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

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.

About [AGTC](#): AGTC is focused on the research and development of novel therapeutics for patients with unmet medical needs utilizing AGTC's proprietary, non-pathogenic adeno-associated virus (AAV) delivery system. AGTC has demonstrated that this system can be used to deliver a normal form of a gene in both animals and humans thus allowing their own body to produce sustained therapeutic levels of important biologics. The Company's most advanced programs in development are treatments for Alpha-1 antitrypsin deficiency, a disease causing a progressive loss of lung function, and Leber's Congenital Amaurosis, an inherited condition causing early blindness. Both utilize AGTC's proprietary AAV system and production methods. AGTC has licensed a significant portion of its intellectual property from the University of Florida where researchers originated this ground-breaking work and has received significant financing from some of the world's leading venture capital firms: InterWest Partners, Intersouth Partners and MedImmune Ventures. For more information see www.agtc.com.

About Foundation Fighting Blindness ([FFB](#)): FFB is the largest source of non-governmental funding for retinal degenerative disease research in the world. The urgent mission of the Foundation Fighting Blindness is to drive the research that will provide preventions, treatments and cures for people affected by retinitis pigmentosa, macular degeneration, Usher syndrome, and the entire spectrum of retinal degenerative diseases. The Foundation has invested over \$140 million to provide seed money for scientific research of diseases of the retina, which cause blindness. Further information is available at www.FightBlindness.org.

About National Neurovision Research Institute ([NNRI](#)): NNRI is a recently-established non-profit support organization of the Foundation Fighting Blindness, the leading non-government funding source for inherited orphan retinal degeneration research. The mission of NNRI is to accelerate the translation of laboratory-based research into clinical trials for treatments and cures of retinal degenerative diseases. It is a medical research institute that obtains support from government agencies, corporations and private foundations. It may also receive royalties or licensing fees from the drug discovery processes and commercialization of new therapies. Further information is available at www.NNRI.info.

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