AGTC Announces Completion of Enrollment in the Two Highest Dose Groups of its Ongoing Phase 1/2 Clinical Trial in Patients with X-Linked Retinitis Pigmentosa

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GAINESVILLE, Fla. and CAMBRIDGE, Mass., Feb. 19, 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 enrollment in the two highest dose groups of its Phase 1/2 clinical trial evaluating the safety and efficacy of sub-retinal injection of AGTC-501 for the treatment of X-linked retinitis pigmentosa (XLRP) caused by mutations in the RPGR gene. The patients in these additional groups received a higher or highest dose of AGTC's XLRP candidate.

"We expect that the information that will be obtained from the two additional dose groups will help to reinforce the 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 these two new dose groups and to report top-line 12-month data for the first four dose groups in the second half of 2020 and intend to initiate a pivotal trial by the end of the year. Combined with the ongoing progress in our two achromatopsia clinical studies, we expect to have multiple data read-outs in 2020 that will build on the momentum we created in January."

AGTC most recently reported data from the ongoing XLRP Phase 1/2 clinical trial in January 2020. Results at the six-month time point in patients dosed centrally were indicative of durable and meaningful improvements in central visual sensitivity, encouraging improvements in Best Corrected Visual Acuity and a favorable safety profile.

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 CNGA3). 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. 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 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.

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.

For AGTC

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