AGTC Clinical Investigators Provide Encore Presentation of Data from the Company's Ongoing XLRP and Achromatopsia Phase 1/2 Trials

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-Company remains on track to present additional 12-month data from the XLRP and achromatopsia clinical trials in the second quarter of 2021-

ALACHUA, Fla. and CAMBRIDGE, Mass., May 03, 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 diseases, today announced an encore presentation of previously reported data from the Company's ongoing clinical trials in X-linked retinitis pigmentosa (XLRP) and achromatopsia (ACHM) at the Association for Research in Vision and Ophthalmology (ARVO) 2021 Virtual Annual Meeting. Paul Yang, MD, PhD, Assistant Professor at Casey Eye Institute, Oregon Health & Science University, will review the six-month data from the Phase 1/2 XLRP trial, on which he is a clinical investigator today in a session scheduled to be held at 4:30-6:00 PM EDT; Mark Pennesi, MD, PhD, Assistant Professor at Casey Eye Institute, Oregon Health & Science University, will provide an overview of the Phase 1/2 ACHM clinical trials including examples of patients who the Company previously defined as responders on Thursday, May 6, 5:15-6:45 PM EDT.

"We are pleased to have the opportunity to share these data with members of the eye health community in this peer-reviewed format, and are especially pleased to demonstrate that the XLRP trial and both ACHM trials show favorable safety profiles out to 12 months," said Sue Washer, President and CEO of AGTC. "We believe that the XLRP data, which includes six-month data from all patients dosed and 12-month data for Groups 2 and 4, are best-in-class, and are excited for the multiple additional XLRP milestones that we anticipate in 2021 and 2022. The 12-month ACHM data are encouraging and have given us important insight that we are considering as we move this program forward."

With respect to anticipated upcoming XLRP milestones, AGTC remains on track to report 12-month data for Groups 5 and 6 from the XLRP Phase 1/2 trial in the second quarter of 2021; 3-month masked interim Skyline data in the fourth quarter of 2021; and 12-month Skyline and 6-month masked interim Vista data in the third quarter of 2022. The Company also remains on track to report additional 12-month data from both ACHM trials in the second quarter of 2021, and preliminary 3-month data from the pediatric cohorts of both ACHM trials in the fourth quarter of 2021.

Phase 1/2 XLRP Data Presented at ARVO
The data from all 28 patients in the XLRP trial across six dose groups, which Dr. Yang will present today and which were previously reported in November 2020, demonstrate a favorable safety profile with no dose-limiting inflammatory responses observed. Dr. Yang also will review efficacy data from centrally dosed patients. At six months, the data showed increased mean sensitivity relative to baseline as assessed by microperimetry, and these results were durable through month 12. We also believe that the best corrected visual acuity (BCVA) assessments in 20 centrally dosed patients at six months provides supportive evidence of improved visual acuity across dose groups, with additional supportive evidence of sustained improvement in eight of nine patients who were available for assessment at month 12. We are not aware of similar findings in clinical trials of competitors developing XLRP gene therapies.

Further a combined analysis of visual sensitivity data from all 19 evaluable centrally dosed patients showed that 10 of 15 evaluable patients in Groups 2, 4, 5 and 6 that meet inclusion criteria for the planned Vista Phase 2/3 trial show robust and durable signs of improvements in visual sensitivity through month six for Groups 5 and 6 and month twelve for Groups 2 and 4. The Company based improvement of visual sensitivity on multiple measures including on a change from baseline in visual sensitivity of at least 7 decibels in at least 5 loci or a statistically meaningful improvement in sensitivity improvement profile between the treated and untreated eyes.

Phase 1/2 ACHM Data Presented at ARVO
AGTC previously reported interim data from its ongoing Phase 1/2 trials in patients with mutations in the ACHMB3 or ACHMA3 gene in January 2021. On May 6, 2021, Dr. Pennesi will review case studies from two patients in the ACHMB3 trial and one patient in the ACHMA3 trial. Data from these case studies and the previously presented data from these trials demonstrate that subretinal injection of AGTC's ACHM candidates has been safe and well tolerated out to 12 months. Results also demonstrate potential improvements in visual sensitivity and photosensitivity in these patients that were identified to have positive results

As previously announced, AGTC is focusing on completing enrollment of pediatric patients in the two highest dose groups in the ACHMB3 and ACHMA3 trials and plans to follow all patients through 12 months. The company has amended the study protocol for these trials to allow enrollment of patients as young as four years of age and to include both functional magnetic resonance imaging (fMRI) and improved color brightness tests. AGTC also plans to work with sites to obtain the best quality multi-focal ERG data possible. The Company is hopeful that these changes, combined with longer follow-up times, will add to the developing body of evidence and supportive anecdotal patient-reported outcomes.

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 and its ACHM clinical programs. Forward-looking statements include information concerning preclinical and clinical product development and regulatory progress, 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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