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The Alpha-1 Project announces first investment in potential treatment for Alpha-1 Antitrypsin Deficiency, major emphysema genetic risk factor

Applied Genetic Technologies Corporation to conduct clinical trials

March 12, 2013 - The Alpha-1 Project (TAP) today announced its first investment in a potential new treatment for Alpha-1 Antitrypsin Deficiency (Alpha-1), the most common known genetic risk factor for emphysema. TAP is partially funding further trials of a gene therapy treatment being developed by Applied Genetic Technologies Corporation (AGTC).

Alpha-1 is a genetic disorder characterized by low levels of or absent alpha-1 protein in the blood. Alpha-1 patients are at risk for severe lung disease as adults and liver disease at any age. Up to 3% of all people diagnosed with COPD may have Alpha-1. The condition can lead to severe disability and reduced life expectancy.

“As The Alpha-1 Project’s first investment, this commission signals our intent to direct research aimed at speeding the development of new therapies for Alpha-1,” said Jean-Marc Quach, Executive Director of TAP.

“We are very optimistic about the progress of our clinical trials,” said Sue Washer, Chief Executive Officer of AGTC. “Investments such as this from TAP will allow us to invest in additional research and get closer to a possible treatment for this often debilitating condition.”

AGTC, a gene therapy development company based in Gainesville, FL, has completed Phase 1 and 2 clinical trials on the treatment of Alpha-1. In these, a harmless viral vector, a tool commonly used by scientists, is used to inject a normal gene into the muscle cells of patients with lung disease due to Alpha-1. “The trials show that the potential treatment is safe, with only mild and short-lived side effects,” Washer said. Additionally, treatment can result in long-term expression of the therapeutic protein, as long as one year from a single injection. This could greatly reduce the burden on the patient and healthcare system from current products, which require weekly injections.

In addition to the funding, TAP will make its scientific advisors available to AGTC and assist with patient recruitment for further trials, thanks to the Alpha-1 Foundation Patient Research Registry.

"We're excited to support this important promising therapeutic approach,” said John Walsh, President and CEO of the Alpha-1 Foundation and member of TAP’s board of directors. “AGTC has been a pioneer in Alpha-1 gene therapy studies for a decade.”

Quach said The Alpha-1 Project’s Scientific and Business Advisory Council (SBAC) is currently evaluating several other projects that could advance new therapies. “Applications are being rigorously reviewed by our Advisory Council, made up of world class scientific researchers and experienced senior executives,” he said. More information on the SBAC membership is available at www.thealpha-1project.com.