Agilis Biotherapeutics and T-TOP Clinical Research Enter into an Agreement for the Development of Gene Therapy Treatment of AADC Deficiency

Companies to collaborate on Clinical Development for Rare CNS Disease

Cambridge, MA, March 22, 2016 7:30 am EST -- (BUSINESS WIRE)—Agilis Biotherapeutics, LLC (Agilis), a biotechnology company advancing innovative gene therapies for rare genetic diseases that affect the central nervous system (CNS), and T-TOP Clinical Research Co, Ltd. (T-TOP), today announced that the companies have entered into an exclusive agreement to advance the clinical development of Agilis' innovative treatment of Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency using gene therapy. In January, Agilis announced an exclusive, worldwide license with National Taiwan University (NTU) for AADC gene therapy developed by Dr. Paul Hwu, Professor of Pediatrics at NTU Children's Hospital. The AADC gene therapy program is, to the Company's knowledge, the most advanced CNS gene therapy program in the world, with the most patients treated to date and follow-up data extending for more than five years in some cases.

Under the agreement, T-TOP will provide clinical, database, regulatory and strategic management services for prior and current AADC clinical studies. In collaboration with National Taiwan University, Agilis and T-TOP will advance current clinical evaluations and a Phase IIb study to build upon the clinical data developed to date. Agilis is evaluating potential early-opportunity registration pathways for the AADC gene therapy in territories around the world, potentially positioning it to be the first CNS gene therapy to seek registration.

"We are excited to embark on the next stage of development for the AADC gene therapy program in collaboration with Dr. Hwu and T-TOP to bring this potentially impactful treatment to patients with this devastating disease," stated Christopher Silber, M.D., Agilis Chief Medical Officer. "Dr. Hwu's pioneering

studies in the treatment of AADC Deficiency using gene therapy have shown encouraging progress in treating this disorder, and represent a seminal step for CNS gene therapy. Given the devastating clinical course of disease for patients with severe AADC deficiency and refractoriness to standard therapy, a gene therapy treatment that could improve the most severe forms of this disorder represents a critical advance for these patients and their families. Having a premier, local partner in Taiwan for ongoing clinical development in the AADC gene therapy program provides enabling capabilities as we seek to position this program for registration."

Mr. Minhsuing Kao, Chairman of T-TOP, noted, "We are pleased to collaborate with Agilis to ensure high quality and rigor in the clinical development of AADC gene therapy, provide expertise in the clinical trial and data management activities required for successful development, and help shepherd the program through the next stages of development toward approval. T-TOP has worked with Dr. Hwu on the AADC program for some time and is familiar with the full requirements of the study protocols for this exciting, innovative potential therapeutic. We look forward to working with Agilis, Dr. Hwu and NTU in bringing AADC gene therapy to patients in need."

In its profound forms, AADC Deficiency causes severe developmental delays, extreme muscle strength and control deficits, and frequent hospitalizations often culminating in premature death within the first decade of life. Dr. Hwu's team has successfully treated 18 subjects in two prospective clinical treatment groups with a single administration of the AADC gene therapy. Treated subjects exhibited substantial gains in motor function and cognitive scales over multiple years following the single gene therapy treatment, as well as evidence of de novo dopamine production by F-DOPA PET imaging and the emergence of dopamine metabolites. In contrast, untreated patients routinely showed continued deterioration as the disease progresses, as observed in natural history cases.

Dr. Hwu commented, "Clinical studies continue to support strong safety and promising efficacy to date, with patients showing persistent improvements in functional, biomarker and imaging measures. Collectively, the data from our studies represent the first time gene therapy has been demonstrated as a potential durable treatment for AADC deficiency, and we are encouraged to advance development and bring this important therapy to patients."

Dr. Mark Pykett, Agilis President and CEO, stated, "The AADC gene therapy program continues to be an important catalyst for Agilis and a landmark in the development of gene therapy treatments for CNS disorders. Given our commitment to this program and our focus on its successful development, we believe it has the potential to be the first CNS gene therapy product approved anywhere in the world. We have been impressed with T-TOP's clinical-regulatory development and their insights and experience in the AADC gene therapy program, as well as the tremendous quality of clinical research being performed in Taiwan. We are thrilled that we can enhance our development efforts with people and organizations as skilled and committed as T-TOP, Dr. Hwu and NTU."

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 6-7 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.

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 technologies are 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 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.

About T-TOP Clinical Research

T-TOP is cooperating with biotech companies to further develop innovative products with its clinical research capability. With professional clinical investigators, ICH-harmonized GCP environment and clinical operation staffs in Taiwan, the new innovative products are successfully translated into clinical deliverables. T-TOP had successfully translated new in vitro diagnostic medical devices, new synbiotics, new chemotherapy adjuvant, new drug indication, etc. into clinical deliverables. The full ability on product evaluation, protocol development, regulatory planning, study conduction, remote data capture, data management, biostatistics, and clinical report writing of T-TOP had accelerated clinical development of innovative products. The clinical report had been submitted to US FDA for license application. For more company information, we invite you to visit the website at www.ttopcro.com.tw.

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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