AGTC Reports Positive 12-Month Data from Highest Dose Groups in its Ongoing XLRP Phase 1/2 Clinical Trial

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-Groups 5 and 6 had a 50% response rate among patients who met the inclusion criteria for the Skyline and Vista trials-

-Best Corrected Visual Acuity (BCVA) data continue to provide supportive evidence of biological response at 12 months-

-Data from a subset of Group 4 patients available for analysis at 24 months also provide preliminary evidence of continued durable responses and continue to demonstrate a favorable safety profile-

-Conference call to review data today at 8:00 AM EDT-

GAINESVILLE, Fla. and CAMBRIDGE, Mass., May 06, 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 additional positive data from the ongoing X-linked retinitis pigmentosa (XLRP) Phase 1/2 clinical trial, including 12-month data from Groups 5 and 6 reflecting a 50% response rate among patients who met the inclusion criteria for the Skyline and Vista trials and 24-month data from two of three Group 4 patients providing preliminary evidence of response durability. The third patient was not a responder at Month 12 and no early data are currently available for the remaining four Group 4 patients. Taken together, these data add to the body of evidence suggesting that durable improvements in visual sensitivity and visual acuity may be achieved in patients receiving AGTC's XLRP product candidate while continuing to demonstrate a favorable safety profile.

"The rate and durability of response for improvements in retinal sensitivity are very promising, as are continued trends of increased visual acuity at Month 12 in some patients," said Robert Sisk, MD, Associate Professor of Ophthalmology, University of Cincinnati and Cincinnati Eye Institute, and an investigator on AGTC's XLRP Phase 1/2 clinical trial. "Gene therapy holds great promise in improving outcomes for these early and moderate staged XLRP patients, who today have no FDA-approved treatment options. I believe that the data presented today are an important advance for the field of gene therapy for retinitis pigmentosa, and I look forward to presenting the full Month 12 Phase 1/2 data at the American Academy of Ophthalmology annual meeting later this year."

"A growing body of data supports the best-in-class potential of our XLRP therapy product candidate and we are executing a robust plan to advance this program as rapidly as possible toward commercialization," said Sue Washer, President and CEO of AGTC. "The 50% response rate observed in Groups 5 and 6, coupled with the 12-month visual sensitivity and visual acuity data from these groups and 24-month data from three Group 4 patients, position the Phase 2/3 Vista trial and expanded Phase 1/2 Skyline trials for success. The clear differences in visual sensitivity and visual acuity between the treated and untreated eyes at 12 months in Groups 5 and 6 demonstrate a biological response to our XLRP candidate."

Groups 5 and 6 Month 12 Data
Data at 12 months were available from seven patients in Group 5 and four patients in Group 6. One patient in Group 5 and two patients in Group 6 would not meet the inclusion criteria for the Skyline and Vista trials, resulting in a total of eight patients who were included in the responder analysis. Four of these eight patients (50%) were considered responders, all four of whom met the strict criteria of at least a 7 decibel (dB) improvement in at least 5 loci. One additional patient did not meet these criteria but had a statistically significant improvement in retinal sensitivity in the treated compared with the untreated eye at 12 months.

Consistent with previously reported 6-month data from Groups 2, 4, 5 and 6, assessment of BCVA in these groups at 12 months continue to provide supportive evidence of improved visual acuity in these patients; the difference between treated and untreated eyes is statistically significant. The Company believes that these data, together with the favorable safety profile, differentiate its XLRP candidate from competitors.

Group 4 Month 24 Data
Data from three of the seven Group 4 patients were available for analysis at Month 24, including two who were responders at Month 12 (one by the 7dB change in at least 5 loci response criteria and the other based on improved retinal sensitivity in the treated compared with the untreated eye). These two patients are still responders at Month 24 according to the same criteria; the third patient who has reached Month 24 was not a responder at Month 12 or Month 24. To the best of the Company's knowledge, this is the first XLRP gene therapy clinical trial to demonstrate continued durability of response at this time point.

Safety Data
Data from all 28 patients across six dose groups continue to demonstrate a favorable safety profile with no dose-limiting inflammatory responses observed. This safety profile, which has shown no clinically significant inflammation not manageable with steroids, continues to be observed out to 24 months.

Upcoming XLRP Clinical Milestones
AGTC believes it has a best-in-class XLRP product candidate that may provide significant benefit to patients with XLRP. The Company expects to:

- Present 12-month trial results from the ongoing Phase 1/2 clinical trial at the American Academy of Ophthalmology Annual Meeting in November 2021;
- Provide Skyline trial results from the 3-month masked interim analysis in 4Q 2021;
- Provide Skyline trial results from the 12-month data in 3Q 2022; and
- Provide Vista trial results from the 6-month masked interim analysis in 4Q 2022.

Conference Call and Webcast
AGTC will host a conference call and webcast to discuss the 12-month data from Groups 5 and 6 and 24-month data from Group 4 from its ongoing Phase 1/2 XLRP clinical trial today at 8:00am ET. To access the call, dial 866-269-4262 (US) or 323-347-3278 (outside of the US) referencing conference ID# 9770776. 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 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 CNGB3 and 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. In recent years AGTC has entered into strategic partnerships with companies including Otonomy, a biopharmaceutical company dedicated to the development of innovative therapeutics for neurotology, and Bionic Sight, 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 the projected timing for its planned Vista (Phase 2/3 XLRP) and Skyline (Expanded Phase 1/2) clinical trials, the timing for reporting data in both its Skyline and Vista trials. 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.

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