April 22, 2016

AGTC Announces Data Evaluating Novel AAV-Based Gene Therapy as a Potential Treatment for Leber Congenital Amaurosis (LCA) and Severe Early-Childhood-Onset Retinal Degeneration (SECORD)

Study results show that experimental gene therapy intervention improved several measures of visual function in majority of patients tested

GAINESVILLE, Fla. and CAMBRIDGE, Mass., April 22, 2016 (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 data evaluating an experimental recombinant AAV vector gene delivery in patients with Leber congenital amaurosis (LCA) or severe early-childhood-onset retinal degeneration (SECORD), two related retinal diseases caused by mutations in the RPE65 gene that cause severe loss of vision in infancy. Results from the study, entitled "Results at 2 Years after Gene Therapy for RPE65-deficient Leber Congenital Amaurosis and Severe Early-Childhood Onset Retinal Dystrophy," were published online in the peer-reviewed journal *Ophthalmology* and will appear in the April print issue of the journal.

"LCA and SECORD are serious retinal degenerative conditions with no current treatments that can lead to total blindness and significantly impair quality of life in affected individuals," said contributing study author Jeff Chulay, M.D., DTM&H, Vice President and Chief Medical Officer of AGTC. "We are encouraged by these results demonstrating that administration of a novel AAV-based gene therapy can improve several measures of abnormal visual function in patients affected by these disorders."

The study was conducted at Oregon Health and Science University (OHSU) Casey Eye Institute (CEI) and University of Massachusetts. The study enrolled eight adults and four children with LCA or SECORD who received a subretinal injection of a recombinant AAV expressing RPE65 (rAAV2-CB-hRPE65) at one of two dose levels in the poorer seeing eye. All subjects then underwent serial assessments of visual function and adverse events during two-year clinical follow-up to evaluate safety and efficacy parameters.

All subjects tolerated the surgery and study agent administration with no treatment-related serious adverse events reported. In the treated eye, best-corrected visual acuity (BCVA) increased in five subjects, static perimetry hill of vision measurements for the central 30° of the visual field increased in six subjects, total visual field hill of vision measurements increased in five subjects, and kinetic visual field area improved in three subjects. One study participant had a decrease in BCVA and two subjects had a decrease in kinetic visual field area. Common adverse events associated with the injection included subconjunctival hemorrhage in eight subjects and ocular hyperemia (redness) in five subjects. The investigators concluded that the treatment with rAAV2-CB-hRPE65 was not associated with serious adverse events and improvement in one or more measures of visual function was observed in 9 of 12 (75 percent) subjects.

"While AGTC has made a strategic decision to focus on commercializing treatments for other orphan indications, these results are promising and add to previously published proof of concept data supporting gene therapy applications for inherited retinal diseases," noted Sue Washer, President and CEO of AGTC. "We have recently achieved several important clinical and regulatory milestones for the product candidates in our pipeline, including our lead product candidates for the treatment of the orphan indications X-linked retinoschisis and achromatopsia, and look forward to announcing continued progress in our primary clinical programs in the coming months."

About AGTC

AGTC is a clinical-stage biotechnology company that uses its proprietary gene therapy platform to develop products designed to transform the lives of patients with severe diseases, with an initial focus in ophthalmology. AGTC's lead product candidates are designed to treat inherited orphan diseases of the eye, caused by mutations in single genes that significantly affect visual function, and which currently lack effective medical treatments.

AGTC's product pipeline includes six named ophthalmology development programs across five targets (X-linked retinoschisis, X-linked retinitis pigmentosa, achromatopsia, wet age-related macular degeneration and blue cone

monochromacy), two non-ophthalmology programs (alpha-1 antitrypsin deficiency and adrenoleukodystrophy) and early research studies in additional indications. AGTC employs a highly targeted approach to selecting and designing its product candidates, choosing to develop therapies for indications having high unmet medical need, clinical feasibility and commercial potential. 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.

Forward Looking Statements

This release contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs. These statements relate to a variety of matters, including but not limited to, the anticipated progress of AGTC's clinical and pre-clinical programs and the anticipated utility of AAV vectors made using AGTC's proprietary manufacturing method and progress of or financial implications of AGTC's various partnership activities. 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, which include, but are not limited to, the following: no gene therapy products have been approved in the United States and AGTC cannot predict when or if it will obtain regulatory approval to commercialize a product candidate; AGTC relies on third parties to conduct, supervise and monitor its clinical trials and to conduct certain aspects of its product manufacturing and protocol development; and increased regulatory scrutiny of gene therapy and genetic research could damage public perception of AGTC's product candidates or adversely affect AGTC's ability to conduct its business. Additional factors that could cause actual results to differ materially from those described in the forward-looking statements are set forth under the heading "Item 1A—Risk Factors" in AGTC's Annual Report on Form 10-K for the fiscal year ended June 30, 2015, 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, AGTC assumes 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 Danielle Lewis (PR)
Lazar Partners Ltd.
T: (212) 867-1768 or (212) 843-0211
dcarey@lazarpartners.com or dlewis@lazarpartners.com

Corporate Contacts:

Larry Bullock
Chief Financial Officer
Applied Genetic Technologies Corporation
T: (386) 462-2204
lbullock@agtc.com

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