ACGT ALLIANCE FOR CANCER GENE THERAPY

Discovering new ways to treat and defeat cancer

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For Immediate Release

FDA Advisory Committee Recommends First-Ever CAR-T Gene Therapy Treatment for Cancer

ACGT Funding Helps Move Pioneering Treatment from Bench to Bedside

STAMFORD, Conn., July 12, 2017 — Today the FDA's Oncologic Drugs Advisory Committee, an independent panel of experts, voted unanimously to recommend to the FDA approval of Novartis' experimental CAR-T therapy called Tisagenlecleucel, also known as CTL019. This form of gene therapy has demonstrated impressive results in hard-to-treat leukemia (ALL) pediatric and adolescent patients who have relapsed or whose cancers have proven resistant to other treatment. One of the panelists Dr. Timothy Cripe of Nationwide Children's Hospital remarked, "this is the most exciting therapy I have seen in my lifetime."

This advisory committee hearing was the last major regulatory milestone before the agency decides in September whether or not to approve the treatment, which would make Novartis' CAR-T therapy the first-ever gene therapy treatment approved by the FDA in US markets. The committee's unanimous positive vote will go a long way towards that approval.

ACGT fellow, Dr. Carl June of the **University of Pennsylvania,** was a leader in developing CTL019 therapy and a pioneer of the novel concept to use a patient's own T cells and genetically modify them to attack the cancer cells. The protocol involves removing a patient's white blood cells called T-cells, genetically modifying them, then infusing the newly transformed cells back into the patient's body following chemotherapy. This process gives the patient's immune system a tumor-attack roadmap for the treatment of leukemia and other cancers, including those of the ovaries and myeloma. Dr. June was one of the first scientists to demonstrate the use of gene transfer therapy to create "serial killer" T cells aimed at cancerous tumors.

Dr. June received two grants from ACGT in 2004 and 2008 for his studies in CAR-T therapy for lymphoma and leukemia, and ovarian cancers. On August 10, 2011, Dr. June's study results were reported in the New England Journal of Medicine and Science Translational Medicine. The results exceeded everyone's wildest expectations.

"The funds from ACGT sustained us. When other organizations, including the NIH, considered gene therapy too risky, ACGT believed in the science and funded us when no one else would," said Dr. June. "ACGT really kept us going and kept the research alive. Without them, we wouldn't have had a clinical trial and I don't think we'd be where we are today."

"In the early 2000's when ACGT was founded, gene therapy was an outlier," noted ACGT co-founder Barbara Netter, who founded ACGT with her late husband Edward in 2001. "Scientists believed in the promise of gene therapy, but after the death of a patient in a clinical trial in 1999, organizations were reluctant to fund this type of work. ACGT believed so strongly in cell and gene therapy and

moved to embrace the science, that it focused all its resources on helping scientists we thought had the most exciting, novel ideas on how to treat cancers. Dr. June was one of those scientists and we were very excited about the potential of his work even back in 2004 when he received his first grant from ACGT."

In a Novartis' sponsored ELIANA study, which formed the basis of Novartis' application of approval, treatment with Tisagenlecleucel resulted in a best overall response rate of 83%, with 63% of successfully infused patients experiencing a complete response. Beyond representing a change in the treatment of relapsed/refractory ALL, approval for Tisagenlecleucel would be a landmark decision — ushering in CAR-T as a new class of personalized cancer treatments. A regulatory approval would also put Novartis at the head of that class, further than other bio-pharma rivals.

"This review by the FDA of CAR-T therapy is a major milestone in the successful treatment of cancer," noted John Walter, CEO and president of ACGT. "If approved, it will be the first-ever true gene therapy treatment made available to the US population and will help accelerate the speed at which we will see even more gene-based therapies come to fruition. It's a very exciting time."

About Alliance for Cancer Gene Therapy (ACGT)
Established in 2001, ACGT is the nation's only non-profit dedicated exclusively to cell and gene therapy treatments for all types of cancer. One hundred percent of contributions go directly to research. Founded by Barbara and Edward (1933-2011) Netter, ACGT is headquartered in Stamford, Connecticut. Since its inception, ACGT has funded some of the underlying science that has resulted in the formation of either licensing agreements or biotech companies including Novartis, Ziopharm, Juno, Turnstone Biologics, all of which are in various stages of bringing new treatments to patients. ACGT has funded 52 grants in the U.S. and Canada to conduct and accelerate critically needed innovative research. 36 of those grants have gone to Young Investigators and 16 grants to Clinical Investigators, totaling nearly $27 million in funding. ACGT is located at 96 Cummings Point Road, Stamford, Connecticut 06902; 203-358-5055. To learn more, visit acgtfoundation.org or join the ACGT community on Facebook, Twitter and YouTube at @acgtfoundation.

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