



June 8, 2017

AGTC Announces Topline Safety Data for X-Linked Retinoschisis Phase 1/2 Study

GAINESVILLE, Fla. and CAMBRIDGE, Mass., June 08, 2017 (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 topline safety data for the dose escalation phase of the company's Phase 1/2 X-linked retinoschisis (XLRs) clinical trial, a program partnered with Biogen. Clinical investigator Mark Pennesi, M.D., Ph.D., Associate Professor in Ophthalmic Genetics and Chief of the Ophthalmic Genetics division at Oregon Health and Science University Casey Eye Institute, will present the data at the Macula Society Annual Meeting in Singapore on Saturday, June 10th after 8:15 a.m. local time.

Dr. Pennesi will present results for the first 12 subjects enrolled in the low, middle and high dose groups of the Phase 1/2 dose escalation study. Several subjects have been followed for more than one year. Mild to moderate ocular inflammation was observed in the treated eye for the majority of patients and resolved or was controlled either without further intervention or after treatment with topical or oral corticosteroids. No treatment related serious adverse events were reported and the treatment was generally well tolerated.

"These findings are consistent with previous results demonstrating that our investigational gene therapy for XLRs was generally well tolerated and demonstrated a good safety profile across treatment groups," said Michael Goldstein, M.D., Chief Medical Officer of AGTC. "Based on these results, and in accordance with a recent review by the Data Safety and Monitoring Committee, we are continuing enrollment at the highest dose in the expansion group and look forward to providing additional updates with respect to safety, potential efficacy and biologic activity endpoints."

A copy of Dr. Pennesi's presentation will be available at <https://www.agtc.com/products/x-Linked-retinoschisis> tomorrow, Friday evening by 8:15 p.m. Eastern Time.

Patients and caregivers interested in participating in or learning about this trial may visit www.agtc.com/patients-and-caregivers or e-mail advocacy@agtc.com.

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 currently lack effective medical treatments.

AGTC's product pipeline includes ophthalmology programs in X-linked retinoschisis (XLRs), X-linked retinitis pigmentosa (XLRP), achromatopsia, wet age-related macular degeneration, and our optogenetics program with Bionic Sight. AGTC's non-ophthalmology programs include its adrenoleukodystrophy program and its otology program, which is in pre-clinical development, and the company expects to advance several otology product candidates into clinical development in the next few years. Each of AGTC's XLRs, XLRP and adrenoleukodystrophy programs is partnered with Biogen. AGTC employs a highly-targeted approach to selecting and designing its product candidates, choosing to develop therapies for indications having high unmet medical need that it believes are clinically feasible and present commercial opportunities. 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.

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: no gene therapy products have been approved in the United States and only two such products have been approved in Europe; AGTC cannot predict when or if it will obtain regulatory approval to commercialize a product candidate; uncertainty inherent in 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, 2016, 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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