AGTC Announces Reaching Enrollment Milestones in Phase 1/2 Clinical Trials

April 8, 2019

GAINESVILLE, Fla., and CAMBRIDGE, Mass., April 08, 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 achieved enrollment milestones in two of its Phase 1/2 clinical trials. The company completed enrollment of the dose escalation portion of the achromatopsia (ACHM) CNGB3 trial, which is evaluating the safety and efficacy of its product candidate (rAAV2tYF-PR1.7-hCNGB3) for the treatment of ACHM caused by mutations in the CNGB3 gene. The company also completed enrollment in the expansion group, which includes pediatric patients, of its product candidate (rAAV2tYF-GRK1-RPGR) for the treatment of X-linked retinitis pigmentosa (XLRP) caused by mutations in the RPGR gene. Both investigational therapies utilize the company's proprietary AAV delivery technology and are administered by subretinal injection.

"Continued success enrolling in all our clinical trials is critical to advancing new and innovative gene therapies for patients with inherited retinal diseases that lack approved treatment options and we are pleased with the progress we have made," said Sue Washer, president and CEO of AGTC. "Achieving these important milestones is a testament to the dedication of the AGTC clinical teams and our clinical investigators, underscoring the support that our investigational therapies have among the patient communities we seek to serve."

The company 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. To date, AGTC has enrolled 12 patients in the ACHM CNGB3 trial and six patients in the ACHM CNGA3 trial. AGTC plans to provide topline interim six-month data for the dose escalation portion of the CNGB3 study in the second half of 2019. To date, AGTC has completed enrollment of 10 and six patients in the dose escalation and expansion portions of the XLRP trial, respectively. The company plans to provide topline interim six-month data for the dose escalation portion of the trial in the third quarter of 2019 and the expansion portion in the fourth quarter.

The primary focus of each trial 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 fields, visual acuity and quality of life.

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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Source: Applied Genetic Technologies Corporation