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AGTC and BCM Families Foundation Announce Collaboration to Develop AAV-Based Gene Therapy for Blue Cone Monochromacy

GAINESVILLE, Fla. and JUPITER, Fla. and CAMBRIDGE, Mass., Jan. 06, 2016 (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 eye diseases, and the BCM Families Foundation, a non-profit organization focused on eradicating Blue Cone Monochromacy (BCM), today announced a collaboration to develop an AAV-based gene therapy for the disease.

Blue Cone Monochromacy, also known as X-linked achromatopsia, is a rare genetic disease of the retina that almost exclusively affects males. It is a hereditary condition linked to the X chromosome that manifests with a partial dysfunction of the cones of the retina. BCM can result in reduced visual acuity, impaired color vision, photosensitivity, myopia and infantile-onset nystagmus. These manifestations are similar to those in achromatopsia, caused by mutations in the CNGB3 or CNGA3 gene, for each of which AGTC has ongoing clinical development activities.

"The BCM Families Foundation is deeply committed to the discovery and development of biomedical and scientific research that can lead to a treatment or a cure for BCM. We are excited to announce this partnership with AGTC, a leader in ophthalmic gene therapy," said Renata Sarno, Ph.D., President of the BCM Families Foundation. "AGTC has deep expertise in the optimization and manufacturing of novel AAV-based therapies for the treatment of inherited retinal diseases, and we are excited to work together on our mutual goal of providing treatment options for individuals affected by BCM."

As part of the collaboration, the BCM Families Foundation will share with AGTC scientific and clinical study data that have been generated through its research efforts, and will also provide patient advocacy and clinical trial support to AGTC. AGTC will be responsible for product development efforts and aspects of the clinical program design and execution.

"We are proud of our many longstanding collaborations with patient advocacy and research organizations, and we are honored to recognize the BCM Families Foundation as a new partner. This new co-development program complements our ongoing research and development in achromatopsia, and will utilize many of the same product components," said Sue Washer, President and CEO of AGTC. "The BCM Families Foundation's deep knowledge of BCM, combined with our resources and technology, has the potential to make a real difference in the lives of patients in this significantly underserved community."

About BCM Families Foundation

The mission of BCM Families Foundation is to eradicate Blue Cone Monochromacy by supporting the most promising biomedical and scientific research in an effort to find a cure. BCM Families Foundation is a United States non-profit organization incorporated in May 2014 by families affected by Blue Cone Monochromacy. Learn more by visiting <http://www.blueconemonochromacy.org/>.

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 in ophthalmology. AGTC's lead product candidates focus on 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 six named ophthalmology development programs across five targets [x-linked retinoschisis (XLRS), x-linked retinitis pigmentosa (XLRP), achromatopsia, wet age-related macular degeneration and now blue cone monochromacy], one non-ophthalmology program (alpha-1 antitrypsin deficiency) and proof-of-concept data in multiple additional indications. AGTC employs a highly targeted approach to selecting and designing its product candidates, choosing to develop therapies for indications having high unmet medical need, clinical feasibility and commercial potential. AGTC has a significant intellectual property portfolio and 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. These statements relate to a variety of matters, including but not limited to: the anticipated benefits to AGTC of its partnership with the BCM Families Foundation. 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, which include, but are not limited to, the following: no gene therapy products have been approved in the United States and AGTC cannot predict when or if it will obtain regulatory approval to commercialize a product candidate; AGTC relies on third parties to conduct, supervise and monitor its clinical trials and to conduct certain aspects of its product manufacturing and protocol development; and increased regulatory scrutiny of gene therapy and genetic research could damage public perception of AGTC's product candidates or adversely affect AGTC's ability to conduct its business. Additional factors that could cause actual results to differ materially from those described in the forward-looking statements are set forth under the heading "Item 1A—Risk Factors" in AGTC's Annual Report on Form 10-K for the fiscal year ended June 30, 2015, 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, 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.

AGTC IR/PR CONTACTS:

David Carey (IR) or Danielle Lewis (PR)

Lazar Partners Ltd.

T: (212) 867-1768 or (212) 843-0211

dcarey@lazarpartners.com or dlewis@lazarpartners.com

AGTC CORPORATE CONTACTS:

Larry Bullock

Chief Financial Officer

Applied Genetic Technologies Corporation

T: (386) 462-2204

lbullock@agtc.com

Stephen Potter

Chief Business Officer

Applied Genetic Technologies Corporation

T: (617) 413-2754

spotter@agtc.com

BCM FAMILIES FOUNDATION PR CONTACT:

Kay McCrary

BCMFF Board Secretary

BCM Families Foundation

T: (803) 479-3605

kaymccrary@bcmfamilies.org

 Primary Logo

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