AGTC Reports Process Development Advances for Manufacturing its XLRP Gene Therapy Candidate in Two Abstracts at ASGCT

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-Improved process and validated expression assay support clinical phase product release for the Vista Phase 2/3 XLRP clinical trial-

GAINESVILLE, Fla. and CAMBRIDGE, Mass., May 11, 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 the presentation of new data from two studies related to improvements in the manufacturing process for rAAV2tYF-GRK1-RPGRco, the Company's gene therapy candidate for the treatment of X-linked retinitis pigmentosa (XLRP). The data are being presented today in two abstracts at the American Society of Gene & Cell Therapy (ASGCT) 24th Annual Meeting, which is being held virtually May 11-14, 2021. One abstract describes improvements to the Company's process for manufacturing its XLRP candidate (Abstract 806) and the other describes the development and validation of a novel expression assay that meets International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) guidelines and is being applied to support clinical phase product release for AGTC's XLRP clinical Phase 2/3 study, Vista (Abstract 884).

"In parallel with the progress we have made in the clinical development of our XLRP candidate, we have continued to invest in and improve our XLRP manufacturing capabilities. The advances reported today will support manufacturing for the Phase 2/3 Vista trial as we work toward finalizing a commercial manufacturing process that could meet potential market demand," said Sue Washer, President and CEO of AGTC. "We expect to report additional clinical and manufacturing advances for our XLRP candidate, which has best-in-class potential, in the months ahead as we continue to drive this program toward commercialization."

Subretinal Rd9 Mouse Study to Compare RPGR Expression Pre- and Post-Manufacturing Process Improvements (Abstract 806)
AGTC has made several improvements to the manufacturing process for its XLRP candidate. These include substituting an anion exchange column for the original cation exchange column, which enables significant improvement in the full/empty capsid ratio, as well as improvements designed to optimize the clarification and affinity steps. This abstract describes a study designed to compare transgene expression between vectors produced with the original and improved processes. Study results show that these changes resulted in an improved full/empty capsid ratio from 30% to greater than 80%, reduced process residuals (Host cell DNA and Protein), and an improved ratio of infectious particles (< 4.0 total particles/infectious particle). Immunohistochemistry staining of RPGR expression was scored as equivalent in retinal area (most showed >50% photoreceptor positivity) and intensity of staining (2.2-2.8 on a 5-point scale) following subretinal injection of XLRP vectors produced under each process into the eyes of Rd9 mutant mice, an animal model for XLRP due to mutations in the RPGR gene. RPGR expression by western blotting was also similar following administration of vector from each process. These findings support that AGTC's novel, more efficient production method yields similar, high-quality XLRP gene therapy vectors for use in future clinical trials.

Expression Assay Development for rAAV Vector Encoding Retinitis Pigmentosa GTPase Regulator (RPGRco) (Abstract 884)
Potency tests, which are critical for measuring rAAV product attributes such as quality, identity, purity, strength, and stability, are multifaceted and require continuous efforts throughout preclinical and clinical phases. This abstract reports the development and validation of a sensitive and specific *in vitro* cell-based assay to measure hRPGRco (codon optimized human retinitis pigmentosa GTPase regulator) mRNA expression from AGTC's XLRP vector, rAAV2tYF-GRK1-RPGRco. Measurement of hRPGRco mRNA expression after transduction with the Company's AAV-RPGR vector was achieved in three phases: cell transduction, cell harvesting/RNA isolation, and detection via gene-specific real-time qualitative polymerase chain reaction (RT-qPCR). Study data demonstrate successful optimization of this gene expression assay, resulting in qualification for use as a functional potency assay according to ICH guidelines. This qualification evaluated the assay's sensitivity, specificity, accuracy, precision, RT-qPCR linearity, and total assay linearity. AGTC is using this assay to support release of XLRP vector material for the Phase 2/3 Vista trial.

Both abstracts are being presented as digital posters today at 8:00 AM EDT. Abstracts for the presentations can be viewed online at: https://annualmeeting.asgct.org/

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 Company's manufacturing capabilities and its potential support market demand and release of XLRP vector material for its planned Vista (Phase 2/3 XLRP) clinical trial. 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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