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Novel Gene-Based Therapy for Achromatopsia Demonstrates Functional Rescue of Cone Cells

Research Led by Scientists From Michigan State University and the University of Pennsylvania Presented at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting

DENVER, May 4, 2015 (GLOBE NEWSWIRE) -- Researchers from Michigan State University and the University of Pennsylvania presented new preclinical data today, evaluating the efficacy of an adeno-associated virus (AAV) vector gene therapy treatment for achromatopsia (ACHM), a rare inherited retinal disease. The investigational gene therapy was developed by Applied Genetic Technologies Corporation (Nasdaq:AGTC), a clinical stage biotechnology company. Study results were presented in an oral session at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting, taking place from May 3 - 7, in Denver.

The abstract (program #2066), titled "Long-term Cone ERG Functional Rescue in CNGB3 Mutant Achromatopsia Dogs by AAV-hCNGB3 Vectors Containing the PR1.7 Promoter and Packaged in AAV5, AAV9 or Mutant AAV2 Capsids," describes a study to evaluate the efficacy of an experimental AAV vector expressing the human cyclic nucleotide gated channel beta 3 (hCNGB3) gene to restore function to cone cells (specialized cells primarily responsible for color vision) in the retinas of dogs affected by ACHM. The study assessed the efficacy of AAV vectors expressing hCNGB3 or codon-optimized hCNGB3 (hCNGB3co) cDNAs, driven by 1.7 kb or 2.1 kb versions of the human red cone opsin promoter (PR1.7 or PR2.1) and packed in AAV5, AAV9 or mutant AAV2 mutant capsids. Results demonstrated that functional rescue of cone cells was observed in nearly 100 percent of eyes treated with AAV5-PR2.1-hCNGB3co (n=3/3), AAV5-PR2.1-hCNGB3 (n=5/5), AAV9-PR1.7-hCNGB3co (n=9/10), AAV2tYF-PR1.7-hCNGB3co (n=10/11), and AAV5-PR1.7-hCNGB3co vector (n=3/3).

"These study results are promising and demonstrate that a novel, AAV-based gene therapy has significant clinical potential in treating achromatopsia," said András Komáromy, DrMedVet, Ph.D., Associate Professor, Michigan State University College of Veterinary Medicine, and co-principal study investigator. "We are encouraged by the possibility of treating serious eye disease with gene therapy innovations. This is an important advancement, especially given the lack of available therapeutic options."

Gustavo Aguirre, VMD, Ph.D., Professor of Medical Genetics and Ophthalmology, University of Pennsylvania School of Veterinary Medicine, and co-principal study investigator added, "We are excited to contribute additional evidence suggesting that investigational gene therapies for treating inherited eye disorders show a high degree of potential. These data highlight the effectiveness of the PR1.7 promoter in restoring cone cell function and support the further development of an AAV-PR1.7-hCNGBco vector for patients with achromatopsia."

ACHM is an inherited retinal disease characterized by complete loss of cone photoreceptor function, and people with ACHM have severe vision loss that renders patients legally blind. ACHM can be caused by mutations in several genes, with mutations in CNGB3 accounting for 50 percent of all cases of ACHM in Western countries. While there is currently no cure for ACHM, AGTC is developing a gene therapy product based on an AAV vector to enable expression of normal CNGB3 protein within cone photoreceptors and restore cone photoreceptor functions in patients. AGTC and its academic collaborators have previously demonstrated that subretinal delivery of gene sequences using an AAV vector restores cone responses in a dog model of ACHM.

"We are grateful to our academic research partners for their study support and guidance and look forward to continuing to advance our pipeline of AAV gene therapy products," said Sue Washer, President and CEO of AGTC. "We remain focused on our goal to provide treatments for serious eye diseases with significant unmet medical need."

About Michigan State University College of Veterinary Medicine

Michigan State University College of Veterinary Medicine, a world-renowned institute of veterinary education, advances knowledge and transforms lives through innovative teaching, research, and outreach. The college is a collaborator in MSU's One Health Initiative, which includes the MSU medical school and the colleges of osteopathic medicine and nursing. With more than 100 years of teaching excellence, CVM continues to prepare leaders, life-savers, and world changers to meet the challenges of Michigan, the nation, and the world. Visit cvm.msu.edu for more information.
About the University of Pennsylvania School of Veterinary Medicine

Penn Vet is a global leader in veterinary medicine education, research, and clinical care. Founded in 1884, Penn Vet is the only veterinary school developed in association with a medical school. The school is a proud member of the One Health Initiative, linking human, animal, and environmental health.

Penn Vet serves a diverse population of animals at its two campuses, which include extensive diagnostic and research laboratories. Ryan Hospital in Philadelphia provides care for dogs, cats, and other domestic/companion animals, handling more than 31,000 patient visits a year. New Bolton Center, Penn Vet’s large-animal hospital on nearly 700 acres in rural Kennett Square, PA, cares for horses and livestock/farm animals. The hospital handles more than 4,000 patient visits a year, while the Field Service treats nearly 36,000 patients at local farms. In addition, New Bolton Center’s campus includes a swine center, working dairy, and poultry unit that provide valuable research for the agriculture industry.

For more information, visit www.vet.upenn.edu.

About AGTC

AGTC is a clinical-stage biotechnology company that uses its proprietary gene therapy platform to develop products designed to transform the lives of patients with severe diseases in ophthalmology. AGTC’s lead product candidates focus on X-linked retinoschisis, achromatopsia and X-linked retinitis pigmentosa, which are inherited orphan diseases of the eye, caused by mutations in single genes that significantly affect visual function and currently lack effective medical treatments. AGTC is also pursuing pre-clinical development of treatments for wet AMD using the company’s experience in ophthalmology to expand into disease indications with larger markets.

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