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Data Published in Molecular Therapy Demonstrate Five-Year Response Following a Single Dose of an Investigational Gene-Based Therapy for Alpha-1 Antitrypsin (AAT) Deficiency

Clinical study led by researchers at the University of Massachusetts Medical School demonstrates sustained protein expression and improvements in multiple indicators of AAT biological activity

GAINESVILLE, Fla. and CAMBRIDGE, Mass. and WORCESTER, Mass., June 08, 2017 (GLOBE NEWSWIRE) -- Applied Genetic Technologies Corporation (NASDAQ:AGTC) and the University of Massachusetts Medical School today announced the publication of data demonstrating sustained protein expression five years following a single intramuscular injection of a gene-based therapy for the treatment of alpha-1 antitrypsin (AAT) deficiency.

The study, "5 Year Expression and Neutrophil Defect Repair After Gene Therapy in Alpha-1 Antitrypsin Deficiency" appears in the June issue of *Molecular Therapy*. AGTC developed the gene-based therapy evaluated in the study, which was led by researchers at the University of Massachusetts Medical School.

Alpha-1 antitrypsin deficiency is an inherited genetic defect that results in severe loss of lung function. The study describes five-year follow-up results of a one-time intramuscular injection of a recombinant adeno-associated virus (AAV)-AAT vector in patients with AAT deficiency who had participated in a Phase 2a trial and had not received subsequent AAT protein therapy. Key findings from the study include:

- Sustained expression of AAT protein over the five-year study period without re-administration of AAV-AAT vector and in the absence of immune suppression or corticosteroid therapy
- Partial correction of disease-associated defects in neutrophils, a type of immune cell that contributes to lung damage in patients with AAT, including neutrophil elastase inhibition, markers of degranulation and membrane-bound anti-neutrophil antibodies
- Evidence of an active regulatory T cell response that contributed to stable gene expression despite the presence of an immune response directed against the AAV vector
- Continuous, steady-state levels of AAT protein without the peak and trough effects observed following infusion of AAT protein replacement therapy

"This is the first publication to demonstrate multi-year persistence of recombinant AAV expression in gene therapy trial participants without immune suppression or corticosteroid therapy," said lead study investigator and senior author Terence R. Flotte M.D., Dean of the School of Medicine, Provost and Executive Deputy Chancellor, and The Celia and Isaac Haidak Professor of Medical Education at the University of Massachusetts Medical School. "We also observed specific regulatory T cell responses directed against AAV1 capsid proteins, and believe that these responses may help the immune system become tolerant to AAV1. This would be beneficial in enabling the development of AAV-based therapies for AAT deficiency that provide durable responses following a single administration and may also allow for repeat dosing regimens with potential to improve long-term outcomes for patients with AAT deficiency. Finally, we have seen partial correction of disease biomarkers resulting from sustained expression of AAT in these patients."

The authors conclude that stable levels of serum AAT achieved in this study over five years may have beneficial clinical effects, despite being below the threshold of what is traditionally considered therapeutic. They hypothesize that continuous expression at a lower level may provide greater clinical benefit compared with levels that fluctuate drastically, as is observed with current AAT replacement therapy. This is analogous to the improvements observed in patients with diabetes receiving continuous insulin infusions compared with those receiving multiple daily insulin injections. Additional studies of intramuscular administration of AAV-AAT will provide further insight into the impact of AAT expression levels on symptoms of AAT deficiency.

"This study adds to the robust and growing body of evidence supporting the clinical utility of AAV vectors produced with AGTC's proprietary manufacturing methods in the treatment of a variety of diseases," said Sue Washer, President and CEO of AGTC. "We remain committed to evaluating the potential of our technology to address unmet needs of patients with serious medical conditions, and are proud to collaborate with leaders in clinical gene therapy, including Dr. Flotte and his

colleagues."

About the University of Massachusetts Medical School

The University of Massachusetts Medical School in Worcester is a world-class research institution, consistently producing noteworthy advances in clinical and basic research. Researchers at UMMS have made pivotal advances in HIV, cancer, diabetes, infectious disease, and in understanding the molecular basis of disease. Programs and centers of international distinction include those devoted to RNA therapeutics; gene therapy; gene function and expression; systems biology; and ALS.

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 X-linked retinoschisis (XLRS), X-linked retinitis pigmentosa (XLRP), achromatopsia, wet age-related macular degeneration, and our 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 partnered 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 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.

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