# AGTC Reports Encouraging Interim Six-Month Data from the Dose Escalation Cohorts of its Ongoing Phase 1/2 Clinical Trials in Achromatopsia

January 23, 2020

*–Encouraging preliminary signs of biologic activity and a favorable safety profile are outcomes that are clinically relevant to patients*

*– Enrollment ongoing in additional dose groups, which will include pediatric patients; interim data from these additional dose groups expected in 2H of 2020*

*– Company to host conference call and webcast with slides today at 8:00 am ET*

*– Company to review data on January 28, 2020, R&D Day*

GAINESVILLE, Fla., and CAMBRIDGE, Mass., Jan. 23, 2020 (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, today reported encouraging interim six-month data from the dose-escalation cohorts of its ongoing Phase 1/2 clinical programs in patients with achromatopsia due to mutation in the ACHM CNGB3 or ACHM CNGA3 genes. The interim results from both studies demonstrate encouraging signs of biologic activity and a favorable safety profile. The company plans to report data from additional dose groups, age groups and time-points in the second half of 2020.

"We are pleased that both of our achromatopsia clinical candidates are demonstrating preliminary signs of biologic activity for patients who have no current treatment options for a disease that significantly compromises their vision and quality of life. We are further encouraged by many patients' real-world positive anecdotes. We are continuing dose escalation in order to determine the highest safe dose with the potential to provide maximal benefit," said Sue Washer, President and CEO of AGTC. "Combined with the positive six-month data presented earlier this month from our ongoing Phase 1/2 clinical trial in patients with X-linked retinitis pigmentosa, the results presented today further validate our AAV vector technology, clinical development expertise and ability to innovate and advance product candidates that have the potential to transform patient care."

The data reported today are from 22 patients who have been dosed in the initial groups of the trials, comprising 13 patients in the ACHM CNGB3 trial and 9 patients in the ACHM CNGA3 trial. Data from both trials continue to demonstrate a favorable safety profile with no dose-limiting inflammatory responses observed.

These data also demonstrate encouraging signs of biologic activity, as shown by positive changes in light discomfort testing and encouraging patient anecdotes describing real-world improvements in visual function. The company is currently dosing two higher dose groups in adults and three groups of pediatric patients at the three highest dose groups in both trials. The company will use the additional data, which is expected to be available in the second half of 2020, to inform decision-making regarding readiness to move the product candidates to pivotal trials.

**Conference Call and Webcast Today at 8:00 am ET**
AGTC will host a conference call and webcast with accompanying slides to discuss the interim six-month data from the dose-escalation cohorts of its Phase 1/2 achromatopsia clinical trials today at 8:00 am ET. To access the call, dial 877-407-6184 (US) or 201-389-0877 (outside of the US). A live webcast will be available in the Events and Presentations section of AGTC's Investor Relations site at http://ir.agtc.com/events-and-presentations. 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.

**AGTC R&D Day**
AGTC will review the interim six-month data from the dose-escalation cohorts of the Phase 1/2 achromatopsia clinical trials at the company's R&D day taking place Tuesday, January 28, 2020, in New York. A live webcast will be available in the Events and Presentations section of AGTC's Investor Relations site at http://ir.agtc.com/events-and-presentations. 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 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, in which it has active clinical trials in 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 other CNS, ophthalmology and indications. The optogenetics program is being developed in collaboration with Bionic Sight. The otology program is being developed in collaboration with Otonomy. 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.

**About Achromatopsia (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 achromatopsia, 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. 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 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: 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; 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, 2019, as Amended, 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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