Applied Genetic Technologies Corporation Logo

AGTC Doses First Patient in Phase 1/2 Clinical Study of Gene Therapy for the Treatment of X-Linked Retinitis Pigmentosa

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GAINESVILLE, Fla., and CAMBRIDGE, Mass., April 18, 2018 (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 dosed the first patient in the Company's Phase 1/2 clinical trial evaluating the safety and efficacy of an investigational AAV-based gene therapy for the treatment of X-linked retinitis pigmentosa (XLRP). The multicenter study will assess AGTC's novel recombinant AAV vector expressing a human RPGR gene (the rAAV2tYF-GRK1-RPGR) in patients with XLRP. The patient is being followed by Dr. David Birch of Retina Foundation of the Southwest and the surgery was performed by Dr. Andreas Lauer of Oregon Health & Science University.

"Dosing the first patient in our Phase 1/2 clinical study is an important step forward in advancing a new gene therapy in individuals with XLRP, a condition with no approved treatment options," said Sue Washer, president and CEO of AGTC. "We are committed to advancing our clinical programs to deliver novel gene-based therapies for inherited orphan diseases and would like to express our deep appreciation to those patients participating in this clinical trial and their contributions to finding a potential treatment."

The Phase 1/2 trial is an open-label, dose escalation study designed to assess the safety and efficacy of subretinal administration of the AAV-based gene therapy in approximately 15 patients diagnosed with XLRP. Trial participants will be enrolled sequentially in four groups. Individuals in Groups 1, 2 and 3 will receive a low, middle and high dose of the investigational study agent, respectively. Patients in Group 4 will receive the maximum tolerated dose as determined by the first three groups. The primary focus of the study will be to assess the safety of the vector through analysis of local (ocular) or systemic treatment-emergent adverse events. Efficacy will be measured by evaluation of changes in retinal structure, function and quality of life.

Along with the XLRS program for which we announced the completion of enrollment last week, the XLRP program is part of AGTC's collaboration with Biogen. Under the terms of the collaboration, AGTC will receive a milestone payment of $2.5 million as a result of enrollment of the first patient in the XLRP trial.

"This is an exciting clinical milestone for one of the first potential treatments of XLRP," said Stephen Rose, Ph.D., chief scientific officer for the Foundation Fighting Blindness (FFB). "The Foundation's registry, *My Retina Tracker*, which is supported in part by a grant from AGTC, continues to provide invaluable support in identifying patients with XLRP, and other inherited retinal diseases. It allows us to inform them of clinical trials, such as this gene therapy in XLRP, that have the potential to transform their lives."

XLRP is an inherited condition that causes progressive vision loss in boys and young men. Characteristics of the disease include night blindness in early childhood and progressive constriction of the visual field. In general, XLRP patients experience a gradual decline in visual acuity over the disease course, which results in legal blindness around the 4th decade of life. Preclinical data in a canine model of XLRP caused by mutations in the RPGR gene indicate that treatment with an AAV-based gene therapy product slowed the loss of visual function.

My Retina Tracker® is an online, confidential patient registry for people affected by inherited retinal diseases. The registry collects data with the intention of using the aggregated knowledge to advance research and to help accelerate clinical trial enrollment by providing a source of information about people impacted by retinal diseases. The registry is free for people affected by inherited orphan retinal diseases, and is designed with state-of-the-art technology to protect user privacy. To learn more about *My Retina Tracker*, visit www.MyRetinaTracker.org or contact the registry's coordinator at coordinator@MyRetinaTracker.org.

AGTC was granted U.S. Food and Drug (FDA) orphan drug designation in 2017, as well as European Commission orphan medicinal product designation in 2016, for its gene therapy product candidate to treat XLRP caused by mutations in the RPGR gene. Orphan drug designation, covered by the U.S. Orphan Drug Act of 1983, is granted to drugs or biologics that treat a rare disease or condition affecting fewer than 200,000 individuals. Products receiving orphan drug designation are eligible to receive market exclusivity for a period of seven years, an exemption from certain taxes and user fees and additional clinical support from FDA.

For more information on AGTC and its pipeline of AAV-based gene therapy candidates in rare disease, please visit www.agtc.com/programs.

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, 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. Information about the Phase 1/2 clinical trial in XLRS can be found at ClinicalTrials.gov under trial identifier number NCT02416622.

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. Information about the Phase 1/2 clinical trial in achromatopsia caused by CNGA3 can be found at ClinicalTrials.gov under the trial identifier number NCT02935517, while the Phase 1/2 clinical trial in achromatopsia caused by CNGB3 can be found under the trial identifier number NCT02599922.

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. Information about the Phase 1/2 clinical trial in XLRP can be found at ClinicalTrials.gov under trial identifier number NCT03314207.

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 September 13, 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.

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