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IND-Enabling Study Data Published in Human Gene Therapy Clinical Development Support Clinical Development of AGTC-402 for the Treatment of CNGA3-deficient Achromatopsia

Preclinical study demonstrates that AGTC-402 significantly improved vision in a large animal model of achromatopsia

GAINESVILLE, Fla. and CAMBRIDGE, Mass., June 15, 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 the publication of IND-enabling preclinical data demonstrating the safety and efficacy of AGTC-402 in the treatment of achromatopsia (ACHM) due to mutations in the CNGA3 gene.

The study, "Safety and Efficacy Evaluation of rAAV2tYF-PR.1-hCNGA3 Vector Delivered by Subretinal Injection in CNGA3 Mutant Achromatopsia Sheep" appears in the June issue of *Human Gene Therapy Clinical Development* and was led by researchers at the Volcani Center and Hebrew University in Israel. The gene-based therapy used in this study, AGTC-402, utilizes AGTC's proprietary AAV technology to deliver a functional copy of the human CNGA3 gene.

"These data, which were included in our Investigational New Drug application filed with the U.S. Food and Drug Administration in October 2016, provide compelling evidence that AGTC-402 has potential as a treatment for achromatopsia due to CNGA3 mutations," said Sue Washer, President and CEO of AGTC. "We are currently scheduling patients for enrollment in our Phase I/II trial in this patient population, and we continue enrollment in our clinical trial for patients with CNGB3 achromatopsia."

Patients and caregivers interested in participating in or learning more about AGTC's clinical trials for patients with ACHM caused by mutations in the CNGB3 and CNGA3 genes may find more information at www.agtc.com/patients-and-caregivers or by contacting advocacy@agtc.com.

Achromatopsia results from mutations in one of several genes. Two of these genes, CNGA3 and CNGB3, encode the alpha and beta subunits, respectively, of an ion channel that is essential for proper function of cone cells within the retina. About 75 percent of ACHM patients have mutations in one of these two genes; the remainder of cases result from mutations in one of several other genes.

The study was conducted in 13 sheep with day-blindness due to mutations in the CNGA3 gene. This large animal model has been used successfully in the evaluation of other AAV-based gene therapies for ACHM. Nine animals received a low (n=4) or high (n=5) dose of AGTC-402 administered as a single subretinal injection to the right eye. A positive efficacy control group of four animals received a different AAV-CNGA3 vector shown to be effective in a previous study. Four animals distributed across the three experimental groups received an injection of carrier vehicle in the left eye. Animals were followed for 13 weeks post-injection.

Key findings from the study include:

- Electroretinography (ERG) testing (30 Hz and CFFF) showed a significant increase in cone responses in treated eyes at Weeks 6 and 12 compared with pre-treatment responses ($P<0.05$) in all groups. ERG responses were also significantly higher at Weeks 6 and 12 when comparing treated and untreated eyes in animals receiving AGTC-402 ($P<0.05$). Animals receiving the positive efficacy control vector had significant increases in the 30 Hz response at Weeks 6 and 12 and in the CFFF response at 12 Weeks ($P<0.05$).
- Maze navigation tests showed significantly shorter navigation times ($P<0.05$) and reduced obstacle collisions in the vector-treated eye for all groups.
- Immunohistochemical analysis showed expression of the human CNGA3 gene in all 13 vector-treated eyes but not in untreated or vehicle treated eyes.
- No serum antibodies against the human CNGA3 protein were detected in any vector-treated animal.
- Subretinal injections were generally well tolerated and were not associated with any systemic toxicity. Most animals

had mild to moderate side effects thought to be related to the surgical procedure, including inflammation, swelling and bleeding, which generally resolved without further intervention.

The authors conclude that the study results support the evaluation of AGTC-402 in clinical trials in individuals affected by ACHM due to CNGA3 mutations, and add that study participants should be carefully monitored for possible inflammatory responses. The planned Phase I/II trial of AGTC-402 includes the prophylactic administration of oral and topical corticosteroids to minimize inflammation at the time of administration.

"These results add to the growing body of preclinical evidence that support the safety and efficacy of AAV-based delivery of the CNGA3 gene for the treatment of achromatopsia," said Michael Goldstein, M.D., Chief Medical Officer of AGTC. "The significant improvements in ERG results and maze navigation times demonstrate that AGTC-402 provided a functional benefit in these animals. We believe that similar benefits may be achievable in achromatopsia patients, and are excited to be screening for enrollment in two clinical trials for this condition."

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 an 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.

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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