



**Agilis Biotherapeutics Updates Progress of its CNS Gene Therapy for  
AADC Deficiency  
BLA Preparation Underway Following Meeting with FDA**

**Cambridge, MA**, September 6, 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 following a recent end-of-phase-II meeting with the U.S. Food and Drug Administration (FDA) covering the clinical, non-clinical and manufacturing data available to date, Agilis will begin preparing a biologics licensing application (BLA) for its gene therapy program for the treatment of Aromatic L-amino acid decarboxylase (AADC) deficiency, AGIL-AADC, targeted for submission in 2018.

“We are pleased that the FDA agreed with our assessment of the potential clinical benefit of AGIL-AADC as observed in treated patients to date, and with the plan to submit a BLA for review,” said Mark Pykett, President and CEO of Agilis.

Twenty-three patients have received AGIL-AADC to date, likely representing the largest cohort of patients in any CNS gene therapy program. Clinical data have been analyzed and presented on patients with severe AADC deficiency from two clinical studies in which each patient received a single administration of the AGIL-AADC gene therapy, an adeno-associated virus (AAV) vector containing the human gene for the AADC enzyme. Data on the first cohort of patients demonstrating sustained improvement in motor function over a 5-year period post-treatment were also presented. The Company has recently completed enrollment of a Phase IIb clinical study for AGIL-AADC which may be extended to permit the treatment of additional patients in need of therapeutic intervention.

“Over the period of observation, we have seen important gains in motor function and a good safety and tolerability profile to date in treated children. Some of the children can maintain head position, sit unassisted, and stand with assistance, milestones these children would never have achieved without treatment,” said Paul Hwu, M.D., Ph.D., Professor of Pediatrics at National Taiwan University Hospital, and study principal investigator.

AGIL-AADC has previously received Orphan Drug Designation and Rare Pediatric Disease (RPD) designation in the U.S., as well as Orphan Medicinal Product status in Europe. The U.S. Orphan designation allows Agilis to leverage the FDA’s Priority Review pathway, hastening patient access to AGIL-AADC by shortening the review of the marketing application by as much as four months. The complementary RPD designation gives Agilis the ability to qualify for a Priority Review Voucher (PRV) upon approval of AGIL-AADC. PRV’s can be redeemed or transferred to a third party to receive Priority Review on a subsequent marketing application for a different product. This voucher will be requested at the time of AGIL-AADC’s marketing application and awarded upon approval of AGIL-AADC.

Agilis’ gene therapy program has been developed in collaboration with National Taiwan University and the U.S. National Institutes of Health’s National Center for Advancing Translational Sciences (NCATS) through a Cooperative Research and Development Agreement (CRADA). NCATS is supporting this work through its [Therapeutics for Rare and Neglected Diseases program](#) (TRND). The University of Florida Powell Gene Therapy Center contributed manufacturing and toxicology work on the initial product.

“An important mission in the TRND program is to help accelerate development of promising treatments for those diseases that are often overlooked, underfunded and continue to have significant unmet medical needs,” said Nora Yang, Ph.D., Director of TRND Portfolio Management and Program Operations.

AADC Deficiency is a rare disease with a devastating clinical course. Symptoms include absence of motor milestone development, seizure-like events (termed oculogyric crisis) and hypotonia described by many 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. “Unfortunately, many of our families wait several years for a diagnosis,” said Lisa Flint, AADC Research Trust Founder and Managing Director. “We are thankful that Agilis has worked closely with the AADC community to bring forward a potential major treatment for our kids.”

### **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’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](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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