AGTC Completes Enrollment of Third Group in the Dose Escalation Portion of the Achromatopsia CNGA3 Phase 1/2 Clinical Study

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GAINESVILLE, Fla., and CAMBRIDGE, Mass., July 23, 2019 (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 of the third group in the dose escalation portion of its Phase 1/2 clinical trial evaluating the safety and efficacy of subretinal injection of (rAAV2tYF-PR1.7-hCNGA3) for the treatment of achromatopsia (ACHM) caused by mutations in the CNGA3 gene.

“This achievement is another demonstration of our continued progress in the clinical development of our three product candidates for the treatment of X-linked retinitis pigmentosa and achromatopsia due to mutations in the CNGA3 or CNGB3 gene,” said Sue Washer, president and CEO of AGTC. “To date, we have dosed 68 patients across our orphan ophthalmology programs. This reflects the strong enthusiasm and support among patients and physicians for novel approaches to treating diseases that have tremendous unmet medical need.”

AGTC is enrolling patients in two parallel Phase 1/2 clinical trials of its product candidates for ACHM caused by mutations in the two most common ACHM genes, CNGB3 and CNGA3. The primary focus of the ACHM trials is assessing the safety of the vector and subretinal delivery procedure through analysis of focal (ocular) and systemic treatment-emergent adverse events. Safety will be monitored by evaluation of ocular and non-ocular adverse events. In addition, each trial will measure biologic activity by assessing changes in a wide number of measures of visual function, including visual acuity, light sensitivity, color vision, and quality of life. The company expects to provide interim dose-escalation data from both trials in the fourth quarter of 2019.

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 trials in X-linked retinitis pigmentosa (XLRP), achromatopsia (ACHM CNGB3 & ACHM CNGA3) and X-linked retinoschisis (XLRS). In addition to its clinical trials, AGTC has preclinical programs in optogenetics, adrenoleukodystrophy (ALD), which is a disease of the central nervous system (CNS) and other ophthalmology and otology indications. The optogenetics program is being developed in collaboration with Bionic Sight. 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.

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.

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