



November 7, 2017

## **AGTC Announces Financial Results and Business Update for the Quarter Ended September 30, 2017**

GAINESVILLE, Fla., Nov. 07, 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 September 30, 2017.

"Progressing our XLRS, XLRP and ACHM clinical studies is our primary focus, while continuing to develop our corporate infrastructure to support these programs and our research efforts in vector design and manufacturing," said Sue Washer, President and CEO of AGTC.

### **Recent Highlights**

- | In October 2017, AGTC and the Medical College of Wisconsin published data in the October issue of *RETINA: The Journal of Retinal and Vitreous Diseases* from a longitudinal study that examined measurements of cone photoreceptor structure in patients with CNGB3-associated achromatopsia (ACHM) over time.
- | In October 2017, safety and efficacy data supporting the initiation of the XLRP phase I/II clinical trial were presented in a poster at the European Society of Gene & Cell Therapy (ESGCT) meeting, entitled "Evaluation of AAV2tYF-GRK1-hRPGRco vectors in a canine model of RPGR-XLRP".
- | Received notice of allowance of U.S. Patent 9,738,826 "Recombinant Virus Production Using Mammalian Cells in Suspension" which covers an efficient and scalable method of AAV manufacturing.

### **Clinical Programs**

The company has three ongoing clinical trials, and an additional trial which will begin enrolling patients in the coming months. The status of these programs is summarized below:

#### XLRS Phase 1/2 Clinical Trial

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

To date, the company has completed enrollment of 12 patients in the first four groups which completes the dose escalation phase of the trial. The company is also enrolling adult patients in the expansion group at the highest dose level and children between the ages of 6 and 18 at the mid-dose level. As of November 7, 2017, a total of 17 adults and one child have been enrolled in the trial across all groups.

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 safe and well tolerated.

#### ACHM CNGB3 Phase 1/2 Clinical Trial

A Phase 1/2 clinical trial evaluating the company's product candidate for achromatopsia (ACHM) caused by mutations in the CNGB3 is currently enrolling patients. To date, the company has enrolled four patients. While reviewing the data from the patients to date, AGTC also analyzed an extensive and growing body of data generated across several different animal disease models. The company found evidence of biological activity at lower vector doses than those originally proposed for the ACHM B3 study and has elected to adjust the dose downward in this dose ranging study. This new dose will also apply to the ACHM A3 study. The company will continue to evaluate emerging clinical and preclinical data to help guide future dosing decisions.

#### ACHM CNGA3 Phase 1/2 Clinical Trial

The company is currently screening patients for enrollment in a Phase 1/2 clinical trial evaluating the company's ACHM CNGA3 product candidate.

#### XLRP Clinical Trial

The company is currently conducting site initiation activities at four clinical sites and plans to initiate a Phase 1/2 clinical study in the United States in the coming months for its X-linked Retinitis Pigmentosa (XLRP) product candidate.

#### **Preclinical Optogenetic Program**

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 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 expect to occur in 2018.

#### **Financial Results for the Quarter Ended September 30, 2017**

**Revenue:** Total revenue for the three months ended September 30, 2017 was \$10.3 million compared to \$11.8 million generated during the comparable period in 2016. Collaboration revenue recorded during the three months ended September 30, 2017 and 2016 resulted primarily from the amortization of upfront fees received under the company's collaboration agreement with Biogen. Non-refundable upfront fees are amortized to collaboration revenue on a straight-line basis over the estimated service period. Extensions to the length of time for the estimated service periods resulted in a decrease in non-refundable upfront fees recognized as revenue during the three months ended September 30, 2017 versus the three months ended September 30, 2016. Grant revenue decreased \$27 thousand during the three months ended September 30, 2017 compared to the same period in 2016, largely attributable to reduced research and development activities on grant-funded projects.

**R&D expenses:** Research and development expenses for the three months ended September 30, 2017 were \$8.3 million, compared to \$5.6 million for the three months ended September 30, 2016, an increase of \$2.7 million. The increase was primarily due to increased spending on general research and discovery programs, increased spending on the company's XLRs and XLRP 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 for the three months ended September 30, 2017 were \$3.7 million, compared to \$2.8 million for the three months ended September 30, 2016, an increase of \$0.9 million. The increase was primarily driven by increased corporate infrastructure, employee-related, and shared based compensation expenses associated with the company's continued expansion and hiring of additional employees.

**Tax Provision:** There was no income tax expense for the three months ended September 30, 2017 compared to \$600,000 for the three months ended September 30, 2016. The income tax expense for the three months ended September 30, 2016 results from the recognition of revenue related to the Biogen agreement for tax purposes, which is accelerated compared to the company's GAAP revenue, resulting in significantly more taxable income than GAAP net income. While the company's taxable income is largely offset by the use of NOLs, AGTC's income tax expense is primarily due to federal alternative minimum tax expense, the apportionment of income to certain state jurisdictions where we do not have NOLs and the recognition of a reserve for uncertain tax positions.

**Net Income or loss:** Net loss was \$1.4 million for the three months ended September 30, 2017 compared to a net income of \$3.0 million for the three months ended September 30, 2016.

**Financial Guidance:** As of September 30, 2017, the company's cash, cash equivalents, and investments amounted to \$129.6 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 \$85 and \$100 million. Any milestone payments received by the company from Biogen before June 30, 2018 would increase these projected cash balances.

#### **Upcoming Investor Conference Participation**

Members of AGTC's senior management team will participate in the following upcoming investor conferences, taking place in New York:

- ┆ Barclays Gene Editing and Gene Therapy Summit, taking place November 30

1 BMO Capital Markets Prescriptions for Success Healthcare Conference, taking place December 14

A live audio webcast of the presentation at the BMO Healthcare Conference will be accessible by visiting [ir.agtc.com/events.cfm](http://ir.agtc.com/events.cfm). A replay will be available on the company's website following the event.

### **Conference Call and Webcast**

AGTC will host a conference call and webcast to discuss financial results for the first fiscal quarter ended September 30, 2017 today at 4:30 p.m. (ET). To access the call, dial 877-407-6184 (US) or 201-389-0877 (outside of the US). The passcode is 13671782. A live webcast will be available in the Events and Presentations section of AGTC's Investor Relations site at [ir.agtc.com/events.cfm](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 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 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: 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 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>September 30, 2017</b>	<b>June 30, 2017</b>
<b>ASSETS</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 48,734	\$ 30,706
Investments	76,469	95,994
Grants receivable	182	174
Prepaid and other current assets	3,228	3,361
Total current assets	128,613	130,235
Investments, net of current portion	4,385	11,749
Property and equipment, net	2,621	2,661
Investment in Bionic Sight	2,000	2,000
Other assets	1,355	1,278
<b>Total assets</b>	<b>\$ 138,974</b>	<b>\$ 147,923</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
<b>Current liabilities:</b>		
Accounts payable	\$ 2,468	\$ 998
Accrued expenses and other liabilities	5,423	6,162
Deferred revenue	14,651	20,996
Total current liabilities	22,542	28,156
Deferred revenue, net of current portion	1,029	4,438
<b>Total liabilities</b>	23,571	32,594
<b>Stockholders' equity:</b>		
Common stock—par value \$.001 per share; shares authorized: 150,000 at September 30, 2017 and June 30, 2017;		
shares issued and outstanding: 18,093 and 18,088 at September 30, 2017 and June 30, 2017, respectively.		
	18	18
Additional paid-in capital	206,408	204,937
Accumulated deficit	(91,023)	(89,626)
Total stockholders' equity	115,403	115,329
<b>Total liabilities and stockholders' equity</b>	<b>\$ 138,974</b>	<b>\$ 147,923</b>

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

In thousands, except per share amounts	<b>For the Three Months Ended September 30,</b>	
	<b>2017</b>	<b>2016</b>
<b>Revenue:</b>		
Collaboration revenue	\$ 10,308	\$ 11,772
Grant and other revenue	7	34
Total revenue	10,315	11,806
<b>Operating expenses:</b>		
Research and development	8,276	5,571
General and administrative	3,706	2,846
Total operating expenses	11,982	8,417
<b>Income (loss) from operations</b>	(1,667)	3,389
<b>Other income:</b>		
Investment income, net	270	236

Total other income, net	270	236
<b>Income (loss) before provision for income taxes</b>	<u>(1,397)</u>	<u>3,625</u>
Provision for income taxes	—	600
<b>Income (loss)</b>	<u>\$ (1,397)</u>	<u>\$ 3,025</u>
<b>Net earnings (loss) per share, basic</b>	<u>\$ (0.08)</u>	<u>\$ 0.17</u>
<b>Net earnings (loss) per share, diluted</b>	<u>\$ (0.08)</u>	<u>\$ 0.16</u>
<b>Weighted average shares outstanding — basic</b>	<u>18,088</u>	<u>18,050</u>
<b>Weighted average shares outstanding — diluted</b>	<u>18,088</u>	<u>18,445</u>

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