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

Ongoing Phase II Trial tests ability to deliver sustainable treatment in patients with Leber congenital amaurosis (LCA) using proprietary AAV technology.

September 13, 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). The grant will fund a Phase II Human Clinical Trial evaluating the safety and efficacy of a treatment for Leber congenital amaurosis (LCA), a genetic retinal disease known to cause blindness at an early age. The clinical trial, coordinated by AGTC, is being conducted at The University of Massachusetts Medical School by Dr. Shalesh Kaushal and Oregon Health & Science University by Dr. Tim Stout.

“We are pleased that the FDA and its grant reviewers recognized AGTC’s expertise in clinical development of treatments for genetic retinal disease,” 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 to larger areas of the retina than in clinical studies performed to date. The ultimate goal of this research is to improve the sight and quality of life of patients affected by this disorder. We continue to be encouraged by the data from this and other trials supporting the ability of rAAV to provide sustained delivery and expression of therapeutic levels of many different biologics in the eye.”

"I am excited about the opportunity to offer my patients with LCA the opportunity to participate in this clinical trial.” said Tim Stout, M.D., vice president and professor of ophthalmology, Casey Eye Institute, OHSU School of Medicine. “The orphan program is an important mechanism that enables clinical development of these kinds of treatments."

LCA is an inherited form of retinal degeneration that usually presents as blindness or severely impaired vision at birth or during the first few months of life. Among the 3,000 patients with LCA in the United States, approximately 8 to 10% are caused by mutations in a single protein called RPE65. These patients have profound impairment of vision, but with the relatively preserved cellular structure necessary for successful gene therapy treatment. No treatment for LCA is currently available, but subretinal delivery of recombinant adeno-associated virus (rAAV) vectors expressing RPE65 has demonstrated substantial restoration of visual function in mouse and dog models of RPE65-associated blindness, and initial clinical trials in small numbers of patients have been encouraging.

Seattle Times article