AGTC Announces Financial Results and Business Update for the Quarter Ended September 30, 2019

November 12, 2019

Company to host R&D Day on January 28, 2020, in New York

GAINESVILLE, Fla. and CAMBRIDGE, Mass., Nov. 12, 2019 (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, 2019.

“Presenting promising topline six-month and preliminary three-month data from the dose-escalation cohorts of our ongoing Phase 1/2 clinical programs in X-linked retinitis pigmentosa and both achromatopsia trials, respectively, was a key milestone for AGTC,” said Sue Washer, President and CEO of AGTC. “We are on track to present additional data from all three programs and are advancing our discussions with the FDA such that we can finalize the design and initiate a pivotal trial in X-linked retinitis pigmentosa in 2020. We also announced the expansion of our preclinical pipeline, including naming a potential treatment for Stargardt disease as our next orphan ophthalmology product candidate, and the establishment of a strategic collaboration with Otonomy. All of these programs give us additional opportunities to leverage our industry-leading AAV platform and expertise to create value for patients and shareholders.”

Recent Highlights

- In September, AGTC announced positive data from the ongoing Phase 1/2 trial in patients with X-linked retinitis pigmentosa (XLRP), demonstrating stabilization of decline in the peripheral vision, improvement in central vision, and a favorable safety profile. Based on its ongoing discussion with physicians and advocacy groups, the company expects these results will be highly meaningful to XLRP patients, who today have no treatment options that address the underlying cause of their disease.

- In September, AGTC announced positive data demonstrating biological activity in its ongoing Phase 1/2 trials in patients with achromatopsia (ACHM) due to mutations in the ACHM B3 or A3 genes.

- AGTC entered into a strategic collaboration with Otonomy, Inc., to co-develop and co-commercialize an AAV-based gene therapy to restore hearing in patients with sensorineural hearing loss caused by a mutation in the gap junction protein beta 2 gene (GJB2) — the most common cause of congenital hearing loss.

- In a poster at the European Society of Gene and Cell Therapy annual meeting, AGTC presented new data from non-clinical studies evaluating the effect of pre-existing anti-AAV antibodies on the transduction and expression efficiency of AAV vectors. Results showed that the presence of neutralizing antibodies (NAbs) to AAV in the serum or in the eye did not affect gene delivery, gene expression, or inflammation following ocular administration of AAV vectors. The data support the safety and efficiency of the company’s AAV vectors independent of Nab levels, which should allow use of these vectors in larger patient populations, including those with pre-existing antibodies against AAV.

- AGTC identified Stargardt disease, an inherited form of macular dystrophy, as the company’s second preclinical ophthalmology program, and reported preclinical data supporting continued development of its Stargardt disease product candidate.

AGTC Clinical Program Update

XLRP Phase 1/2 Clinical Trial
As of November 12th, a total of 24 patients have been dosed in the trial. AGTC continues to enroll patients to create a robust data set.

In September 2019, AGTC reported data from 17 patients, which continued to demonstrate a favorable profile for the XLRP candidate, with no dose-limiting inflammatory responses observed and no secondary inflammatory responses requiring re-administration of any steroid in any patients dosed to date. Efficacy analyses showed stable visual function in all eight patients dosed peripherally and for whom six-month data were available, as measured by visual fields and best-corrected visual acuity (BCVA). Evidence of improved visual function was also observed in preliminary data from nine centrally dosed patients at the three-month time point, with all nine patients demonstrating stable or improving visual acuity, a result that has not been reported by others.

ACHM Phase 1/2 Clinical Trials

As of November 12th, a total of 29 patients have been dosed in both trials. AGTC continues to enroll patients to create a robust data set. Safety data from these patients continue to demonstrate a favorable profile for each of the ACHM candidates, and the Data Safety Monitoring Committee has supported continued dose escalation and dosing of pediatric patients.

In September 2019, AGTC reported data from 14 patients dosed across the B3 and A3 Phase 1/2 trials. One of three patients at the middle dose level

in each trial and two of three patients at the high dose level in the ACHM B3 trial have shown clinically meaningful improvements in light discomfort, defined as greater than one log lux change from baseline at three months. These early data suggest a potential for meaningful benefit for these patients.

Preclinical Programs

As announced in September 2019, 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, which include the previously announced program in adrenoleukodystrophy (ALD) as well as two additional rare genetic CNS indications that have substantial patient populations and well-defined clinical phenotypes. We also have collaborations with Otology and Bionic Sight for genetic forms of hearing loss and optogenetics, respectively.

On November 5, 2019, AGTC identified Stargardt disease as its second preclinical ophthalmology program and reported that its dual AAV vector system effectively expressed full-length ABCA4 protein in the retinal tissue of non-human primates following subretinal injection. Most cases of Stargardt disease result from mutations in the ABCA4 gene, and absence of functional ABCA4 protein results in the accumulation of toxic substances in photoreceptor cells. The non-human primate study provides a solid foundation on which to continue advancing this program to human clinical trials, and AGTC is conducting additional studies in support of a potential Investigational New Drug Application submission to the U.S. Food and Drug Administration.

Financial Results for the Quarter Ended September 30, 2019

R&D Expenses: Research and development expenses were $8.6 million for the three months ended September 30, 2019, compared to $10.1 million for the three months ended September 30, 2018. The decrease of $1.5 million for the quarter was primarily due to decreased XLRP sublicense expenses associated with a milestone payment from Biogen in 2018, decreased discovery spending associated with the company's pre-clinical ophthalmology programs, and decreased XLRS spending associated with completing enrollment of the XLRS Phase 1/2 trial in April 2018.

G&A Expenses: General and administrative expenses for the three months ended September 30, 2019, were $3.3 million, compared to $3.2 million for the three months ended September 30, 2018, an increase of $0.1 million. The increase was primarily driven by higher employee-related and other G&A expenses partially offset by decreased shared based compensation expenses.

Net Loss: Net loss was $11.6 million for the three months ended September 30, 2019, compared to net income of $1.2 million for the three months ended September 30, 2018.

Financial Guidance: As of September 30, 2019, the company's cash, cash equivalents, and investments amounted to $71.1 million. The company believes these funds will be sufficient to allow AGTC to generate data from its ongoing clinical programs, to initiate activities to ensure efficient transition into pivotal trials and fund the currently planned research and discovery programs into the first half of 2021. The company expects total cash, cash equivalents and investments as of June 30, 2020, to be between $30 and $40 million.

AGTC Hosting New York R&D Day on January 28, 2020

AGTC will host an R&D Day on Tuesday, January 28, 2020, in New York. The R&D Day will feature presentations from AGTC's management team and key opinion leaders specializing in the field of retinal diseases covering XLRP, ACHM and the company's preclinical programs. Additional details will be provided in the coming weeks.

Conference Call and Webcast
AGTC will host a conference call and webcast to discuss financial results for the first fiscal quarter ended September 30, 2019 today at 4:30pm 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 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, in which it has active clinical trials in 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) and other CNS, ophthalmology and other indications. The optogenetics program is being developed in collaboration with Bionic Sight. The otology program is being developed in collaboration with Otonomy. 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.

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 (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. 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 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, 2019, as amended, 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.

Financial tables follow

APPLIED GENETIC TECHNOLOGIES CORPORATION
BALANCE SHEETS
(Unaudited)

In thousands, except per share data	**September 30, 2019**	**June 30, 2019**
ASSETS		
Current assets:		
Cash and cash equivalents	$ 16,272	$ 26,703
Investments	54,839	55,292
Grants receivable	13	13
Prepaid and other current assets	2,054	2,276
Total current assets	73,178	84,284
Property and equipment, net	4,137	4,430
Intangible assets, net	1,062	1,013
Investment in Bionic Sight	1,936	1,945
Right of use asset – operating lease	3,654	—
Right of use asset – finance lease	114	—
Other assets	544	544
Total assets	$ 84,625	$ 92,216
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,338	$ 1,331
Accrued and other liabilities	7,185	8,024
Lease liability - operating	641	—
Lease liability - finance	46	—
Total current liabilities	9,210	9,355
Lease liability – operating, net of current portion	5,052	—
Lease liability – finance, net of current portion	75	—
Other liabilities	2,315	4,152
Total liabilities	16,652	13,507
Stockholders' equity:		
Preferred stock, par value $.001 per share, 5,000 shares authorized; no shares issued and outstanding	—	—
Common stock, par value $.001 per share, 150,000 shares authorized; 18,238 and 18,226 shares issued; 18,218 and 18,207 shares outstanding at September 30, 2019 and June 30, 2019, respectively	18	18
Additional paid-in capital	215,168	214,324
Shares held in treasury of 20 and 19 at September 30, 2019 and June 30, 2019, respectively	(88)	(85)
Accumulated deficit	(147,125)	(135,548)
Total stockholders' equity	67,973	78,709
Total liabilities and stockholders' equity	$ 84,625	$ 92,216

APPLIED GENETIC TECHNOLOGIES CORPORATION
STATEMENTS OF OPERATIONS
(Unaudited)

In thousands, except per share amounts	Three Months Ended September 30, 2019	2018
Revenue:		
Collaboration revenue	$ –	$ 14,025
Grant and other revenue	–	9
Total revenue	–	14,034
Operating expenses:		
Research and development	8,642	10,065
General and administrative and other	3,348	3,213
Total operating expenses	11,990	13,278
Income/(loss) from operations	(11,990)	756
Other income:		
Investment income, net	446	471
Other expense	(2)	–
Total other income, net	444	471
Income/(loss) before provision for income taxes	(11,546)	1,227
Provision for income taxes	21	19
Income/(loss) before equity in net losses of affiliate	(11,567)	1,208
Equity in net losses of affiliate	(10)	(8)
Net income/(loss)	$ (11,577)	$ 1,200
Weighted Average Shares Outstanding:		
Basic	18,212	18,128
Diluted	18,212	18,158
Net earnings/(loss) per common share:		
Basic	$ (0.64)	$ 0.07
Diluted	$ (0.64)	$ 0.07

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