AGTC Announces Financial Results and Business Update for the Fourth Quarter and Fiscal Year Ended June 30, 2019

September 26, 2019

Company announces promising topline six-month dose escalation data from its ongoing Phase 1/2 clinical trials for X-linked retinitis pigmentosa (XLRP)

Preliminary three-month XLRP dose expansion data demonstrates improvement in 50% of centrally dosed patients

Early data from both achromatopsia (ACHM) programs indicate biologic activity

Company to host conference call and webcast with slides today at 4:30pm ET

GAINESVILLE, Fla., and CAMBRIDGE, Mass., Sept. 26, 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 fourth quarter and fiscal year ended June 30, 2019. The company also provided topline six-month data from the dose escalation groups of the Phase 1/2 XLRP program as planned. Additionally, the Company shared a preliminary look at three-month data from the XLRP dose expansion group. Both data sets demonstrated a favorable safety profile with stability of visual function in peripherally dosed patients and improvement of visual function in 50% of centrally dosed patients. AGTC also provided an early look at data from the achromatopsia B3 and A3 trials showing signs of clinically meaningful improvements in light discomfort.

"The encouraging signs of stable or improved visual function, as measured by visual fields and visual acuity analysis in the XLRP trial and improvements in light discomfort in both ACHM trials, further increase our confidence in the promise of our gene therapies to make a difference to patients in need," said Sue Washer, President and CEO of AGTC. "Taken together, we believe these data put AGTC in a favorable position to move its current clinical programs forward and bring important therapies to patients who today lack effective treatment options. AGTC has already engaged in pivotal trial planning as well as assay validation and manufacturing, such that we will be ready to move forward rapidly as soon as the remaining data are available. We are also announcing important new programs in our preclinical pipeline that leverage our unique capabilities in vector design and manufacturing, and provide a significant platform for growth beyond our current clinical programs."

AGTC Clinical Program Update

X-linked Retinitis Pigmentosa (XLRP) Phase 1/2 Clinical Trial
A total of 22 patients have been dosed to date. Safety data from all 22 patients continue 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. Data from 17 of the 22 patients, comprising top-line interim efficacy results for ten patients (eight dosed peripherally and two dosed centrally) who have been analyzed at the six-month time point and preliminary data from an additional seven centrally dosed patients who have been analyzed at the three-month time point demonstrated the following results:

- The eight patients from the dose escalation portion of the study who were dosed peripherally showed stable visual function at six months as measured by visual fields and best corrected visual acuity (BCVA).
- Preliminary data from nine centrally dosed patients at the three-month time point (seven who have completed three-month evaluation and two who have completed six-month evaluation) showed evidence of improvement in visual function.

  - Measurable improvements were observed in visual sensitivity for four of the eight (50%) patients (one centrally dosed patient was not evaluable for visual sensitivity).
  - Patients with improvement in visual sensitivity within the treatment area that is beyond the testing variability on at least two different test dates are defined as responders.
  - Three of eight (38%) patients had greater than five loci in the treated area with at least a 7 decibel increase from baseline, at three months compared with 33% reported by others.
  - All nine patients treated centrally also had stable or improving visual acuity, a result which has not been reported by others.

"Maintenance of peripheral vision and improvements in central vision are important and clinically meaningful. Reductions in these capabilities are hallmarks of this degenerative disease and negatively impact patients' ability to engage in daily living and work." said Dr David Birch, Scientific Director, Retinal Foundation of the Southwest.

AGTC believes that the biologic activity observed to date in this clinical trial, especially if sustained through the 12-month timepoint, supports our belief that this treatment may offer a stabilization of peripheral vision as well as improvement in central vision with a favorable safety profile that could be of tremendous clinical benefit to patients.

Achromatopsia (ACHM) Phase 1/2 Clinical Trials
To date, 27 patients have been dosed across the B3 and A3 Phase 1/2 trials. Safety data from these patients continue to demonstrate a favorable profile for each of the ACHM candidates, and the Data Safety Monitoring Committee (DSMC) has supported continued dose escalation and dosing of pediatric patients. Early data suggest meaningful benefit from treatment for the patients. 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.

"The evidence of biological activity observed in both the XLRP and ACHM programs furthers our confidence in the design of these trials and the potential to advance our programs to pivotal trials." said Theresa Heah, MD, MBA, Chief Medical Officer. "As we analyze the ACHM data, our belief is that we will see this type of meaningful improvement in light discomfort as well as other measures of visual function for these patients."

Preclinical Programs
As AGTC's clinical programs demonstrate the potential of its technology platform, the company continues to advance a diverse portfolio of preclinical programs, including two ophthalmology programs, one of which targets the dry form of age-related macular degeneration (AMD), which accounts for 90 percent of the 24 million AMD cases globally; continued research in the field of otology, and three programs targeting central nervous system (CNS) disorders. The CNS programs 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. During today's conference call, the company will briefly review these programs, which leverage the company's deep capabilities in AAV gene therapy manufacturing and technology and represent important opportunities to create unique therapeutic solutions for patients.

Financial Results for the Fourth Quarter and Fiscal Year Ended June 30, 2019

Revenues: Revenue was $0.4 million for the fourth quarter of 2019 and $41.7 million for the year ended June 30, 2019, compared to $5.4 million and $24.2 million in the comparable periods in fiscal year 2018. Revenue primarily consists of license and related services, development services and milestone revenue. The increase of $17.5 million for the year ended June 30, 2019 compared to the same period in 2018 was primarily due to recognizing revenue of $20.4 million as a result of the termination of the Collaboration Agreement with Biogen, and recognizing revenue of $8.3 million associated with the receipt of a $10.0 million milestone payment from Biogen during the first quarter of fiscal year 2019, partially offset by decreased license and related service revenue due to the Company's revised pattern of revenue recognition under ASC 606, Revenue from Contracts with Customers.

R&D Expenses: Research and development expenses were $8.3 million for the fourth quarter of 2019 and $33.2 million for the year ended June 30, 2019, compared to $8.8 million and $32.2 million in the comparable periods in fiscal year 2018. The increase of $1.0 million for the full year was primarily due to incurring sublicense expense of $2.3 million associated with receiving a milestone payment from Biogen and increased employee-related costs, partially offset by decreased pre-clinical R&D spending.

G&A Expenses: General and administrative expenses were $3.5 million for the fourth quarter of 2019 and $12.9 million for the fiscal year ended June 30, 2019, compared to $3.4 million and $14.4 million in the comparable periods in fiscal year 2018. The decrease in general and administrative expenses for the full year was primarily driven by decreased employee-related and share-based compensation expenses.

Net Loss: Net loss was $10.5 million for the fourth quarter of 2019 and $2.0 million for the year ended June 30, 2019, compared to net loss of $6.6 million and $21.3 million in the comparable periods in 2018.

Financial Guidance: As of June 30, 2019, the company's cash, cash equivalents, and investments amounted to $82.0 million. The company believes these funds will be sufficient to allow AGTC to generate data from its ongoing clinical programs, to move the pre-clinical optogenetic program in collaboration with Bionic Sight into the clinic 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.

Conference Call and Webcast

AGTC will host a conference call and webcast with accompanying slides to review the Phase 1/2 XLRP and ACHM data and discuss financial results for the fourth quarter and fiscal year ended June 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 and slide presentation 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, where 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 otology indications. The optogenetics program is being developed in collaboration with Bionic Sight. 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 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 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.

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, 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	June 30, 2019	June 30, 2018
ASSETS		
Current assets:		
Cash and cash equivalents	$ 26,703	$ 31,065
Investments	55,292	73,840
Grants receivable	13	210
Prepaid and other current assets	2,276	4,009
Total current assets	84,284	109,124
Property and equipment, net	4,430	5,254
Intangible assets, net	1,013	968
Investment in Bionic Sight	1,945	1,980
Other assets	544	1,206
Total assets	$ 92,216	$ 118,532
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,331	$ 945
Accrued and other liabilities	8,024	7,155
Deferred revenue	—	6,295
Total current liabilities	9,355	14,395
Deferred revenue, net of current portion	—	610
Other liabilities	4,152	4,345
Total liabilities	13,507	19,350
Stockholders' equity:		
Common stock, par value $.001 per share, 150,000 shares authorized; 18,226 and 18,137 shares issued; 18,207 and 18,126 shares outstanding at June 30, 2019 and June 30, 2018, respectively	18	18
Additional paid-in capital	214,324	210,139
Shares held in treasury of 19 and 11 at June 30, 2019 and June 30, 2018, respectively	(85)	(49)
Accumulated deficit	(135,548)	(110,926)
Total stockholders' equity	78,709	99,182
Total liabilities and stockholders' equity	$ 92,216	$ 118,532

APPLIED GENETIC TECHNOLOGIES CORPORATION
STATEMENTS OF OPERATIONS
(Unaudited)

In thousands, except per share amounts	Three Months Ended June 30, 2019	2018	Year Ended June 30, 2019	2018
Revenue:				
Collaboration revenue	$ –	$ 5,331	$ 41,128	$ 24,057
Grant and other revenue	406	85	564	129
Total revenue	406	5,416	41,692	24,186
Operating expenses:				
Research and development	8,332	8,827	33,183	32,181
General and administrative and other	3,514	3,368	12,859	14,389
Total operating expenses	11,846	12,195	46,042	46,570
Income/(loss) from operations	(11,440)	(6,779)	(4,350)	(22,384)
Other income:				
Investment income, net	505	435	2,009	1,301
Other expense	446	(114)	446	(125)
Total other income, net	951	321	2,455	1,176
Income/(loss) before provision for income taxes	(10,489)	(6,458)	(1,895)	(21,208)
Provision/(benefit) for income taxes	19	138	76	72
Income/(loss) before equity in net losses of affiliate	(10,508)	(6,596)	(1,971)	(21,280)
Equity in net losses of affiliate	(6)	(16)	(35)	(20)
Net income/(loss)	$ (10,514)	$ (6,612)	$ (2,006)	$ (21,300)
Weighted Average Shares Outstanding				
Weighted average shares outstanding - basic	18,184	18,112	18,157	18,105
Weighted average shares outstanding - diluted	18,184	18,112	18,157	18,105
Net earnings/(loss) per common share				
Net earnings/(loss) per share, basic	$ (0.58)	$ (0.36)	$ (0.11)	$ (1.18)
Net earnings/(loss) per share, diluted	$ (0.58)	$ (0.36)	$ (0.11)	$ (1.18)

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