AGTC Announces First Reported Improvements in Visual Sensitivity for Achromatopsia (ACHM) patients from its Ongoing Clinical Trials

January 27, 2021

-Preliminary results suggest sustained improvements to 12-months in visual sensitivity, as measured by full field static perimetry, supported in some patients by other endpoints-

-Company plans longer term follow-up, dosing of younger pediatric patients, and addition of new brain imaging and color brightness tests-

GAINESVILLE, Fla., and CAMBRIDGE, Mass., Jan. 27, 2021 (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, is today providing an update on its ongoing clinical trials in patients with achromatopsia (ACHM). The Company believes the additional data reported today provides the first reported quantitative evidence of improvements in visual sensitivity, supports the positive patient reported outcomes and provides a path forward to collect additional data to fully realize the potential of this treatment.

"Encouraging data from a careful patient-by-patient analysis of both the ACHMB3 and ACHMA3 trials provide additional support of our ACHM gene therapies," said Sue Washer, President and CEO of AGTC. "We currently plan to focus on completing enrollment of pediatric patients in the two highest dose groups of both trials. In addition, we have amended the study protocol for these trials to allow enrollment of patients as young as 4 years of age and to include both functional magnetic resonance imaging of the brain and improved color brightness tests. We are hopeful that these changes, combined with longer follow-up times, will add to the developing body of evidence and supportive anecdotal patient-reported outcomes."

In January 2020, AGTC provided 3-month ACHM data indicating evidence of biologic activity in the dose escalation portions in both our ACHMB3 and ACHMA3 trials, based on improvements in light discomfort.

In November 2020, the Company provided additional 12- and 6-month data across dose groups in both trials, including one group of pediatric patients. While some patients showed improvements in at least one measure of visual function, no consistent sustained improvements were observed within the dose groups analyzed on a groupwise basis. Anecdotal statements, however, and assessments from patient-reported outcome surveys continued to provide us with confidence that patients were subjectively experiencing improved vision in their treated eye.

AGTC is now reporting preliminary results based on a patient-by-patient analysis of data from both ACHMB3 and ACHMA3 trials. For ACHMB3, this consists of 12month data from 15 patients, 9month data from five patients, 6month data from three patients and 3month data from three patients, for a total of 26 patients across all dose groups. These results reflect a further analysis of the data discussed in November 2020, together with new data that became available in January 2021. Seven of the 16 patients in the three highest dose groups in the ACHMB3 trial showed improvements in visual sensitivity, in the treated area, as measured by static perimetry. No consistent results were seen in the other dose groups. In a subset of these patients with evaluable multi-focal electroretinograms, improvements in electrical signaling were measurable in the same treated area.

For ACHMA3, the new data analysis consists of 12month data from 10 patients, 9month data from four patients, 6month data from one patient and 2 or 3-month data from three patients, for a total of 18 patients across all dose groups. One additional patient did not have evaluable data. In the 16 patients in the four highest dose groups, three patients showed improvements in visual sensitivity, in the treated area, as measured by static perimetry. No consistent results were seen in other dose groups. None of these three patients with improvements in visual sensitivity had evaluable ERGs.

AGTC currently plans to focus on completion of enrollment of pediatric patients in the two highest dose groups in our ACHMB3 and ACHMA3 trials and following all patients through 12 months. The Company has amended the study protocol for these trials to allow enrollment of patients as young as 4 years of age and to include both functional magnetic resonance imaging (fMRI) and improved color brightness tests. AGTC will also work with sites to obtain the best quality multi-focal ERG data possible. The Company is hopeful that these changes, combined with longer follow-up times, will add to the developing body of evidence and supportive anecdotal patient-reported outcomes. The Company expects to report 12month data from the adult patients in both trials in second quarter of calendar year 2021, and preliminary 3-month data from the pediatric patients in both trials is anticipated in the fourth quarter of calendar year 2021, dependent on any effects of the COVID pandemic.

About AGTC

AGTC is a clinical-stage biotechnology company developing genetic therapies for people with rare and debilitating ophthalmic, otologic and central nervous system (CNS) diseases. AGTC is a leader in designing and constructing all critical gene therapy elements and bringing them together to develop customized therapies that address real patient needs. The Company's most advanced clinical programs leverage its best-in-class technology platform to potentially improve vision for patients with an inherited retinal disease. AGTC has active clinical trials in X-linked retinitis pigmentosa (XLRP) and achromatopsia (ACHM CNGB3 & ACHM CNGA3). Its preclinical programs build on the Company's industry leading AAV manufacturing technology and scientific expertise. AGTC is advancing multiple important pipeline candidates to address substantial unmet clinical need in optogenetics, otology and CNS disorders.

About ACHM

ACHM (achromatopsia) is an inherited retinal disease, which is present from birth and is 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 ACHM, even under subdued light conditions, is usually about 20/200, a level at which people are considered legally blind.

Forward-Looking Statements

This release contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs, including statements regarding the projected timing for enrollment of patients in and data from, and the potential of, its ACHM clinical programs. Forward-looking statements include

information concerning possible or assumed future results of operations, financial guidance, business strategies and operations, preclinical and clinical product development and regulatory progress, potential growth opportunities, potential market opportunities, the effects of competition and the impact of the COVID-19 pandemic, including the impact on its ability to enroll patients. 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: gene therapy is still novel with only a few approved treatments so far; AGTC cannot predict when or if it will obtain regulatory approval to commercialize a product candidate or receive reasonable reimbursement; uncertainty inherent in clinical trials and the regulatory review process; risks and uncertainties associated with drug development and commercialization; the direct and indirect impacts of the ongoing COVID-19 pandemic on the Company's business, results of operations, and financial condition; 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 AGTC's most recent annual and subsequently filed quarterly reports 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.

IR/PR CONTACTS:
David Carey (IR) or Glenn Silver (PR)
Lazar FINN Partners
T: (212) 867-1768 or (646) 871-8485
david.carey@finnpartners.com or glenn.silver@finnpartners.com

Corporate Contacts:
Bill Sullivan
Chief Financial Officer
Applied Genetic Technologies Corporation
T: (617) 843-5728
bsullivan@agtc.com

Stephen Potter
Chief Business Officer
Applied Genetic Technologies Corporation
T: (617) 413-2754
spotter@agtc.com

Source: Applied Genetic Technologies Corporation