Positive 18-Month Data from Ongoing Phase 1/2 Clinical Study of AGTC-501 Presented at the Association for Research in Vision and Ophthalmology 2022 Annual Meeting

May 4, 2022

– Data reconfirm previously highlighted safety and efficacy potential of AGTC-501 as a treatment for X-linked retinitis pigmentosa (XLRP) –

GAINESVILLE, Fla., and CAMBRIDGE, Mass., May 04, 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 announced the presentation of additional positive findings from the ongoing X-linked retinitis pigmentosa (XLRP) Phase 1/2 study of AGTC-501, including 18-month safety and efficacy data, at the Association for Research in Vision and Ophthalmology (ARVO) 2022 Annual Meeting, May 1-4, 2022 in Denver, Colorado.

The data presented at ARVO update previously reported data from this non-randomized, open-label Phase 1/2 study that the Company believes showed both safety and biological activity of AGTC-501 12 months after treatment. The 18-month data appear to show safety and efficacy signals similar to the study's 12-month findings, including improvements to the ellipsoid zone (EZ). In patients with XLRP, the EZ, a defined region within the photoreceptor layer of the retina, degenerates over time and is eventually lost. Eighteen months after treatment, of the eyes in the study with visible foveal EZ at baseline that underwent subretinal administration of AGTC-501, two thirds showed recovery of foveal EZ and nearly half had improved EZ appearance, which correlated with improvement in macular sensitivity.

"These results indicate sustained durability of improved visual function over 18 months and suggest evidence of biological activity for this XLRP gene therapy," said Paul Yang, M.D., Assistant Professor of Ophthalmology at Oregon Health & Science University's Casey Eye Institute, and the trial's principal investigator. "Because there are currently no approved treatment options for patients with XLRP, this data provides an important step toward a potential treatment for patients with vision loss due to XLRP."

At 18 months post treatment, AGTC-501 appeared to be well-tolerated across a wide dose range. The majority of adverse events were mild to moderate in severity, including those related to the subretinal injection procedure and importantly, immunological assessments did not indicate safety concerns.

"We continue to be enthusiastic about our lead candidate AGTC-501. The data presented today combined with the prior 12-month data from this trial are a strong indicator of the potential of both AGTC-501 and our gene therapy platform," said Sue Washer, President and Chief Executive Officer of AGTC. "These data, coupled with other data from our ongoing clinical trials, reassure us that we are on the right path to bringing lifechanging therapies for rare retinal diseases to patients and we look forward to sharing the three-month interim data from the SKYLINE Phase 2 expansion portion of this trial in the second quarter of calendar 2022 and 24-month results from this Phase 1/2 trial in the third quarter of calendar year 2022."

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 multiple clinical trials in XLRP, the potential of AGTC-501 as a treatment for XLRP, and whether these results will support future regulatory filings. 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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