AGTC Reports Positive Six-Month Data from its Ongoing Phase 1/2 Clinical Trial in X-Linked Retinitis Pigmentosa

January 9, 2020

- Data suggest durable and meaningful improvements in central visual sensitivity
- Secondary data showed encouraging improvements in Best Corrected Visual Acuity
- All patients dosed continue to demonstrate a favorable safety profile
- Company plans to initiate pivotal trial by the end of 2020
- Company to host conference call and webcast with slides today at 8:00 am ET

GAINESVILLE, Fla., and CAMBRIDGE, Mass., Jan. 09, 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 positive interim six-month data from its ongoing Phase 1/2 clinical program in X-linked retinitis pigmentosa (XLRP). The results show that patients treated centrally with its product candidate demonstrated durable improvement in visual function six months after dosing. These data reinforce the promising efficacy and safety results reported in September 2019 and will help to design the XLRP pivotal trial that is currently being planned to initiate by the end of 2020. The company also remains on track to report interim six-month data from the dose escalation cohorts of both of its ongoing trials in achromatopsia later this month.

“These promising results further demonstrate that our XLRP candidate has tremendous potential to provide meaningful benefit to XLRP patients who today have no treatment options,” said Sue Washer, President and CEO of AGTC. “The positive results observed to date give us confidence that the data as a whole will support advancement of our XLRP clinical program to a pivotal trial in 2020.”

Data from 17 of the 25 patients have been previously reported in September 2019 and demonstrated a favorable safety profile. Of the 17, eight peripherally treated patients showed stable visually function through six months and nine centrally treated patients showed improvement in visual function as measured by microperimetry and/or BCVA through three months.

At the six-month time-point for these same nine centrally dosed patients:

- Measurable improvements were observed in visual sensitivity for four of the evaluable eight patients. These are the same four patients discussed in September and indicate encouraging signs of a durable biologic effect.
  - Patients are defined as responders with improvement in visual sensitivity within the treatment area that is beyond the testing variability on at least two different test dates.
- All nine patients treated centrally also had stable or improving visual acuity, 78% saw a 5 letter or more improvement, a result not reported in other XLRP trials.
- Patients who improved in either visual sensitivity or BCVA also anecdotally report noticeable improvement in visual function including greater clarity and reduced night blindness.

Preliminary data for additional patients enrolled at a new higher dose group are consistent with previous data.

Safety data from all 25 patients dosed to date continue to demonstrate a favorable profile for the XLRP candidate, with no dose-limiting inflammatory responses observed and no secondary inflammatory responses requiring re-administration of any steroid in any patients.

The company is scheduling additional patients for enrollment during the first quarter of the 2020. These patients will enable AGTC to generate the most robust set of data possible as the company moves forward with planning for a pivotal trial and eventual BLA application.

“The sustained improvement in visual sensitivity in centrally dosed patients are compelling and, if confirmed in a pivotal trial, would be highly meaningful to patients,” said Dr. Paul Yang, MD, PhD, Assistant Professor of Ophthalmology at the Casey Eye Institute, Oregon Health & Science University in Portland. “Additionally, this is the first investigational therapy for XLRP to report on encouraging improvements in visual acuity. The combination of improved visual function across two endpoints in centrally treated patients and the previously reported stabilization of visual function in peripherally dosed patients, suggest that this gene-based therapy has the potential to be an important new approach to treating XLRP.”

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 expansion cohort of its Phase 1/2 XLRP clinical trial today 8:00am 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.

Tuesday January 28, 2020 R&D Day
AGTC plans to review the data from the XLRP and achromatopsia Phase 1/2 clinical programs at an R&D Day on Tuesday, January 28, 2020 beginning at 7:30 am ET in New York City. Members of the financial community interested in attending this event can register on the Events and Presentations page of the AGTC website or by clicking here.

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 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 (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. 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, 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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Source: Applied Genetic Technologies Corporation