



August 10, 2017

## **AGTC Files Investigational New Drug Application for the Treatment of X-linked Retinitis Pigmentosa Caused by Mutations in the RPGR Gene**

GAINESVILLE, Fla., and CAMBRIDGE, Mass., Aug. 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 that it has filed an Investigational New Drug application (IND) with the U.S. Food and Drug Administration to conduct a Phase 1/2 clinical trial of the company's gene therapy product candidate for the treatment of X-linked retinitis pigmentosa (XLRP) caused by mutations in the RPGR gene.

XLRP is an inherited condition that causes progressive vision loss, beginning with night blindness in young boys followed by progressive constriction of the field of vision. Affected men progress to legal blindness by their early forties. Preclinical data indicated that treatment with an investigational gene therapy product slowed the loss of visual function in canines with XLRP caused by mutations in the RPGR gene. XLRP and X-linked retinoschisis (XLRs) are the two lead development programs within AGTC's collaboration and license agreement with Biogen, Inc. (NASDAQ:BIIB), to develop gene-based therapies for multiple ophthalmic diseases.

"We are deeply committed to advancing therapies to address the unmet needs of patients with rare inherited retinal disorders, and filing our fifth IND in this field represents a significant milestone that may ultimately improve the lives of individuals affected by XLRP," said Sue Washer, President and CEO of AGTC. "Gene therapy clinical trials continue to rapidly progress, making this an exciting time for AGTC, the clinical community and patients affected by ocular conditions with no currently available treatments."

AGTC plans to initiate a clinical study evaluating the safety and efficacy of its proprietary gene therapy for XLRP caused by mutations in the RPGR gene in the United States in the coming months.

AGTC is currently scheduling patients for enrollment in clinical trials for XLRs as well as for achromatopsia caused by mutations in the CNGB3 and CNGA3 genes. Patients and caregivers interested in participating in or learning about AGTC's current and upcoming clinical trials may visit [www.agtc.com/patients-and-caregivers](http://www.agtc.com/patients-and-caregivers) or e-mail [advocacy@agtc.com](mailto:advocacy@agtc.com).

### **About AGTC**

AGTC is a clinical-stage biotechnology company that uses its proprietary gene therapy platform to develop products designed to transform the lives of patients with severe diseases, with an initial focus in ophthalmology. 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 product pipeline includes ophthalmology programs in XLRs, XLRP, achromatopsia, wet age-related macular degeneration, and an optogenetics program with Bionic Sight. AGTC's non-ophthalmology programs include its adrenoleukodystrophy program and its otology program, which is in pre-clinical development, and the company expects to advance several otology product candidates into clinical development in the next few years. Each of AGTC's XLRs, XLRP and adrenoleukodystrophy programs is part of its collaboration and license agreement with Biogen. 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 products.

### **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 updated by the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended March 31, 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.*

IR/PR CONTACTS:

David Carey (IR)  
Lazar Partners Ltd.  
T: (212) 867-1768  
dcarey@lazarpartners.com

Lazar Partners for AGTC (PR)  
T: (212) 867-1762  
AGTC\_PR@lazarpartners.com

CORPORATE CONTACTS:

Stephen Potter  
Chief Business Officer  
Applied Genetic Technologies Corporation  
T: (617) 413-2754  
spotter@agtc.com