Agilis Biotherapeutics Presents at International Rare Diseases Research Consortium Meeting

Recognition as an Invited Presentation Highlights Company's Growing Position in Gene Therapy and Rare Diseases

Cambridge, MA, February 17, 2017--Agilis Biotherapeutics (Agilis), a biotechnology company advancing innovative DNA therapeutics for rare genetic diseases that affect the central nervous system (CNS), announced today that the Company was invited to present at the prestigious Third International Rare Diseases Research Consortium (IRDiRC) meeting in Paris, France February 8-9, 2017. Dr. Jodi Cook, Agilis Chief Operating Officer, presented "Gene Therapy for Neurological Disorders: A Promising Novel Treatment for AADC Deficiency," as part of the Therapeutics Research Track on February 8.

IRDiRC is an invitation-only organization that teams up researchers and organizations investing in rare diseases. Members are from government, academia, industry and patient organizations that work on a global scale to promote organizational goals. Having met the IRDiRC's original goals several years early, the purpose of the 2017 meeting was to share global research successes in rare diseases in the areas of diagnostics and therapeutics and establish a new set of overarching goals.

"We were honored to have been invited and given the opportunity to present Agilis' novel approach to gene therapy programs for rare diseases to a truly global and innovative consortium, said Jodi Cook, PhD. "We were thrilled to share our promising developments in the treatment of AADC-Deficiency, an ultra-rare debilitating, often life-limiting disease, with this global community. Our aim is to improve the lives of children and families all over the world

who suffer with this condition."

Agilis' clinical program for gene therapy for AADC Deficiency has treated 21 subjects in three prospective cohorts using a single administration of the gene therapy candidate, AGIL-AADC. Some of these subjects have been followed for more than five years with evidence of substantial gains in motor and cognitive function over multiple years, as well as *de novo* dopamine production visualized by F-DOPA PET imaging and the emergence of dopamine metabolite biomarkers. In contrast, untreated subjects typically do not achieve critical developmental milestones, as observed in natural history cases.

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, seizure-like 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. Patients with severe forms usually die before the age of 7 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.

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. 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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