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ACGT ALLIANCE FOR CANCER GENE THERAPY

Advancing molecular therapies for cancer

January 21, 2014

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IMMUNOTHERAPY LEADS THE WAY

An 8-year old girl with acute lymphocytic leukemia was expected to succumb within days, and after gene therapy treatment at the University of Washington, Seattle, she has been in complete remission for two years. 120 patients in total have participated in similar trials across the country with similar results.



Dr. Laurence Cooper

"It's exciting to see these encouraging initial results with engineered immune cells, particularly such a durable response among patients who have very aggressive disease that has relapsed after standard treatments," says Dr. Laurence Cooper, MD, PhD, ACGT Fellow and Professor in the Department of Pediatrics Patient Care, MD Anderson Cancer Center in Houston.

At the National Cancer Institute, 11 patients with lymphoma and four with chronic lymphocytic leukemia were treated with immunotherapy drugs - six had full remission and six partial. Similar studies are underway at Memorial Sloan-Kettering Cancer Center, MD Anderson Cancer Center, University of Pennsylvania, and Baylor University, and thus far, all treatments have been positive, with little or no relapse. Much of the original research was funded by ACGT. Immunotherapy treatments for blood and bone marrow cancers filter a patient's blood to re-engineer T-cells which, once injected back into the patient, within just a few days, turn into an army of killer soldiers on the offense that also remain on defense. These treatments may soon yield the first FDA approved drug - stay tuned!

MARCHING INTO THE FUTURE

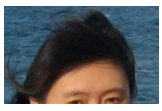
In their year-end issue, *Science Magazine* named Cancer Immunotherapy the "Breakthrough Science of the Year."



ACGT has been a proponent of immune-mediated research for a decade, including trials at the University of Pennsylvania and Memorial Sloan-Kettering that prove the science to be among the most effective therapies for cancer. Immunotherapy harnesses the body's immune system to destroy cancers, and protect against resurgence, with limited treatment and with few if any side effects. "Oncologists, a grounded-in-reality bunch, say a corner has been turned and we won't be going back." Read the full article on the *Science* site.

"We are on the cusp of a golden age of cancer prevention and treatment thanks to the promise of gene and cell therapy."
- Michel Sadelain, MD, PhD, Memorial Sloan-Kettering Cancer Center, 2004 ACGT Fellow - Gene Therapy for Lymphoma and Leukemia Award.

TWO YOUNG INVESTIGATOR GRANTS ANNOUNCED



Fan Yang, PhD, Assistant Professor, Orthopedic Surgery and Bioengineering, Stanford University, has been awarded a $250,000 grant to fund research into



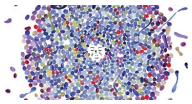
a new treatment for pediatric brain cancer, the leading fatality from childhood cancer. Pediatric brain tumors are highly invasive and penetrate the delicate brain stem, making them especially difficult to treat. The study will deploy stem cells targeted to the tumor, in conjunction with chemotherapy. Yang earned a PhD and completed her residency at Johns Hopkins University and was a fellow at MIT.



Arnob Banerjee, MD, PhD, Assistant Professor, Hematology and Oncology, at the University of Maryland, will use his $250,000 grant award to study methods to optimize immunology for many forms of cancer. Immune-mediated treatments are the most advanced form of gene therapy, activating the body's immune system to reject and/or eradicate tumor cells. This study aims to defuse obstacles to treatment by opening T-cell pathways. Banerjee earned his MD/PhD at Columbia University and was a resident, fellow and instructor at the University of Pennsylvania.

Young Investigator grants fund 2 - 3 year studies by tenure-track assistant professors exploring cancer cell and gene therapies at the best research institutions in North America. In total, 29 Young Investigator grants have been awarded for innovative research into new treatments for cancer.

EVERYONE SEEMS TO HAVE CANCER



In a January 5th article "Why Everyone Seems to Have Cancer" in the *New York Times,* George Johnson, author of "The Cancer Chronicles", points out the 20% decline in cancer fatality in the last twenty years, based largely on early detection. As a result, more Americans are living with cancer and this too takes a tremendous toll on patients and loved ones. Remission is not a cure, and over 13 million Americans living with cancer fear recurrence or deal with the lingering toxicity of treatments. We want people to live longer and also live healthier lives.

Help us support breakthrough research that is changing the way cancer is treated and defeated. Donate in honor or in memory of a loved one, host an event, tell your friends, and keep posted on progress at www.acgtfoundation.org, on Facebook and Twitter@acgtfoundation.

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