AGTC Announces Financial Results and Business Update for the Quarter Ended March 31, 2021

May 17, 2021

- Reported 50% response rate in visual sensitivity among patients in highest dose groups in ongoing Phase 1/2 clinical trial of its XLRP gene therapy candidate for patients who met inclusion criteria for Skyline and Vista trials -

- Company on track to provide multiple data readouts for its XLRP and ACHM clinical programs in 2021 and 2022 -

- Company plans to lease 21,000 square foot build-to-suit cGMP manufacturing facility adjacent to its Florida facility -

- Company to host conference call and webcast today at 4:30 p.m. ET -

GAINESVILLE, Fla. and CAMBRIDGE, Mass., May 17, 2021 (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 retinal diseases, today announced financial results for the quarter ended March 31, 2021.

"The positive data from our ongoing Phase 1/2 XLRP clinical trial and planned expansion of our manufacturing and analytics capabilities give us tremendous momentum as we seek to advance our XLRP candidate toward commercialization," said Sue Washer, President and Chief Executive Officer of AGTC. "We have great confidence in our XLRP program as well as our broader portfolio of clinical and preclinical programs, which is reflected in our commitment to lease a build-to-suit cGMP manufacturing and quality control facility. We have several XLRP clinical milestones ahead in 2021 and 2022 that we expect will continue to differentiate our XLRP candidate. We also remain on track to share multiple data readouts from our ongoing achromatopsia Phase 1/2 clinical trials in 2021."

X-linked Retinitis Pigmentosa (XLRP)
In May 2021, the Company reported 12-month data from the highest dose Groups 5 and 6 in the ongoing Phase 1/2 XLRP clinical trial. A total of eight patients across both groups met the inclusion criteria for the Skyline and Vista trials and were evaluated for response. Four of these eight patients (50%) met the response criteria of at least a 7 decibel (dB) improvement in at least 5 loci, and one additional patient, did not meet these criteria, but had a statistically significant improvement in retinal sensitivity in the treated eye compared with the untreated eye at 12 months.

The Company also reported preliminary 24-month data for three of the seven patients in Group 4 who were evaluable at this time point. Two of these three patients, both of whom were responders at 12 months, continued to be responders at 24 months (one by the 7dB change in at least 5 loci criteria and the other with a statistically significant improvement in retinal sensitivity in the treated eye compared with the untreated eye). These results provide preliminary evidence of continued durability. The third patient was not a responder at 12 or 24 months. To the best of the Company's knowledge, this is the first XLRP gene therapy clinical trial to demonstrate durability of response at this time point.

Consistent with previously reported 6-month data from Groups 2, 4, 5 and 6, Best Corrected Visual Acuity (BCVA) assessments at 12 months in these groups continued to provide supportive evidence of improved visual acuity in these patients, with a statistically significant difference between the treated and untreated eyes. This type of improvement has not been reported in other XLRP clinical trials and the Company believes that these data, together with the favorable safety profile, differentiate its XLRP candidate from competitors.

The Company's XLRP candidate, which is administered via subretinal injection, has best-in-class potential that may provide significant benefit to patients with XLRP. The Company expects to:

- Present 12-month trial results from the ongoing Phase 1/2 clinical trial at the American Academy of Ophthalmology Annual Meeting in November 2021;
- Provide Skyline trial results from the 3-month masked interim analysis in 4Q 2021;
- Provide Skyline trial results from the 12-month data in 3Q 2022; and
- Provide Vista trial results from the 6-month masked interim analysis in 4Q 2022.

Achromatopsia (ACHM)
In January 2021, the Company announced the first reported quantitative evidence of improvement in visual sensitivity, as measured by full field static perimetry, supported in some patients by other endpoints. Seven of the 16 patients in the three highest dose groups in the ACHMB3 trial showed these improvements in visual sensitivity in the treated area. No consistent results were seen in the other dose groups. In a subset of these patients with evaluable multi-focal electroretinograms (mfERG), improvements in electrical signaling were measurable in the same treated area.

For ACHMA3, of the 16 patients in the four highest dose groups, three patients showed improvements in visual sensitivity in the treated area, as measured by static perimetry. No consistent results were seen in other dose groups. None of these three patients with improvements in visual sensitivity had evaluable mfERGs.

AGTC currently plans to focus on completing enrollment of pediatric patients in the two highest dose groups in its ACHMB3 and ACHMA3 trials, subject to potential delays caused by the COVID-19 pandemic, and to follow all patients through 12 months. The Company expects to:

- Provide 12-month data from the adult patients in both trials in 2Q 2021; and
- Provide preliminary 3-month data from the pediatric patients in both trials in 4Q 2021.

Manufacturing Facility

Last week, the Company announced that it had initiated plans to lease a build-to-suit 21,000 square foot current Good Manufacturing Practices (cGMP) manufacturing and quality control facility adjacent to its Florida facility to prepare for potential late-stage development of its XLRP and ACHM programs. Leasing this cGMP facility is part of the Company's strategy to enable more rapid filing of a Biologics Licensing Application and allow for an expedited commercial launch of its XLRP candidate if approved by the United States Food and Drug Administration (FDA). The cGMP facility is also expected to support more rapid advancement of the Company's product pipeline while providing supply chain redundancy and reducing manufacturing risk. The Company anticipates that the build-out of the new manufacturing and quality control facility will be completed during the second half of calendar year 2022. The Company plans to support this strategic investment through robust tenant improvement allowances, tiered rental rates during construction and its restructured loan agreement with Hercules Capital, Inc.

Financial Results for the Three and Nine Months Ended March 31, 2021

Revenue: There was no revenue for the three and nine months ended March 31, 2021. There was no revenue in the three months ended March 31, 2020 and $2.5 million of revenue in the nine months ended March 31, 2020. Revenue during the nine months ended March 31, 2020 was primarily due to $2.2 million of non-cash collaboration revenue in connection with in-kind contributions made to Bionic Sight, LLC pursuant to a collaborative agreement.

R&D Expenses: Research and development expenses for the three and nine months ended March 31, 2021 were $11.0 million and $34.4 million, respectively, compared to $8.3 million and $25.3 million, respectively, during the comparable 2020 periods. The increase of $9.1 million during the 2021 nine-month period was primarily due to increased external XLRP spending for planned manufacturing, clinical site preparation and other activities related to the Skyline and Vista trials, partially offset by decreased external ACHM spending.

G&A Expenses: General and administrative expenses for the three and nine months ended March 31, 2021 were $3.5 million and $10.3 million, respectively, compared to $3.1 million and $9.5 million, respectively, during the comparable 2020 periods. The increase of $0.8 million during the 2021 nine-month period was primarily due to higher fees from outside legal counsel and other costs, partially offset by lower employee-related expenses.

Investment Income, net: Investment income, net for the three and nine months ended March 31, 2021 declined by $0.3 million and $1.0 million, respectively, when compared to the comparable 2020 periods, which was primarily due to lower interest rates in the marketplace.

Interest Expense: Interest expense for the three and nine months ended March 31, 2021 increased by $0.3 million and $1.0 million, respectively, when compared to the comparable 2020 periods due to the loan agreement that the Company entered into on June 30, 2020.

Net Loss: The Company's net loss for the three and nine months ended March 31, 2021 was $14.9 million and $45.7 million, respectively, compared to $11.2 million and $31.4 million, respectively, during the comparable 2020 periods.

Financial Guidance: As of March 31, 2021, the Company's cash, cash equivalents and investments totaled $111.0 million. The Company believes that these funds, plus $10.0 million in additional borrowings under the recently amended loan agreement with Hercules Capital., will be sufficient to allow the Company to generate data from its ongoing and planned clinical programs, support the Company's strategic investment in a cGMP manufacturing and quality control facility, and fund currently planned research and discovery programs into calendar year 2023.

R&D Day Information

AGTC plans to review the data and provide the latest updates on the XLRP and achromatopsia clinical programs at an R&D Day on Thursday, July 22, 2021 beginning at 10:00 a.m. ET. AGTC management and clinical trial investigators will present.

Chief Financial Officer Transition

AGTC announced today that Bill Sullivan, Chief Financial Officer and Treasurer, will be leaving the Company effective June 9, 2021 to pursue the same position at an early-stage oncology company.

"Bill has been instrumental in leading our finance team through multiple rounds of financings, enabling us to advance our best-in-class clinical and preclinical programs," said Ms. Washer. "On behalf of the Company, I wish Bill continued success and thank him for his many contributions to AGTC."

"I am grateful for my time at AGTC and I have really enjoyed working with Sue and the entire team at AGTC," said Mr. Sullivan. "With a strong balance sheet, differentiated XLRP data, the recent strategic investment in manufacturing, and a promising pipeline, the future for AGTC is bright. I sincerely look forward to seeing AGTC achieve future success."

Jerry Reynolds, Vice President, Accounting, has been appointed to serve as Chief Accounting Officer and Treasurer, effective upon Mr. Sullivan's departure, and will assume certain key financial responsibilities for AGTC, including SEC reporting, which are consistent with his current responsibilities. The finance and accounting team will report to Mr. Reynolds, who will report directly to Ms. Washer.

The Company has initiated a search process to identify a new Chief Financial Officer.

Conference Call and Webcast

AGTC will host a conference call and webcast to discuss financial results for the quarter ended March 31, 2021 today at 4:30 p.m. 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. AGTC'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 and 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 press 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, the potential of its ACHM clinical programs and its ability to enroll pediatric patients, and AGTC's planned build-to-suit lease, the expected timing for the build-out of the facility subject to the lease and its potential to support AGTC's pipeline 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 AGTC's 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: risks related to new construction; 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
CONDENSED BALANCE SHEETS
(Unaudited)

In thousands, except per share data	March 31, 2021	June 30, 2020
ASSETS		
Current assets:		
Cash and cash equivalents	$ 89,531	$ 38,463
Investments	21,496	41,995
Prepaid and other current assets	1,997	2,506
Total current assets	113,024	82,964
Property and equipment, net	4,019	4,311
Intangible assets, net	1,291	1,098
Investment in Bionic Sight, LLC	8,021	8,096
Right-of-use assets – operating leases	3,158	3,422
Right-of-use asset – finance lease	46	80
Other assets	107	348
Total assets	$ 129,666	$ 100,319
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,926	$ 1,355
Accrued and other liabilities	13,504	10,502
Lease liabilities – operating	1,072	1,058
Lease liability – finance	51	48
Total current liabilities	16,553	12,963
Lease liabilities – operating, net of current portion	3,540	4,070
Lease liability – finance, net of current portion	—	38
Long-term debt, net of debt discounts and deferred financing fees	9,929	9,677
Other liabilities	2,644	2,555

Total liabilities	32,666	29,303
Stockholders' equity:		
Preferred stock	—	—
Common stock	42	25
Additional paid-in capital	324,302	252,519
Treasury stock	(211)	(88)
Accumulated deficit	(227,133)	(181,440)
Total stockholders' equity	97,000	71,016
Total liabilities and stockholders' equity	$ 129,666	$ 100,319

APPLIED GENETIC TECHNOLOGIES CORPORATION
CONDENSED STATEMENTS OF OPERATIONS
(Unaudited)

	Three Months Ended March 31,		Nine Months Ended March 31,	
In thousands, except per share data	**2021**	**2020**	**2021**	**2020**
Revenue:				
Collaboration and milestone revenue	$ —	$ —	$ —	$ 2,297
Grant revenue	—	—	—	156
Total revenue	—	—	—	2,453
Operating expenses:				
Research and development	10,960	8,308	34,397	25,325
General and administrative and other	3,528	3,134	10,268	9,490
Total operating expenses	14,488	11,442	44,665	34,815
Loss from operations	(14,488)	(11,442)	(44,665)	(32,362)
Other income (expense), net:				
Investment income, net	13	281	106	1,063
Interest expense	(330)	(2)	(997)	(6)
Other income	—	6	—	6
Total other income (expense), net	(317)	285	(891)	1,063
Loss before provision for income taxes	(14,805)	(11,157)	(45,556)	(31,299)
Provision for income taxes	21	21	62	63
Loss before equity in net losses of an affiliate	(14,826)	(11,178)	(45,618)	(31,362)
Equity in net losses of an affiliate	(25)	(11)	(75)	(27)
Net loss	$ (14,851)	$ (11,189)	$ (45,693)	$ (31,389)
Weighted average shares outstanding:				
Basic	36,751	22,272	29,431	19,558
Diluted	36,751	22,272	29,431	19,558
Net loss per common share:				
Basic	$ (0.40)	$ (0.50)	$ (1.55)	$ (1.60)
Diluted	$ (0.40)	$ (0.50)	$ (1.55)	$ (1.60)

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