AGTC Announces Financial Results and Business Update for the Quarter Ended March 31, 2020

May 13, 2020

- Company on track to provide multiple data readouts for XLRP and ACHM clinical programs in 2H 2020 -

- Company on track for End of Phase 2 meeting in Q2 2020 -

- Company sees minimal COVID-19 impact to date -

- Company to host conference call and webcast today at 8:00am ET -

GAINESVILLE, Fla. and CAMBRIDGE, Mass., May 13, 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 March 31, 2020.

"AGTC started 2020 with significant momentum, announcing sustained improvements in visual function for four of eight centrally dosed patients in our XLRP trial and encouraging preliminary signs of biologic activity in our ACHM trials. The company also completed enrollment in the XLRP trial, completed adult enrollment in both ACHM trials, and strengthened our balance sheet with a successful financing in February," said Sue Washer, President and CEO of AGTC. "We remain on track to provide multiple data readouts for both our XLRP and ACHM clinical programs in the second half of 2020, including top-line 12-month data from the peripherally and centrally-dosed initial groups and interim data from the two new higher dose groups in the XLRP trial as well as interim data in the new adult groups in both ACHM trials. Our industry-leading XLRP data package has us well-positioned for an End of Phase 2 meeting with the FDA in the second quarter of 2020. The biological activity observed to date in our XLRP clinical trial reinforces our optimism that the program will move into a pivotal trial by the end of 2020."

Recent Highlights

X-linked Retinitis Pigmentosa (XLRP)

- In January, AGTC announced positive interim six-month data from its ongoing Phase 1/2 clinical program in XLRP. The results show that four of eight evaluable patients treated centrally with the company's product candidate demonstrated durable improvement in visual sensitivity six months after dosing. All patients demonstrated a favorable safety profile for the XLRP candidate, with no dose-limiting inflammatory responses observed and no secondary inflammatory responses requiring re-administration of any steroids in any patients. Preliminary data also showed that all nine centrally dosed patients had stable or improving visual acuity at the six-month time point, a result that has not been reported by others.
- In February, AGTC completed enrollment in the two highest dose groups of its XLRP trial, bringing the total number of patients dosed to 28.
- An End of Phase 2 meeting with the FDA is planned for the second quarter of 2020.
- The company plans to release additional data in the second half of 2020 and initiate a pivotal trial by the end of 2020.

Achromatopsia (ACHM)

- In January, 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. The interim three-month results from both studies demonstrated encouraging signs of biologic activity based on improvements in light discomfort. Interim six-month data from both trials continue to demonstrate a favorable safety profile with no dose-limiting inflammatory responses.
- 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 three pediatric patients dosed in each trial to date. The company expects that pediatric enrollment will continue to be challenging.
- The company plans to release additional data for the adult dose groups in the second half of 2020, which will be used to inform decision-making regarding readiness to move the product candidates to pivotal trials.

Preclinical Programs

As previously announced at AGTC's R&D Day on January 28, 2020, 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, frontotemporal dementia (FTD) and amyotrophic lateral sclerosis (ALS), that have substantial patient populations and well-defined clinical phenotypes. AGTC also has collaborations with Otology and Bionic Sight for genetic forms of hearing loss and optogenetics, respectively.

COVID-19 Business Update

Despite the worldwide impact of the COVID-19 pandemic, AGTC remains on track and expects to report data from its XLRP program and both ACHM programs in the second half of 2020. The company already completed enrollment in the XLRP Phase 1/2 clinical trial and enrollment in all dose groups for adult patients of both ACHM trials. The company also closed a $34.8 million financing in February, providing funding to support its ongoing clinical programs.

AGTC is taking appropriate measures to ensure that the health and safety of its employees and patients are protected. The majority of the company's personnel have been working from home and those corporate facilities and clinical testing sites that are open are operating with the utmost caution and in-line with government guidelines. These precautions and pandemic-related travel restrictions have created challenges for new patients to meet with clinicians and receive proper evaluations.

Financial Results for the Three and Nine Months Ended March 31, 2020

Revenue: Total revenue for the three and nine months ended March 31, 2020 was nil and $2.5 million, respectively, compared to $21.3 million and $41.3 million, respectively, during the comparable 2019 periods. Revenue for the nine months ended March 31, 2020 was primarily $2.2 million of non-cash consideration collaboration revenue in connection with the in-kind contributions made to Bionic Sight. Thereafter, no additional collaboration revenue will be recognized in connection with the Bionic Sight agreement.

R&D Expenses: Research and development expenses for the three and nine months ended March 31, 2020 were $8.3 million and $25.3 million, respectively, compared to $7.2 million and $24.9 million, respectively, during the comparable 2019 periods. The increase of $0.4 million during the nine month period was primarily due to increased ACHM Phase 1/2 clinical trial expenses associated with increased patient enrollment and new site activations and increased employee-related costs, partially offset by decreased sublicense expense associated with receiving a milestone payment from Biogen in the previous fiscal year.

G&A Expenses: General and administrative expenses for the three and nine months ended March 31, 2020 were $3.1 million and $9.5 million, respectively, compared to $3.1 million and $9.3 million, respectively, during the comparable 2019 periods.

Net Income (Loss): Net loss for the three and nine months ended March 31, 2020 was $11.2 million and $31.4 million, respectively. Net income for the three and nine months ended March 31, 2019 was $11.5 million and $8.5 million, respectively.

Financial Guidance: As of March 31, 2020, the company's cash, cash equivalents and investments totaled $84.5 million. The company believes these funds will be sufficient to allow AGTC to generate data from its ongoing clinical programs, initiate a pivotal trial on XLRP and to fund currently planned research and discovery programs into the second half of 2021.

Conference Call and Webcast

AGTC will host a conference call and webcast to discuss financial results for the third fiscal quarter ended March 31, 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. Initially focusing on ophthalmology, our goal is to preserve or, hopefully, be able to improve vision in some cases. AGTC has active clinical trials in X-linked retinitis pigmentosa and achromatopsia (ACHM CNGB3 & ACHM CNGA3). Our pre-clinical programs build on our industry leading AAV manufacturing technology and 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 (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 reporting data and the commencement of pivotal clinical trials. 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. 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	March 31, 2020	June 30, 2019
ASSETS		
Current assets:		
Cash and cash equivalents	$ 58,491	$ 26,703
Investments	26,026	55,292
Grants receivable	—	13
Prepaid and other current assets	2,942	2,276
Total current assets	87,459	84,284
Property and equipment, net	4,216	4,430
Intangible assets, net	1,176	1,013
Investment in Bionic Sight	8,115	1,945
Right-of-use assets – operating leases	3,503	—
Right-of-use asset – finance lease	91	—
Other assets	544	544
Total assets	$ 105,104	$ 92,216
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 3,013	$ 1,331
Accrued and other liabilities	9,434	8,024
Lease liabilities – operating	1,054	—
Lease liability – finance	47	—
Total current liabilities	13,548	9,355
Lease liabilities – operating, net of current portion	4,329	—
Lease liability – finance, net of current portion	51	—
Other liabilities	2,357	4,152
Total liabilities	20,285	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; 25,784 and 18,226 shares issued; 25,764 and 18,207 shares outstanding at March 31, 2020 and June 30, 2019, respectively	25	18
Additional paid-in capital	251,819	214,324
Shares held in treasury of 20 and 19 at March 31, 2020 and June 30, 2019, respectively	(88)	(85)
Accumulated deficit	(166,937)	(135,548)
Total stockholders' equity	84,819	78,709
Total liabilities and stockholders' equity	$ 105,104	$ 92,216

APPLIED GENETIC TECHNOLOGIES CORPORATION
STATEMENTS OF OPERATIONS
(Unaudited)

In thousands, except per share amounts	Three Months Ended March 31, 2020	2019	Nine Months Ended March 31, 2020	2019
Revenue:				
Collaboration revenue	$ —	$ 21,207	$ 2,297	$ 41,128
Grant and other revenue	—	111	156	158
Total revenue	—	21,318	2,453	41,286
Operating expenses:				
Research and development	8,308	7,203	25,325	24,851
General and administrative and other	3,134	3,110	9,490	9,345
Total operating expenses	11,442	10,313	34,815	34,196
Income (loss) from operations	(11,442)	11,005	(32,362)	7,090
Other income:				
Investment income, net	281	512	1,063	1,504
Other income	4	—	—	—
Total other income, net	285	512	1,063	1,504
Income (loss) before provision for income taxes	(11,157)	11,517	(31,299)	8,594
Provision for income taxes	21	19	63	57
Income (loss) before equity in net losses of an affiliate	(11,178)	11,498	(31,362)	8,537
Equity in net losses of an affiliate	(11)	(9)	(27)	(29)
Net income (loss)	$ (11,189)	$ 11,489	$ (31,389)	$ 8,508
Weighted Average Shares Outstanding				
Basic	22,272	18,166	19,558	18,149
Diluted	22,272	18,324	19,558	18,322
Net income (loss) per common share				
Basic	$ (0.50)	$ 0.63	$ (1.60)	$ 0.47
Diluted	$ (0.50)	$ 0.63	$ (1.60)	$ 0.46

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