Alliance for Cancer Gene Therapy Funds Promising Leukemia Study – Patients in Remission More Than Two Years after Gene Therapy Treatment at UPenn

The Associated Press

STAMFORD, Conn.--(BUSINESS WIRE)--Dec 10, 2012--Alliance for Cancer Gene Therapy (ACGT), the nation's only non-profit organization dedicated exclusively to funding cell and gene therapy research for cancer, is excited to play a major role in the recent leukemia study pioneered by scientists at the Perelman School of Medicine at the University of Pennsylvania. ACGT was the initial funding arm for the study using immune-mediated gene therapy for leukemia and lymphoma. This study illustrates the successful and sustained demonstration of how gene therapy uses the body's own T-cells and turns them into weapons aimed directly at cancer.

Initial ACGT grants were awarded in 2004 to Dr. Carl June of the Abramson Family Cancer Research Institute at the University of Pennsylvania, and to Dr. Michel Sadelain, of Memorial Sloan-Kettering Cancer Center, Gene Therapy & Gene Expression Laboratory in New York City. Preliminary results were issued by Dr. Carl June and the University of Pennsylvania in August 2011, with additional results released this week and presented by Dr. Carl June at the American Society of Hematology's Annual Meeting and Exposition being held in Atlanta, Georgia.

The clinical trial participants, all of whom had advanced cancers, included ten adult patients with chronic lymphocytic leukemia, were treated at the Hospital of the University of Pennsylvania (HUP) and two children with acute lymphoblastic leukemia were treated at the Children's Hospital of Philadelphia (CHOP). Two of the first three patients treated with the protocol at HUP – whose cases were detailed in – remain healthy and in full remission more than two years after their treatment, with the engineered cells still circulating in their bodies. Currently, nine out of 12 of the participants show their disease in remission.

"The discovery of successful cancer gene therapy treatments are what my husband and I hoped for when we founded ACGT a decade ago," noted Barbara Netter, president and co-founder of the Alliance for Cancer Gene Therapy. "We knew it would be an uphill battle. ACGT was the only organization willing to take the risk when others were not. With federal funds decreasing, and the realization that pharmaceutical companies will not participate in the research phase until marketable and mass-produced treatments are created, we seized the chance to make a difference. My late husband Edward Netter (1932-2011), was a true visionary in the field of medical research. He would be so thrilled by the progress ACGT has made possible." ACGT is currently funding 17 clinical trials in cancer cell and gene therapy targeting numerous types of cancers. Since its inception, ACGT has awarded more than $23 million in grants to 39 investigators to treat 11

different types of cancer. In 2012, ACGT also awarded a $500,000 grant to a clinical translational study on pancreatic cancer and has great expectations for its outcome. In ACGT's 2011 grant cycle, 87 scientists from throughout the U.S. responded with grant applications for ACGT's Young Investigator Grants, making 2011 one of the most sought-after funding year in ACGT history. The ACGT funded clinical studies are already showing promise, especially in the treatment of leukemia, lung, melanoma and prostate cancers.

"It is so exciting that the pathfinder role ACGT played by provided the seed money for the University of Pennsylvania trial has led to these stunning successes for leukemia treatment," said Dr. Savio Woo, chairman of ACGT's Scientific Advisory Council, and founding Chair of the Department of Gene and Cell Medicine at Mt. Sinai School of Medicine in New York City.

Woo noted that 100 percent of all funds donated to ACGT go directly toward funding innovative cancer gene therapy research grants. ACGT's Scientific Advisory Council, which comprises some of the best scientific minds and thought leaders with major U.S. medical institutions, oversee all phases of the ACGT grant process. Through this rigorous review, ACGT is able to identify and fund studies with the most potential for positive and innovative outcomes for treating cancer using cell and gene therapy.

The University of Pennsylvania's initial study was funded primarily by ACGT. The most recent clinical trial was also supported by ACGT, the Leukemia & Lymphoma Society (Dr. June is the leader of one of the LLS's grants), and the National Institutes of Health. In addition, Novartis announced this summer that it would fund additional research at the University of Pennsylvania to further study the immunotherapies and has acquired exclusive rights to market the treatment.

Alliance for Cancer Gene Therapy (ACGT) is the nation's only non-profit dedicated exclusively to cell and gene cancer therapy research. One hundred percent of all contributions to ACGT go directly to research and fund grants with leading scientists in the U.S., representing such institutions as Harvard Medical School, Johns Hopkins University School of Medicine, Mayo Clinic, St. Jude's Children's Hospital, Duke University, The Salk Institute, University of Pennsylvania, Memorial Sloan-Kettering, Stanford University, Dana Farber Cancer Center, University of California San Diego, University of Pittsburgh, and the University of Chicago. A rigorous grant review by Scientific Advisory Council ensures the most promising projects are rewarded. To learn more about the leukemia study at the University of Pennsylvania, and about the Alliance for Cancer Gene Therapy (ACGT), visit www.acgtfoundation.org or call 203.358.8000.

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