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AGTC Receives Grant from the Foundation Fighting Blindness for Pre-Clinical Study of Gene Therapy for Eye Disease

Pre-clinical trial in animal models tests ability to deliver sustainable treatment in patients with X-Linked Retinoschisis (XLRS) using proprietary AAV gene delivery technology.

September 21, 2011 - 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 $1.5 million from the Foundation Fighting Blindness to fund a pre-clinical trial evaluating the safety and efficacy of a gene therapy treatment for X-linked Retinoschisis (XLRS), a genetic eye disease affecting over 35,000 patients in the US and Europe. The work is being coordinated by AGTC in collaboration with researchers at Oregon Health & Science University Casey Eye Institute Dr. David Wilson and The University of Florida, Dr. William Hauswirth. The grant is part of the Foundation Fighting Blindness' effort to fund research projects with strong potential to enter into human clinical trials within three years.

"Translating the advances in genetics of eye disease to potentially curative treatments provides hope to the patients and families affected by these conditions," says Dr. David Wilson, Director, Casey Eye Institute. "We are delighted to be partnering with AGTC and FFB in what is the most exciting and meaningful work being done in ophthalmology and medicine at this time."

"AGTC's emerging gene therapy is an elegant approach to treating a devastating retinal disease like XLRS, because one treatment may last several years or even a lifetime," says Stephen Rose, Ph.D., chief research officer, Foundation Fighting Blindness. "Furthermore, AGTC has the production resources, clinical development knowledge, and regulatory expertise to move this promising approach through clinical trials once proof of principle is established in the laboratory."

"We are thrilled that the FFB and its grant reviewers recognize AGTC's expertise in development of treatments for rare genetic diseases and our ongoing commitment to ophthalmology patients," said Sue Washer, President and CEO of AGTC. "This funding will enable us to finish final testing of the treatment in animal models of XLRS as well as complete the pre-clinical safety studies required by the FDA in order to start a Phase 1 human clinical trial. The ultimate goal is to improve these patients' quality of life; there is currently no treatment available for the thousands of young boys losing their sight due to XLRS."

XLRS, an inherited genetic condition, is a leading cause of juvenile macular degeneration in males. It is caused by mutations in a gene, called RS1, which results in splitting of the layers of the central retina. Patients typically begin to experience progressive loss of vision between the ages of 5 and 10, and other early symptoms include the inability to focus both eyes and roving, involuntary eye movements. No treatment for XLRS is currently available. AGTC's potential treatment uses an adeno-associated virus (AAV) — a safe, manmade virus — that delivers healthy copies of the XLRS gene to the cells of the retina, replacing the defective copies of the gene. A single treatment can halt the disease for several years, perhaps a lifetime. The AAV delivery system is successfully being used in clinical trials of Leber congenital amaurosis gene therapy that have restored vision in more than 40 adults and children who were virtually blind.