



AGTC Announces Completion of Enrollment in All Adult Dose Groups of its Ongoing Phase 1/2 Clinical Trials in Patients with Achromatopsia

March 16, 2020

Enrollment ongoing in pediatric dose groups

Interim data of all adult dose groups expected in 2H of 2020

GAINESVILLE, Fla. and CAMBRIDGE, Mass., March 16, 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 announced that it has completed the planned enrollment in all dose groups for adult patients (age 18 years or older), including the two higher dose groups, of its Phase 1/2 clinical programs with achromatopsia due to mutation in the ACHM CNGB3 or ACHM CNGB3 genes. The company continues to enroll pediatric patients at the higher dose groups in both trials.

"We expect the information to be obtained from these adult dose groups, as well as the additional pediatric data, will help to reinforce the encouraging preliminary data generated to date and previously reported in [September 2019](#) and [January 2020](#)," said Sue Washer, president and CEO of AGTC. "We plan to report interim data from all adult dose groups in the second half of 2020 and to use the data to inform decision-making regarding readiness to move the product candidates to pivotal trials. In addition to being on track for our End of Phase 2 submission for our X-linked retinitis pigmentosa (XLRP) clinical study, we expect to have multiple achromatopsia data read-outs in 2020 that will build on the momentum we created in January."

AGTC most recently reported interim six-month data from the dose escalation cohorts of its ongoing ACHM Phase 1/2 clinical trials in [January 2020](#). Results from both studies demonstrated encouraging signs of biologic activity as shown by positive changes in light discomfort testing as well as encouraging patient anecdotes describing real-world improvements in visual function. A favorable safety profile with no dose-limiting inflammatory responses was observed.

About AGTC

AGTC is a clinical-stage biotechnology company that uses a proprietary gene therapy platform to develop transformational genetic therapies for patients suffering from rare and debilitating diseases. Its initial focus is in the field of ophthalmology, where it has active clinical trial programs in X-linked retinitis pigmentosa (XLRP) and achromatopsia (ACHM CNGB3 & ACHM CNGB3). In addition to its clinical trials, AGTC has preclinical programs in optogenetics, otology and adrenoleukodystrophy (ALD), frontotemporal dementia (FTD) and amyotrophic lateral sclerosis (ALS), which are diseases of the central nervous system (CNS), and other ophthalmology indications including Stargardt's and dry age-related macular degeneration (dry AMD). The optogenetics program is being developed in collaboration with Bionic Sight. The otology program is being developed in collaboration with Otonomy. AGTC has a significant intellectual property portfolio and extensive expertise in the design of gene therapy products including capsids, promoters and expression cassettes, as well as expertise in the formulation, manufacture and physical delivery of gene therapy products.

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 and the effects of competition. 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; 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 Annual Report on Form 10-K for the fiscal year ended June 30, 2018, 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.

IR/PR CONTACTS:

David Carey (IR) or Glenn Silver (PR)

Lazar FINN Partners

T: (212) 867-1768 or (646) 871-8485

david.carey@finnpartners.com or glenn.silver@finnpartners.com

Corporate Contacts:

Bill Sullivan
Chief Financial Officer
Applied Genetic Technologies Corporation
T: (617) 843-5728
bsullivan@agtc.com

Stephen Potter
Chief Business Officer
Applied Genetic Technologies Corporation
T: (617) 413-2754
spotter@agtc.com



Source: Applied Genetic Technologies Corporation