AGTC Provides Trial Design Update for its Phase 2/3 XLRP Clinical Trial, New Data on XLRP Higher Dose Group and Financial Results for the Fourth Quarter and Year Ended June 30, 2020

September 9, 2020

- Planned Phase 2/3 trial expected to include approximately 60 patients and a responder analysis based on at least 7 decibel improvement in visual sensitivity in at least 5 loci across two active dose groups and one control group -

- Data from Group 5 supports use of higher dose level in Phase 2/3 trial -

- Company on track to provide multiple data readouts for XLRP and ACHM clinical programs in 4Q 2020 -

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

GAINESVILLE, Fla. and CAMBRIDGE, Mass., Sept. 09, 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 provided additional information about the proposed design of the planned Phase 2/3 trial for its X-linked retinitis pigmentosa (XLRP) clinical program, which is expected to commence in the first quarter of 2021, and new preliminary data on a higher dose Group 5 from the ongoing Phase 1/2 XLRP trial. The Company also announced financial results for the fourth quarter and fiscal year ended June 30, 2020.

"We are excited to close out our 2020 fiscal year with a path toward initiating a Phase 2/3 trial for our XLRP program, and to provide new preliminary data for the higher dose Group 5 in our Phase 1/2 trial, which supports inclusion of that dose level in the Phase 2/3. In addition, we remain on track to report additional data readouts from all three of our clinical programs in the fourth quarter of the 2020 calendar year," said Sue Washer, President and CEO of AGTC. "The advancements we are making in our clinical programs, highlighted by the proposed design of our planned Phase 2/3 XLRP trial, underscore our continued progress."

Recent Highlights

X-linked Retinitis Pigmentosa (XLRP)

The proposed design of the XLRP Phase 2/3 trial is expected to include approximately 60 patients randomized across three arms: a low-dose group (1.2E+11 vg/mL the Group 2 dose from the ongoing Phase 1/2 trial), a high-dose group (1.1E+12 vg/mL the Group 5 dose from the ongoing Phase 1/2 trial), and an untreated control group. The primary endpoint will be based on visual sensitivity, with a responder defined as having at least 7 decibel improvement in visual sensitivity in at least 5 loci at month 12, which is intended to represent a clinically meaningful benefit. Responder rates in each active arm will be compared to responder rates in the control arm. The Company plans to submit a 6-month interim analysis of the data from the Phase 2/3 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 will discuss with FDA the possibility of finalizing dose selection for the treatment of the contralateral eye based on this 6-month interim data. The Company expects to begin enrolling patients in 1Q 2021 and to provide results from the 6-month interim analysis in 3Q 2022, dependent on future effects of the COVID-19 pandemic on clinical trial enrollment.

- Analysis of visual sensitivity data from patients in the ongoing Phase 1/2 XLRP trial shows that 7 of 15 patients in Groups 2, 4 and 5 are responders at month 6 (month 3 in one case) based on the responder criteria defined above. Focusing on the Phase 1/2 Group 5 dose group, a dose level that the Company is planning to use as the high dose in the Phase 2/3 trial, 4 of 7 patients met the response criteria. Of note, using the planned inclusion/exclusion criteria for the Phase 2/3 trial, one Phase 1/2 Group 5 patient would be removed such that the responder rate would be 4 of 6, or 67%.

- The Company also plans to expand its ongoing Phase 1/2 trial to include approximately 12 additional patients who will be masked and randomized to doses of 1.2E+11 vg/mL (Group 2 in original trial plan) and 1.1E+12 vg/mL (Group 5 in original trial plan). The Company expects to begin enrolling these additional patients in 4Q 2020 and to provide results from a 3-month interim analysis in 4Q 2021, dependent on future effects of the COVID-19 pandemic on clinical trial enrollment.

- AGTC remains on track to provide 12-month data from the ongoing Phase 1/2 trial by 4Q 2020 for Groups 1-4 to evaluate durability of effect and continued safety, as well as full 6-month data analysis for Groups 5 and 6 to evaluate safety and efficacy at higher doses.

- The Company remains on-track to have clinical trial material produced in time for the initiation of the Phase 2/3 trial with its advanced manufacturing process that provides improved yields, purity and potency.

Achromatopsia (ACHM)

- In January 2020, AGTC announced encouraging interim data from the dose-escalation cohorts of its ongoing Phase 1/2 clinical programs in patients with ACHM due to mutations in the ACHM CNGB3 or ACHM CNGA3 genes. In March, AGTC completed the planned enrollment in all dose groups for adult patients (age 18 years or older), including the two higher dose groups, of both ACHM trials, bringing the total number of adults dosed to 15 in the ACHM A3 trial and 22 in the

ACHM B3 trial. Pediatric dosing is ongoing with four pediatric patients dosed in each trial to date. The Company expects that pediatric enrollment may continue to be challenging.

- The Company plans to release additional data for the adult dose groups in 4Q 2020, which will be used to inform decision-making regarding readiness to move the product candidates to pivotal trials.

Preclinical Programs

AGTC's preclinical pipeline includes two ophthalmology programs, one of which targets the dry form of age-related macular degeneration (AMD), and three programs targeting central nervous system (CNS) disorders. The CNS programs target adrenoleukodystrophy (ALD) and two additional rare genetic CNS indications—frontotemporal dementia (FTD) and amyotrophic lateral sclerosis (ALS)—that have substantial patient populations and well-defined clinical phenotypes. AGTC also has collaborations with Otonomy and Bionic Sight for genetic forms of hearing loss and optogenetics, respectively.

Mobile Vision Testing Program

In June 2020, AGTC announced that it launched a mobile vision testing program to conduct follow-up assessments during the COVID-19 pandemic for patients enrolled in the Company's ongoing clinical trials in XLRP and ACHM. The mobile vision testing program is available to patients enrolled in AGTC's clinical trials across the United States so that they are able to maintain their follow-up study assessments while COVID-19 restrictions remain in effect. To date, more than 25 patients have been seen in the mobile vision testing program.

Financial Results for the Fourth Quarter and Fiscal Year Ended June 30, 2020

Revenue: There was no revenue for the fourth quarter of 2020 and $2.5 million for the year ended June 30, 2020, compared to $0.4 million and $41.7 million in the comparable periods in fiscal year 2019. Revenue for the year ended June 30, 2020 was primarily $2.2 million of non-cash collaboration revenue in connection with the in-kind contributions made to Bionic Sight.

R&D Expenses: Research and development expenses were $10.5 million for the fourth quarter of 2020 and $35.8 million for the year ended June 30, 2020, compared to $8.3 million and $33.2 million in the comparable periods in fiscal year 2019. The increase of $2.6 million during the full year period was primarily due to increased external XLRP spending primarily related to Phase 2/3 activities, increased employee-related costs and increased external spending related to ACHM, primarily due to patient enrollment. These expenses were partially offset by decreased external research and discovery spending and share-based compensation expenses.

G&A Expenses: General and administrative expenses were $4.1 million for the fourth quarter of 2020 and $13.6 million for the fiscal year ended June 30, 2020, compared to $3.5 million and $12.9 million in the comparable periods in fiscal year 2019. The increase in general and administrative expenses for the full year was primarily driven by increased employee-related expenses partially offset by a decrease in share-based compensation expenses.

Net Income (Loss): Net loss was $14.5 million for the fourth quarter of 2020 and $45.9 million for the year ended June 30, 2020, compared to net loss of $10.5 million and $2.0 million in the comparable periods in 2019.

Financial Guidance: As of June 30, 2020, the Company's cash, cash equivalents and investments totaled $80.5 million. The Company believes these funds will be sufficient to allow AGTC to generate data from its ongoing clinical programs, initiate a Phase 2/3 on XLRP trial and to fund currently planned research and discovery programs into the fourth quarter of calendar year 2021.

Conference Call and Webcast

AGTC will host a conference call and webcast with accompanying slides to review the Phase 2/3 XLRP trial design and discuss financial results for the fourth quarter and fiscal year ended June 30, 2020 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 4th 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.

About Achromatopsia (ACHM)

Achromatopsia is an inherited retinal disease, which is present from birth and is characterized by the lack of cone photoreceptor function. The condition results in markedly reduced visual acuity, extreme light sensitivity causing day blindness, and complete loss of color discrimination. Best-corrected visual acuity in persons affected by achromatopsia, even under subdued light conditions, is usually about 20/200, a level at which people are considered legally blind.

Forward-Looking Statements

This release contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs, including statements regarding the timing for and expected expansion of its planned Phase 2/3 XLRP clinical trial, the timing for reporting data in both its XLRP and ACHM clinical programs and the funding needs for these 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.

APPLIED GENETIC TECHNOLOGIES CORPORATION
BALANCE SHEETS
(Unaudited)

In thousands, except per share data	June 30, 2020	2019
ASSETS		
Current assets:		
Cash and cash equivalents	$ 38,463	$ 26,703
Investments	41,995	55,292
Grants receivable	—	13
Prepaid and other current assets	2,506	2,276
Total current assets	82,964	84,284
Property and equipment, net	4,311	4,430
Intangible assets, net	1,098	1,013
Investment in Bionic Sight, LLC	8,096	1,945
Right-of-use assets - operating leases	3,422	—
Right-of-use asset - financing lease	80	—
Other assets	348	544
Total assets	$ 100,319	$ 92,216
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,355	$ 1,331
Accrued and other liabilities	10,502	8,024
Lease liabilities - operating	1,058	—
Lease liability - finance	48	—
Total current liabilities	12,963	9,355
Lease liabilities - operating, net of current portion	4,070	—
Lease liability - finance, net of current portion	38	—
Long-term debt, net of debt discounts and deferred financing fees	9,677	—
Other liabilities	2,555	4,152
Total liabilities	29,303	13,507
Stockholders' equity:		
Preferred stock, par value $0.001 per share, 5,000 shares authorized; no shares issued and outstanding	—	—
Common stock, par value $0.001 per share, 150,000 shares authorized; 25,813 and 18,226 shares issued; 25,793 and 18,207 shares outstanding at June 30, 2020 and 2019, respectively	25	18
Additional paid-in capital	252,519	214,324
Shares held in treasury of 20 and 19 at June 30, 2020 and 2019, respectively	(88)	(85)
Accumulated deficit	(181,440)	(135,548)
Total stockholders' equity	71,016	78,709
Total liabilities and stockholders' equity	$ 100,319	$ 92,216

APPLIED GENETIC TECHNOLOGIES CORPORATION
STATEMENTS OF OPERATIONS
(Unaudited)

In thousands, except per share data	Three Months Ended June 30, 2020	2019	Year Ended June 30, 2020	2019
Revenue:				
Collaboration revenue	$ –	$ –	$ 2,297	$ 41,128
Grant and other revenue	–	406	156	564
Total revenue	–	406	2,453	41,692
Operating expenses:				
Research and development	10,453	8,332	35,778	33,183
General and administrative and other	4,127	3,514	13,617	12,859
Total operating expenses	14,580	11,846	49,395	46,042
Loss from operations	(14,580)	(11,440)	(46,942)	(4,350)
Other income (expense), net:				
Investment income, net	122	505	1,185	2,009
Other income (expense), net	(5)	446	(5)	446
Total other income (expense), net	117	951	1,180	2,455
Loss before provision for income taxes	(14,463)	(10,489)	(45,762)	(1,895)
Provision for income taxes	20	19	83	76
Loss before equity in net losses of an affiliate	(14,483)	(10,508)	(45,845)	(1,971)
Equity in net losses of an affiliate	(20)	(6)	(47)	(35)
Net loss	$ (14,503)	$ (10,514)	$ (45,892)	$ (2,006)
Weighted average shares outstanding:				
Basic	25,769	18,184	21,102	18,157
Diluted	25,769	18,184	21,102	18,157
Net loss per common share:				
Basic	$ (0.56)	$ (0.58)	$ (2.17)	$ (0.11)
Diluted	$ (0.56)	$ (0.58)	$ (2.17)	$ (0.11)

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