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AGTC and National Neurovision Research Institute Collaborate, Funding Research in two Genetic Retinal Diseases

Combined Funds and Resources to be used to further demonstrate the AGTC's AAV system's ability to deliver sustainable treatments for human retinal diseases.

June 29, 2009 - Applied Genetic Technologies Corporation, (AGTC), a privately-held, clinical stage biotechnology company developing novel systems to deliver human therapeutics, announces that AGTC has entered into an agreement with the National Neurovision Research Institute (NNRI), the clinical trial support organization for the Foundation Fighting Blindness (FFB), to collaborate in experiments using the AAV delivery system in the treatment of two genetic retinal diseases known to cause blindness at an early age. The research will be coordinated by AGTC and will be conducted at The University of Florida, Oregon Health & Science University, The University of Pennsylvania, and The University of British Columbia.

The collaboration will focus on development of treatments for two of the more common genetic retinal diseases that cause blindness at a very early age: X-Linked Retinoschisis (XLRS) and Achromatopsia. "We are delighted to expand our strong relationship with the FFB and the NNRI through this collaboration" said Sue Washer, President and CEO of AGTC. "We continue to be encouraged by the data supporting the AAV vector system's ability to provide sustained delivery and expression of therapeutic levels of many different biologics in the eye with minimal observable toxicity to date in either animal or human testing. There are hundreds of thousands of patients suffering from retinal diseases who currently have no treatment options and this research collaboration is another step towards using the AAV delivery system to address this unmet need and improve the patients' quality of life."

"This collaboration is a tremendous boost for the development of gene therapy products for retinal degenerative diseases and NNRI's partnership with AGTC accelerates these emerging treatments into and through the clinical trial process" said Stephen Rose, Ph.D., Chief Research Officer, Foundation Fighting Blindness. "It affirms the great potential for science guided foundations and academic researchers to work in partnership with commercial firms like AGTC that have the commitment and experience to bring these promising treatments directly to patients.

XLRS is an inherited form of retinal degeneration affecting young boys. Patients present with poor vision either in infancy or at school age. Visual acuity usually worsens during the teenage years and then stabilizes until complicated by vitreous hemorrhage or retinal detachment during adulthood. There is no treatment available for the retinal degeneration in XLRS, which affects approximately 34,000 patients in the US and Europe. Previous research has shown promising signs of efficacy in mouse models and this collaboration will explore safety and efficacy in primates.

Achromatopsia is an inherited condition that presents at birth with impaired visual acuity, lack of color discrimination and extreme light sensitivity resulting in daytime blindness. There is no specific treatment for Achromatopsia, although deep red tinted spectacles or contact lenses can reduce symptoms of light sensitivity. Approximately 22,000 patients in the US and Europe suffer from this disease. Previous research has shown promising signs of efficacy in dog models and this collaboration will enable expanded safety and efficacy studies.