AGTC Joins the My Retina Tracker® Program as a New Scientific Collaborator

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GAINESVILLE, Fla., and CAMBRIDGE, Mass., Oct. 07, 2020 (GLOBE NEWSWIRE) -- Applied Genetic Technologies Corporation (AGTC), Blueprint Genetics, and Foundation Fighting Blindness, announce today that AGTC, a biotechnology company conducting human clinical trials of adeno-associated virus (AAV)-based gene therapies for the treatment of rare diseases, will join the My Retina Tracker® Program as a new scientific collaborator to enhance development of its investigational gene therapies for inherited retinal diseases (IRDs). The My Retina Tracker Program, a collaboration between Blueprint Genetics, Foundation Fighting Blindness and InformedDNA, is an open access, no-cost program that offers individuals with IRDs easy access to high-quality genetic diagnostics and genetic counseling. This program has become one of the largest volume genetic testing initiatives for IRDs globally.

As a scientific collaborator, AGTC will have access to specific deidentified genetic data and expert healthcare providers to help AGTC rapidly identify potential candidates eligible for their clinical trials. AGTC's initial focus in the collaboration will be X-linked retinitis pigmentosa (XLRP), specifically *RPGR*-associated disease. Disease-causing variants in *RPGR* are the third most common cause of IRDs. AGTC has initiated an expansion of its Phase 1/2 gene therapy clinical trial and is planning to initiate a Phase 2/3 clinical trial in early 2021 in males with XLRP with a pathogenic variant in the *RPGR* gene.

"We are delighted to be among the scientific collaborators in the My Retina Tracker Program who share our mission of improving the vision of patients with IRDs. Patient registries are a rich source of information for sponsors of clinical trials, particularly for rare diseases, where patient identification can be more challenging," said **Sue Washer**, President and CEO of AGTC. "The critical genetic data from the My Retina Tracker Program will benefit current and future patients as it is an important step in being able to participate in clinical trials that may lead to effective treatments for these diseases. We thank all the registrant participants for their contribution to a greater understanding of these conditions."

Patients taking part in the My Retina Tracker Program are offered Blueprint Genetics' 322-gene panel that includes comprehensive coverage of major IRD genes, noncoding variants, copy number variants and mitochondrial genome analysis. One of the hallmarks of the panel is the high performance of *RPGR* testing, including the difficult-to-sequence ORF15 region which harbors approximately 80% of disease-causing *RPGR* variants. Blueprint Genetics has done extensive work to develop high-quality testing capabilities for this gene using a unique NGS-based approach in order to maximize XLRP diagnostic yield. The My Retina Tracker Program has also received excellent feedback regarding the comprehensive genetic counseling provided by InformedDNA. All patients taking part in the program are provided no-cost, post-test genetic counseling by expert genetic counselors from InformedDNA.

"With the very strong focus on gene –specific and gene variant–specific therapies dominating the late preclinical and clinical trials pipelines, knowing the genetic basis of disease is an important component of any inherited retinal disease diagnosis," said **Dr. Brian Mansfield**, executive vice president research and interim chief scientific officer of the Foundation Fighting Blindness. "The Foundation is delighted to partner with industry partners, like AGTC, and other non-profit foundations, to be able to make genetic testing accessible to people with an inherited retinal disease—at no cost to patients. By partnering with industry sponsors to proactively genotype people with these rare diseases, our partners not only provide a valuable service to affected people but also significantly reduce their overall expense in finding potential clinical trial participants, compared to solo screening programs."

"Over the past few years, we have invested in improving the quality and performance of genetic analysis of this challenging *RPGR* gene. It has been a privilege to witness the significance of these efforts to patients and is even more rewarding now as we have opportunities to collaborate with biopharmaceutical companies like AGTC that are developing product candidates with the potential to change the lives of these patients with *RPGR*-targeted gene therapy," said Blueprint Genetics Executive Medical Director, **Dr. Tero-Pekka Alastalo.**

About the My Retina Tracker® Program

The My Retina Tracker® Program offers open access, no-cost genetic testing and genetic counseling for individuals living in the United States with a clinical diagnosis of an inherited retinal disease. The program provides people with these diseases access to the highest quality genetic testing and genetic counseling. InformedDNA provides genetic counseling by certified genetic counselors with inherited retinal disease expertise. Although not required for participation, this program also offers an easy opportunity to join the Foundation Fighting Blindness My Retina Tracker Registry which gives individuals the opportunity to share their deidentified information and contribute to focus groups, patient journey analyses, research studies, and, when available, invitations to apply for enrollment in relevant natural history studies and clinical trials.

For more information on the My Retina Tracker Program, please visit https://blueprintgenetics.com/my-retina-tracker-program/

For more information on My Retina Tracker Registry, please visit: FightingBlindness.org/my-retina-tracker-registry

About AGTC

AGTC is a clinical-stage biotechnology company developing genetic therapies for people with rare and debilitating ophthalmic, otologic and central nervous system (CNS) diseases. AGTC is a leader in designing and constructing all critical gene therapy elements and bringing them together to develop customized therapies that address real patient needs. The Company's most advanced clinical programs leverage its best-in-class technology platform to potentially improve vision for patients with inherited retinal diseases. AGTC has active clinical trials in X-linked retinitis pigmentosa and achromatopsia (ACHM CNGB3 & ACHM CNGA3). Its pre-clinical programs build on the Company's industry leading AAV manufacturing technology and scientific expertise. AGTC is advancing multiple important pipeline candidates to address substantial unmet clinical need in larger ophthalmology indications, optogenetics, otology and CNS disorders. www.agtc.com

About the Foundation Fighting Blindness
Established in 1971, the Foundation Fighting Blindness is the world's leading private funding source for retinal degenerative disease research. The Foundation has raised more than $800 million toward its mission of accelerating research for preventing, treating, and curing blindness caused by the entire spectrum of retinal degenerative diseases including: retinitis pigmentosa, age-related macular degeneration, Usher syndrome, and Stargardt's disease. Visit FightingBlindness.org for more information.

About Blueprint Genetics
Blueprint Genetics, a Quest Diagnostics company, is a leading specialty genetics and bioinformatics company focused on providing genetic testing for inherited diseases. The company is based in Helsinki with a US hub in Seattle, and a customer base spanning over 70 countries. www.blueprintgenetics.com

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