AGTC Reports 12-Month Data from its Ongoing Phase 1/2 Achromatopsia Clinical Trials Showing Biologic Activity in Patients with Mutations in the ACHM B3 Gene

June 24, 2021

- Activities to support the next stage of clinical development of ACHM B3 candidate are ongoing-

- Dosing of pediatric patients in ACHM B3 program and ACHM A3 program is expected to be completed in August 2021 -

- Conference call to review data today at 8:00am ET -

GAINESVILLE, Fla. and CAMBRIDGE, Mass., June 24, 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 retinal diseases, today reported 12-month data from its ongoing achromatopsia (ACHM) Phase 1/2 clinical trials, including data from all adult patients and low-dose pediatric patients. For its ACHM B3 candidate, results demonstrate biologic activity based on improvements in visual sensitivity in the treated area measured by static perimetry and light discomfort measured by the Ocular Photosensitivity Analyzer (OPA) and are supported by anecdotal patient reports. In addition, the safety profile of the Company's ophthalmic gene therapy platform remained favorable. Based on these data, AGTC intends to advance the ACHM B3 trial to the next stage of clinical development. The path forward for ACHM A3 will be determined after additional pediatric patient data and pre-clinical studies are available and can be evaluated.

"The results regarding responders in the ACHM B3 trial are encouraging," said Rachel Huckfeldt, MD, PhD, Assistant Professor of Ophthalmology at Harvard Medical School and an investigator on the ongoing AGTC achromatopsia Phase 1/2 trials. "The data from the ACHM B3 trial support further clinical investigation of this candidate, and data from additional pediatric patients may support the focused development of the ACHM A3 candidate specifically in younger patients."

"We are incredibly pleased with these data, which further support AGTC as a leader in the field of ophthalmic gene therapy. Our strong capabilities lead to differentiated products based on capsid and vector engineering, robust manufacturing, and rigorous preclinical evaluation of our product candidates in validated models of retinal diseases," said Sue Washer, President and CEO of AGTC. "We believe that these data provide important validation for the broad potential of our AAV technology platform and build on and extend the favorable safety and efficacy profiles observed to date in the clinical trials of our candidate for X-linked retinitis pigmentosa."

ACHM B3 12-Month Results
12-month data, which are available for 25 patients, consisting of 21 adult and 4 pediatric (<18 years of age) patients, show continued improvements on perimetry for higher dose and younger patients and include positive patient anecdotes. Improvements in retinal sensitivity as measured by static perimetry were seen in the treated eye compared with the untreated eye in four of 11 patients from the high-dose and pediatric cohorts. Improvements in light discomfort as measured by OPA, were also observed in six of these 11 patients.

Improvements in light discomfort also were observed in the untreated eye of these patients, which we believe may suggest possible cortical adaptation within the brain. There is no evidence that this observation is due to the presence of the ACHM B3 vector in the untreated eye. At the Company's R&D Day, which is scheduled to take place on Thursday, July 22, 2021, beginning at 10:00 AM ET, an external expert is scheduled to provide a more detailed analysis of the bilateral light discomfort finding and its potential causes. This will be in addition to clinical investigators presenting a detailed patient by patient review of all the data to date.

Based on these data, AGTC plans to advance its ACHM B3 program to the next stage of clinical development. Toward this end, the Company is drafting an End-of-Phase 2 (EOP2) briefing packet to submit to the U.S. Food and Drug Administration, developing assays for pivotal ready testing, and planning production of clinical trial material.

AGTC plans to continue to collect data from younger pediatric patients that may provide additional supportive data for the development of ACHM B3, but this data will not be a gating factor for the Company's current clinical development activities.

ACHM A3 12-Month Results
12-month data are available for 20 patients, including 16 adults and 4 pediatric patients. The results at this time point do not show consistent evidence of ACHM A3 candidate biologic activity, although we believe that the patient-reported anecdotes continue to be encouraging. In contrast with patients in the B3 trial, the majority of whom have mutations that results in the complete absence of B3 protein, the majority of A3 patients have mutations that result in the production of non-functional protein. The results observed to date in the Phase 1/2 ACHM trials suggest that the presence of these non-functional proteins may interfere with the activity of the vector expressed ACHM A3 protein. There will be an external expert at R&D Day to provide a deeper analysis.

Safety Data
Consistent with earlier data from the Company's ACHM Phase 1/2 trials and data out to 24 months for its X-linked retinitis pigmentosa candidate, 12-month ACHM data continue to support a favorable safety profile for AGTC's AAV technology platform. No serious adverse events (SAEs) related to the ACHM candidates were reported; one SAE was related to the surgical procedure and has resolved, two SAEs were related to the use of steroids and one has resolved, and one is improving.

Upcoming ACHM Clinical Milestones
AGTC believes it has a best-in-class ACHM B3 product candidate that may provide significant benefit to patients. The Company expects to report 3-month data from younger pediatric patients in both the ACHM B3 and ACHM A3 trials in the fourth quarter of 2021.

Conference Call and Webcast
AGTC will host a conference call and webcast to discuss the 12-month data from its ongoing Phase 1/2 ACHM clinical trials today at 8:00am ET. The live webcast will be available in the Events and Presentations section of the Investor Relations page at http://ir.agtc.com/events-and-presentations. To access the call, dial 877-407-6184 (US) or 201-389-0877 (outside of the US). Please log in approximately 10 minutes prior to the scheduled start time.

The archived webcast will be available in the Events and Presentations section of the Company's website.

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. AGTC'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 B3 and ACHM A3). 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. In recent years AGTC has entered into strategic partnerships with companies including Otonomy, Inc., a biopharmaceutical company dedicated to the development of innovative therapeutics for neurotology, and Bionic Sight, LLC, an innovator in the emerging field of optogenetics and retinal coding.

Forward-Looking Statements
This release contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs, including statements regarding AGTC's proposed clinical development of ACHM B3, planned pediatric surgeries for its ACHM clinical programs and the potential that future pediatric data will be supportive of future development of ACHM A3, the potential results of both its ACHM trials, its ability to enroll patients for its clinical trials, regulatory progress, potential growth and market opportunities, and the effects of competition. 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 our 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 our most recent annual or quarterly report and in other reports we have 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 Contact:
Stephen Potter
Chief Business Officer
Applied Genetic Technologies Corporation
T: 617-413-2754
spotter@agtc.com

Source: Applied Genetic Technologies Corporation