AGTC Treats First Patient of Second Cohort in Phase 1/2 Clinical XLRP Study; Earns $10 Million Enrollment Milestone

July 23, 2018

Company Provides Pipeline Enrollment Update for Clinical Trials in Inherited Retinal Diseases

GAINESVILLE, Fla. and CAMBRIDGE, Mass., July 23, 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 enrolled the first patient of the second cohort 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). Under the terms of its collaboration with Biogen (NASDAQ: BIIB), AGTC will receive a milestone payment of $10 million.

"We are pleased to announce this important milestone under our collaboration with Biogen and remain on track to complete the dose escalation portion of the trial in the first quarter of 2019," said Sue Washer, President and CEO of AGTC.

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 patients diagnosed with XLRP caused by mutations in the RPGR gene. The primary focus of the study will be to assess the safety of the vector and subretinal delivery procedure through analysis of focal (ocular) and systemic treatment-emergent adverse events. Potential efficacy will be measured by evaluation of changes in retinal structure, visual function and quality of life.

Ms. Washer continued, "In addition to achieving this milestone for our XLRP study, we are improving execution through enhanced clinical operations, clinical outreach and patient advocacy efforts to support enrollment of our three additional clinical trials. We remain grateful to our team, the investigators and the patients who have volunteered for these studies for their commitment to accelerating the development of gene therapies for inherited retinal diseases."

In addition to the XLRP study, the company has ongoing clinical trials in X-linked retinoschisis (XLRS), and achromatopsia (ACHM).

XLRS Phase 1/2 Clinical Trial
Earlier this year, AGTC completed its target enrollment of 27 patients in the Phase 1/2 clinical trial for its XLRS product candidate as part of the company's collaboration with Biogen. The primary endpoint of this clinical trial is safety, and available data thus far have shown that the XLRS product candidate is generally safe and well tolerated. In addition to safety, this trial will measure biologic activity by assessing changes in visual function, retinal structure and quality of life. The company expects to provide topline six-month data across both safety and biologic activity endpoints by the end of 2018 with the primary analysis of the full twelve-month active trial data six months later.

ACHM Phase 1/2 Clinical Trials
The company is presently enrolling patients in two parallel Phase 1/2 clinical trials of its product candidates for achromatopsia (ACHM) caused by mutations in the two most common ACHM genes, CNGB3 and CNGA3. In the ACHM CNGB3 trial, AGTC has completed enrollment of seven patients. The company expects to complete the dose escalation portion of the CNGB3 trial in the first quarter of 2019. The ACHM CNGA3 trial has enrolled and dosed two patients.

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

About X-linked Retinitis Pigmentosa (XLRP)

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. 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.

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.

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.

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 (CNS), 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, and the optogenetics program is being developed in collaboration with Bionic Sight. 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.

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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