AGTC Announces Topline Interim Six-Month Data from Phase 1/2 X-Linked Retinoschisis Clinical Study; Termination of Biogen Collaboration

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- Results from XLRS study support general safety and tolerability of AGTC's gene delivery platform but did not demonstrate signs of clinical activity at interim six-month time point -
- AGTC to regain rights to five clinical and preclinical development programs; Biogen terminates collaboration agreement -
- AGTC on schedule to advance its Phase 1/2 clinical studies in achromatopsia and X-linked retinitis pigmentosa; three data releases expected in 2019 -
- AGTC to host conference call and webcast today at 5:00 p.m. ET -

GAINESVILLE, Fla., and CAMBRIDGE, Mass., Dec. 12, 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 reported topline interim six-month data from its Phase 1/2 clinical trial of rAAV2tYF-CB-hRS1, an investigational AAV-based gene therapy delivered via intravitreal injection for X-linked retinoschisis (XLRS) due to mutations in the RS1 gene. Results from the study show that rAAV2tYF-CB-hRS1 is generally safe and well-tolerated, but no signs of clinical activity were observed at six-months. As per the study protocol, AGTC will continue to monitor enrolled patients at scheduled visits through the end of the study.

AGTC also announced that the company will regain full rights to the XLRS and X-linked retinitis pigmentosa (XLRP) programs and the three other partnered discovery programs following Biogen's termination of the collaboration agreement, which will be effective on March 8, 2019. The company will complete patient monitoring activities on the XLRS program according to the clinical protocol but will not further develop the product. AGTC plans to advance the XLRP program as previously disclosed, and will determine next steps for the three discovery programs over the next several months.

"The data from the XLRS trial support the general safety and tolerability of our gene delivery platform and further reinforce our confidence in our ongoing Phase 1/2 trials in achromatopsia (ACHM) and XLRP," said Sue Washer, President and CEO of AGTC. "We are extremely grateful for the commitment of the patients, families, patient advocacy organizations and our clinical collaborators, which was crucial to completing enrollment of this Phase 1/2 trial. Through this study, we have deepened our understanding of the challenges that patients with inherited ophthalmic diseases face every day. It has also provided us with valuable information and insights in designing and analyzing clinical trials for inherited retinal diseases. We remain dedicated to leveraging our gene delivery platform to improve the care and outcomes for patients with ACHM, XLRP and other ophthalmic diseases."

Ms. Washer continued: "We appreciate our collaboration with Biogen and the support they have provided in advancing our joint efforts. AGTC will regain full rights to the XLRP Phase 1/2 clinical program, which has completed its dose escalation phase ahead of our previous guidance and is moving directly to an expansion phase in 2019. AGTC's two ongoing clinical programs in ACHM are proceeding as previously disclosed and the company is expecting data readouts from all three trials in 2019. We will also regain control of the preclinical programs in adrenoleukodystrophy (ALD) and two ophthalmic programs. After reviewing all available data for these programs, we will decide which to independently move forward into IND-enabling studies."

Topline XLRS Six-Month Clinical Data
The Phase 1/2 XLRS 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 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, respectively, of the investigational study agent. Patients in Group 4 received the maximum tolerated or high dose as determined by the first three groups. In addition, a group of pediatric patients was enrolled at the middle dose. The primary endpoint of this study was designed to evaluate safety and tolerability, whereas secondary efficacy outcomes included changes in visual acuity, visual field, schisis (cystic) cavity size and ERG b-wave amplitude, among others. A total of 27 subjects were treated and all subjects completed study visits through at least month six. AGTC will continue to monitor study participants as required in the study protocol.

Key study findings include:

- There were no signs of clinical activity over the six-month interim analysis period
- There were no study discontinuations and no dose-related toxicities. Mild to moderate ocular inflammation resolved with and without steroid treatment
- Baseline patient demographics were relatively well-balanced

In summary, Ms. Washer notes: "Based on AGTC's XLRS data and other recently reported intravitreal injection data, it is clear that the intravitreal injection used in our XLRS program created a challenge compared with our other clinical programs, all of which utilize a subretinal injection. While clinical plans for XLRP and ACHM are supported by robust data from naturally occurring large animal models, such models do not exist for XLRS. AGTC will benefit from our experience with the XLRS trial data because it strengthens our ability to effectively analyze and interpret a variety of important ophthalmic endpoints that have not commonly been used in inherited retinal diseases. I believe this expertise will give us an advantage in supporting future development of our diverse ophthalmic pipeline across multiple products."

The company plans to present data from the trial at an upcoming medical or scientific meeting in 2019. For more information on AGTC and its pipeline of AAV-based gene therapy candidates in rare diseases, please visit https://agtc.com/programs/.

Financial Guidance
AGTC's previous financial guidance, even in light of Biogen's termination of the collaboration agreement, remains unchanged. As of September 30,

2018, the company's cash, cash equivalents and investments totaled $105.4 million. The company believes these funds will be sufficient to allow AGTC to generate data from its ongoing clinical programs, to move the preclinical optogenetic program in collaboration with Bionic Sight into the clinic and fund the currently planned research and discovery programs for at least the next two years. The company expects total cash, cash equivalents and investments as of June 30, 2019, to be between $65 and $75 million.

Conference Call and Webcast Information
AGTC's management will host a conference call today at 5:00 p.m. ET. A question and answer session will follow AGTC's remarks. To access the call, dial 877-407-6184 (US) or 201-389-0877 (outside of the US). A live webcast will be available in the Events and Presentations section of AGTC's Investor Relations site at http://ir.agtc.com/events-and-presentations. Please log in approximately 10 minutes prior to the scheduled start time.

The archived webcast will be available in the Events and Presentations section of the company's website.

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), other ophthalmology indications and otology. The optogenetics program is being developed in collaboration with Bionic Sight. 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.

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, including regarding which programs, if any, will be advanced in the future, 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 June 30, 2018, 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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