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AGTC Presents New Data on its AAV-Based Gene Therapies for the Treatment of Achromatopsia and X-Linked Retinitis Pigmentosa at the American Society of Gene and Cell Therapy 20th Annual Meeting

Results support AGTC's clinical development strategies in both conditions

Phase 1/2 trial in patients with achromatopsia due to CNGB3 mutations is currently enrolling patients; Phase 1/2 trial in patients with CNGA3-related achromatopsia is scheduling patients for enrollment

WASHINGTON, May 11, 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 the presentation of new data from studies in animal models of achromatopsia (ACHM) and X-linked retinitis pigmentosa (XLRP) that support the company's clinical development programs in these indications. The data were presented at the American Society of Gene and Cell Therapy 20th Annual Meeting, taking place in Washington, D.C., May 10-13.

ACHM and XLRP are rare inherited retinal diseases. ACHM results from mutations in either of the CNGB3 or CNGA3 genes. Mutations in these genes account for approximately 75 percent of the total achromatopsia patient population. Individuals with achromatopsia have markedly reduced visual acuity, extreme light sensitivity, and complete loss of color discrimination.

AGTC is currently enrolling patients in a clinical trial for its CNGB3 gene-related ACHM treatment candidate, and is currently scheduling patients to be enrolled in a clinical trial for its CNGA3 gene-related ACHM treatment candidate. Patients and caregivers interested in participating in or learning more about these trials may find more information at www.agtc.com/patients-and-caregivers or by contacting advocacy@agtc.com.

XLRP affects boys, causing night blindness by the time they are ten, and progresses to legal blindness by their early forties. AGTC is developing a gene-based therapy for XLRP in collaboration with Biogen and expects to file an Investigational New Drug (IND) Application with the U.S. Food and Drug Administration for this product candidate this year.

Lisa R. Keyes, Ph.D., Research Scientist at AGTC, will present the ACHM data in an abstract titled, "Evaluating Safety and Efficacy of the AAV2tYF-PR1.7-CNGA3 Vector in CNGA3-Deficient Sheep" (Abstract #299) today in an oral session from 4:15 p.m. to 4:30 p.m. EDT. These data are from a study that assessed toxicity, CNGA3 expression and efficacy of two subretinally administered vectors [AAV2tYF-PR1.7-hCNGA3 and AAV5-PR2.1-hCNGA3 (a vector previously shown to rescue cone photoreceptor responses)] in an animal model of ACHM, over a 12-week evaluation period.

No systemic toxicity was associated with treatment and no consistent test article-related effects were observed. Two out of five animals treated with the higher dose of AAV2tYF-PR1.7-CNGA3 had microscopic findings of outer retinal atrophy, with or without inflammatory cells in the retina and choroid that were considered procedural- and/or test article-related. All vector-treated eyes demonstrated CNGA3 expression, and developed cone-mediated electroretinogram (ERG) responses with no change in rod-mediated ERG responses. Improvements in maze navigation times and obstacle collisions were observed in all vector-treated eyes compared with control eyes and with pre-dose results in the treated eyes. The researchers conclude that these results support the use of AAV2tYF-PR1.7-hCNGA3 in clinical studies in patients with achromatopsia caused by mutations in CNGA3.

"The improvements in maze navigation times, obstacle collisions and ERG responses observed with the product candidate in this study suggest that AAV-based gene therapy has important potential in the treatment of ACHM resulting from mutations in the CNGA3 gene," said Sue Washer, President and CEO of AGTC. "The favorable tolerability profile observed in this study also supports the use of this vector construct in human clinical trials. These study findings provided the basis for the design of the Phase 1/2 clinical trial of our gene-based therapy for ACHM resulting from CNGA3 mutations, which is currently scheduling patients for enrollment."

Jilin Liu, Associate Scientist at AGTC, will present the XLRP data in an abstract titled, "Evaluation of AAV2tYF-GRK1-RPGR Vectors in a Canine Model of *RPGR*-XLRP" (Abstract #692) in a poster session May 12, from 5:45 p.m. to 7:45 p.m. EDT.

The poster will include results from a study evaluating the efficacy of two vectors (AAV2tYF-GRK1-RPGRco and AAV2tYF-GRK1-RPGRstb) containing the AAV2tYF capsid, human GRK1 promoter and a codon-optimized or stabilized version of the human RPGR gene administered subretinally in an animal model of mid-stage XLRP resulting from mutations in the RPGR gene. In this model, mid-stage disease occurs when animals are approximately 12 weeks of age and is associated with an approximate 40% loss of photoreceptors. Two animals per group received RPGRco in the right eye and RPGRstb in the left eye at each of three dose levels. Rescue of photoreceptor structure was assessed by clinical examination and histology and/or immunohistochemistry on retinal cryosections eight weeks post injection.

No abnormal ophthalmic findings were noted in any eyes at the middle- or low-dose levels. Fundoscopic examination at 8 weeks post-dosage showed signs of retinal detachment and inflammation in the eyes injected with the high dose of either RPGRco or RPGRstb. Dose-dependent RPGR transgene expression was observed with both vectors, with greater RPGR expression noted in eyes injected with RPGRco compared with contralateral eyes injected with RPGRstb at the same dose levels. Correction of rod opsin and middle/long wavelength cone opsin mislocalization was demonstrated in all AAV-RPGR treated eyes. Researchers conclude that the results demonstrate greater RPGR expression with RPGRco compared with RPGRstb, and that the middle doses of both vectors resulted in optimal correction at mid-stage disease with limited inflammation in this animal model of XLRP.

Data from both the ACHM and XLRP studies were also presented earlier in the week at ARVO 2017, the Association for Research in Vision and Ophthalmology Annual Meeting, which took place in Baltimore from May 7-11.

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 our 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 is partnered 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 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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