AGTC Reports Additional Positive Data from its Phase 1/2 Clinical Trial in Patients with X-Linked Retinitis Pigmentosa

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- Durable improvements observed in visual sensitivity and visual acuity over a wide dose-range with a favorable safety profile -

- Recent FDA interactions clarify clinically meaningful improvement on microperimetry and no further comments or questions regarding the company's pre-clinical or CMC plans -

- Company plans to initiate enrollment in the planned Phase 1/2 expansion trial (Skyline) in 4Q 2020 and the Phase 2/3 trial (Vista) in 1Q 2021 -

- Company to host management update and webcast with slides today at 8:00am ET -

GAINESVILLE, Fla. and CAMBRIDGE, Mass., Nov. 11, 2020 (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 positive data from its ongoing Phase 1/2 clinical program in patients with X-linked retinitis pigmentosa (XLRP). Visual sensitivity, visual acuity and safety data were reported for 12-month timepoints for Groups 2 and 4, and 6-month time points for Groups 5 and 6. Eight of the 11 patients in Groups 5 and 6 would meet the eligibility criteria for AGTC's future XLRP trials, and five of these eight (62%), met the definition for response based on an improvement of at least 7 decibels in at least 5 loci. The Company plans to initiate its Skyline trial by enrolling approximately 12 additional patients total in Group 2 (1.2E+11 vg/mL) and Group 5 (1.1E+12 vg/mL). In addition, the Company remains on track to initiate enrollment in its planned Vista trial in 1Q 2021.

"The high proportion of responders from multiple dose groups with sustained durability of improved visual function over 12 months is compelling evidence of biological activity for this XLRP gene therapy," said Dr. Paul Yang, Assistant Professor of Ophthalmic Genetics and Immunology at Casey Eye Institute (Oregon Health and Science University). "With further investigation, there is a high likelihood that this gene therapy candidate could become a meaningful treatment for patients with XLRP."

XLRP Phase 1/2 6-Month and 12-Month Data
Data from all 28 patients across six dose groups continue to demonstrate a favorable safety profile with no dose-limiting inflammatory responses observed.

At the 12-month time-point for the nine centrally dosed patients in Groups 2 and 4:

- Measurable improvements were observed in visual sensitivity for two of the evaluable eight patients, while a third patient identified as a responder at 6-months fell just below the cut-off. The Company believes this represents an encouraging sign of a durable biologic effect.
    - Patients are defined as responders when at least 5 loci within the central 36 loci of the perimetry grid increase by at least 7 decibels.
- Eight of nine patients treated centrally also had stable or improving visual acuity, a result not reported in other XLRP trials.

At the 6-month time-point for the 11 centrally dosed patients in Groups 5 and 6:

- Measurable improvements were observed in visual sensitivity for five of the 11 patients.
    - Patients are defined as responders when at least 5 loci within the central 36 loci of the perimetry grid increase by at least 7 decibels.
    - Three of the 11 patients in these groups, who were not responders, would not meet the inclusion criteria for future trials meaning that five of eight patients, or 62%, would be considered responders.
- Nine of the 11 patients treated centrally also had stable or improving visual acuity, a result not reported in other XLRP trials.

Further a combined analysis of visual sensitivity data from all 19 evaluable centrally dosed patients shows that 10 of 15 evaluable patients in Groups 2, 4, 5 and 6 that meet inclusion criteria for the planned Vista Phase 2/3 trial show robust and durable signs of improvements in visual sensitivity through month 6 for Groups 5 and 6 and month 12 for Groups 2 and 4. The Company is basing improvement of visual sensitivity on multiple measures including on a change from baseline in visual sensitivity of at least 7 decibels in at least 5 loci or a statistically meaningful improvement in sensitivity improvement profile between the treated and untreated eyes.

"These updated data provide important evidence that our XLRP product candidate provides durable improvements in multiple endpoints that are also meaningful to patients," said Sue Washer, President and CEO of AGTC. "We are especially pleased to see that 62% of patients with appropriate baseline characteristics in Groups 5 and 6 show biologic activity of our product across multiple measures of visual sensitivity and that there are encouraging trends in visual acuity. This result and the continued favorable safety profile further increase our confidence in the potential of our XLRP gene therapy to become the industry-leading treatment for this disease, for which there are currently no therapies."

Planned XLRP Phase 2/3 Vista Trial
The proposed design of the XLRP Phase 2/3 or Vista trial is currently expected to include approximately 60 patients randomized across three arms: a

low-dose group (the 1.2E+11 vg/mL, Group 2 dose from the ongoing Phase 1/2 trial), a high-dose group (the 1.1E+12 vg/mL, Group 5 dose from the ongoing Phase 1/2 trial), and an untreated control group. The primary endpoint will be based on visual sensitivity defined having at least a 7 decibel improvement in visual sensitivity in at least 5 pre-specified loci at month 12. This primary endpoint was informed by recent comments that the Company received from the Food and Drug Administration (FDA) on what evidence would help support a showing of a clinically meaningful improvement on microperimetry at the Group 2 and Group 5 doses. Importantly, the Company plans to use this endpoint as one of several measures of visual sensitivity that have the potential to support a clinically meaningful benefit. The Company plans to compare the responder rates in each active arm to responder rates in the control arm of the Vista trial. The Company plans to submit a 6-month interim analysis of the data from the Vista trial, together with complete 12-month data from the Skyline trial to the FDA to obtain feedback on the Company's development plan to support approval. Based on any FDA feedback, the Company may modify the final trial design, enrollment numbers, and/or statistical analysis plan. The Company also plans to discuss with the FDA dose selection for the treatment of the contralateral eye. The Company expects to initiate enrollment in the Vista trial in 1Q 2021 and to provide results from the 6-month interim analysis in 3Q 2022.

The Company remains on-track to have clinical trial material produced in time for the initiation of the Vista trial through its advanced manufacturing process that provides improved yields, purity and potency.

Conference Call and Webcast Today at 8:00 am ET
AGTC will host a conference call and webcast with accompanying slides to discuss the 12-month and 6-month data from its ongoing Phase 1/2 XLRP clinical trial today at 8:00am ET. 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 developing genetic therapies for people with rare and debilitating ophthalmic, otologic and central nervous system (CNS) diseases. AGTC is a leader in designing and constructing all critical gene therapy elements and bringing them together to develop customized therapies that address real patient needs. The Company's most advanced clinical programs leverage its best-in-class technology platform to potentially improve vision for patients with an inherited retinal disease. AGTC has active clinical trials in X-linked retinitis pigmentosa and achromatopsia (ACHM CNGB3 & ACHM CNGA3). Its pre-clinical programs build on the Company's industry leading AAV manufacturing technology and scientific expertise. AGTC is advancing multiple important pipeline candidates to address substantial unmet clinical need in optogenetics, otology and CNS disorders.

About X-linked Retinitis Pigmentosa (XLRP)
XLRP is an inherited condition that causes progressive vision loss in boys and young men. Characteristics of the disease include night blindness in early childhood and progressive constriction of the visual field. In general, XLRP patients experience a gradual decline in visual acuity over the disease course, which results in legal blindness around the 4^{th} or 5^{th} decade of life. AGTC was granted U.S. Food and Drug Administration (FDA) orphan drug designation in 2017, as well as European Commission orphan medicinal product designation in 2016, for its gene therapy product candidate to treat XLRP caused by mutations in the RPGR gene.

Forward-Looking Statements
This release contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs, including statements regarding the projected timing for its planned Vista (Phase 2/3 XLRP) and Skyline (Expanded Phase 1/2) clinical trials, the timing for reporting data in both its Skyline and Vista trials and its ACHM clinical programs. Forward-looking statements include information concerning possible or assumed future results of operations, financial guidance, business strategies and operations, preclinical and clinical product development and regulatory progress, potential growth opportunities, potential market opportunities, the effects of competition and the impact of the COVID-19 pandemic, including the impact on its ability to enroll patients. 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; the direct and indirect impacts of the ongoing COVID-19 pandemic on our business, results of operations, and financial condition; 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 our most recent annual or quarterly report and in other reports we have 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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