



## AGTC Announces Financial Results and Business Update for the Quarter Ended March 31, 2018

May 8, 2018

GAINESVILLE, Fla. and CAMBRIDGE, Mass., May 08, 2018 (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, 2018.

"Along with our collaboration partner, Biogen, we have achieved several critical milestones recently, including the completion of enrollment in our XLRs Phase 1/2 clinical trial and dosing the first patient in our XLRP trial," said Sue Washer, president and CEO of AGTC. "We expect these positive trends to continue as we realize improved execution through enhanced clinical operations, clinical outreach and patient advocacy efforts."

### Recent Highlights

- AGTC completed enrollment of the Phase 1/2 clinical study of investigational gene therapy in patients with X-linked retinoschisis (XLRs), in collaboration with Biogen.
- AGTC dosed the first patient in the Phase 1/2 clinical trial evaluating the safety and biologic activity of an investigational AAV-based gene therapy for the treatment of X-linked retinitis pigmentosa caused by mutations in the RPGR gene (XLRP) and, as a result, received a \$2.5M milestone payment under its collaboration agreement with Biogen.
- In March, AGTC sponsored a subretinal surgical summit in Chicago, Illinois, to discuss and develop best practices for subretinal administration of viral vectors in gene therapy.

### Clinical Programs

The company has four ongoing clinical trials.

#### XLRs Phase 1/2 Clinical Trial

In April, AGTC completed its target enrollment of 27 patients in the Phase 1/2 clinical trial for its XLRs product candidate as part of the company's collaboration with Biogen.

The primary endpoint of this clinical trial is safety, and available data thus far have shown that the XLRs product candidate is generally safe and well tolerated. In addition to safety, this trial will measure biologic activity by assessing changes in visual function, retinal structure and quality of life. The company expects to provide topline six-month data across both safety and biologic activity endpoints by the end of 2018 with the primary analysis of the full twelve-month active trial data six months later.

#### XLRP Phase 1/2 Clinical Trial

In April, the company enrolled the first patient in a Phase 1/2 clinical trial of its product candidate for XLRP and earned a milestone payment of \$2.5M from Biogen as part of its ongoing collaboration. The company has two sites open for enrollment in this trial with more sites expected to open over the next several months. The company expects to complete the dose escalation portion of the XLRP trial in the first quarter of 2019.

#### ACHM Phase 1/2 Clinical Trials

The company is presently enrolling patients in two parallel Phase 1/2 clinical trials of its product candidates for achromatopsia (ACHM) caused by mutations in the two most common ACHM genes, CNGB3 and CNGB3. In the ACHM CNGB3 trial, AGTC has completed enrollment of four patients in one dose group and one patient in a new lower dose group. The company expects to complete the dose escalation portion of the CNGB3 trial in the first quarter of 2019. The ACHM CNGB3 trial has enrolled one patient.

#### Surgical Summit and Clinical Infrastructure Enhancements

AGTC has taken several steps during the past quarter to more predictably and efficiently enroll patients in all of the company's ongoing clinical trials. This included expanding patient recruitment efforts, initiating new clinical trial sites and enhancing clinical support and infrastructure activities. In March, the company also sponsored a subretinal surgical summit that brought together highly experienced, world-renowned vitreoretinal surgeons to discuss best practices for subretinal surgery techniques used in gene therapy. Information from this summit will be used to optimize the subretinal delivery of AGTC's ACHM and XLRP product candidates and to educate and train additional surgeons in subretinal delivery.

#### AGTC-Bionic Sight Collaboration

Through the AGTC-Bionic Sight collaboration, the companies are pursuing the development of a novel optogenetic therapy that leverages AGTC's extensive experience in gene therapy and ophthalmology and Bionic Sight's pioneering neuro-prosthetic device and algorithm for retinal coding to develop a product to treat patients with advanced retinal disease. AGTC is working with Bionic Sight to file their IND for this product candidate, which the companies anticipate will take place in the second half of 2018.

### Financial Results for the Three and Nine Months Ended March 31, 2018

**Revenues:** Total revenue was \$3.6 million for the three months ended March 31, 2018 and \$18.8 million for the nine months ended March 31, 2018, compared to \$8.4 million and \$31.1 million in the comparable periods in 2017. Revenue primarily consists of non-refundable upfront fees received under the company's collaboration with Biogen, which are amortized to collaboration revenue on a straight-line basis over the estimated service period, and development services revenue, which primarily consists of reimbursement of development activities under the Biogen Collaboration. Amortization revenue decreased \$14.1 million for the nine months ended March 31, 2018 compared to the same period in 2017 primarily due to reaching the end of the XLRP service period in the first quarter of fiscal year 2018, and to a lesser extent, due to changes in estimates associated with the period of performance under the XLRs and preclinical programs. Development services revenue increased \$1.9 million for the nine months ended

March 31, 2018 compared to the same period in 2017 primarily due to activities associated with the initiation of a Phase 1/2 clinical trial for XLRP.

**R&D Expenses:** Research and development expenses were \$7.4 million for the three months ended March 31, 2018 and \$23.4 million for the nine months ended March 31, 2018, compared to \$6.3 million and \$17.9 million in the comparable periods in 2017. The increase for the nine-month period was primarily due to increased spending on general research and discovery programs, increased spending on the company's clinical programs and increased employee-related expenses associated with the hiring of additional employees to support clinical trial execution and research and development activities.

**G&A Expenses:** General and administrative expenses were \$3.9 million for the three months ended March 31, 2018 and \$11.0 million for the nine months ended March 31, 2018, compared to \$2.9 million and \$8.5 million in the comparable periods in 2017. The increase for the nine-month period was primarily due to increased employee-related and corporate expenses to support the company's continued expansion.

**Tax Provision:** For the three and nine months ended March 31, 2018, the company recorded an income tax provision of \$0.7 million and (\$66,000), respectively. For the same periods in 2017, the income tax provision was \$0.6 million and \$1.8 million. The income tax expense for the three months ended March 31, 2018 was primarily driven by the apportionment of income to certain state jurisdictions where the company had not generated net operating losses (NOL's). The income tax benefit for the nine months ended March 31, 2018 was primarily due to certain tax credit carryforwards becoming refundable under The Tax Cuts and Jobs Act of 2017, offset by income tax expense for the three months ended March 31, 2018.

**Net Income or Loss:** The net loss was \$8.1 million for the three months ended March 31, 2018 and the net loss was \$14.7 million for the nine months ended March 31, 2018 compared to a net loss of \$1.2 million and net income \$3.6 million in the comparable periods in 2017.

**Financial Guidance:** As of March 31, 2018, the company's cash, cash equivalents and investments amounted to \$111.8 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 for at least the next two years. The company expects total cash, cash equivalents and investments as of June 30, 2018 to be between \$95 and \$105 million. These projected cash balances include the \$2.5 million milestone payment, less sublicensing fees of approximately 23%, associated with dosing of the first patient in the XLRP trial, but do not include the \$10 million milestone payment, less such fees, associated with dosing of the fourth patient in the XLRP trial, as we are uncertain as to the timing of this milestone payment.

#### **Conference Call and Webcast**

AGTC will host a conference call and webcast to discuss financial results for the third fiscal quarter ended March 31, 2018 today at 4:30pm ET. To access the call, dial 877-407-6184 (US) or 201-389-0877 (outside of the US). The passcode is 13678555. 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, where it has active clinical trials in X-linked retinoschisis (XLRs), 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 otology. The clinical-stage XLRs and XLRP programs, the discovery program in ALD and two additional ophthalmology programs are being developed in collaboration with Biogen. In addition to its product pipeline, 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 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 reduced 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: 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, 2017, 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	<b>March 31, 2018</b>	<b>June 30, 2017</b>
<b>ASSETS</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 47,633	\$ 30,706
Investments	64,167	95,994
Grants receivable	133	174
Prepaid and other current assets	3,968	3,361
Total current assets	115,901	130,235
Investments, net of current portion	—	11,749
Property and equipment, net	5,526	2,661
Investment in Bionic Sight	1,995	2,000
Other assets	2,025	1,278
<b>Total assets</b>	<b>\$ 125,447</b>	<b>\$ 147,923</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
<b>Current liabilities:</b>		
Accounts payable	\$ 1,322	\$ 998
Accrued and other liabilities	8,035	6,162
Deferred revenue	8,188	20,996
Total current liabilities	17,545	28,156
Deferred revenue, net of current portion	912	4,438
Other liabilities	2,294	—
<b>Total liabilities</b>	<b>20,751</b>	<b>32,594</b>
<b>Stockholders' equity:</b>		
Common stock—par value \$.001 per share; shares authorized: 150,000: shares issued and outstanding: 18,121 and 18,088 at March 31, 2018 and June 30, 2017, respectively	18	18
Additional paid-in capital	209,015	204,937
Shares held in treasury of: 6 and 0 at March 31, 2018 and June 30, 2017 respectively	(23 )	— )
Accumulated deficit	(104,314 )	(89,626 )
Total stockholders' equity	104,696	115,329
<b>Total liabilities and stockholders' equity</b>	<b>\$ 125,447</b>	<b>\$ 147,923</b>

**APPLIED GENETIC TECHNOLOGIES CORPORATION**  
**CONDENSED STATEMENTS OF OPERATIONS**  
**(Unaudited)**

In thousands, except per share amounts	<b>For the Three Months Ended March 31,</b>		<b>For the Nine Months Ended March 31,</b>	
	<b>2018</b>	<b>2017 (as adjusted)</b>	<b>2018</b>	<b>2017 (as adjusted)</b>
<b>Revenue:</b>				
Collaboration revenue	\$ 3,588	\$ 8,297	\$ 18,727	\$ 30,959
Grant and other revenue	15	91	43	169
Total revenue	3,603	8,388	18,770	31,128
<b>Operating expenses:</b>				
Research and development	7,353	6,303	23,355	17,916
General and administrative and other	3,946	2,921	11,020	8,507
Total operating expenses	11,299	9,224	34,375	26,423
Income (loss) from operations	(7,696 )	(836 )	(15,605 )	4,705

**Other income:**

Investment income, net	325	236	866	700
Other expense	—	—	(10)	—
Total other income, net	325	236	856	700
Income (loss) before provision for income taxes and equity in net earnings (losses) of affiliate	(7,371)	(600)	(14,749)	5,405
Provision (benefit) for income taxes	725	600	(66)	1,800
Income (loss) before equity in net losses of affiliate	(8,096)	(1,200)	(14,683)	3,605
Equity in net losses of affiliate	(5)	—	(5)	—
Net income (loss)	\$ (8,101)	\$ (1,200)	\$ (14,688)	\$ 3,605

**Weighted Average Shares Outstanding**

Weighted average shares outstanding - basic	18,112	18,081	18,098	18,068
Weighted average shares outstanding - diluted	18,112	18,081	18,098	18,408

**Net income (loss) per common share**

Net income (loss) per share, basic	\$ (0.45)	\$ (0.07)	\$ (0.81)	\$ 0.20
Net income (loss) per share, diluted	\$ (0.45)	\$ (0.07)	\$ (0.81)	\$ 0.20

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