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AGTC and Collaborators Present New Data on the Natural History of X-Linked Retinoschisis (XLRS) at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting

Results expected to enhance development of AGTC's XLRS gene therapy candidate; Phase 1/2 clinical trial currently enrolling patients at multiple locations across the United States

BALTIMORE, May 09, 2017 (GLOBE NEWSWIRE) -- Researchers at the Casey Eye Institute, the Retina Foundation of the Southwest, the Kellogg Eye Center and 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, reported results from a study of the natural history of X-linked retinoschisis (XLRS) and the impact of carbonic anhydrase inhibitors (CAIs) on disease progression. The data were presented this week at ARVO 2017, the Association for Research in Vision and Ophthalmology Annual Meeting, taking place in Baltimore from May 7-11.

Maria A. Parker, M.D., Senior Project Manager at the Casey Eye Institute at the Oregon Health and Science University, presented the data in a poster titled "Natural History and Effect of Carbonic Anhydrase Inhibitor Use in X-Linked Retinoschisis" (Abstract #1490).

XLRS is characterized by abnormal splitting of the layers of the retina, resulting in poor visual function in young boys, which can ultimately result in legal blindness in adult men. The study was designed to characterize the natural history of XLRS and to determine the effect of CAIs on retinal function and structure in XLRS patients.

This observational study enrolled 56 patients six years of age and older (average 30.0 years) with a confirmed mutation in the RS1 gene. Of the 56 patients, 18 had no CAI use prior to or during the study (Group A), 18 had previously used CAIs and continued to do so during the study (Group B) and 20 had no history of CAI use but began these medications at the start of the study (Group C). All patients underwent functional [best corrected visual acuity (BCVA)] and structural [macular cystic cavity volume (CCV) calculated from spectral domain optical coherence tomography] evaluations at baseline, 6, 12 and 18 months, and Group C patients underwent additional exams at 1 and 3 months after starting CAI therapy.

There were no significant differences in BCVA or CCV within each group at subsequent evaluations compared with baseline values. Comparison of Group C with Groups A and B at each follow up examination also showed no statistically significant differences in BCVA or CCV, although there was a suggestion of improved visual acuity in Group C earlier in the study. Researchers conclude that these results demonstrate that XLRS is stable over an 18-month time period and that topical CAI use was not associated with improvement in visual function or macular cyst volume at one year. However, they also noted that some individuals treated with CAIs demonstrated notable improvements at earlier time points, suggesting that these medications may have more nuanced effects.

"There are limited data available on the natural course of XLRS, or the impact of using CAIs on disease progression, likely because XLRS is a rare condition," said Sue Washer, President and CEO of AGTC. "This lack of information is one hurdle to overcome as we develop new treatment approaches. We believe these study results are an important advance in our understanding of the natural progression of XLRS, and will enhance our efforts to develop our AAV-based XLRS gene therapy candidate, which is currently being evaluated in a Phase 1/2 clinical trial."

AGTC is currently enrolling patients in a clinical trial for its XLRS product candidates, as part of the company's collaboration with Biogen. Patients and caregivers interested in participating in or learning more about this trial may learn more at www.agtc.com/patients-and-caregivers or by contacting 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.

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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