

MOMENTUM

Accelerating Progress for Cancer Gene Therapies

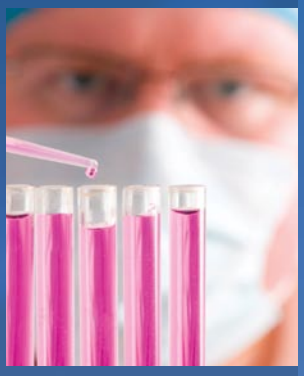
ACGT®
National grants for cancer research
ALLIANCE FOR CANCER GENE THERAPY

2008 Annual Report

MOMENTUM

Objects or concepts moving forward are said to have



Alliance for Cancer Gene Therapy, Inc. [ACGT] is a 501[c][3] non-profit public charity founded in 2001 by Barbara and Edward Netter to facilitate funding for high potential research into gene therapy, in the hopes of realizing an effective alternative to the treatment and management of cancer.

ACGT is approved by the coalition of the Better Business Bureau and the National Charities Information Bureau for meeting the highest standards of governance, effectiveness, public communications and financial responsibility.

MOMENTUM

Success can be defined as that moment when momentum arrives at its destination – when the train enters the station, when the home run hitter arrives at home plate, when theory becomes reality.

Momentum builds on itself, unless slowed by an opposing force. As the only public charity in the country devoted solely to cell and gene therapies for cancer, we are the engine of momentum.

In just seven years, ACGT has become a positive force for molecular medicine, working with countless institutions, hundreds of the best scientists and physicians, and thousands of supporters and beneficiaries.

The science and technology will continue to gain momentum, if only we have the collective determination and funding to keep moving forward.

MISSION

The mission of ACGT is to support the extraordinary potential offered by cell and gene therapies to accelerate effective and safe treatment of all types of cancer.

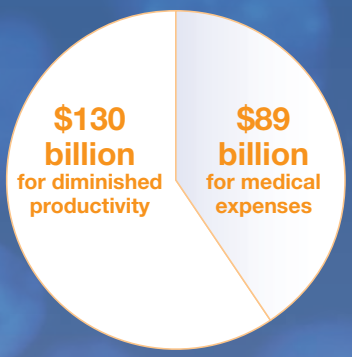
To this end, ACGT will identify, fund and monitor innovative research that meets a rigorous set of standards and promotes a new paradigm for cancer treatment.

Through this endeavor, ACGT will educate the public about the potential for cancer gene therapy and facilitate greater collaboration among the scientific and philanthropic communities to foster the best possible research and ultimately the best treatment.

1.4 million new cases of cancer were diagnosed in the United States in 2008.

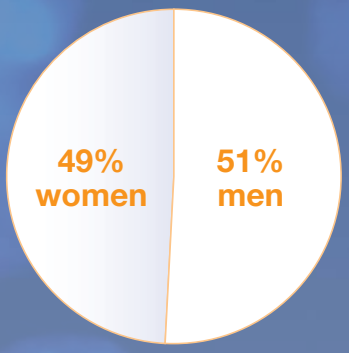
Nearly **600,000** cancer patients will die this year.

CANCER MUST BE STOPPED



The National Institutes of Health estimates the overall cost of cancer was $219.2 billion in 2007.

Of the 10 million people living with cancer, only 1 in 10 is a long-term survivor.



Cancer is gender-blind.

Strides have been made in early detection and drug therapies, but most treatments fail to attack cancer at the core: in the genes.

Statistical information excerpted from the American Cancer Society: *Cancer Facts & Figures 2008*

Cancer is recession-proof. 160 people will be diagnosed as you read this report. Each day this year, 3,835 will discover they have cancer. Another 11,507 will die this week. We cannot promise a breakthrough this year or next year, or the next, but we can promise you that great strides are being made every day by scientists all over the country researching what we believe is the future for cancer treatment: gene and cell therapies. Because we know now for certain that cancer is a gene-based disease.



ACGT is the only public charity funding research exclusively into cell and gene therapies for cancer. We support many of the leading researchers in the field and see progress being made every day. We founded this organization based on the integration of science, philanthropy and good business practices, and the result has been a research program with integrity and promising results. Our accomplishments have been recognized by the coalition of the Better Business Bureau and the National Charity Information Bureau, by the media, and the more than 100 research institutions that are studying molecular medicine.

This organization has momentum and the science has momentum. Most importantly, we have confidence in cell and gene therapies as the future for cancer treatment. Thank you for being part of the momentum fueling our journey, and for your support – past, present and future.

Barbara Netter
Edward Netter

Edward and Barbara Netter,
Co-Founders, Alliance for Cancer Gene Therapy

MOMENTUM

The potential for molecular medicine is also the challenge: gene and cell therapies attack only the cancer without damage to other parts of the body, but getting the right remedy to the right gene requires new thinking and new technologies. We have been optimistic from the start and continue to be optimistic. Why? Because more than 800 gene therapy trials are ongoing, of which two-thirds are for cancer. More and more, we receive reports of positive outcomes. Thousands of patients have been treated with very few adverse effects. And, gene therapies have been applied for almost all forms of cancer, as well as diseases like muscular dystrophy, and Parkinson's and conditions such as blindness. Our goal is to identify and fund the most promising cell and gene therapies for cancer and monitor progress to ensure that every dollar is spent wisely and effectively. ACGT is well established and highly respected, and we work closely with the best and the brightest of medical scientists and institutions. We're in the business of challenging the status quo and creating entirely new options, but we cannot do this alone.



This is not the time to slow down, despite economic challenges. This is not the time to forsake the future for immediacy. This is the moment to step up and maintain the momentum we have created. We must keep our eye on the prize because people are dying and cancer demands new treatments. The potential is within our sights. Stay with us - we are on our way.

Margaret Cianci
Executive Director

ACGT 2002-2008

504 Applications representing 131 Institutions

32 Research Projects Funded:

Discovery [Basic]	15
Pre-Clinical Phase	6
Phase I Trials	10
Phase II Trials	1

ACGT ACCELERATES RESEARCH IN 2008

76 Applications representing 52 Institutions

7 Finalists

1 Funded Project

$19.8 MILLION GRANTED

Young Investigator Grants:
21 $ 9.3 million

Investigator Grants:
11 $10.5 million

Institutions with ACGT Fellows: 25

100%



of donations to ACGT go directly to research. Separate funding is provided to cover all administrative and fundraising costs.

ACGT RESEARCH FELLOWS 2002 – 2008

2008

Dr. Carl June
INSTITUTION: University of Pennsylvania Abramson Family Cancer Research Institute
FOCUS: Ovarian Cancer
RESEARCH: Immune-mediated Gene Therapy
AWARD: Recipient of The Joan Miller & Linda Bernstein Gene Therapy Ovarian Cancer Award

2007

Antonio E. Chiocca, MD, PhD
INSTITUTION: Ohio State University Research Foundation
FOCUS: Glioma
RESEARCH: Tumor Specific Replicating Viruses & Bacteria

Ronald Levy, MD
INSTITUTION: Stanford University
FOCUS: Lymphoma
RESEARCH: Immune-mediated Gene Therapy & Cancer Vaccines

Clodagh O'Shea, PhD
INSTITUTION: Salk Institute for Biological Studies
FOCUS: Breast Cancer
RESEARCH: Tumor Specific Replicating Viruses & Bacteria

Khalid Shah, PhD
INSTITUTION: Harvard Medical School, Massachusetts General Hospital
FOCUS: Brain Cancer
RESEARCH: Tumor Targeting
AWARD: Recipient of The Swim Across America Young Investigator Award

2006

Roberto Cattaneo, PhD
INSTITUTION: Mayo Clinic College of Medicine
FOCUS: Lymphoma/Leukemia
RESEARCH: Tumor Specific Replicating Virus

George Coukos, MD, PhD
INSTITUTION: University of Pennsylvania Abramson Family Cancer Research Institute
FOCUS: Ovarian & Peritoneal Cancer
RESEARCH: Anti-angiogenesis
AWARD: Recipient of The Dr. Judah Folkman Angiogenesis Award for Cancer Gene Therapy

Maciej S. Lesniak, MD
INSTITUTION: University of Chicago
FOCUS: Brain Cancer
RESEARCH: Tumor Targeting & Vector Development

Kah-Whye Peng, PhD
INSTITUTION: Mayo Clinic Cancer Center
FOCUS: Myeloma
RESEARCH: Tumor Specific Replicating Viruses & Bacteria

Eckhard Podack, MD, PhD
INSTITUTION: University of Miami Miller School of Medicine
FOCUS: Lung Cancer
RESEARCH: Immune-mediated Gene Therapy

Harald Sauthoff, MD
INSTITUTION: New York University
FOCUS: Breast/Lung Cancer
RESEARCH: Tumor Targeting & Vector Development

2005

Biao He, PhD
INSTITUTION: Pennsylvania State University
FOCUS: Breast Cancer
RESEARCH: Tumor Specific Replicating Virus

Thomas J. Kipps, MD, PhD
INSTITUTION: University of California, San Diego, Moores Cancer Center
FOCUS: Lymphoma/Leukemia
RESEARCH: Immune-mediated Gene Therapy

Hyam I. Levitsky, MD
INSTITUTION: Johns Hopkins University School of Medicine
FOCUS: Lymphoma/Leukemia
RESEARCH: Immune-mediated Gene Therapy

Miguel Sena-Esteves, PhD
INSTITUTION: Massachusetts General Hospital/Harvard Medical School
FOCUS: Brain Cancer
RESEARCH: Oncogenesis

Koji Tamada, MD, PhD
INSTITUTION: University of Maryland
FOCUS: Lymphatic Systems
RESEARCH: Immune-mediated Gene Therapy

Xianzheng Zhou, MD, PhD
INSTITUTION: University of Minnesota – Twin Cities
FOCUS: Blood, Lymphoma/Leukemia
RESEARCH: Immune-mediated Gene Therapy

Yiping Yang, MD, PhD
INSTITUTION: Duke University
FOCUS: Lymphoma/Leukemia
RESEARCH: Immunotherapy

2004

Chien-Fu Hung, PhD
INSTITUTION: Johns Hopkins University School of Medicine
FOCUS: Ovarian Cancer
RESEARCH: Immunotherapy

Mukesh Jain, MD, FAHA
INSTITUTION: Case Western Reserve University
FOCUS: Metastatic Cancer
RESEARCH: Anti-angiogenesis

Carl H. June, MD
INSTITUTION: University of Pennsylvania Abramson Family Cancer Research Institute
FOCUS: Lymphoma/Leukemia
RESEARCH: Immune-mediated Gene Therapy

Suzie Pun, PhD
INSTITUTION: University of Washington
FOCUS: Metastatic Cancer
RESEARCH: Targeted Non-Viral Delivery
AWARD: Recipient of The Patricia Zoch Tate Gene Therapy Award

Michel Sadelain, MD, PhD
INSTITUTION: Memorial Sloan Kettering Cancer Center
FOCUS: Lymphoma/Leukemia
RESEARCH: Immune-mediated Gene Therapy

Jian Yu, PhD
INSTITUTION: University of Pittsburgh School of Medicine, Hillman Cancer Center
FOCUS: Lung Cancer
RESEARCH: Oncogenesis

2003

Laurence Cooper, MD, PhD
INSTITUTION: University of Texas, MD Anderson Cancer Center
FOCUS: Lymphoma/Leukemia
RESEARCH: Immune-mediated Gene Therapy

Timothy Lane, PhD
INSTITUTION: University of California, Los Angeles
FOCUS: Breast Cancer
RESEARCH: Anti-angiogenesis
AWARD: Recipient of The Kimberly Lawrence Netter Gene Therapy Breast Cancer Award

Todd R. Reilly, PhD
INSTITUTION: Johns Hopkins University [thru 2006]
FOCUS: Breast Cancer
RESEARCH: Immune-mediated Gene Therapy

Katherine Ryman, PhD
INSTITUTION: Louisiana State University Medical Center, Shreveport
FOCUS: Prostate Cancer
RESEARCH: Tumor Specific Replicating Viruses

Robert Vonderheide, MD, D.Phil.
INSTITUTION: University of Pennsylvania Abramson Family Cancer Research Institute
FOCUS: Neuroblastoma
RESEARCH: Immune-mediated Gene Therapy

2002

Jeffrey S. Bartlett, PhD
INSTITUTION: Children's Research Institute, Columbus
FOCUS: Ovarian Cancer
RESEARCH: Vector Development

Andrew M. Davidoff, MD
INSTITUTION: St.Jude Children's Research Hospital
FOCUS: Neuroblastoma
RESEARCH: Anti-angiogenesis

Thomas S. Griffith, PhD
INSTITUTION: University of Iowa
FOCUS: Prostate Cancer
RESEARCH: Immune-mediated Gene Therapy

Read more about ACGT Research Fellows at www.acgtfoundation.org

MOMENTUM

EDUCATING THE PUBLIC ON THE PROMISE OF GENE THERAPIES

WINTER 2008 **At an educational forum in January at Greenwich Library in Connecticut,** George Coukos, MD, PhD, of the University of Pennsylvania Abramson Family Cancer Research Institute, and an ACGT Research Fellow, presented the status of his ground-breaking trials for vaccines to combat ovarian cancer. The science of immunotherapy utilizes the patient's own tumor cells to trigger an immune response. He was joined by Leisha Emens, MD, PhD, of the Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins University who discussed the development of a therapeutic vaccine for particularly aggressive breast cancers, based on starving blood cells from the cancer (anti-angiogenesis). Ovarian cancer is one of the silent killers, claiming 60% of victims, in contrast to breast cancer where a reduced mortality rate is based largely on improved diagnostics, resulting in treatment in early stages. There are currently several promising research projects and clinical trials exploring the potential for gene therapies for both ovarian and breast cancers. The discussion was moderated by Dr. Stephen Eck, ACGT Scientific Advisory Council member and Vice-President of Translational Medicine and Pharmacogenomics at Eli Lilly and Company.

ACGT attended the Health Research Alliance conference in March in Washington, D.C. "Accelerating Medical Discovery Through Strategic Philanthropy" drew hundreds of participants and speakers in the field of health research from the non-profit sector as well as industry, academia and government. While foundation giving has been on the rise since 1995, grants to biomedical research have not kept pace, a subject of particular interest to the 64 not-for-profit organizations attending. Discussions included opportunities in science, strategic planning in philanthropy, training the translational researchers of tomorrow, grant-making and managing change.



"Because of gene therapy, I am standing here talking to you tonight."

– Connie Burnett-West, *participant in gene therapy trial for lung cancer.*

SPRING 2008 **Jack Roth, MD, FACS, member of the ACGT Scientific Advisory Council,** and director of the W. M. Keck Center for Innovative Cancer Therapies at the University of Texas M. D. Anderson Cancer Center in Houston, reported on cutting edge research for lung cancer to a packed crowd at the Bruce Museum in Greenwich, Connecticut in April. Dr. Roth, a leading researcher in tumor suppressor gene therapy for cancer, has focused his studies on the genetic events involved in lung carcinogenesis, with treatment that not only is proving successful, but has no side effects, as it treats only the cancer. Roth was joined by cancer survivor Connie Burnett-West who, at the age of 54, received the devastating news that she had tumors in both lungs with little hope for recovery. Not a candidate for surgery or radiation, she endured two chemotherapy trials, which she said almost killed her, before she was recommended to a gene therapy trial at Mary Crowley Medical Research Center at Baylor University Medical Center in Dallas. Using a gene suppressor treatment called GVAX, cancer cells were surgically removed from her lungs and used to create a vaccine. In 2000, she received six injections in six months and has been tumor-free ever since.

"The greater our funding to accelerate research, the closer we come to our goal of wiping out this disease, which is presently of epidemic proportions."

– Barbara Netter, *ACGT Co-Founder and Chair of Special Events*



At the annual conference of the American Society for Gene Therapy in Boston in May, ACGT Research Fellow Maciej Lesniak, MD, received the Outstanding New Investigator Prize. Dr. Lesniak, an Assistant Professor at the University of Chicago, is researching tumor targeting and vector development for brain cancer with funding from an ACGT Young Investigator grant. At a roundtable discussion hosted by ACGT, research scientists discussed critical issues affecting the progress of accelerating clinical translation and drug delivery.

SUMMER 2008 **110 cancer survivors, or supporters of survivors, swam, floated or boated on Long Island Sound in June** to help raise funds for cancer gene therapy research. Among the swimmers were Olympic silver medalist Janel Jorgensen, Executive Director of Swim Across America, who was also on hand to present awards for achievement, and Olympic swimmer Glenn Mills. Participants raised over $100,000, an amount matched by corporate sponsorships and supporting contributions, for a total of $200,000. Proceeds from the event will continue to fund Dr. Khalid Shah, an Assistant Professor at Harvard Medical School and physician at Massachusetts General Hospital, who is exploring gene therapy alternatives for the treatment of brain tumors. Swim Across America, founded in 1987 by Connecticut natives Matt Vossler and lifelong friend Jeff Keith, a cancer survivor, has generated over $20 million through swims of all types

"Those of us in the know, know that gene therapy is going to be the thing that finally solves the problem for us." – Robert Easton, *Chair of the Board, Gilda's Club New York City*

throughout the country, hoping to raise awareness for cancer research, prevention and treatment. ACGT Board member John Sites of Wexford Capital, LLC, and wife Cindy were joined by Reuben Mark, former CEO of Colgate-Palmolive, and wife Arlene, as Honorary Chairs.

ACGT is the first organization listed under Research Resources on the "Stand Up 2 Cancer" website, and we were proud to be part of this landmark media event. Hosted by the anchors of all three network news programs, and watched by millions around the world, the telethon not only raised millions of dollars, but re-invigorated a global commitment to win the war on cancer.

***US News and World Report* in September 2008** presented their rankings of the nation's top cancer centers. We are proud to be funding research projects at 11 of the top 12 institutions.

FALL 2008 **ACGT co-founder, Edward Netter, was profiled in the September issue of *Fairfield County Business Journal.*** An article entitled "The Philanthropist's Journey" focused on the weaving of philanthropy with sound business principles. In just seven years, ACGT has become a force for cancer research, an acknowledged innovator and an inspiration to anyone who cares about making a difference not only in people's lives, but in how medical research is conducted. Mr. Netter has proven that, with passion and purpose, one can move an idea towards realization.

At an October event co-sponsored by ACGT and Foundation Source entitled *Fast-Tracking Medical Cures through Philanthropy,* ACGT co-founder Edward Netter spoke as a founder of a medical research foundation. He was joined by Greg Simon, President of FasterCures, whose mission is to accelerate the discovery and development of new medical research techniques, and is using a unique assessment metric to cultivate donor confidence in organizations like ACGT that follow best practices. Mr. Netter addressed the importance of blending philanthropy with good business practices.

Dr. Harald Sauthoff, a 2006 ACGT Young Investigator and Assistant Professor of Medicine, Pulmonary and Critical Care at NYU School of Medicine, spoke at a symposium at Gilda's Club in New York City in November. His research into gene therapies for breast and lung cancer centers around the p53 tumor suppressor gene which, when turned on, activates other genes as a defense against cancer, an important innovation in immunotherapy.

"Looking into the future we will see more powerful therapeutics using genes and cells. The research is exciting – and we are at a critical juncture."



– Dr. Savio Woo, *Professor and Chairman of the Department of Gene and Cell Medicine at Mount Sinai School of Medicine*

ACGT Scientific Advisory Council Chair, Dr. Savio Woo, Professor and Chairman of the Department of Gene and Cell Medicine at Mount Sinai School of Medicine, was honored at the Gilda's Club annual gala in November for his pioneering work on human molecular medicine and gene therapies for inherited disorders. "Cancer has been with us for the longest time, but we all know that therapies have side effects and morbidity," said Dr. Woo in his remarks. "Looking into the future, we will see more powerful therapeutics using genes and cells. The research is exciting – and we are at a critical juncture."



IN MEMORIAM

Our friend and colleague, Dr. Judah Folkman, passed away in January 2008. For 36 years, often in the face of great skepticism, the renowned researcher at Harvard University and Children's Hospital Boston believed and ultimately proved that tumors could be stopped by cutting off their blood supply, in effect strangulating cancer. Known as the father of anti-angiogenesis, he left behind a blueprint for an entirely new approach to the treatment of many diseases, and a loyal following of molecular biologists who will sustain his legacy. Dr. Folkman was a founding member of ACGT's Scientific Advisory Council and in 2006 was honored at the Fifth Anniversary Dinner with the organization's first Lifetime Achievement Award for discovery and visionary leadership.

"Judah Folkman embodied the passion, dedication and perseverance essential to scientific discovery and advancement. Like any true leader, he led by example, inspiring the many research scientists that follow in his path. I was proud to know him and grateful for his commitment to gene therapy and to ACGT." – Edward Netter, *ACGT Co-Founder*

MOMENTUM

MOVING THE SCIENCE OF GENE THERAPY RAPIDLY FROM THE LABORATORY TO HUMAN TRIALS



Combination Therapies for Lymphoma

Dr. Roberto Cattaneo, Mayo Clinic

Expanding on previous studies, current research utilizes a combination chemotherapy regimen for non-Hodgkin and Mantle Cell lymphomas. A recombinant measles virus vector, armed with the prodrug convertase purine nucleoside phosphorylase, delayed cancer growth in mice. Research has been modified to include chemotherapy drugs given before the virus treatment to enhance viral replication and therapeutic efficiency. Not only were the primary tumors destroyed within three weeks, but secondary disease symptoms were significantly reduced, more than doubling the survival rate. This research seeks to generate and use virus-producing enzymes that can activate and enhance the cancer killing effects of chemotherapeutic agents.



Accelerating T-Cell Production

Dr. Laurence Cooper, Children's Cancer Hospital, M. D. Anderson

Because many gene therapies are dependent on the engineering of new T-cells, increasing the speed of production is crucial to rapid treatment. A new high throughput medical electroporation device [HitMeD] significantly reduces the time needed to produce genetically altered T-cells for preclinical research. These manipulated T-cells are reintroduced into the patient and used to attack and kill tumors, while keeping risk to the patient at a minimum. In previous research, T-cells were found to lose their fighting power quickly, so the new device, which processes the cells 100 times faster than standard commercial technologies, will have vast benefits to future research. A Phase I trial for leukemia patients will open later this year. This research aims to improve the accessibility of cancer patients to large, healthy populations of their own immune cells that have been altered by gene therapy to temporarily increase the ability to attack and kill cancer cells.



Vaccine for Lung Cancer

Dr. Eckhard Podack, University of Miami

In an on-going trial for a cellular vaccine for the treatment of non-small cell lung cancer, six of the first seven patients have been vaccinated with no toxicity. The vaccine utilizes lung tumor cell secretions that facilitate the generation of killer cells. The injection protocol has been amended to allow weekly vaccinations for a total of 18 injections per patient, based on findings that suggested more frequent injections increase efficacy. This form of lung cancer is especially virulent, with a median survival time of 4 – 6 months. Non-small cell lung cancers are being treated with novel gene therapy methods that produce tumor cell secretions, which enhance the generation of tumor killing cells in the immune system.



Non-Viral Vector Development

Dr. Suzie Pun, University of Washington

Recipient of The Patricia Zoch Tate Gene Therapy Award

Nanoparticles are promising agents for drug delivery to tumors. However, the smaller size of nanoparticles is a double-edged sword: although the size allows for the delivery of anticancer agents with reduced side effects, it also prevents efficient penetration into the tumor tissue. The overall goal of this research has been to develop ways to improve penetration of nanoparticles to tumors. To do this, a new cell culture model was developed to allow analysis of tumor delivery without having to go to mouse models. Second, nanoparticles were coated with an enzyme that helped it to "chew through" tumor tissue, improving penetration into tissue. It was also demonstrated that nanoparticles could be modified with a bacterial protein called ActA, which uses cell machinery to form "actin-comet tails" and rocket through cell cytoplasm with more efficiency than unmodified nanoparticles. This research is directed at substantially improving the efficiency of gene therapy through delivery systems that overcome physical barriers.

"These results are particularly important as we build on our understanding of the microenvironments that allow tumors to grow and spread. The same zone of resistance approach could also be applied to the treatment of other solid tumors."

– Miguel Sena-Esteves, PhD, *Massachusetts General Hospital*

"Our goal is to provide therapy to patients closer to their time of need. The HitMeD processes a larger volume of T-cells in a continuous fashion over a much shorter time than we can achieve with commercial devices. We hope that will translate to better treatment opportunities for relapsed patients."

– Laurence Cooper, MD, PhD, *University of Texas M. D. Anderson Cancer Center*



Surrounding the Tumor Dr. Miguel Sena-Esteves, Massachusetts General Hospital

Researchers are developing a gene therapy for brain tumors that delivers a cancer-fighting gene to the normal tissue surrounding the tumor to keep it from spreading. In studies with mice, inducing brain cells to secrete human interferon-beta suppressed and eliminated the growth of glioblastoma cells implanted nearby within a very short period after injection of the viral vector into the brain. Importantly, this treatment eradicated the brain tumors completely and mice survived indefinitely, while untreated mice bearing brain tumors died within a month or less. In other studies of this difficult-to-treat cancer, the patient's immune system has targeted the viral vector used to deliver cancer-fighting genes. In this study, researchers used adeno-associated virus (AAV) vectors, which are capable of modifying normal brain cells to permanently produce therapeutic proteins, in this case an anti-tumor agent. Gene therapy vectors are being used to instruct normal brain cells to produce human beta-interferon, which suppresses and eliminates the growth of glioblastoma tumor cells in the brain.



Brain Tumor Selective Therapies Dr. Khalid Shah, Massachusetts General Hospital

Recipient of The Swim Across America Young Investigator Award

Mesenchymal stem cells (MSC) are emerging as novel cell-based delivery agents. In a recent study, engineered MSC were shown to retain their core properties, survive longer in mice with gliomas than in the normal brain and migrate extensively toward the tumor. The study also shows that MSCs are resistant to cell death protein, TRAIL, and can be engineered to produce a secretable, potent version of TRAIL. MSC releasing TRAIL were shown to induce apoptosis in both malignant and invasive cells of glioblastomas in the brains of tumor bearing mice. The fate of both the tumors and therapeutic MSC in the brain could be followed in real time by non-invasive imaging. The study demonstrates the efficacy of both diagnostic and therapeutic MSC that will serve as a foundation for developing stem cell-based therapies for many cancers. This research explores the utility of Mesenchymal Stem Cells as drug delivery vehicles for killing primary tumor mass and invading tumor cells in the brain.



Optimizing Immunotherapy Dr. Yiping Yang, Duke University

Progress has been made in a continuing effort to characterize the conditions that will optimize immuno-therapy treatment for lymphomas. In this study, progress has been made in developing vaccine strategies to overcome T-cell tolerance. Findings in several areas will pave the way to new vaccine strategies designed to overcome T-cell tolerance, including a greater understanding of clonal expansion and memory formation. These studies enhance previous research to delineate several important parameters that can influence the potency of cancer vaccines. This effort is being made to use gene therapy to enhance the potency of cancer vaccines by overcoming T-cell tolerance, a key factor in the efficiency of the immune system.

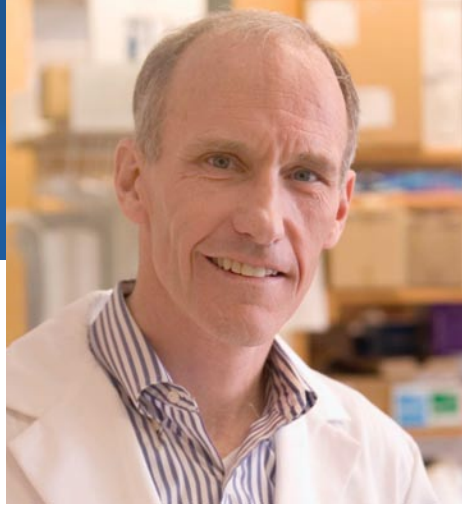


T-Cell Gene Transfer Dr. Xianzheng Zhou, University of Minnesota

A non-viral DNA gene delivery vehicle, aptly named the Sleeping Beauty Transposon System, mediates gene integration and long-term expression. The system has been tested to express both a therapeutic gene and a reporter gene in umbilical cord blood-derived T-cells to generate an anti-leukemia response. The engineered T-cell subsets not only kill cancer cells, they produce anti-tumor cytokines. In studies this year, adopted transfer of these T-cells in tumor-bearing mice inhibited tumor growth and prolonged survival. This research seeks to develop novel non-virus vectors for introducing genes into cells of the immune system that kill leukemia cells and also elicit the production of proteins that inhibit tumor growth.

MOMENTUM

A MEMORIAL GRANT FACILITATES TRIALS



Dr. June is the Director of Translational Research, Abramson Family Cancer Research Institute, University of Pennsylvania, and recipient of The Joan Miller and Linda Bernstein Gene Therapy Ovarian Cancer Award.

Carl June, MD, Director of Translational Research and Professor of Pathology and Laboratory Medicine at the University of Pennsylvania's Abramson Family Cancer Research Institute, is the recipient of The Joan Miller and Linda Bernstein Gene Therapy Ovarian Cancer Award. Dr. June was first awarded a 2004 ACGT Investigator Grant for innovative research into immunotherapy to combat leukemia. Now, expanding his efforts to include ovarian cancer, he focuses on adoptive immunotherapy, using the power of the immune system to kill tumor cells once they have been identified as a threat. One of the leading researchers in gene therapies, he has contributed significantly to the exploration of the genetic alteration of T-cells to become "guided missiles" against cancer. "I wake up every morning wondering how to basically put the immune system on steroids," Dr. June says.

Ovarian cancer is the most common lethal cancer for women. Relapse rates are high and while initially chemotherapy-sensitive, the response is poor once the cancer returns, resulting in a 3 to 5-year survival rate. Each year, roughly 20,000 women will be diagnosed and more than 15,000 will die.

In May 2008, made possible by ACGT Board member Eric Rothfeld, Dr. June received a 3-year grant funded by contributions made in honor of two victims of ovarian cancer: Joan Miller and Linda Bernstein. These two women succumbed to ovarian cancer early in their lives, within five years of each other. "I've seen the devastation to my friends who passed away and to their families," Rothfeld says. Hoping to help build the momentum, he facilitated this special grant and he is most pleased with Dr. June's progress. "I think gene therapy has a real chance of a breakthrough here."

In clinical trials beginning later this year, Dr. June's research team will remove T-cells from patients, manipulate them in the laboratory, adding the engineered genes, and transfer them back to the patient, triggering an immune system response and hoping for a repeat of pre-clinical experiments that resulted in the eradication of large ovarian tumors.

The research is not in isolation. Dr. June is also pressing for a greater understanding not only of the tumor but the tumor's microenvironment. "Evidence is accumulating that the tumor milieu is affected not only by the malignant cells, but the presence of inflammatory cells, vascular endothelium, pericytes and differing stromal cells." Dr. June believes that future work will focus on identifying biomarkers that may predict response to particular therapeutic approaches, further permitting a more targeted treatment with greater efficacy.

"We cannot do this on our own. The vector alone costs $400,000. Philanthropy is critical to this sort of research and we are extremely grateful to ACGT."

– Dr. Carl June, MD

Members of the Miller family visited with Dr. June and his team last July and were most impressed with a presentation and tour of the lab. "They were able to see us at work in the blood bank as we removed white cells and infused them with killer cells," Dr. June said. "Our whole research team was there and they found the visit especially motivating. Keeps your eye on the ball."

Working closely with fellow Penn physician and ACGT Research Fellow Dr. George Coukos, who is studying an anti-angiogenic approach to ovarian cancer, Dr. June says that there is a wonderful environment at the University for translational science. "We are all on one campus and we operate in a matrix rather than departmental organization, which facilitates an infrastructure meant to make things happen." I'm very grateful to the Abramson family and to ACGT, as we cannot do this work without their support.

"I think gene therapy has a real chance of a breakthrough here."

– Eric Rothfeld, *ACGT Board Member, Chairman, REI Capital, LLC, NYC*

FINANCIAL REPORT

APRIL 30, 2008 AND 2007

CONDENSED STATEMENTS OF ACTIVITIES	2008	2007
Support and revenue:		
Contributions	$3,216,189	$3,312,876
Contributions for administrative expenses (specially designated)	207,622	217,100
Contributed services	277,994	264,392
Interest and dividend income	163,562	180,995
Realized and unrealized gains on investments, net	32,396	308,741
TOTAL SUPPORT AND REVENUE	**3,897,763**	**4,284,104**
Expenses:		
Program services:		
Research grants and awards	3,351,997	3,522,732
Management and general	373,331	228,828
Fundraising	45,719	195,425
TOTAL EXPENSES	**3,771,047**	**3,946,985**
Increase in total net assets	126,716	337,119
Total net assets at beginning of year	2,269,275	1,932,156
TOTAL NET ASSETS AT END OF YEAR	**$2,395,991**	**$2,269,275**

CONDENSED STATEMENTS OF FINANCIAL POSITION	2008	2007
Assets:		
Cash and cash equivalents	$3,668,183	$3,115,698
Contributions receivable, net	1,730,669	1,974,315
Investments, at fair value	3,173,439	3,126,407
TOTAL ASSETS	**$8,572,291**	**$8,216,420**
Liabilities and net assets:		
Liabilities:		
Grants payable	$6,151,248	$5,941,775
Accounts payable and accrued expenses	25,052	5,370
TOTAL LIABILITIES	**6,176,300**	**5,947,145**
TOTAL NET ASSETS	**2,395,991**	**2,269,275**
TOTAL LIABILITIES AND NET ASSETS	**$8,572,291**	**$8,216,420**

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

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Because cancer does not recognize or react to economic cycles, it is imperative that we stay the course. So much progress has been made, thanks in large part to the commitment of our many supporters, and continued funding is critical to the realization of this amazing medical science. We are grateful for your confidence in ACGT as a charity worthy of your selective giving. We understand how difficult it is at times to make such decisions, as we also engage in a rigorous selection process, crucial to the success of our grants program. We take seriously the confidence which you have placed in us, and as such allocate every dollar with great care and oversight. We remain steadfast in our mission and hope that the strength of our program and its results will encourage you to include ACGT as a recipient of your future generosity, in whatever form you choose. With your support, we intend to continue to identify the most promising cell and gene therapy research and maintain our momentum, because cancer must be stopped. Thank you.



Virginia Boldt
ACGT Director of Development & Strategic Planning

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There are many moments in our lives that we wish to honor someone special, especially those who have struggled with cancer. A financial gift is a thoughtful way to support a cause near to their hearts. A birthday, anniversary, Mother's or Father's Day, or any special occasion, is an opportunity to show you care, and at the same time, make a difference for the future of medical science. We will send them an acknowledgement of your thoughtful tribute.

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"Gene and cell therapies attack cancer and only the cancer and offer great hope to very soon make cancer a manageable disease and ultimately a cure."

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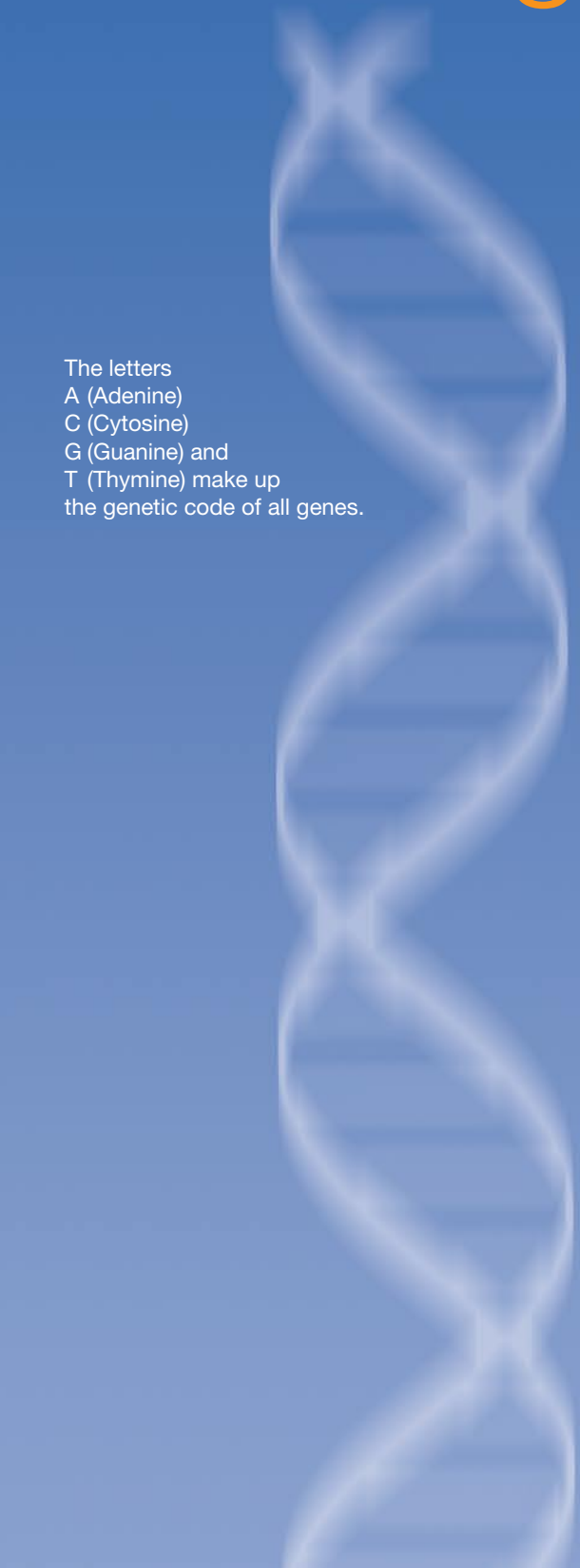
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The letters
A (Adenine)
C (Cytosine)
G (Guanine) and
T (Thymine) make up
the genetic code of all genes.

Information At Your Fingertips

The Alliance for Cancer Gene Therapy website offers content to help research scientists, cancer patients, medical practitioners, donors and interested parties access current information about cell and gene therapy and the battle against cancer.

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