Agilis Biotherapeutics Launches Awareness Campaign for AADC Deficiency at Society for Inherited Metabolic Disorders Annual Meeting

Meeting focuses on bringing awareness to inborn errors of metabolism

Lynnfield, MA, March 22, 2018 -- 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 its disease awareness campaign for AADC deficiency was initiated at the 40th Annual Meeting of the Society for Inherited Metabolic Disorders (SIMD), March 11-14, 2018, in San Diego, CA. Agilis is developing innovative gene therapies for patients with CNS diseases for which few, if any, treatment options exist. Agilis’ lead program (AGIL-AADC) is in AADC deficiency. AADC deficiency is a rare disease with a devastating clinical course arising from mutation in the DDC gene encoding the AADC enzyme responsible for the synthesis of dopamine and other neurotransmitters in the brain. Symptoms of AADC deficiency include the absence of motor milestone development, seizure-like events (termed oculogyric crisis) and hypotonia often described as “floppiness”. Because of the nature of symptoms, the disease can be confused with other, better known disorders such as cerebral palsy, making diagnosis difficult and often leading to a protracted diagnostic odyssey in correctly identifying the disease.

The SIMD is a research- and practice-oriented conference that raises understanding of diseases of inborn errors of metabolism and facilitates communication between physicians and researchers. Attendees include physicians, geneticists, researchers, genetic counselors, nurses and nutritionists, and the conference includes sessions on politics, economics, newborn screening, genomics, genomic therapies, and dietary interventions. Agilis representatives shared information on AADC deficiency and provided participants with publications about the disease state, potential areas for mis-diagnosis, and screening and diagnostic tests.
“We are proud to participate in this conference and demonstrate our Company’s dedication to disease education so that patients with AADC deficiency can get properly diagnosed at as early an age as possible. As we advance our mission to develop treatments for rare diseases of the CNS, we will continue to actively engage the physician community in discussing the needs of patients with these devastating illnesses,” commented Dr. Markus Peters, Agilis Chief Commercial Officer.

Based on a successful end-of-phase II meeting with the FDA, Agilis is preparing a Biologics License Application (BLA), with a target submission by year-end 2018.

**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. The Company is preparing a BLA for its AADC deficiency investigational gene therapy and anticipates approval in 2019.

We invite you to visit our website at [www.agilisbio.com](http://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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