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AGTC and the Medical College of Wisconsin Announce Publication of Natural History Data Examining Foveal Cone Structure in Patients with CNGB3-associated Achromatopsia

GAINESVILLE, Fla. and CAMBRIDGE, Mass., Oct. 06, 2017 (GLOBE NEWSWIRE) -- Applied Genetic Technologies Corporation (Nasdaq:AGTC), a biotechnology company conducting human clinical trials of adeno-associated virus (AAV)-based gene therapies for the treatment of rare diseases, and the Medical College of Wisconsin (MCW) today announced the publication of data from a longitudinal study that examined measurements of cone photoreceptor structure in patients with CNGB3-associated achromatopsia (ACHM) over time. The peer-reviewed study results appear in the current issue of *RETINA: The Journal of Retinal and Vitreous Diseases.*

"Emerging gene therapies for retinal diseases are designed to enable production of functional copies of proteins in cells that have absent or nonfunctional versions of the protein," said Joseph Carroll, Ph.D., Richard Schultz, MD/Ruth Works Professor in Ophthalmology, professor of ophthalmology & visual sciences, biophysics, and cell biology, neurobiology and anatomy, and director of the Advanced Ocular Imaging Program, MCW. "The success of these therapies requires the presence of cells that can undergo functional correction after the cells produce the protein. Consequently, remnant cone structure could serve as an indicator of the potential for response to therapy. The results of this first longitudinal study of foveal cone density in patients with CNGB3-associated achromatopsia provide important information about cone structure and how it may change over time."

Researchers at MCW used high-resolution imaging tools, including optical coherence tomography (OCT) and adaptive optics scanning light ophthalmoscopy (AOSLO), to evaluate outer nuclear layer (ONL) thickness, ellipsoid zone (EZ) band disruption and peak foveal cone density in 41 patients with CNGB3-associated ACHM over a period of 6—26 months. ONL thickness increased slightly compared to baseline (0.184 µm/month, $p = 0.02$), EZ grade remained unchanged for 34/41 subjects, and peak foveal cone density did not significantly change over time (mean change 1% per 6 months, $p = 0.126$). Study researchers concluded that foveal cone structure showed little or no change in this group of subjects over the time scales investigated, though longer-term follow-up is needed.

"These results will help inform current and planned clinical trials to evaluate AGTC's gene-based therapies for CNGB3-associated achromatopsia," said study co-author Jeffrey D. Chulay, M.D., DTM&H, Executive Director of Clinical Strategy at AGTC. "The study demonstrates that the retinas of patients have substantial numbers of residual cone photoreceptors that remained constant over the time period examined, suggesting that gene-based therapies may benefit a broad segment of patients with CNGB3-associated achromatopsia."

AGTC is currently is currently enrolling patients in clinical trials for achromatopsia caused by mutations in the CNGB3 and CNGA3 genes. Patients and caregivers interested in participating in or learning about AGTC's current and upcoming clinical trials may visit www.agtc.com/patients-and-caregivers or e-mail advocacy@agtc.com.

About Achromatopsia

Achromatopsia is an inherited retinal disease present from birth and characterized by the lack of cone photoreceptor function. The condition results in markedly reduced visual acuity, extreme light sensitivity causing day blindness, and complete loss of color discrimination. Best-corrected visual acuity in persons affected by achromatopsia, even under subdued light conditions, is usually about 20/200, a level at which people are considered legally blind. The incidence rate for achromatopsia is approximately one in 30,000 people, and it is estimated that there are approximately 10,000 people in the United States and 17,000 people in Europe with achromatopsia. Mutations in the CNGB3 or CNGA3 genes together cause about 75% of cases of achromatopsia.

About AGTC

AGTC is a clinical-stage biotechnology company that uses a proprietary gene therapy platform to develop transformational genetic therapies for patients suffering from rare and debilitating diseases. Its initial focus is in the field of ophthalmology, where it has active clinical trials in X-linked retinoschisis (XLRS), X-linked retinitis pigmentosa (XLRP), and achromatopsia (ACHM CNGB3 & ACHM CNGA3). In addition to its clinical trials, AGTC has preclinical programs in optogenetics, adrenoleukodystrophy (ALD), which is a disease of the central nervous system (CNS), and otology. The clinical-stage XLRS

and XLRP programs, the discovery program in ALD and two additional ophthalmology programs are being developed in collaboration with Biogen. In addition to its product pipeline, AGTC has a significant intellectual property portfolio and extensive expertise in the design of gene therapy products including capsids, promoters and expression cassettes, as well as expertise in the formulation, manufacture and physical delivery of gene therapy products.

Forward Looking Statements

This release contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs. Forward-looking statements include information concerning possible or assumed future results of operations, business strategies and operations, preclinical and clinical product development and regulatory progress, potential growth opportunities, potential market opportunities and the effects of competition. Forward-looking statements include all statements that are not historical facts and can be identified by terms such as "anticipates," "believes," "could," "seeks," "estimates," "expects," "intends," "may," "plans," "potential," "predicts," "projects," "should," "will," "would" or similar expressions and the negatives of those terms. Actual results could differ materially from those discussed in the forward-looking statements, due to a number of important factors. Risks and uncertainties that may cause actual results to differ materially include, among others: no gene therapy products have been approved in the United States and only two such products have been approved in Europe; AGTC cannot predict when or if it will obtain regulatory approval to commercialize a product candidate; uncertainty inherent in the regulatory review process; risks and uncertainties associated with drug development and commercialization; factors that could cause actual results to differ materially from those described in the forward-looking statements are set forth under the heading "Risk Factors" in the Company's Annual Report on Form 10-K for the fiscal year ended June 30, 2017, as filed with the SEC. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent management's plans, estimates, assumptions and beliefs only as of the date of this release. Except as required by law, we assume no obligation to update these forward-looking statements publicly or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

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