help accelerate and de-risk promising treatments for those diseases that are often overlooked and continue to have significant unmet medical needs."

Applicants to the TRND program go through a competitive process to be selected. TRND scientists select the most promising proposals for the prestigious public-private partnership.

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 seizures 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 six-seven 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, LLC

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

For more information, contact:

NIH/NCATS/TRND

https://ncats.nih.gov

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