May 10, 2017

AGTC Announces Financial Results and Business Update for the Quarter Ended March 31, 2017

GAINESVILLE, Fla. and CAMBRIDGE, Mass., May 10, 2017 (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, 2017.

"In the past quarter, we completed enrollment in the dose escalation portion of the Phase 1/2 clinical trial for our XLRS product candidate and are scheduling patients for enrollment in XLRS as well as for ACHMB3 and ACHMA3 Phase 1/2 clinical trials," said Sue Washer, President and CEO of AGTC. "We are also looking forward to filing the IND for our XLRP program later in 2017 and continue to advance our preclinical programs in these potentially transformative therapies."

AGTC's lead product candidates are designed to treat inherited orphan diseases of the eye, caused by mutations in single genes that significantly affect visual function and currently lack effective medical treatments.

AGTC's pipeline includes ophthalmology programs in X-linked retinoschisis (XLRS), X-linked retinitis pigmentosa (XLRP), achromatopsia (ACHM) caused by mutations in the CNGB3 and CNGA3 genes, wet age-related macular degeneration and an optogenetics program.

Recent Highlights

- The company is scheduling patients for enrollment in the expansion group of the Phase 1/2 clinical trial for its XLRS product candidate at the highest of the three dose levels tested to date.
- AGTC is scheduling patients for enrollment in both its ACHMB3 and ACHMA3 Phase 1/2 clinical trials.
- The company and Bionic Sight are preparing for a pre-IND meeting with the FDA to discuss the IND-enabling studies for an optogenetic product candidate for patients with advanced retinal disease.
- AGTC continues to progress on its initiative in capsid and promoter selection, assay development and process development, keeping the company at the forefront of AAV technology. Testing in non-human primates comparing novel capsids to naturally occurring capsids is ongoing and preliminary cell culture testing of novel synthetic promoters is demonstrating promising results.
- AGTC was awarded U.S. patent 9,534,225, entitled "Condon optimized nucleic acid encoding a retinitis pigmentosa GTPase regulator (RPGR)." This patent adds to the intellectual property portfolio related to the company's XLRP product candidate.

XLRS Phase 1/2 Clinical Trial

AGTC is currently enrolling patients in a Phase 1/2 clinical trial for its XLRS product candidate as part of the company's collaboration with Biogen.

To date, the company has completed dosing in the first three groups. The company expects to provide a data update, across both safety and potential efficacy endpoints, for these first 12 patients this summer.

The Data Safety and Monitoring Committee (DSMC) met recently to review the current safety data set including the most recent high-dose group and the Company has initiated enrollment in the expansion group at the high dose.

The primary endpoint of this clinical trial is safety, and available data thus far have shown that AGTC's XLRS product candidate has been generally well tolerated. As previously disclosed, mild to moderate ocular inflammation occurred in the majority of treated patients and resolved either without treatment or after treatment with topical or oral corticosteroids. The frequency and intensity of inflammation to date has been similar at all three dose levels tested.

ACHM B3 Phase 1/2 Clinical Trial

A Phase 1/2 clinical trial evaluating the company's ACHMB3 product candidate is enrolling. The company has enrolled three patients in the low dose group. The company plans to enroll two additional patients in the low dose group who will both be treated by a surgeon who treated one of the earlier patients in this group, such that a total of three patients will be treated by a single surgeon.

ACHM A3 Phase 1/2 Clinical Trial

Based on the experience with the ACHMB3 trial, the Company plans to use a single surgeon in the near term to treat patient in the ACHMA3 trial. Screening is on-going and the company does not expect enrollment to be limited by enrollment of the additional patients in the ACHMB3 trial.

Preclinical Programs

For the X-linked retinitis pigmentosa, or XLRP, program, IND-enabling toxicology studies are underway in two relevant disease models. After completion of these studies, the company expects to submit an IND to the FDA in the third quarter of 2017.

In January of this year the company entered into a collaboration agreement with Bionic Sight to develop an optogenetic product candidate for patients with advanced retinal disease. Through the AGTC-Bionic Sight collaboration, the companies will seek to develop a new optogenetic therapy that leverages AGTC's deep experience in gene therapy and ophthalmology and Bionic Sight's innovative neuro-prosthetic device and algorithm for retinal coding. The company and Bionic Sight intend to request a pre-IND meeting with the FDA to discuss the IND-enabling studies for this program and the expectation is that an IND will be filed in 2018.

Financial Results for the Quarter Ended March 31, 2017

Total revenue for the three months ended March 31, 2017 was $8.4 million compared to $12.0 million generated during the same period in 2016. Collaboration revenue recorded during the three months ended March 31, 2017 and 2016 resulted primarily from the amortization of upfront fees received under our collaboration agreement with Biogen which began in August 2015. Grant revenue decreased $24 thousand during the three months ended March 31, 2017 compared to the same period in 2016, largely attributable to reduced research and development activities on grant-funded projects.

Research and development expense for the three months ended March 31, 2017 decreased by $1.6 million to $6.3 million compared to the same period in 2016. The period-over-period decrease was largely driven by the reduction of licenses and related fees which were lower during the three months ended March 31, 2017 compared to the three months ended March 31, 2016 due to technology access fees that were incurred in 2016 and did not recur in 2017. In addition, outside program costs decreased during the three months ended March 31, 2017 compared to the three months ended March 31, 2016, due largely to temporary delays encountered during the conduct of Phase 1/2 human clinical trials for our XLRS and CNGB3-related ACHM product candidates. The impact of these decreases was partially offset by higher employee-related costs that were driven primarily by hiring additional employees.

General and administrative expense for the three months ended March 31, 2017 increased by $0.5 million to $2.9 million compared to the same period in 2016. The increase was primarily driven by higher share-based compensation and employee-related costs which resulted from hiring additional employees to support our continued expansion. Nominal increases in professional fees and other expenses were noted during the three months ended March 31, 2017 compared to the three months ended March 31, 2016.

For the three months ended March 31, 2017, the company recorded a net loss after income taxes of $0.8 million, compared to net income after taxes of $2.0 million for the same period of 2016.

Financial Results for the Nine Months Ended March 31, 2017

Total revenue for the nine months ended March 31, 2017 was $31.1 million compared to $35.2 million generated during the same period in 2016. Collaboration revenue recorded during the nine months ended March 31, 2017 and 2016 resulted primarily from the amortization of upfront fees received under our collaboration agreement with Biogen which began in August 2015. Grant revenue decreased $0.4 million during the nine months ended March 31, 2017 compared to same period in 2016, largely attributable to reduced research and development activities on grant-funded projects.

Research and development expense for the nine months ended March 31, 2017 decreased by $14.2 million to $17.9 million compared to the same period in 2016. The decrease was largely driven by the impact of collaboration-related costs and license fees that were incurred in 2016 as a result of our entry into and receipt of fees and payments under the collaboration agreement with Biogen and which did not recur in 2017. Also, licenses and related fees decreased during the nine months ended March 31, 2017 compared to the nine months ended March 31, 2016 due to technology access fees

that were incurred in 2016 and did not recur in 2017. In addition, outside program costs decreased in 2017 compared to 2016 due largely to temporary delays encountered during the conduct of Phase 1/2 human clinical trials for our XLRS and CNGB3-related ACHM product candidates. The impact of these decreases was partially offset by higher employee-related and share-based compensation expenses that were driven primarily by hiring additional employees and the incremental impact of new share-based incentives awarded in 2017, as well as higher laboratory supplies and other costs during 2017, when compared to 2016.

General and administrative expense for the nine months ended March 31, 2017 increased by $0.8 million to $8.5 million compared to the same period in 2016. The increase was primarily driven by higher employee-related and share-based compensation costs which resulted from hiring additional employees to support our continued expansion, partially offset by lower legal and professional fees and other expenses. The higher legal and professional fees incurred in 2016 were largely attributable to professional consultations associated with the negotiation and entry into our collaboration with Biogen.

For the nine months ended March 31, 2017, the company recorded net income after income taxes of $4.9 million, compared to a net loss after income taxes of $4.1 million in the same period of 2016.

As of March 31, 2017, the company's cash, cash equivalents, and investments amounted to $148.7 million. The company believes these cash, cash equivalents, and investments will be sufficient to enable it to advance planned preclinical studies and clinical trials for its lead product candidates and currently planned discovery programs for at least the next two years.

Conference Call and Webcast

AGTC will host a conference call and webcast to discuss financial results for the third fiscal quarter ended March 31, 2017 today at 4:30pm ET. To access the call, dial 866-565-7742 (US) or 614-999-1914 (outside of the US). The passcode is 14485210. A live webcast will be available in the Events and Presentations section of AGTC's Investor Relations site at http://ir.agtc.com/events.cfm. 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 its proprietary gene therapy platform to develop treatments designed to transform the lives of patients with severe diseases, with an initial focus in ophthalmology. AGTC's product pipeline includes ophthalmology programs in X-linked retinoschisis (XLRS), X-linked retinitis pigmentosa (XLRP), both partnered with Biogen, achromatopsia caused by mutations in the CNGB3 and CNGA3 genes, wet age-related macular degeneration, and our collaborative optogenetics program with Bionic Sight. AGTC's non-ophthalmology programs include its adrenoleukodystrophy program, also partnered with Biogen, and its otology research program. The otology programs are in pre-clinical development and the company expects to advance several otology product candidates into clinical development in the next few years. AGTC employs a highly-targeted approach to selecting and designing its product candidates, choosing to develop therapies for indications having high unmet medical need that it believes are clinically feasible and present commercial opportunities. 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 treatments.

About X-linked Retinoschisis (XLRS)

XLRS is an inherited retinal disease caused by mutations in the RS1 gene, which encodes the retinoschisin protein. It is characterized by abnormal splitting of the layers of the retina, resulting in poor visual acuity in young boys, which can progress to legal blindness in adult men.

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.

About X-linked Retinitis Pigmentosa (XLRP)

XLRP is an inherited condition that causes boys to develop night blindness by the time they are ten and progresses to legal blindness by their early forties.

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, 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: no gene therapy products have been approved in the United States and only two such products have been approved in Europe; AGTC cannot predict when or if it will obtain regulatory approval to commercialize a product candidate; uncertainty inherent in 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, 2016, as amended and 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
CONDENSED BALANCE SHEETS
(Unaudited)

In thousands, except per share data	March 31, 2017	June 30, 2016
ASSETS		
Current assets:		
Cash and cash equivalents	$ 35,500	$ 28,868
Investments	96,228	69,664
Grants receivable	169	954
Prepaid and other current assets	3,797	3,089
Total current assets	135,694	102,575
Investments	16,995	74,183
Property and equipment, net	2,720	2,627
Intangible assets, net	1,264	1,321
Other assets	2,107	91
Total assets	$ 158,780	$ 180,797
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 817	$ 1,331
Accrued and other liabilities	6,372	6,514
Deferred revenue	24,818	46,898
Total current liabilities	32,007	54,743
Deferred revenue, net of current portion	8,411	16,766
Total liabilities	40,418	71,509
Stockholders' equity:		
Common stock—par value $.001 per share; shares authorized: 150,000 at March 31, 2017 and June 30, 2016; shares issued and outstanding: 18,084 and 18,077, respectively, at March 31, 2017; shares issued outstanding: 18,053 and 18,048, respectively, at June 30, 2016.	18	18
Additional paid-in capital	203,492	199,303
Accumulated deficit	(85,148)	(90,033)
Total stockholders' equity	118,362	109,288
Total liabilities and stockholders' equity	$ 158,780	$ 180,797

APPLIED GENETIC TECHNOLOGIES CORPORATION
CONDENSED STATEMENTS OF OPERATIONS
(Unaudited)

In thousands, except per share amounts	For the Three Months Ended March 31, 2017	For the Three Months Ended March 31, 2016	For the Nine Months Ended March 31, 2017	For the Nine Months Ended March 31, 2016
Revenue:				
Collaboration revenue	$ 8,297	$ 11,882	$ 30,959	$ 34,717
Grant and other revenue	91	115	169	531
Total revenue	8,388	11,997	31,128	35,248
Operating expenses:				
Research and development	6,303	7,868	17,916	32,108
General and administrative and other	2,921	2,362	8,507	7,716
Total operating expenses	9,224	10,230	26,423	39,824
(Loss) income from operations	(836)	1,767	4,705	(4,576)
Other income:				
Investment income, net	236	202	700	467
Total other income, net	236	202	700	467
Provision for income taxes	221	—	519	—
Net (loss) income	$ (821)	$ 1,969	$ 4,886	$ (4,109)
Net (loss) income per share, basic and diluted	$ (0.05)	$ 0.11	$ 0.27	$ (0.23)
Weighted average shares outstanding - basic	18,081	18,033	18,068	17,735
Weighted average shares outstanding - diluted	18,081	18,472	18,408	17,735

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