AGTC Formalizes Patient Advisory Council to Provide Insights and Guidance on Patient Perspectives for Pipeline Therapies

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Patient Advisory Council to include advocates from the inherited retinal diseases global community

New Patient Advisory Council to focus initially on Company's clinical stage programs for X-linked retinitis pigmentosa (XLRP)

GAINESVILLE, Fla., and CAMBRIDGE, Mass., Aug. 12, 2020 (GLOBE NEWSWIRE) -- Applied Genetic Technologies Corporation (Nasdaq: AGTC), a biotechnology company focused on developing adeno-associated virus (AAV) based gene therapies for the treatment of rare inherited diseases, today announced the formation of a Patient Advisory Council to build on its focus of incorporating the patient and caregiver voice into the Company's culture and clinical and pre-clinical programs.

Engagement with patients and caregivers has provided AGTC with substantive information that has guided its clinical trial design, enhanced its understanding of retinal disorders, and inspired its patient centric culture. The Council, spearheaded by Jill Dolgin, PharmD, Head of Patient Advocacy at AGTC, is comprised of individuals with inherited retinal diseases (IRDs) and members from the global community of organizations that represent them.

"Patient engagement will continue to be a critical success factor for our programs moving forward, and we are very pleased to formalize the establishment of this distinguished group of patient experts, especially as we move into the next phase of development for our X-linked retinitis pigmentosa gene therapy candidate," said Sue Washer, President and CEO of AGTC.

In July, AGTC announced next steps in the clinical development of the Company's potential treatment of XLRP caused by mutations in the RPGR gene following receipt of written feedback from the U.S. Food and Drug Administration. AGTC is expanding the ongoing Phase 1/2 trial to dose additional patients in two masked dosing arms to collect additional functional data. In parallel, a planned Phase 2/3 trial, which is expected to begin in Q1 2021, will be designed to evaluate sustained efficacy across multiple measures of potential benefit in patients with XLRP.

"We are delighted to be a part of this advisory council established by AGTC to lend our voice and collective experience that spans more than five decades in the search for treatments to address blindness and vision loss," said Brian Mansfield, PhD, Executive Vice President of Research and Interim Chief Scientific Officer at the Foundation Fighting Blindness. "Having the patient's perspective at the center of clinical drug development is a crucial component for addressing the unmet needs of patients within the inherited retinal diseases community."

Organizations and advocate members of the Patient Advisory Council include:

Organization	Name	Title
Foundation Fighting Blindness	Brian Mansfield, PhD	Executive Vice President of Research, Interim Chief Scientific Officer, and oversees the My Retina Tracker Registry patient database
	Todd Durham, PhD	Vice President, Clinical Outcomes Research
	Michelle Glaze	Associate Director, Professional Outreach, and an individual affected by retinitis pigmentosa (RP)
	Richard Faubion	Senior Director, Development; individual affected by XLRP, and a stem cell transplant clinical trial participant
Fighting Blindness Canada	Shari Shaw, MHSc	Health Information Officer, and an individual with RP
Retina International	Avril Daly	CEO; Vice President, Board of Directors of the European Organization for Rare Diseases (Eurordis), and an individual affected by RP
Sofia Sees Hope	Laura Manfree	President and Founder, and parent of a child with Leber Congenital Amaurosis (LCA)
	Alison Lynch, JD	Disabilities attorney, individual affected by achromatopsia, and non-gene therapy clinical trial participant

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 investigational technology platform to potentially improve vision for patients with an inherited retinal disease. 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 optogenetics, otology and CNS disorders.

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 Administration (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.

Forward-Looking Statements

This release contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs, including statements regarding the timing for and expected expansion of its XLRP clinical development program, the timing for reporting data in its XLRP and ACHM clinical programs, and its ability to enroll patients, effectively design and successfully complete its ongoing clinical trials. Forward-looking statements include information concerning possible or assumed preclinical and clinical product development and regulatory progress, future results of operations, financial guidance, business strategies and operations, potential growth opportunities, potential market opportunities, the effects of competition and the impact of the COVID-19 pandemic. 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; the direct and indirect impacts of the ongoing COVID-19 pandemic on the Company's business, results of operations, and financial condition; 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 most recent annual or quarterly report and in other reports AGTC has 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, AGTC assumes 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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