AGTC to Present Three-Month Interim Results from the SKYLINE Clinical Trial of AGTC-501 for the Treatment of X-Linked Retinitis Pigmentosa at the 45th Macula Society Annual Meeting

June 6, 2022

GAINESVILLE, Fla., and CAMBRIDGE, Mass., June 06, 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 an encore presentation of 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), at the 45th Macula Society Annual Meeting taking place June 8-11, 2022, in Berlin, Germany.

“We are excited to share these data from the Skyline clinical trial with the international ophthalmology community, as we believe robust improvements in visual sensitivity, along with a favorable safety profile, show great promise for AGTC-501 as a potential treatment for patients with XLRP”, said Dr. Susan Schneider, Chief Medical Officer of AGTC. “We look forward to sharing future updates as we continue to progress AGTC-501 through the Vista Phase 2/3 clinical trial and towards a potential BLA submission.”

Presentation details are as follows:

Subretinal Gene Therapy Drug AGTC-501 for X-Linked Retinitis Pigmentosa Phase 2
Randomized, Controlled, Masked Multi-center Clinical Trial (Skyline) Interim Efficacy Results
Presenter: Andreas Lauer, MD, Chair, Department of Ophthalmology, Casey Eye Institute - Oregon Health & Science University
Session Date/Time: June 8, 2022; 6:06-6:52 p.m. CET
Presentation Date/Time: 6:06-6:15 p.m. CET
Location: The Ritz-Carlton, Berlin, Main Room

Additional information can be found on the 45th Scientific Program schedule.

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 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, including but not limited to XLRP, 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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