AGTC Announces Significant Productivity and Quality Enhancement To Its Proprietary Manufacturing Process

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Upgraded Manufacturing Process Being Used For Production Of Pivotal Stage Clinical Trial Materials

GAINESVILLE, Fla., and CAMBRIDGE, Mass., June 22, 2020 (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 provided an update on the continued productivity and quality improvements made in its proprietary manufacturing platform that is currently being used to create clinical trial material for the Company's planned pivotal clinical trials in X-linked retinitis pigmentosa (XLRP).

"Continuous improvements to our proprietary HSV-based manufacturing process have been a critical AGTC objective for more than a decade, and our efforts have resulted in AAV vector quality and yields that we believe exceed the performance and economics of other currently available approaches to produce AAV gene therapy products, particularly those using adherent cell platforms," said Dr. Dave Knop, PhD, Head of Process Development at AGTC. "We are now achieving finished product specifications that demonstrate nearly 90% full capsids with extremely low residuals, many of which fall below the level of detection, resulting in purity levels exceeding 97%. This is in addition to yields that are more than 10-fold higher than what we achieved in our Phase 1/2 manufacturing campaigns. What this means is we are already at commercial scale manufacturing for our ophthalmology programs since we can produce thousands of low cost doses from a 40L bioreactor, and our scalable bioreactor format is designed to enable us to meet the production demands for large market indications and indications that may require substantially higher dosing."

Dr. Knop and Sue Washer, President and CEO of AGTC, recently authored an article on the challenges of AAV manufacturing at larger scale in the June issue of *BioProcess International*.

"The future of AAV-based gene therapy products will require high quality, robust and consistent manufacturing technology that can deliver safe and efficacious therapies in a cost-effective manner," said Ms. Washer. "Our long-standing focus on manufacturing improvements and building our manufacturing-related intellectual property portfolio have enabled us to establish a leadership position that we believe will support our growth and success in 2020 and beyond."

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. Initially focusing on ophthalmology, our goal is to preserve or, hopefully, be able to improve vision in some cases. AGTC has active clinical trials in X-linked retinitis pigmentosa and achromatopsia (ACHM CNGB3 & ACHM CNGA3). Our pre-clinical programs build on our industry leading AAV manufacturing technology and expertise. AGTC is advancing multiple important pipeline candidates to address substantial unmet clinical need in optogenetics, otology and CNS disorders.

About X-linked Retinitis Pigmentosa (XLRP)
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 decade of life. AGTC was granted U.S. Food and Drug (FDA) orphan drug designation in 2017, as well as European Commission orphan medicinal product designation in 2016, for its gene therapy product candidate to treat XLRP caused by mutations in the RPGR gene.

About Achromatopsia (ACHM)
Achromatopsia is an inherited retinal disease, which is present from birth and is characterized by the lack of cone photoreceptor function. The condition results in markedly reduced visual acuity, extreme light sensitivity causing day blindness, and complete loss of color discrimination. Best-corrected visual acuity in persons affected by achromatopsia, even under subdued light conditions, is usually about 20/200, a level at which people are considered legally blind.

Forward-Looking Statements
This release contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs. 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. 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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