AGTC Announces Completion of Enrollment of Phase 1 / 2 Clinical Study of Investigational Gene Therapy in Patients with X-linked Retinoschisis (XLRS)

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GAINESVILLE, Fla. and CAMBRIDGE, Mass., April 10, 2018 (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 the completion of enrollment in a clinical study of the company's gene therapy product candidate, in collaboration with Biogen, for the treatment of x-linked retinoschisis (XLRS). This multicenter study is designed to evaluate the collaboration's AAV vector expressing retinoschisin (rAAV2tYF-CB-hRS1) in patients with XLRS caused by mutations in the RS1 gene. Topline data are anticipated by Q4 2018 with the final analysis at the twelve-month time point.

"There are currently no FDA approved treatment options for XLRS, a leading cause of macular degeneration in young men," said Sue Washer, president and CEO of AGTC. "The completion of enrollment in this AAV trial represents another significant achievement in our gene therapy clinical development program- a milestone that may improve the lives of individuals affected by XLRS."

The Phase 1/2 trial is an open-label, dose escalation study designed to assess the safety and efficacy of intravitreal administration of the AAV-based gene therapy in approximately 27 patients diagnosed with XLRS caused by mutations in the RS1 gene. Trial participants were enrolled sequentially in four groups. Individuals in Groups 1, 2 and 3 received a low, middle or high dose of the investigational study agent. Patients in Group 4 received the maximum tolerated dose as determined by the first three groups. In addition, a group of pediatric patients were enrolled at the middle dose. Although the primary endpoint of this study is designed to evaluate safety, efficacy will also be measured by an evaluation of changes in visual structure, function and quality of life.

XLRS is an inherited early onset retinal degenerative disease and the leading cause of juvenile macular degeneration in males. Characterized by abnormal splitting of the layers of the retina, the disease begins early in childhood and causes poor visual acuity in young boys which may materialize into legal blindness in adulthood. The disease begins early in childhood and affected boys typically have best-corrected visual acuity of 20/60 to 20/120 at initial diagnosis. Severe complications such as retinal hemorrhage or retinal detachment occur in up to 40% of patients, especially in older individuals.

For more information on AGTC and its pipeline of AAV-based gene therapy candidates in rare disease, please visit www.AGTC.com/products.

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 retinoschisis (XLRS), X-linked retinitis pigmentosa (XLRP), and achromatopsia (ACHM CNGB3 & ACHM CNGA3). 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 otology. The clinical-stage XLRS and XLRP programs, the discovery program in ALD and two additional ophthalmology programs are being developed in collaboration with Biogen. In addition to its product pipeline, 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 Retinoschisis (XLRS)
XLRS is an inherited retinal disease caused by mutations in the RS1 gene, which encodes the retinoschisin protein. It is characterized by abnormal splitting of the layers of the retina, resulting in poor visual acuity in young boys, which can progress to legal blindness in adult men. Information about the Phase 1/2 clinical trial in XLRS can be found at ClinicalTrials.gov under trial identifier number NCT02416622.

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. Information about the Phase 1/2 clinical trial in achromatopsia caused by CNGA3 can be found at ClinicalTrials.gov under the trial identifier number NCT02935517, while the Phase 1/2 clinical trial in achromatopsia caused by CNGB3 can be found under the trial identifier number NCT02599922.

About X-linked Retinitis Pigmentosa (XLRP)
XLRP is an inherited condition that causes boys to develop night blindness by the time they are ten and progresses to legal blindness by their early forties. Information about the Phase 1/2 clinical trial in XLRP can be found at ClinicalTrials.gov under trial identifier number NCT03314207.

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, 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 September 13, 2017, as 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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