AGTC Announces Positive Three-Month Interim Results from Skyline Trial of AGTC-501 for the Treatment of X-Linked Retinitis Pigmentosa

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– 62.5% of Patients in Dose Group B Were Responders for Improvements in Visual Sensitivity, the Study's Primary Efficacy Endpoint –

– Continued Strong Safety Data –

– Robust Safety and Efficacy Data Consistent with Previously Reported Results from the Phase 1/2 Trial in XLRP –

– Company to Host Conference Call to Review Data on May 17 at 8:00 a.m. ET –

GAINESVILLE, Fla. and CAMBRIDGE, Mass., May 16, 2022 (GLOBE NEWSWIRE) -- Applied Genetic Technologies Corporation (Nasdaq: AGTC), a clinical stage biotechnology company focused on the development of adeno-associated virus (AAV)-based gene therapies for the treatment of rare and debilitating diseases with an initial focus on inherited retinal diseases, today reported positive three-month interim data from its ongoing Phase 2 Skyline trial of AGTC-501, a recombinant AAV viral vector-based gene therapy targeting mutations in the RPGR gene in patients with X-linked retinitis pigmentosa (XLRP).

There were robust improvements in visual sensitivity, the trial's primary efficacy endpoint, in multiple patients three months after dosing, with a 62.5% response rate in dose group B and a 25% response rate in dose group A. This is well above the statistically significant 50% response rate the Vista Phase 2/3 trial for XLRP is powered to detect. The Company considers a responder to be a patient who has an improvement in visual sensitivity as assessed by microperimetry of at least 7 decibels (dB) in at least 5 loci. Importantly, for responders in this trial, the number of loci that improved by at least 7 dB was between 9 and 17. It is also notable that the mean visual sensitivity of the entire macula increased and the area of the macula with visual sensitivity also increased in the patients who were responders. Although these results exceeded the current standard set by the United States Food and Drug Administration (FDA) that at least 5 loci increase by at least 7 dB, the improved loci were not pre-specified, as also required in the FDA standard.

There was a significant difference in visual sensitivity as assessed by MAIA microperimetry in the treated eyes compared with the untreated eyes of all Skyline responders at 3 months. In the Phase 1/2 trial, patients who were responders at 3 months remained responders at 12 months. Based on the durability of the Phase 1/2 improvements in visual sensitivity, we believe the results observed at 3 months in the Skyline trial will be consistent at 12 months. Additional data related to the Skyline trial is available on the AGTC website or by clicking here.

Consistent with the previously reported Phase 1/2 results, AGTC-501 was generally well-tolerated in this trial with no clinically relevant safety concerns attributed to the study agent. The majority of adverse events were non-serious and mild to moderate in severity. The Company believes that the favorable safety profile is largely the result of a strong focus on product design, extensive pre-clinical testing, enhanced inclusion and exclusion criteria, and surgical consistency.

"The meaningful response rate in visual sensitivity seen in this interim Skyline data is very encouraging, as are the favorable safety data," said Dr. Robert Sisk, MD, FACS, FASRS, Director of Pediatric Vitreoretinal Surgery and Director of Ophthalmic Genetics – Cincinnati Children's Hospital and the Cincinnati Eye Institute and an investigator in the trial. "Patients with XLRP have no FDA-approved options to treat this devastating disease and we believe the results presented today show great promise to provide outcomes that are truly meaningful and potentially life-changing to patients."

Skyline is a 14 patient Phase 2 expansion of the Company's Phase 1/2 clinical trial, in which patients are randomized to either a high or low dose of AGTC-501 with the main objective to evaluate the efficacy, safety and tolerability of AGTC-501. The primary endpoint is the proportion of treated eyes that demonstrate improvement from baseline in visual sensitivity at 12 months with secondary endpoints (also at 12 months) that include improvements in best-corrected visual acuity (BCVA) and the patient's ability to navigate a mobility maze more successfully under varying light and challenge conditions. The interim analysis includes 13 male pediatric and adult patients that were treated over a 10-fold dose range in two groups. The fourteenth patient had not yet reached the three-month time point at the cut-off date for the interim analysis. Patients in the Skyline trial were younger than the patients in the comparable dose groups from the Phase 1/2 clinical trial with better BCVA and better mean visual sensitivity at baseline than in the previously reported Phase 1/2 study. Given the higher baseline BCVA, improvements in BCVA at three months were less pronounced than in the Phase 1/2 clinical trial. More than half of the patients, including four of the six responders in visual sensitivity, showed improvement in the mobility maze based on light levels passed, improved speed and/or reduced errors.

"We are incredibly excited by the compelling interim results seen in the Skyline trial, including strong safety data and robust improvements in visual sensitivity with a clear difference between the two dose groups. These results add to the growing body of evidence supporting the best-in-class potential of AGTC-501 for the treatment of XLRP," said Sue Washer, President and Chief Executive Officer of AGTC. "We believe that if we achieve similar results in the Vista Phase 2/3 clinical trial, we will have a broad and compelling body of data to support the submission of a BLA to the FDA, and to enable a differentiated and highly competitive product profile."

Conference Call and Webcast
AGTC will host a conference call and webcast to review the updated and previously reported interim study results tomorrow, May 17, at 8:00 a.m. 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.

About X-Linked Retinitis Pigmentosa (XLRP)
X-linked Retinitis Pigmentosa (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 or 5th decade of life.

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 with the potential to address unmet patient needs. AGTC's most advanced clinical programs leverage its best-in-class technology platform to potentially improve vision for patients with inherited retinal diseases. AGTC has active clinical trials in X-linked retinitis pigmentosa (XLRP) and achromatopsia (ACHM CNGB3). 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 needs in optogenetics, otology and CNS disorders, and has entered strategic collaborations with companies including Bionic Sight, an innovator in the emerging field of optogenetics, and retinal coding and Otonomy, Inc., a biopharmaceutical company dedicated to the development of innovative therapeutics for neurotology. For more information, please visit https://agtc.com/.

Forward-Looking Statements
This release contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs, including statements about the potential of the company's gene therapy platform and the strength of interim results from the Skyline Trial in XLRP, the potential of AGTC-501 as a treatment for XLRP, the ability to use the interim Skyline results as a predictor of the success of the final Skyline and Vista clinical trial results and whether these results will support future regulatory filings for AGTC-501. 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; risks and uncertainties related to funding sources for our development programs; 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 report on Form 10-K, as it may be supplemented by subsequent periodic reports 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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