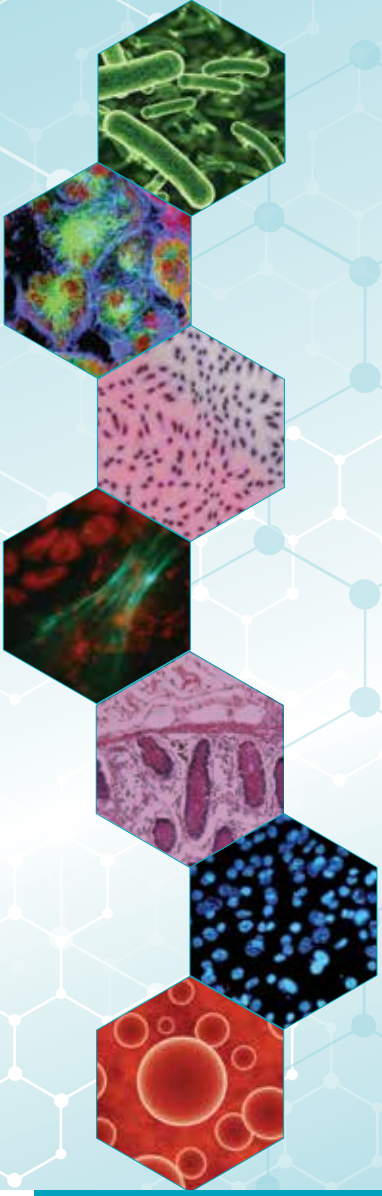
Treat Cancer to Defeat Cancer



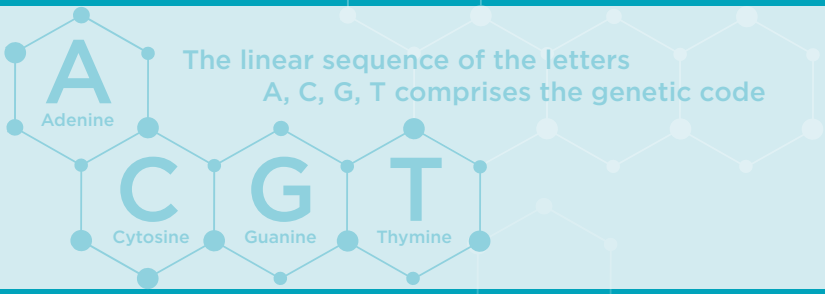
ACGT® ALLIANCE FOR CANCER GENE THERAPY

Discovering new ways to treat and defeat cancer

2016

#acgtProgressReport

Alliance for Cancer Gene Therapy is the only charitable organization in the nation dedicated exclusively to funding and fostering research into cell and gene therapies for cancer.



Our mission is to seek out and support revolutionary scientific research into the causes, treatment and prevention of all types of cancer, using cells and genes as medicine.

To this end we fund and monitor studies and trials that meet the highest possible standards, educate the public on the potential for targeted medicine, and facilitate greater collaboration among the scientific and philanthropic communities to promote progress.

A 501 [c] [3] public charity founded in 2001 by Barbara and Edward Netter. Approved by the coalition of Better Business Bureau and the National Charities Information Bureau.

#acgtIntheNews

Cancer Moonshot Takes Aim.
Vice President Joseph Biden announced the start of a national public access medical database to centralize genomic and clinical data, opening with 12,000 patients from the National Cancer Institute. The initiative, funded through the NIH, aims to bring to market by 2020 more effective therapies for cancer.

***New York Times* series reports on immunology breakthroughs.**
Citing long-term remissions for blood cancers, science reporters demystify the science and spotlight ACGT Fellows Dr. Carl June and Dr. Michel Sadelain for advances in the field.

First Oncolytic Viral Therapy approved for Melanoma.
The FDA approved Imlygic from Amgen, the first drug using a genetically modified virus to treat melanoma.

Drug Giants Kite Pharma, Novartis & Juno Therapeutics announced hope for approvals in the next year or so for immunotherapy treatments for various cancers.

ACGT Fellows featured for revolutionary approach. Dr. Meenakshi Hegde and Dr. Nabil Ahmed reported progress using an approach to destroy antigens that prevent treatment for glioblastoma, one of the more deadly brain cancers.

Rally for Medical Research on Capitol Hill lobbied legislators to secure $2 billion more for medical research threatened by budget cuts.

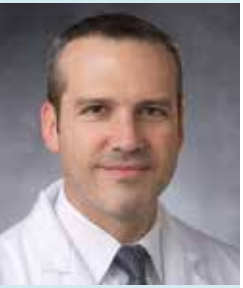
#acgtGrantAwards

Young Investigator Awards fund $250,000 over three years for innovative laboratory research at a dedicated research institute.

Yvonne Yu-Hsuan Chen, MS, PhD

Assistant Professor, Chemical & Biomolecular Engineering, University of California, Los Angeles

Immunotherapy for Lymphoma/Leukemia. Dr. Chen focuses on T-cell therapy using a patient's own immune cells to attack cancer. Reprogrammed cells contain a synthetic protein that binds to disease markers to attack cancer cells. She studies patients who evidence a specific marker in lymphoma and leukemia that prevents a response to conventional therapies. She aims to refine the technique and develop a screening method for consistently high impact. Dr. Chen earned an MS and PhD in Chemical Engineering at the California Institute of Technology and held postdoctoral positions at Seattle Children's Research Institute and Harvard Medical School.



Brent Hanks, MD, PhD

Assistant Professor, Duke University Medical Center

A Vaccine for Melanoma. Melanoma has been steadily increasing and once spread, patients have less than a 10% prognosis for recovery. Dr. Hanks studies cancer pathways that block the immune system, particularly a fatty acid transporter that plays a crucial role. The hope is a vaccine to neutralize the barrier, so the body's natural killer T-cells can destroy cancer cells. Dr. Hanks served as Fellow and Resident at Duke University Medical Center after earning an MD in Medicine and a PhD in Cancer Immunology at Baylor College of Medicine.

Samuel G. Katz, MD, PhD

Assistant Professor, Pathology, Yale University

Immunotherapy for Blood Cancers; Swim Across America Award. Immunotherapy often relies on a dual-action protein known as a chimeric antigen receptor [CAR] to recognize a tumor as a threat. Dr. Katz's study uses RNA to target blood cancer to maximize impact and minimize effect on healthy cells. Dr. Katz earned his MD in Medicine and a PhD in Genetics at Harvard Medical School, Division of Medical Sciences, served a residency and fellowship at Brigham and Women's Hospital, and post-doctoral fellowship at Dana Farber Cancer Institute.

Crystal Mackall, MD

Professor, Pediatrics/Internal Medicine, Stanford University

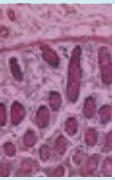
Immunology for Osteosarcoma; The Wendy Walk-ACGT Sarcoma Grant. Wendy Walk is a non-profit founded in 2010 by Wendy Landes' children. The joint Clinical Investigator Award funds $500,000 over three years for translational research from the lab to clinical trial.

Osteosarcoma is a lethal bone cancer that attacks children. Dr. Mackall's research uses a patient's engineered T-cells to destroy cancer cells. She received her MD from Northeastern Ohio University and served a fellowship in pediatric hematology/oncology and a post-doctoral fellowship at the National Cancer Institute. She recently was Head of the Immunology Section and Chief of the Pediatric Oncology Branch at the NCI.

For a complete listing of ACGT grantees, visit www.acgtfoundation.org

#acgtImmunotherapy

At the forefront of cell and gene therapy is immunotherapy, also known as immune-mediated therapy or immunology. Because cancer is organic and because cancer cells are even more tenacious than normal cells in defending intervention, the immune system is inactive with cancers. Immunology aims to awaken the immune system to the threat and deactivate the inhibitors that block the invasion of killer T-cells.



"This is a sea change – melanoma, kidney, lung, esophageal, bladder, liver, neck and head cancers all respond to immunotherapy."

– Dr. Michael Lotze, Member, ACGT Scientific Advisory Council, Lion Biotechnologies, Inc.

Immunology is a concept that has been around for many years, but advanced technology has made it possible for tremendous progress in just the last decade.

This approach to gene therapy is a noninvasive procedure: doctors extract a patient's own cells then inject the cells back after re-engineering them in the laboratory. The immune system recognizes the threat and the cancer target is clearly marked.

CAR-T cell therapy has shown remarkable success treating B-cell hematologic malignancies such as forms of leukemia and lymphoma. The technique focuses on the receptors in T-cells that lock into protein fragments found on the surface of abnormal cells. In this way, modified T-cells attack the cancer and only the cancer, without the toxicity of traditional treatment. Results may range from 40-90% response within days or weeks. More funding for research and trials are needed with solid cancers.

The next challenge is to match the most effective treatment to each individual.

#acgtOncolyticViralTherapy

Also called OV or virotherapy, this treatment uses a virus to awaken the immune system. Viruses are harmless, once decoded, and there are over 3500 known viruses with the potential to be therapeutic agents.

Viruses spread rapidly within cancer cells and signal the immune system to attack the invader.

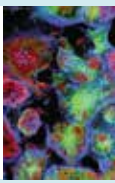
A simple concept, contemplated a century ago, but only possible now through the advances of genetic engineering and the mapping of the human genome.

In research, oncolytic viruses have an exemplary safety record. Just this year, virotherapy was successfully used to treat glioblastoma, a deadly brain cancer, as well as melanoma, blood, lung and colon cancers.

Researchers throughout the world are studying the distinctions between types of cancers to learn how to better use viruses to penetrate cancer cells and stimulate an immune response.

"Cancer patients who previously had no opportunity to survive are alive, particularly those with leukemia and melanoma, and some solid cancers."

– Dr. John Bell, Member, ACGT Scientific Advisory Council, Ottawa Hospital Research Institute



Major pharmaceutical companies are looking closely at the potential for virotherapy drugs like Imlygic, an OV treatment approved for metastatic melanoma.

The challenge is to match the right virus to the cancer and to learn how to mass manufacture viral agents to treat large numbers of patients.

#acgtCRISPR

Clustered Regularly Interspaced Short Palindromic Repetition [CRISPR] is the talk of the medical science community. What is it? A fantastically precise tool for genetic editing.

The mapping of the human genome opened the door to revolutionary treatments and provides an opportunity to target a cancer treatment to match the genetic profile. CRISPR will allow us to make miniscule changes in the DNA that can stop cancer in its tracks.

The technique was revealed just three years ago by researchers using a DNA cut and paste with bacteria and viruses. They realized they could also edit human genes.

"The most exciting aspect of gene therapy is identifying the unique attributes of each patient, so we can better target the treatment and with fewer side effects."

– Dr. Stephen Eck, Member, ACGT Scientific Advisory Council, Astellas Pharma, Inc.

Once the cancerous cell is cut, it will repair itself or the body's natural killer T-cells will take aim to destroy diseased cells. In effect, we will chisel cancer out of DNA.

The discipline is so new that scientists from around the world gathered this year in Washington, DC for the first international summit on human gene editing. They agreed on several principles not the least of which is not to use the tool for human genetic engineering. On the other hand, CRISPR is being used to stimulate cancers in laboratory animals, for the purpose of study.

CRISPR has the potential to battle all forms of disease as well as genetic disorders. The opportunities are limitless.

#acgtHighlights

John Walter named ACGT CEO/President. The former CEO of the Leukemia and Lymphoma Society, John dramatically expanded research collaborations between LLS and biotech and academia, and spearheaded record revenue growth. John previously worked with the March of Dimes, Donaldson, Lufkin & Jenrette, and Bristol-Myers Squibb. He succeeds Barbara Netter, who continues her leadership role as Honorary Chairman.

Scientific Symposium on Research Status and Challenges. ACGT Research Fellows, Board and Scientific Advisory Council Members, and special guests, focused on next steps for cell and gene therapies. All agreed that the research has broken treatment barriers and human trials have saved or extended lives. The challenge ahead is to refine therapeutic design and delivery systems, and match treatment to the cancer profile and to the individual.

Chau Q. Khuong Joins the Board of Directors. A partner at OrbiMed Advisors, a global health care investment firm, Chau has been an active lead investor in innovative drug development. He earned a BS in molecular, cellular and developmental biology, and an MPH, with a focus on infectious disease, at Yale University.

10th Annual Swim Across America Greenwich-Stamford Event a Great Success. The popular event on behalf of ACGT put total revenues over the decade at $3 million+. Funds raised this year continue to support three Swim Across America-ACGT grants for brain cancer, blood cancer and pediatric brain cancer treatments.

#acgtSupporters 2015-16

Thank you for your support. Every penny counts and every penny goes directly to research – a separate fund supports administrative expenses. Stay with us, we are SO close.

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#acgtFinancialReport

Fiscal years ended April 2015 and 2016

CONDENSED STATEMENTS OF ACTIVITIES	2016	2015
Support and revenue:		
Contributions	$950,413	$960,174
Contributions for administrative expenses (specially designated)	313,443	364,150
Contributed services	461,802	357,585
Interest and dividend income	101,788	102,399
Realized and unrealized gains on investments, net	(362,703)	160,067
TOTAL SUPPORT AND REVENUE	1,464,743	1,944,375
Expenses:		
Program services:		
Research	1,626,508	1,739,756
Management and general	219,584	225,863
Fundraising	154,222	103,799
TOTAL EXPENSES	2,000,314	2,069,418
(Decrease) increase in total net assets	(535,571)	(125,043)
Total net assets at beginning of year	3,857,659	3,982,702
TOTAL NET ASSETS AT END OF YEAR	$3,322,088	$3,857,659

CONDENSED STATEMENTS OF FINANCIAL POSITION	2016	2015
Assets:		
Cash and cash equivalents	$1,259,779	$646,150
Contributions receivable, net	3,861,376	4,361,280
Investments, at fair value	10,633	24,523
Accounts receivable and prepaid expenses		
TOTAL ASSETS	$5,131,788	$5,031,953
Liabilities and net assets:		
Liabilities		
Grants payable	$56,401	$35,041
Accrued expenses	1,753,299	1,139,253
TOTAL LIABILITIES	1,809,700	1,174,294
TOTAL NET ASSETS	3,322,088	3,857,659
TOTAL LIABILITIES AND NET ASSETS	$5,131,788	$5,031,953

Alliance for Cancer Gene Therapy, Inc.'s complete financial statements are available upon request.

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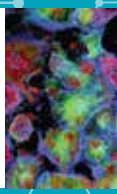
"Right now, we are extending lives using cell and gene therapies as an adjunct to chemotherapy, and soon, based on new research, clinical trials and advanced technology, these therapies will take the lead and save lives."

– Dr. Joseph Glorioso, University of Pittsburgh



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