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For Immediate Release

First-Ever Cancer Gene Therapy Approved by FDA *Early Research Funding Provided by ACGT*

Stamford, Conn., August 30, 2017 — Today the FDA launched a new era in cancer treatment with its approval of the first cancer gene therapy drug in the United States. With the FDA's approval of Novartis' Kymriah (tisagenlecleucel) for pediatric and young adult patients with a form of acute lymphoblastic leukemia (ALL), Alliance for Cancer Gene Therapy (ACGT), one of the original funders of the early work around CAR T, believes that today is a monumental turning point in cancer treatment.

The approved CAR T gene therapy drug, now known as Kymriah, received early funding from ACGT in 2004, while in development by Dr. Carl June of the University of Pennsylvania. ACGT funded Dr. June's work before the government (NIH) or pharmaceutical companies would. Novartis ultimately partnered with Dr. June and the Penn Medicine team to fund clinical trials that showed an exceptional 83 percent success remission rate with pediatric and young adult patients with a form of acute lymphoblastic leukemia (ALL). With this FDA approval, cancer centers around the country will be able to make this specialized therapy readily available to ALL patients with no other treatment options.

"We're entering a new frontier in medical innovation with the ability to reprogram a patient's own cells to attack a deadly cancer," said FDA Commissioner Scott Gottlieb, M.D., in an announcement made by the FDA. "New technologies such as gene and cell therapies hold out the potential to transform medicine and create an inflection point in our ability to treat and even cure many intractable illnesses."

Dr. Carl June, an ACGT Research Fellow/Scientific Advisory Council Member, led the research and trials leading to this breakthrough at the University of Pennsylvania. He stated, "When other organizations, including the NIH, considered gene therapy too risky, ACGT believed in the science and funded us when no one else would. 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."

ACGT's late co-founder Edward Netter and his wife Barbara founded ACGT in 2001 and Dr. Carl June was one of ACGT's first grant recipients. The Netters founded ACGT after the death of their daughter-in-law Kimberly from breast cancer. After her passing, the Netters became convinced that there was a better way to treat cancer. They believed gene and cell therapy held the key.

"My only regret is that my husband Edward did not live to see the fulfillment of his vision, as he sadly passed away in 2011, only months before Dr. June's initial research was published in the *New England Journal of Medicine*," said Barbara Netter, ACGT's co-founder. "My husband Edward Netter was one of the primary promulgators of gene therapy — before anyone else was interested. He was

always thinking about gene therapy and knew that it was the key to unlocking better and more effective treatments for cancer.”

There are a number of other CAR T therapies awaiting approval, including for the treatment of CLL and other blood cancers.

“This approval by the FDA of Kymriah CAR-T therapy is a major milestone in the successful treatment of cancer,” noted John Walter, CEO and president of ACGT. “This is 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 55 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 19 grants to Clinical Investigators, totaling over \$28 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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