AGTC Announces Updated Development Plan for its X-Linked Retinitis Pigmentosa Clinical Program, Including Q1 2021 Start of Planned Phase 2/3 Trial

July 22, 2020

FDA feedback allows for forward program development

Company reiterates that its favorable safety profile and its advanced manufacturing and analytics capabilities enable rapid clinical development

GAINESVILLE, Fla. and CAMBRIDGE, Mass., July 22, 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 announced next steps in the clinical development of the Company's potential treatment of X-linked retinitis pigmentosa (XLRP) caused by mutations in the RPGR gene following receipt of written feedback from the FDA. The revised development plan, which includes immediate expansion of the current trial in parallel with the planned Phase 2/3 trial, will be designed to evaluate sustained efficacy across multiple measures of potential benefit in patients with XLRP.

In lieu of an in-person meeting likely due to limitations imposed by COVID-19, the FDA provided comprehensive written feedback regarding the design and execution of a registration trial and future regulatory submissions. The Company continues to move forward as planned with manufacturing, clinical site preparation and other activities to enable initiation of the studies as quickly as possible.

"We are pleased with the productive feedback from the FDA and are modifying our development program based on their recommendations to advance our XLRP gene therapy candidate," said Sue Washer, President and CEO of AGTC. "Based on data available to date, we believe we have the potential for a best-in-class product when important factors such as visual sensitivity improvements, BCVA and safety are considered, which could provide meaningful benefit to patients with XLRP who today have no treatment options."

AGTC is expanding its ongoing Phase 1/2 trial immediately and plans to dose approximately 20 patients in two masked dosing arms to collect additional functional data, including a mobility test added as a supplemental endpoint. The Company expects to begin dosing in Q4 2020.

For late stage studies, the FDA has indicated in its written feedback, which is consistent with how others in the XLRP gene therapy space are analyzing data, that a change in visual sensitivity of 7 decibels or greater in at least 5 loci would be clinically meaningful. AGTC has previously reported visual sensitivity as a mean over an entire treated area, but believes multiple patients already evaluated in the ongoing Phase 1/2 trial would meet the FDA's definition. The Company's revised Phase 2/3 trial design is also expected to include two masked active arms in addition to a control group, with visual sensitivity as the primary endpoint and several supplemental endpoints such as the mobility test. AGTC expects to begin this trial in Q1 2021.

Further information on the protocols for these trials including patient numbers, timelines and corporate cash guidance will be provided in the Company's 10K filing for the fiscal year ending June 30, 2020. In addition, AGTC remains on track to provide multiple data readouts for both its XLRP and ACHM clinical programs in the second half of 2020. These readouts will include data from the two higher dose groups in the XLRP Phase 1/2 trial.

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. Initially focusing on ophthalmology, our goal is to preserve or, hopefully, be able to improve vision in some cases. AGTC has active clinical trials in X-linked retinitis pigmentosa and achromatopsia (ACHM CNGB3 & ACHM CNGA3). Our pre-clinical programs build on our industry leading AAV manufacturing technology and expertise. AGTC is advancing multiple important pipeline candidates to address substantial unmet clinical need in optogenetics, otology and CNS disorders.

About X-linked Retinitis Pigmentosa (XLRP)
XLRP is an inherited condition that causes progressive vision loss in boys and young men. Characteristics of the disease include night blindness in early childhood and progressive constriction of the visual field. In general, XLRP patients experience a gradual decline in visual acuity over the disease course, which results in legal blindness around the 4th decade of life. AGTC was granted U.S. Food and Drug Administration (FDA) orphan drug designation in 2017, as well as European Commission orphan medicinal product designation in 2016, for its gene therapy product candidate to treat XLRP caused by mutations in the RPGR gene.

Forward-Looking Statements
This release contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs, including statements regarding the timing for and expected expansion of its XLRP clinical development program, the timing for reporting data in its XLRP and ACHM clinical programs, and its ability to enroll patients, effectively design and successfully complete its ongoing clinical trials. Forward-looking statements include information concerning possible or assumed preclinical and clinical product development and regulatory progress, future results of operations, financial guidance, business strategies and operations, potential growth opportunities, potential market opportunities, the effects of competition and the impact of the COVID-19 pandemic. 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 the Company's most recent annual or quarterly report and in other reports AGTC has 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, AGTC assumes 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:
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