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AGTC Receives Second Grant from Food and Drug Administration for Clinical Study of Genetic Respiratory Disease

Ongoing Phase II Trial tests ability to deliver sustainable treatment in patients with Alpha 1 Antitrypsin Deficiency (AAT) using proprietary AAV technology.

September 16, 2010 - Applied Genetic Technologies Corporation, a privately-held, clinical stage biotechnology company developing novel systems to deliver human therapeutics, announces that it has received a grant of $1 million from the Food and Drug Administration (FDA Orphan Drugs Program) to fund a Phase II Human Clinical Trial evaluating the safety and efficacy of a treatment for Alpha 1 Antitrypsin Deficiency (AAT), a genetic disease known to result in serious lung and/or liver disease. The clinical trial is being coordinated by AGTC at The University of Massachusetts by Dr. Terrence R. Flotte and Cincinnati Children's Hospital Medical Center by Dr. Bruce C. Trapnell.

"We are thrilled that the FDA and its grant reviewers continue to recognize AGTC's expertise in clinical development of treatments for rare genetic diseases with this, our second grant award this year," said Sue Washer, President and CEO of AGTC. "This funding will enable us to complete enrollment of the Phase 2 trial that is designed to deliver recombinant adeno-associated virus (rAAV) vectors at higher doses than in previous clinical studies. The ultimate goal is to improve these patients' quality of life as current treatments are costly and in short supply."

"The tremendous potential of recombinant AAV vector technology to impact human disease symptoms has recently been demonstrated in a number of early phase clinical trials, particularly in the retina and brain," said Terence R. Flotte, MD, dean of the School of Medicine and provost & executive deputy chancellor of the University of Massachusetts Medical School. "We are excited at the opportunity to determine whether this technology can safely and effectively be brought to bear on genetic emphysema due to alpha-1 antitrypsin deficiency, a relatively common but much under-recognized disease."

AAT is an inherited, genetic condition characterized by reduced levels of a required protein leading to increased risk of developing emphysema and liver disease. It affects approximately 100,000 people in the US. The current therapy, requiring weekly intravenous infusions, is in short supply and not all patients are able to receive treatment. AAT is a lifelong chronic disease that decreases lung function, causing 2.7% of deaths due to obstructive pulmonary disease among persons in the 35- to 44-year-old age group, and death due to lung disease in 72% of Alpha-1 patients. Pre-clinical studies in animals support the safety and efficacy of the treatment and the Phase I clinical trial demonstrated safety and sustained expression of the AAT protein.