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AGTC Secures $37.5 Million Series B Funding

Clinical stage gene therapy company will use financing, led by Alta Partners and S.R. One, to fund studies for the treatments of rare human diseases

November 15, 2012 - Applied Genetic Technologies Corporation, (AGTC), a privately-held, clinical stage biotechnology company developing gene therapy products to treat rare retinal diseases, announced today that it has secured $37.5 million in a Series B round of financing.

Alta Partners (ALTA) and S.R. One, Limited (SROne) led the financing, with new investor Osage University Partners joining existing investors InterWest (IW), Intersouth (IS) and MedImmune Ventures (MV) in the round. The funding will allow AGTC to continue development of its Phase 2 program in Alpha-1 Antitrypsin Deficiency (Alpha-1) and initiate full development of potential treatments for two orphan ophthalmology indications, Achromatopsia (ACHM) and X-Linked Rentinoschisis (XLRS).

"We are strong believers in the business model of developing treatments for genetic disorders," said Ed Hurwitz, General Partner of Alta Partners. "Based on encouraging clinical results from AGTC and others, we concluded that a large set of genetically defined diseases could be cured using AGTC's proprietary vectors and manufacturing technologies. The Series B financing is designed to move several of AGTC's programs through proof of concept as well as to allow the company to leverage its manufacturing and development infrastructure with partners to accelerate a broad portfolio of curative products."

ACHM is an inherited genetic condition that presents at birth with impaired visual acuity. Most patients are legally blind, lacking color discrimination and experiencing extreme light sensitivity, resulting in daytime blindness. ACHM is caused by mutations in a group of genes which make the cone cells concentrated in the central retina non-functional. There is no treatment for Achromatopsia, although deep red tinted spectacles or contact lenses can reduce symptoms of light sensitivity. Approximately 22,000 patients in the US and Europe suffer from this disease.

AGTC's potential treatment uses an adeno-associated virus (AAV), a safe, man-made virus that delivers healthy copies of the ACHM gene to the cells of the retina, replacing the defective copies of the gene. A single treatment is expected to halt the disease for several years, perhaps a lifetime. The AAV delivery system is successfully being used in clinical trials of Leber congenital amaurosis gene therapy that have restored vision in more than 50 adults and children who were virtually blind. Previous research has shown promising signs of efficacy in dog models of ACHM.

XLRS, an inherited genetic condition, is a leading cause of juvenile macular degeneration in males. It is caused by mutations in the RS1 gene, which results in the layers of the central retina splitting. Patients typically begin to experience progressive vision loss between the ages of 5 and 10. Other early symptoms include the inability to focus both eyes and roving, involuntary eye movements. No treatment for XLRS is currently available. Approximately 35,000 patients in the US and Europe suffer from this disease. Previous research has shown promising signs of efficacy in rodent models of XLRS.