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AGTC Announces U.S. FDA Orphan Drug Designation for Gene Therapy to Treat X-Linked Retinitis Pigmentosa

GAINESVILLE, Fla. and CAMBRIDGE, Mass., Aug. 03, 2017 (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 the U.S. Food and Drug Administration (FDA) has granted an orphan drug designation for its gene therapy product candidate for the treatment of X-linked retinitis pigmentosa (XLRP) caused by mutations in the RPGR gene. In June 2016 the company announced that the European Commission (EC) granted orphan medicinal product designation for the same indication.

XLRP is an inherited condition that causes progressive vision loss, beginning with night blindness in young boys followed by progressive constriction of the field of vision. Affected men become legally blind at an average of about 45 years of age. The most common form of XLRP is caused by mutations in the RPGR gene. Preclinical data indicate that treatment with a gene therapy product slowed the loss of visual function in a canine model of XLRP due to mutations in the RPGR gene. XLRP and X-linked retinoschisis (XLRS) are the two development programs within AGTC's collaboration and license agreement with Biogen Inc. (NASDAQ:BIIB), to develop gene-based therapies for multiple ophthalmic diseases.

"Receiving U.S. orphan drug designation for our fourth gene therapy treatment candidate demonstrates our ongoing commitment to addressing the unmet needs of people with rare inherited diseases," said Sue Washer, President and CEO of AGTC. "XLRP affects approximately 20,000 people in the US and Europe. We are on track to file an Investigational New Drug application for this program this year, and this orphan drug designation will help to accelerate the regulatory review process as we work to bring a new treatment option to XLRP patients."

AGTC has also been granted orphan drug designation from the FDA and EC for its gene therapy product candidates for the treatment of X-linked retinoschisis and for the treatment of achromatopsia caused by mutations in the CNGA3 and CNGB3 genes.

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 accelerated regulatory review process, an exemption from certain taxes and user fees and additional clinical support from FDA.

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 an 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 are part of its collaboration and license agreement 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 updated by the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended March 31, 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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