AGTC Announces Financial Results and Business Update for the Quarter Ended September 30, 2020

November 16, 2020

GAINESVILLE, Fla. and CAMBRIDGE, Mass., Nov. 16, 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 announced financial results for the quarter ended September 30, 2020. The Company is also providing an update on its ongoing clinical trials in patients with X-linked retinitis pigmentosa (XLRP) and achromatopsia (ACHM).

"The updated XLRP data that we reported last week provide evidence that our XLRP product candidate provides durable improvements in visual sensitivity and visual acuity over a wide dose range and has a favorable safety profile," said Sue Washer, President and CEO of AGTC. "Data publicly reported by our competitors this weekend at the American Academy of Ophthalmology (AAO) Annual Meeting further increase our confidence that we have the industry-leading XLRP program. We are on track 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. Additionally, new data from our ongoing ACHM trials have provided important insights for the potential development of our ACHM product candidates. We are pleased that we met our goal of reporting out data from all three ongoing clinical trials in 4Q 2020 and are now focused on advancing the Skyline and Vista trials for XLRP as rapidly as possible."

XLRP
On November 11, 2020, the Company reported additional positive data from its Phase 1/2 clinical trial in patients with XLRP that indicated durable improvements observed in visual sensitivity and visual acuity over a wide dose range with a favorable safety profile out to month 12 in two of the dose groups. Based on comparison to publicly released data reported by competitors this weekend at the AAO Annual Meeting, the Company believes it has a best-in-class product that may provide significant benefit to patients with XLRP.

ACHM
Today the Company is reporting 12-month ACHM data in the original three dose groups (low, medium and high), as well as 6- to 9-month data at two higher dose groups (higher and highest groups in the January 2020 data release), and data for 6 pediatric subjects (three in each study; 12-17 years old) at the first of three planned dose levels. While some patients showed improvements in at least one measure of visual function, no consistent sustained improvements were observed based on current assessments within the dose groups tested. However, anecdotal statements and assessments from patient-reported outcome surveys continue to provide the Company with confidence that patients are subjectively experiencing improved vision. Based on the characteristics of ACHM, the Company continues to believe that longer treatment durations and/or focusing on younger patients may be necessary to fully realize the potential of this treatment. To this end, the Company intends to complete the planned enrollment of pediatric patients in the two highest dose groups of the ongoing ACHM clinical trials and has amended the study protocol to allow enrollment of patients as young as four years of age and to include additional assessments such as functional MRI (fMRI) and improved color brightness tests.

Financial Results for the Three Months Ended September 30, 2020

R&D Expenses: Research and development expenses were $11.6 million for the quarter ended September 30, 2020 compared to $8.6 million during the comparable period in the prior fiscal year. The increase of $3.0 million was primarily due to increased external XLRP spending (primarily related to Skyline and Vista activities) and increased external spending related to ACHM (primarily due to patient enrollment and deployment of the Company's mobile vision center). These expenses were partially offset by a reduction in employee-related costs and decreased external research and discovery spending on other programs.

G&A Expenses: General and administrative expenses were $3.4 million for the quarter ended September 30, 2020 compared to $3.3 million during the comparable period in the prior fiscal year. The increase of $0.1 million was primarily due to higher fees from outside legal counsel in the 2020 period, partially offset by lower employee-related expenses and share-based compensation expense.

Interest Expense: Interest expense increased by $0.3 million during the quarter ended September 30, 2020 when compared to the comparable period in the prior fiscal year due to the loan agreement that the Company entered into on June 30, 2020.

Net Loss: The Company's net loss was $15.4 million and $11.6 million for the quarters ended September 30, 2020 and 2019, respectively.

Financial Guidance: As of September 30, 2020, the Company's cash, cash equivalents and investments totaled $66.6 million. We believe that these funds will be sufficient to allow the Company to generate data from its ongoing clinical programs, initiate the Skyline and Vista studies and fund currently planned research and discovery programs into the fourth quarter of calendar year 2021.

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 (XLRP) and achromatopsia (ACHM CNGB3 & ACHM CNGA3). Its preclinical 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 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 or 5th 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 ACHM
ACHM 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 ACHM, 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 projected timing for its planned Vista (Phase 2/3 XLRP) and Skyline (Expanded Phase 1/2 XLRP) clinical trials, the timing for reporting data in both its Skyline and Vista trials and the potential of 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 the Company's 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 AGTC's most recent annual and subsequently filed quarterly reports 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	**September 30, 2020**	**June 30, 2020**
ASSETS		
Current assets:		
Cash and cash equivalents	$ 31,570	$ 38,463
Investments	34,987	41,995
Prepaid and other current assets	1,984	2,506
Total current assets	68,541	82,964
Property and equipment, net	4,314	4,311
Intangible assets, net	1,136	1,098
Investment in Bionic Sight, LLC	8,067	8,096
Right-of-use assets – operating leases	3,337	3,422
Right-of-use asset – finance lease	69	80
Other assets	151	348
Total assets	$ 85,615	$ 100,319
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,654	$ 1,355
Accrued and other liabilities	10,246	10,502
Lease liabilities – operating	1,063	1,058
Lease liability – finance	49	48
Total current liabilities	13,012	12,963
Lease liabilities – operating, net of current portion	3,898	4,070
Lease liability – finance, net of current portion	26	38
Long-term debt, net of debt discounts and deferred financing fees	9,759	9,677
Other liabilities	2,718	2,555
Total liabilities	29,413	29,303
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; 25,901 and 25,813 shares issued; 25,860 and 25,793 shares outstanding at September 30, 2020 and June 30, 2020, respectively	25	25
Additional paid-in capital	253,208	252,519

Treasury stock at cost; 41 and 20 shares at September 30, 2020 and June 30, 2020, respectively	(211)	(88)
Accumulated deficit	(196,820)	(181,440)
Total stockholders' equity	56,202	71,016
Total liabilities and stockholders' equity	$ 85,615	$ 100,319

APPLIED GENETIC TECHNOLOGIES CORPORATION
STATEMENTS OF OPERATIONS
(Unaudited)

In thousands, except per share data	**Three Months Ended September 30, 2020**	**2019**
Revenue:		
Collaboration revenue	$ —	$ —
Grant revenue	—	—
Total revenue	—	—
Operating expenses:		
Research and development	11,626	8,642
General and administrative and other	3,436	3,348
Total operating expenses	15,062	11,990
Loss from operations	(15,062)	(11,990)
Other income (expense), net:		
Investment income, net	64	446
Interest expense	(332)	(2)
Total other income (expense), net	(268)	444
Loss before provision for income taxes	(15,330)	(11,546)
Provision for income taxes	21	21
Loss before equity in net losses of an affiliate	(15,351)	(11,567)
Equity in net losses of an affiliate	(29)	(10)
Net loss	$ (15,380)	$ (11,577)
Weighted average shares outstanding:		
Basic	25,818	18,212
Diluted	25,818	18,212
Net loss per common share:		
Basic	$ (0.60)	$ (0.64)
Diluted	$ (0.60)	$ (0.64)

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