Dr. Carl June, a two-time ACGT grant award recipient, has one trial currently underway and another nearly ready for a 2011 start – both the result of his ground-breaking research to harness the power of the immune system to recognize and destroy tumor cells. June began his investigation on leukemia, expanded into ovarian cancer and has recently added pancreatic cancer to the protocol for the next trial.

Early results of the trial underway for leukemia is promising, reports Dr. June, and we look forward to announcing the results next year. This immunotherapy trial expands on previous research conducted by Dr. June and his team at the University of Pennsylvania, and is designed to further test the central hypothesis that antitumor effects can be augmented by the direct injection into a tumor using a patient’s own genetically re-engineered T-cells to destroy cancer cells expressing the tumor antigen mesothelin, which is almost always over-expressed in these cancers, among others.

Dr. June is the recipient of a 2004 Investigator grant for Lymphoma/Leukemia, and the 2008 Joan Miller and Linda Bernstein Gene Therapy Ovarian Cancer Award. We applaud his commitment and his contributions to the field of cancer gene therapies, and to his considerable progress this year:

- Pre-trial research using the same immunotherapy approach with metastatic pancreatic cancer was so encouraging that the trial protocol has been expanded to include patients with ovarian, as well as pancreatic cancers, both “silent killers” and especially difficult to treat;
- Clinical trial preparations are complete, awaiting only final manufacturing and testing of the lentiviral vector;
- To accelerate initiation of the protocol, a lentiviral vector pilot manufacturing facility has been established at the Children’s Hospital of Philadelphia that will produce the vectors for this study. In the past year, the team also developed a parallel GMP (Good Manufacturing Practices) manufacturing method for cell modification using RNA transfection, which offers a more easily transitioned platform for testing multiple constructs, an enhanced safety profile for high risk gene products, and lower cost;
- One paper summarizing the new technology has recently been published and another is currently under review; and,
- Dr. June has applied for NIH funding for a mesothelioma trial, which is a further extension of this research and these trials, supported by ACGT.

“On behalf of our team at Penn, I want to thank you for your generous support on this project.

We cannot do this on our own – philanthropy is critical to this sort of research and we are extremely grateful to ACGT.”

— Dr. Carl June,
University of Pennsylvania
Abramson Family Cancer Research Institute

**THE MISSION OF ACGT** is to support the extraordinary potential offered by cell and gene-based therapies to accelerate effective and safe treatment of all types of cancer.
Khalid Shah, PhD

Recipient of the Swim Across America Young Investigator Award, Harvard Medical School, Massachusetts General Hospital
Focus: Brain Cancer

Dr. Shah’s research endeavors to harness the power of patient derived mesenchymal stem cells [MSC] to destroy malignant brain tumors. The study focuses on developing stem cells to ultimately deliver drugs that can stop tumor cells from proliferating and kill them, while leaving normal brain cells intact. He and his team have recently shown that engineered MSC retain their core properties, survive longer in mice with gliomas than in the normal brain and migrate extensively toward the tumor. Combined with state-of-the-art imaging technology, they also show that the fate of stem cells and their therapeutic impact can be tracked simultaneously. In the next 3 -5 years, Dr. Shah envisions a therapeutic modality in which the main tumor mass will be removed at the time of surgery and therapeutic stem cells introduced near the remaining tumor cells thus eliminating the risk of recurrence.

Clodagh O’Shea, PhD

Salk Institute for Biological Studies
Focus: Breast Cancer

What might cancer and the common cold have in common? Plenty. Previous research at the Salk Institute indicated that a novel mechanism used by adenovirus [the common cold] to sidestep the cell’s suicide program might go a long way to explain how tumor suppressor genes are silenced in tumor cells and pave the way for a new type of targeted cancer therapy. The p53 tumor suppressor pathway is inactivated in almost every human cancer, allowing cells to escape normal controls and evolve into tumors. Yet there is still no rationally designed targeted cancer therapy to treat patients based on the loss of p53 because it is extremely challenging to tackle something that is no longer there. However, it has been discovered that, just like cancer cells, the cold virus relies on these genes to shut down or get out of the way, and this study aims to develop viruses that specifically replicate within p53 mutant tumor cells to implode them from the inside out. The most important finding thus far: adenovirus brings along another protein that neutralizes p53 through a completely different mechanism, and prevents it from turning on its target genes in the genome. These discoveries have provided Dr. O’Shea and her team the opportunity over the next year to test the selectivity and efficacy of these novel viral therapies in pre-clinical studies.

Yiping Yang, MD, PhD

Duke University
Focus: Lymphoma/Leukemia

Dr. Yang reports that significant progress has been made towards an optimized immunotherapy treatment for lymphoma. The research is designed to develop vaccines to overcome T-cell tolerance, including a greater understanding of clonal expansion and memory formation. Dr. Yang and his team are attempting to improve the efficacy of treating pre-established lymphomas and define the parameters for a clinical trial, based on findings that support the potential treatment of patients using a Dendritic Cell vaccine co-administered with a TLR ligand. To-date, the team has published 14 peer-reviewed papers in prestigious journals including PNAS, PLoS, Blood, and the Journal of Immunology. Dr. Yang has his sights set on a clinical trial and will continue to progress in his effort to use gene therapy to enhance the potency of cancer vaccines and elevate the immune system as the prime warrior against cancer.

Follow the Progress at www.acgtfoundation.org

You can follow us at Twitter, Facebook and YouTube. Add your email address to receive our email blasts. Learn more about the revolutionary science of molecular medicine, make a donation, read our annual report, or discover news about research and clinical trials.
News from the Board

Welcome, New Board Members

Peter Glicklich, Esq. is the managing partner of the New York office of Davies, Ward Phillips & Vineberg LLP, a Canadian-based law firm specializing in mergers and acquisitions, cross-border transactions, real estate, infrastructure and related matters. Previously he was a partner at Roberts & Holland, LLP, a private client tax-focused law firm. Mr. Glicklich graduated cum laude from Harvard Law School and summa cum laude from the University of Wisconsin, Madison, with a degree in economics. He is on the board of advisors of the Whitehead Institute of Biomedical Research in Cambridge, MA and serves as Finance VP of the International Fiscal Association, USA Branch.

Peter Hearn has served as Chief Executive Officer at Willis Re, the reinsurance unit of Willis Group Holdings, since 2006. Willis is a leading brokerage firm that develops and delivers professional insurance, reinsurance, risk management, financial and human resource consulting and actuarial services to corporations, public entities and institutions around the world. Mr. Hearn joined the firm as Senior Vice President in January, 1994 to establish and manage the Philadelphia office, was appointed Eastern Region Manager later that year and Executive Vice President in 1997. In 1999, Mr. Hearn was appointed one of three global/national production leaders, and in 2000 was assigned overall responsibility for directing new business production efforts for global, national and regional property/casualty business. Previously he worked in the reinsurance business in London and subsequently with Towers Perrin in Philadelphia. In 2004 Mr. Hearn was appointed President of Willis Re, Inc. and in 2005 the CEO of their U.S. reinsurance operations. He holds a BA degree in History and Political Science from Lake Forest College.

Mike Moen is Consul and Head of Economic Affairs for the Ontario Ministry of Economic Development and Trade at the Canadian Consulate in New York City. He manages trade, investment, economic and business interests for the Province of Ontario in New York, New Jersey and Connecticut. Mr. Moen is engaged to advance Ontario’s political and investment objectives to government and commercial stakeholders. He works with senior executives at Fortune 500 companies in life sciences, information and technology, financial services, advanced manufacturing and renewable energy. Previously, Mr. Moen worked at Citigroup, Goldman Sachs and CIBC. He holds an MS in Global Affairs from NYU, MBA from IUM in Monaco, and BAcc from University of Minnesota.

Martin Winter is a Managing Director with Alvarez & Marsal, and co-head of the Healthcare Industry Group. He brings more than 30 years of broad business and diverse industry sector experience and an extensive operating background. An expert in corporate reorganization, he aided HealthSouth in its complex out-of-court restructuring following discovery of massive financial fraud, and has led the reorganization of a variety of healthcare organizations. Mr. Winter received a BS in economics from the Wharton School of the University of Pennsylvania and an MA in economics from the Graduate School of Arts and Sciences at the University of Pennsylvania. He is a member of the Board and Chairman of the Audit Committee of American Independence Corp., a NASDAQ-listed publicly traded health insurance company.

I have said this before and will say it again that the ACGT grant jump started my independent career. It is the vision of the Founders/Boards and wonderful people on your Staff that ensure the continued success of ACGT. I will keep ACGT in mind and strive to translate my research into ways to help cancer patients, and support other new investigators, whenever possible.

– Jian Yu, Ph.D., Assistant Professor of Pathology, University of Pittsburgh Cancer Institute
ACGT Fellows at Top Cancer Hospitals

US News & World Report released their annual ranking of the nation’s best cancer hospitals and we’re pleased to report that ACGT Research Fellows are affiliated with 11 of the 12 institutions rated among the very best.

Martha Zoubek A journalist and philanthropist, Martha Zoubek appreciates the importance of spreading the word on the science of molecular medicine. Currently president of the Wrightson-Ramsing Foundation, which supports cutting-edge cancer research, she brings extensive experience in publishing, media and education. Martha began her professional career as a writer and editor with McGraw-Hill and was a longtime associate and now contributing editor of Greenwich magazine. She founded the Education Department at the Bruce Museum in Greenwich, CT and launched the Brucemobile, an outreach program bringing science and the arts to schools and into the community. In 2008, Martha received the YMCA Spirit of Greenwich award for her volunteerism, honoring her contributions to the Bruce Museum, Arch Street Teen Center, Greenwich Arts Council, the Breast Cancer Alliance and the Grace Notes, a therapeutic music organization. She holds a bachelor’s degree in English from Smith College.

Many Thanks

We bid farewell to retiring board member Richard Murphy, President of Murphy & Associates, Boston, with great appreciation for his commitment to cell and gene therapy and his service to the ACGT Board.

In Memoriam: Diane W. Darst

A founding board member and dear friend, Diane W. Darst of Greenwich, CT, passed away in June. Mrs. Darst was an active member of the community and a long-standing proponent of new alternatives for cancer treatment. In her honor, the Board has established a resolution that expresses its admiration and appreciation for her “enthusiasm, insight and commitment” that helped ACGT grow into a national cancer research charity. Mrs. Darst’s service to ACGT included fundraising leadership and the establishment and refinement of board policies. She will be deeply missed.

Endowment Established with a Special Gift

We are pleased to report that an endowment fund is now in place to help support future research. The fund was made possible by an anonymous gift of $1 million, a tribute to all that has been accomplished in our first nine years and to perpetuate the future of molecular medicine. As we approach the mark of our first decade of funding and fostering cell and gene therapies, an endowment is an important step towards sustaining innovative research.

We invite you to contribute to the cause by not only helping to fund annual grants, but to ensure our future. Make a donation to the endowment, or include us in your estate planning by forming a charitable trust for ACGT or with a legacy gift in perpetuity.

Contact
Margaret Cianci, Executive Director
203.358.8000
Swimmers Break the Record

Swim Across America Makes a Big Splash for ACGT

The Fourth Annual Swim Across America for ACGT held June 26th raised a record $280,000 to support novel research into cell and gene therapies. Previous funding made it possible for our Research Fellow, Dr. Khalid Shah, to progress further towards developing therapies to eradicate brain cancers, and a new grant recipient will be announced shortly. In total, Swim Across America has contributed $845,000 to support ACGT-funded research and we’re already planning for next year to break the million dollar mark. Many thanks to the 2010 Greenwich-Santa Barbara, CA — Founders Edward and Barbara Netter hosted an educational forum featuring ACGT 2003 Research Fellow Laurence Cooper, MD, PhD. The event was presented for the benefit of the Teddy Bear Foundation which supports the families of children with cancer. Dr. Cooper is Associate Professor, Department of Pediatrics Patient Care, at the University of Texas M D Anderson Center, Houston. A physician and scientist, his focus is the development and implementation of innovative therapies to treat cancers that afflict children. Dr. Cooper has made great strides towards genetically modifying a patient’s own cells to attack and destroy tumors. He recently established a new system to manufacture cells 100 times faster than standard technologies. Once re-engineered, these cells are reintroduced to the patient to inspire the immune system to attack and kill tumors, with minimum side effects or risk. In his clinical practice, he takes special care of the most medically fragile patients undergoing bone marrow transplantation.

Stamford Event Committee chaired by Jacque Lang and Kerry Anderson, Honorary Chairs Donna de Varona and John Pinto, Jonathan Sackler, Arlene and Reuben Mark, and Cindy and John Sites, to our generous sponsors, countless volunteers, and to the 170 swimmers — you make a real difference.

ACGT Coast to Coast

Santa Barbara, CA — Founders Edward and Barbara Netter hosted an educational forum featuring ACGT 2003 Research Fellow Laurence Cooper, MD, PhD. The event was presented for the benefit of the Teddy Bear Foundation which supports the families of children with cancer. Dr. Cooper is Associate Professor, Department of Pediatrics Patient Care, at the University of Texas M D Anderson Center, Houston. A physician and scientist, his focus is the development and implementation of innovative therapies to treat cancers that afflict children. Dr. Cooper has made great strides towards genetically modifying a patient’s own cells to attack and destroy tumors. He recently established a new system to manufacture cells 100 times faster than standard technologies. Once re-engineered, these cells are reintroduced to the patient to inspire the immune system to attack and kill tumors, with minimum side effects or risk. In his clinical practice, he takes special care of the most medically fragile patients undergoing bone marrow transplantation.

Breakefield On Track to Head American Society of Gene and Cell Therapy

Congratulations to Dr. Xandra O. Breakefield, member of our Scientific Advisory Council, who has been elected the 2010-2011 Vice President for the American Society of Gene and Cell Therapy. The Society is a professional medical and scientific organization with over 1,900 members and is dedicated to the understanding, development and application of genetic and cellular therapies. This VP position is the first year of a three-year cycle leading to the presidency of the organization. She also serves as an ex-officio member of most standing committees. Dr. Breakefield is Professor of Neurology at Harvard Medical School and Geneticist in the Neurology and Radiology Services where she directs the Laboratory of Experimental Gene Therapy at Massachusetts General Hospital.

“Most drugs for childhood cancers were developed in the 50’s... we need to re-energize the drumbeat and shift resources towards the cancers that kill the very young."

– Laurence Cooper, MD, PhD., Section Chief, Cell Therapy, Children’s Cancer Hospital, Division of Pediatrics, Department of Immunology, U.T. MD Anderson Cancer Center, Houston, TX

Amelia Island, FL — At the annual Philanthropy Roundtable Conference, ACGT Founders Edward and Barbara Netter served as hosts at a breakfast for family foundations. The Roundtable is a national association of individual donors, corporate giving officers and foundation trustees and staff who support their mutual goals of making a difference through charitable giving. Says President John Templeton Jr., MD, “The Roundtable is the leading voice in philanthropy for objectivity, integrity, open-mindedness, and a spirit of stewardship with a commitment to measurable results.” Learn more at www.philanthropyroundtable.org.
Alliance for Cancer Gene Therapy
Ninety six Cummings Point Road
Stamford, Connecticut 06902
203 358 8000
www.acgtfoundation.org

You Can Help Visit www.acgtfoundation.org or call Margaret Cianci at 203.358.8000

ACGT by the Numbers

37 major grants in nine years:
24 Discovery, 13 Translational

$22.35 million dollars awarded; ACGT Research Fellows attracted $58 million in complimentary funding.

ACGT Fellows conduct research at 27 of the 150 universities and research institutions in the US, Canada and Puerto Rico engaged in the investigation of cell and gene therapies for cancer.

17 human trials approved of which 11 trials started – 113 patients enrolled.

118 publications in leading professional journals acknowledging ACGT

100% of donations to ACGT go directly to research. Separate funding is provided to cover all administrative and fundraising expenses.

Savio L.C. Woo, Ph.D.
Council Chairman
Mount Sinai School of Medicine

Stuart A. Aaronson, M.D.
Mount Sinai School of Medicine

Xandra O. Breakefield, Ph.D.
Massachusetts General Hospital, Harvard Medical School

Lieping Chen M.D., Ph.D.
Johns Hopkins University

Stephen L. Eck, M.D., Ph.D.
Eli Lilly & Company

Joseph C. Glorioso III, Ph.D.
University of Pittsburgh

Carl H. June, M.D.
University of Pennsylvania

Michael T. Lotze, M.D.
University of Pittsburgh

Robert L. Martuza, M.D., F.A.C.S.,
Massachusetts General Hospital, Harvard Medical School

A. Dusty Miller, Ph.D.
Fred Hutchinson Cancer Research Center

John Nemunaitis, M.D.
Mary Crowley Medical Research Centers

Drew M. Pardoll, M.D., Ph.D.
Johns Hopkins University

Jack A. Roth, M.D., F.A.C.S.
University of Texas

MD Anderson Cancer Center

Stephen J. Russell, M.D., Ph.D.
Mayo Clinic

Michel Sadelain, M.D., Ph.D.
Memorial Sloan-Kettering Cancer Center

Thomas J. Wickham, Ph.D.
Merrimack Pharmaceuticals

George D. Yancopoulos, M.D., Ph.D.
Regeneron Pharmaceuticals, Inc.

In Memoriam
Judah Folkman, M.D.,
Children’s Hospital, Harvard Medical School

Margaret C. Cianci, Executive Director
H. William Smith, Esq., Legal/Secretary
Christine A. Herman, Finance/Treasurer
Grace Pedersen, Foundation Administrator
Betty Condon, Development Associate
Phyllis Schondorf, Outreach Coordinator