



THE CATALYST:

FUNDING AND FACILITATING CANCER GENE THERAPY

2007 ANNUAL REPORT

ACGT®

National grants for cancer research

ALLIANCE FOR CANCER GENE THERAPY

OUR MISSION

The mission of ACGT is to support the extraordinary potential offered by gene therapy as a revolutionary model for effective and safe treatment for 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 community to foster the best possible research and ultimately the best treatment.

"Gene therapy for cancer is revolutionary because it can lead to the eradication of tumors in ways that are distinct from existing treatment protocols that include surgery, radiation, chemotherapy and antibody therapy. This novel treatment can be used by itself and in conjunction with the existing therapies to achieve optimal outcome in patients including substantially prolonged survival."

Savio Woo, PhD
Professor, Mt. Sinai School of Medicine, Chairman, ACGT Scientific Advisory Council

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.

ACGT: A CHARITABLE BUSINESS MODEL

As is often the case, the formation of a charitable entity is the result of not one but several experiences or events. We have endured enormous personal loss in our families as a result of cancer, and we have seen so many others devastated by the disease. The loss of our daughter-in-law was a seminal moment. We had to do something, but what?

Recognizing that cancer has been treated in the same basic way for half a century, we set forth in pursuit of an alternative. We discovered, as we often have in our lives, that the fulfillment of one's goals relies on tenacity as much as passion. We spent a good deal of time evaluating the science of cancer and analyzing the current mode of treatment, which led to the conclusion that treating cancer symptoms through surgery, radiation and chemotherapy, while offering some benefits, do not address the root cause of the disease, which we know now is in the genes. We were convinced six years ago, and remain certain, that gene therapy is the future for cancer treatment.

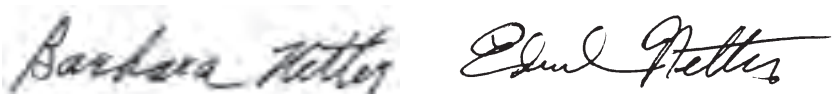
Gene therapy goes directly to the cause of cancer, not the symptoms. It is both logical and reflective of recent discoveries in molecular medicine and the mapping of the human genome, as well as new technologies. Traditional treatments are the result of scientific discovery prior to these achievements, thus without the benefit of modern wisdom.

We've been involved in several businesses in our lives and determined early on to establish an organization rooted in the same basic principles of any successful enterprise: strong management, solid resources, cost efficiencies, wise investments, effective marketing, and the careful monitoring of results. What this means at ACGT is that every dollar invested in this organization has the best possible potential for return, which means the best people at the best institutions doing the best research and, perhaps most importantly, moving rapidly towards human trials.

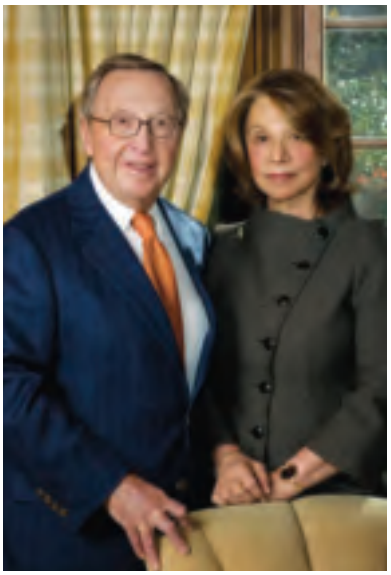
Beyond good intentions, we knew we needed to set a very high standard for the work. We established a Scientific Advisory Council, guided by Dr. Savio Woo, to provide rigorous oversight of our grants program. We have been blessed with an enlightened and prestigious Board of Directors. Our donors, many who have also experienced the tragedy of cancer, want to be part of advancing a science that offers a new prototype for cancer treatment. And, we now have excellent working relationships with nearly all of the national universities and institutions researching gene therapies.

Like all business enterprises, there are disappointments, and like all scientific methods, there are constant challenges to get it right. There is risk, but the reward has limitless potential. Now that we've charted a new course, we need to stay the course. We believe in the promise of gene therapy and we are seeing that promise realized in new ways every day.

Sincerely,



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



"We discovered, as we often have in our lives, that the fulfillment of one's goals relies on tenacity as much as passion...

We were convinced six years ago, and remain certain, that gene therapy is the future for cancer treatment."



"We are always mindful that innovation is the first step... we aim to divide our funding nearly equally between research into discovery of new practices and research approaching clinical trials."

ACGT: AN AGENT FOR CHANGE

ACGT serves as a change agent. However, in the process of fostering change, our organization has evolved from an agency of intention to a force for the advancement of molecular medicine.

As ACGT is the only public charity dedicated exclusively to cancer gene therapy research, we have quickly become the "go to" organization for anyone interested in this form of treatment. From medical researchers to professionals and patients, our organization and our website are a vital link between the science and the potential, the past and the future.

The evidence is in the numbers. In our first six years, we have distributed $18.8 million in support of 31 research projects. These projects represent only 7% of the 428 applications for funding and of course we hope to raise that rate over time. This is especially imperative as many of the projects were unfunded finalists, and we look forward to the day that we can support all worthy proposals.

Further evidence of our role as catalytic agent is the fact that proposals have been received from 117 of an estimated universe of 135 national institutions pursuing cancer gene therapies. This includes 34 of the 39 National Cancer Institute [NCI] designated Comprehensive Cancer Centers, which have applied for grants. These centers undertake research and also provide direct services to patients, and are expected to conduct "early-phase innovative trials" as well as outreach and education activities.

In addition, 19 of the 21 member National Comprehensive Cancer Network [NCCN] institutions have applied to ACGT for grants. NCCN is an alliance of leading cancer centers, and is an authoritative source of information for patients and health professionals.

Although we are all eager to move research rapidly towards trials, and ultimately medical practice, we are always mindful that innovation is the first step. This is critical for young investigators who have increasingly limited access to funding. To this end, we aim to divide our funding nearly equally between research into discovery of new practices and research approaching clinical trials.

We are also proud that ACGT grant awards have funded or facilitated ten clinical trials. This means that a number of patients are moving into remission for extended periods of time, without compromising their general health or lifestyles, and that scientific hypotheses are now closer to probabilities.

2007 was a year of great accomplishments, as you will read in the pages to follow. We are proud to be the catalyst for change, and look forward to funding and supporting more innovation and investigation, with the support of our collaborators, colleagues and donors. Thank you all.



Margaret C. Cianci
Executive Director



"'Translational' is the defining adjective for the 21st century research. It means turning laboratory 'eureka' moments quickly and safely into 'thank goodness' moments for patients and families."

NIH Grant Planning
Statement, 2006



ACGT 6 YEARS OF

2002
27 Institutions
45 Applicants
18 Finalists
3 Young Investigator Grants

2003
36 Institutions
44 Applicants
21 Finalists
5 Young Investigator Grants

2004
53 Institutions
70 Applicants
32 Finalists
4 Young Investigator Grants
2 Investigator Grants

SINCE 2001

31
RESEARCH PROJECTS

117
PRESTIGIOUS MEDICAL INSTITUTIONS

428
APPLICATIONS RECEIVED

$18.8
MILLION GRANTED

NUMBER OF GRANTS TO DATE

Young Investigator	21	$ 9.3 million
Investigator	10	$ 9.5 million
Total Grants	31	$18.8 million

PROPOSALS FROM MOST NATIONAL INSTITUTIONS RESEARCHING CANCER GENE THERAPY

Number of Applicant Institutions	117
Institutions with ACGT Fellows	25

ACGT GRANTS SUPPORT DISCOVERY AND TRANSLATION

TYPE OF RESEARCH

Discovery (Basic)	15
Pre-Clinical Phase	6
Phase I Trials	9
Phase II Trial	1

"Of all the therapies we have, perhaps the one that has greatest promise is gene therapy; because, in effect, we simply smooth the exit for cancer cells and show them the door."

Michael T. Lotze, MD
University of Pittsburgh Medical Center
ACGT Scientific Advisory Council Member

PROGRESS

2005

61 Institutions
75 Applicants
30 Finalists
4 Young Investigator Grants
3 Investigator Grants

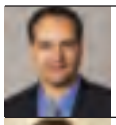
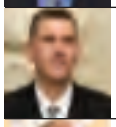
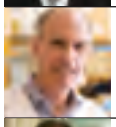
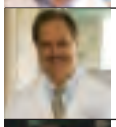
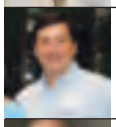
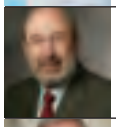
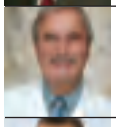
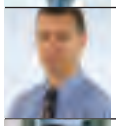
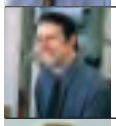
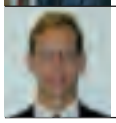
2006

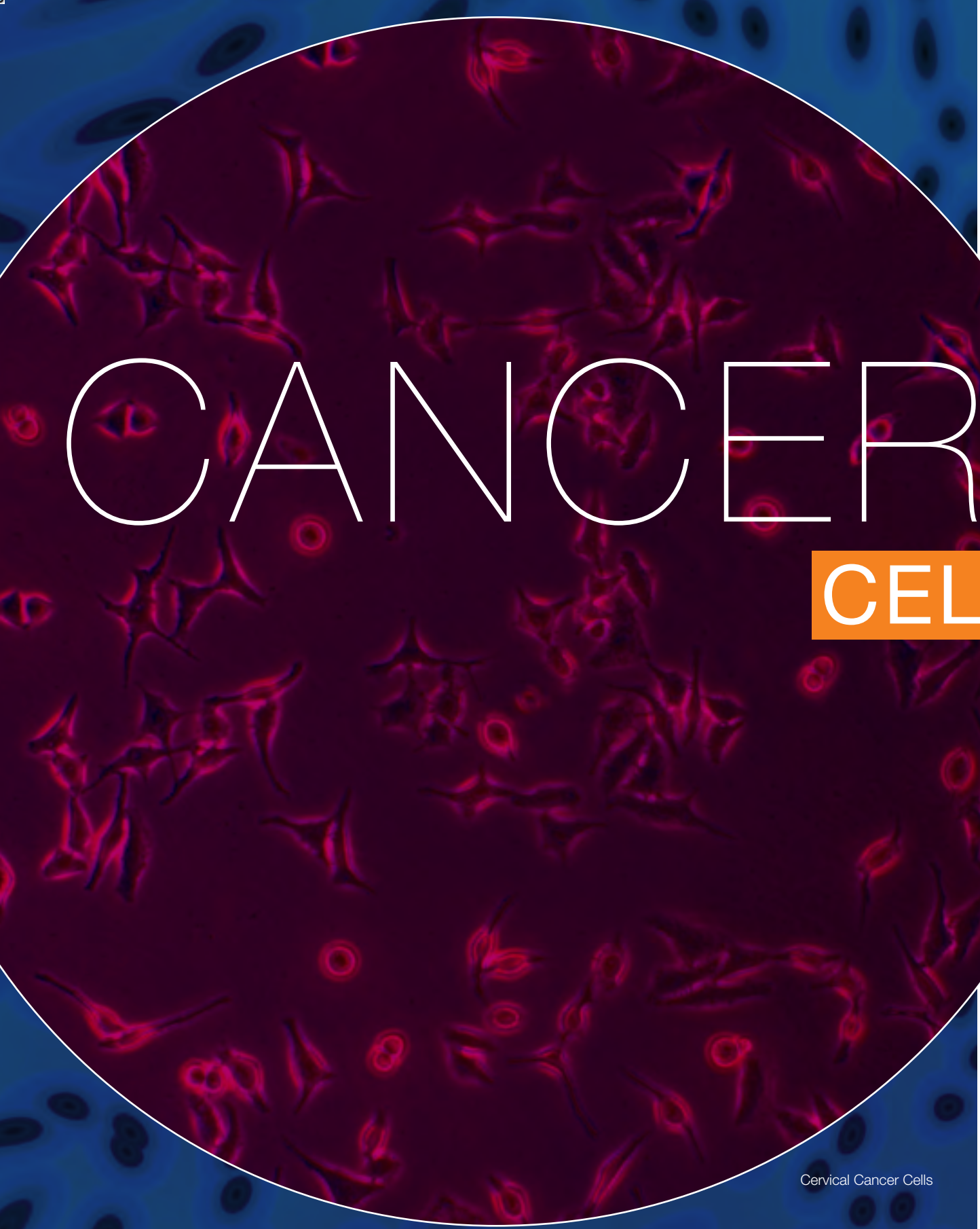
65 Institutions
84 Applicants
31 Finalists
3 Young Investigator Grants
3 Investigator Grants

2007

79 Institutions
110 Applicants
18 Finalists
2 Young Investigator Grants
2 Investigator Grants

CLINICAL TRIALS FACILITATED BY ACGT FUNDED RESEARCH

	Antonio Chiocca, MD, PhD	Ohio State University Research Foundation	Brain Cancer
	Thomas Griffith, PhD	University of Iowa	Prostate Cancer
	Carl June, MD	University of Pennsylvania	Lymphoma & Leukemia
	Thomas Kipps, MD, PhD	University of California San Diego	Lymphoma & Leukemia
	Hyam Levitsky, MD	Johns Hopkins University	Lymphoma & Leukemia
	Ronald Levy, MD	Stanford University	Lymphoma & Leukemia
	Eckhard Podack, MD, PhD	University of Miami Miller School of Medicine	Lung Cancer
	Richard Reilly, PhD (Elizabeth M. Jaffee, MD)	Johns Hopkins University	Breast Cancer
	Michel Sadelain, MD, PhD	Memorial Sloan Kettering Cancer Center	Lymphoma & Leukemia
	Robert Vonderheide, MD, PhD	University of Pennsylvania	Neuroblastoma



CANCER CELLS

Cervical Cancer Cells

Human cells operate on a cycle – a tightly regulated process. Each body, and each part of the body, follows a clearly defined regimen for the development and maintenance of healthy cells, which is the basis for healthy lives. Some cells, like the skin, grow and divide throughout a lifetime. Others, like those of the brain or nervous system, complete growth by adulthood. When the roughly 300 types of cells in humans behave as they are meant to behave, all is well.

However, there are cells in each body that violate the rules and behave badly. Tumor-suppressing genes, which are the cell's natural brakes, work to slow or stop cell growth, but when these fail, cells can divide without limitation, with potentially disastrous results.

Gene therapy is the re-regulation of the body's cell division system: a potentially powerful antidote to cancer, and the subject of cancer gene therapy research throughout the world. Cancer gene therapies have the potential not only to tame cells gone wild and restore the body to good health, but eventually to reform their errant ways before cancer results.

GONE WILD

The three levels of cell dysfunction are as follows:

1. Benign cells divide at an abnormally high rate, but remain in the same location and often appear normal. Benign cells only cause trouble as they increase in number, creating tumors, because tumors may impact nearby organs. As a rule, benign tumors are not invasive.
2. Dysplasia cells are benign but look abnormal, and may threaten health. They are not yet out of control and thus not cancer; however, they lurk in the body as a potential source of cancer and the sooner they are attended to, the better.
3. Malignant cells run amok. They divide uncontrollably and do not follow regulations for timely cell death. Malignant tumors lack form, blending into the surrounding tissue, thus predatory to other parts of the body, what is called metastasis.

GENE THERAPIES ATTACK ALL FORMS OF CANCER

CARCINOMA
Cancer of the skin, nervous system, intestinal or respiratory tract

SARCOMA
Cancer associated with connective tissue such as muscle and bone

LEUKEMIA
Cancer of the blood

LYMPHOMA
Cancer in glands that otherwise fight infections

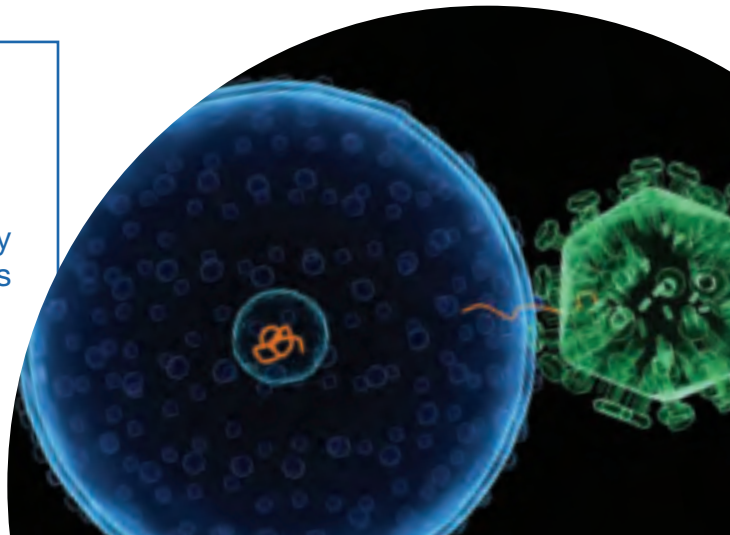
MYELOMA
Cancer that starts in the bone marrow

GENE THERAPIES FIGHT CANCER AT THE SOURCE

- Healthy genes delivered directly to the tumor to facilitate cancer cell death [Apoptosis].
- Treatment that triggers the immune system to recognize and destroy cancer cells [Immunotherapy].
- Inhibitors that strangulate cancer cells by cutting off their blood supply [Anti-angiogenesis].
- Inserting genes to improve the effectiveness of chemotherapy or radiation treatments.
- An inoculation that will re-regulate the cell division system to ultimately prevent cancer.

"My real interest is how do killer cells kill other cells. Now, killer cells are a very important component of the immune system. And it's by now I think accepted by a large number in the community that killer cells are really the primary weapon against cancer if the immune system is involved."

Eckhard Podack, MD, PhD
University of Miami, Miller School of Medicine
2006 ACGT Research Fellow





For the most up-to-date information on cancer gene therapy trials, please visit www.acgtfoundation.org

OF THE 1,300 CLINICAL GENE THERAPY TRIALS CURRENTLY UNDERWAY, TWO THIRDS ARE RELATED TO CANCER

TRIALS PROVE THE SCIENCE OF CANCER GENE THERAPY

Gene therapy research is rapidly moving along a continuum from basic scientific study to laboratory research, to clinical trials with both domestic animals and humans, and ultimately to clinical practice.

CLINICAL TRIALS ARE CRITICAL TO CONFIRMING RESULTS OF BASIC SCIENTIFIC STUDIES.

A clinical trial is a short-term biomedical experiment with patients that follows very strict governmental rules, so that a drug or treatment may be properly evaluated. Patients continue to work with their primary care physician during the trial and often continue with other medications or treatments.

TRIALS EVALUATE ALL ASPECTS OF MEDICAL CARE.

Trials assess treatment alternatives, innovations in diagnostics and screening, prevention methods, and ways to improve the quality of life for those with chronic illnesses.

TRIALS WILL DEFINE THE FUTURE OF CANCER GENE THERAPY.

Trials answer critical questions related to the safety, toxicity and efficacy of a treatment or drug. Participants in trials have the opportunity to be among the first beneficiaries of new treatments, and also contribute to future alternatives for cancer patients of all types.

CLINICAL TRIALS ARE CONDUCTED IN PHASES.
OVER 800 CANCER GENE THERAPY TRIALS ARE UNDERWAY

Phase	Typical scenario	Cancer gene therapy trials
Phase I	Phase I trials test an experimental drug or treatment with a small group of people for the first time, primarily to determine a safe dosage range and identify potential side effects.	269
Phase II	Phase II trials enroll a larger number of patients to determine effectiveness and further evaluate safety.	441
Phase III	Phase III is conducted with groups as large as several thousand, to confirm the results of previous studies.	82
Phase IV	Phase IV trials define additional information required for government approval, including a drug's risks, benefits and optimal use.	65
Total number of cancer gene therapy trials in progress		**857**

Source: www.clinicaltrials.gov, March 2008

ACGT FACILITATES CANCER GENE THERAPY TRIALS

Following are highlights of trials conducted in 2007, which were either funded by ACGT grants or implemented as a result of previously funded research.

TRIAL TARGETS LEUKEMIA

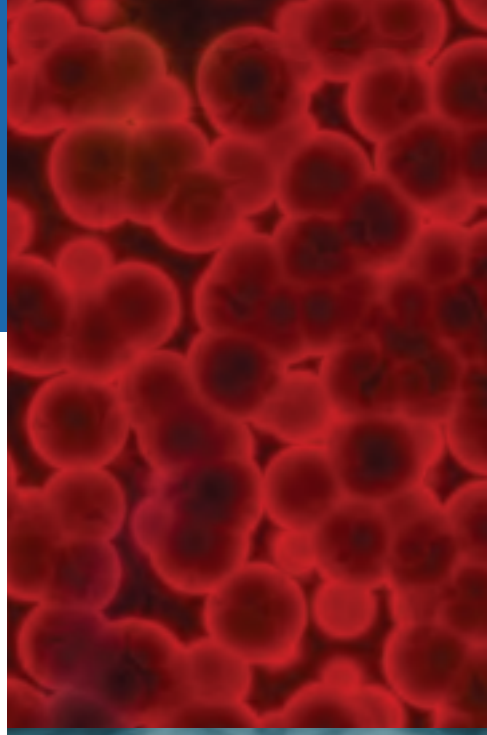
Patients with chronic lymphocytic leukemia who have chosen to forego chemotherapy or for whom treatments have failed, have an opportunity to participate in a new clinical trial at the Moores Cancer Center at the University of California, San Diego. Participants will receive a vaccine designed to stimulate the body's own immune system to combat the disease. According to the trial's principal investigator, Januario Castro, MD, the trial will be the first of its kind in that patients who qualify will receive a single injection. The treatment, known as ISG35, short for Immune Stimulatory Factor 35, is an offspring of technology developed by Dr. Thomas Kipps, Deputy Director for Research at the Moores Cancer Center, an ACGT 2005 Fellow, based on research into immune-mediated gene therapy. ACGT has provided funding for the Phase I trial.

TRIAL AIMS TO DESTROY LUNG CANCER

Dr. Eckhard Podack at the University of Miami, a 2006 Fellow, is conducting trials for a vaccine for non-small cell lung cancer patients, for whom no effective therapy is available. The gp96-vaccine was produced at the University's Miller School of Medicine facilities, and tested for sterility and safety. The purpose of the study is to determine whether the vaccine is able to induce an immune response resulting in the generation of killer cells to destroy cancer cells. In addition, the study will evaluate the extended life-span of patients, for whom the median survival rate is otherwise only 4-6 months. Preliminary testing reports no adverse affect to patients.

VETERINARY TRIALS APPLICABLE TO HUMAN CANCERS

The University of Pennsylvania's Abramson Family Cancer Research Institute has joined forces with The University of Pennsylvania School of Veterinary Medicine to implement gene therapy clinical trials with dogs that have lymphoma. Funded by ACGT, the first small-scale study has proven promising, and research with canines is an important learning opportunity, as their cancers frequently mimic those of humans. Dr. Robert Vonderheide has been developing a cancer vaccine to stimulate an animal's immune system to recognize and attack cancer anywhere in the body, preventing relapse of the original malignancy, which usually happens within a year after dogs receive chemotherapy. Of the first ten dogs treated, one has been healthy for two years, and three others healthy for more than a year. Of the roughly 65 million dogs and 32 million cats in America, the National Cancer Institute estimates that roughly six million of each will be diagnosed annually with cancers. The next step is preparation for human trials.







2007 RESEARCH GRANT AWARDS

INVESTIGATORS

Investigator Grants are made possible by the *ACGT Fund for Advancement,* which awards up to $1 million for 3-5 year projects entering clinical translation. All grant applications undergo rigorous scrutiny by our Scientific Advisory Council, which recommends finalists to the Board of Directors for funding.



"This project was reviewed and approved by the NGVL (National Gene Vector Lab) for funding in 2005, with a score of outstanding, but the scarcity of funding at the NIH has practically halted the development of this promising therapeutic."

Antonio E. Chiocca, MD, PhD
Chairman, Department of Neurological Surgery,
Ohio State University Research Foundation

Tumor Specific Replicating Viruses and Bacteria for Glioma

Dr. Chiocca's research focuses on glioma, a malignant brain tumor that is one of the most incurable forms of cancer. His research team has employed tumor-selective viruses to attack the tumor and also increase the cancer's receptivity to chemotherapy. Successful studies in mice will now be replicated in a Phase I clinical trial with humans, which will involve injecting the brain tumor with the MGH2 virus in combination with two chemotherapeutic agents. This multimodal viro and gene-based therapy will attack multiple vulnerabilities simultaneously.



"The innovative aspects of our study are that it utilizes a novel, non-viral gene transfer technology and takes advantage of the unique immunologic setting of patients who have undergone bone marrow stem-cell transplantation."

Ronald Levy, MD
Professor of Medicine, Oncology, Stanford University

Gene Transfer Vaccine for Lymphoma

Mantle cell lymphoma has the worst prognosis of all forms of the disease. The usual treatment, which involves both high-dose chemotherapy and bone marrow stem-cell transplant, eliminates much of the tumor, but also destroys a patient's immune system, leaving them vulnerable to other life-threatening conditions. And, even with aggressive therapy, residual cancer cells remain and cause recurrence within a fairly short time. This study will utilize a gene-engineered lymphoma vaccine to induce the patient's immune system to eliminate residual lymphoma cells. Trials of this approach in animals have demonstrated impressive results. The proposed Phase II trial will be for patients with newly diagnosed mantle cell lymphoma. A vaccine for each patient will be created from their own tumor cells. Then the vaccine will be tested in the context of conventional high dose chemotherapy.

"ACGT continues to impress me. The Scientific Advisory Council is outstanding as are the grants given to the established and young investigators. I am always pleased to see forward-looking foundations support a fellows program to stimulate growth in the field and to ensure its future."

Dr. Richard A. Murphy
Retired President and Chief Executive Officer of the Salk Institute for Biological Studies, 2000-2007 and Interim President of the California Institute for Regenerative Medicine. Elected to ACGT Board of Directors commencing in 2008.

YOUNG INVESTIGATORS

Young Investigator grants are made possible by the *ACGT Fund for Discovery,* which awards up to $500,000 to tenure-track assistant professors doing groundbreaking research to uncover new methods of treatment.



"To develop these viral cancer therapies we will exploit the striking similarities that exist between adenoviral infected cells and tumor cells. Both subvert many of the same cellular checkpoints, including p53, with one difference: in tumor cells, the key cellular players are targeted via mutations, while adenoviruses use viral proteins to achieve the same end."

Clodagh O'Shea, PhD
Assistant Professor, Salk Institute for Biological Studies, Molecular and Cell Biology Laboratory

Next Generation Oncolytic Adenoviruses for Breast Cancer

Most commonly used chemotherapies are little more than DNA poisons that slow down tumor growth but do not ultimately cure patients. Chemotherapy also has devastating side effects, especially tragic in children. There is a desperate need to identify new drugs and therapeutic modalities that ablate cancer cells while leaving normal cells unharmed. The p53 tumor suppressor pathway is inactivated by mutations or gene deletions in almost every form of human cancer, yet there are no effective therapies to treat p53-defective tumors. It is a major challenge to develop small molecule therapies to a cellular target that is either lost or inactivated. This research will develop new potent sub-group C and B oncolytic adenoviruses that depend specifically on the loss of p53 functions to drive their tumor selective replication. Each time a virus infects a p53 mutant cancer cell and successfully multiplies, the virus ultimately kills the cancer cell by bursting it open to release thousands of viral progenies, which are ready to seek out remaining tumor cells and distant micro-metastases. These next generation p53-tumor selective replicating viruses have the potential to help save the lives of many cancer patients.



"Although these studies are experimental, we can envision a therapeutic modality in which the main tumor mass in the brains of patients will be removed at the time of surgery and therapeutic neural stem cells will be introduced near the remaining tumor cells, eliminating the risk of recurrence."

Khalid Shah, PhD
Assistant Professor, Harvard Medical School, Massachusetts General Hospital
Recipient of the Swim Across America Young Investigator Award

Targeting Cell Survival and Apoptotic Pathways in Brain Tumors

Malignant glioblastomas have virtually defied all therapeutic modalities to-date. Successful therapies will require novel strategies targeting the underlying pathogenesis which may ultimately offer an innovative approach. This research will use neural stem cells to deliver therapeutic drugs to stop tumor cells from proliferating and simultaneously kill them, while leaving the normal brain cells intact. The study will also utilize novel in vivo imaging methods to monitor the location and size of tumors and track the migration of neural stem cell delivery vehicles in experimental animal models. Dr. Shah hopes to realize a major impact in developing a more efficient means of eradicating gliomas and saving the lives of brain cancer patients.



"For years we have used chemicals to treat the problem of cancer spreading from the local tumor; however, these chemicals are not very effective against cancer cells and highly toxic to normal cells. Gene therapy selectively targets abnormal biologic pathways in the cancer cell and may stimulate immune responses to cancer which will not harm normal cells. Thus gene therapy for cancer is potentially more effective and much less toxic than current treatments."

Jack Roth, MD, F.A.C.S.,
University of Texas MD Anderson Cancer Center, ACGT Scientific Advisory Council Member

WELCOME OUR NEW SCIENTIFIC ADVISORY COUNCIL MEMBERS

17 ACGT Scientific Advisory Council members, who serve without remuneration, are among the nation's most preeminent physicians and researchers in oncology and gene therapy. Council members establish scientific criteria for review of all grants, develop strict accountability guidelines, and review progress reports. Guided by Chairman Dr. Savio Woo, Professor, Mt. Sinai School of Medicine, Council members submit recommendations for funding to the Board of Directors for approval.



"Many genetically engineered viruses have been shown to destroy tumors in mice. There is therefore no question that they will eventually prove to be useful in the treatment of human cancer. Our current challenge is to understand the barriers to success that are absent in tumor-bearing mice but present in cancer patients, and to devise new treatment strategies to address them."

Stephen J. Russell, MD, PhD

Director, Molecular Medicine and Professor of Medicine, Mayo Clinic, Rochester, MN

Dr. Stephen Russell graduated in Medicine from Edinburgh University, Scotland, with distinctions in microbiology and surgery. He later earned his PhD at the University of London after researching the retroviral/parvoviral transfer of interleukin genes to cancer cells as a novel approach to immunotherapy, which led to a clinical gene therapy trial at Marsden Hospital, England. Dr. Russell held appointments at hospitals throughout England and Scotland, before moving to the states to join the Mayo Clinic. In addition to his role as Chair of the Department of Molecular Medicine Program, he holds the Richard O. Jacobsen Chair of Molecular Medicine and serves as consultant to the Department of Hematology. His research interests include engineering viral tropisms, evading antiviral immune responses and the development of new methods for noninvasive in vivo monitoring of gene expression. His long-term research aim is to develop injectable gene therapy vectors for the treatment of disseminated malignancies, particularly multiple myeloma, and demonstrate their efficacy in clinical trials.



George D. Yancopoulos, MD, PhD

Chief Scientific Officer and President, Regeneron Laboratories, Tarrytown, NY

Dr. George Yancopoulos graduated as valedictorian of both the Bronx High School of Science and Columbia College, and earned his advanced degrees at Columbia University's College of Physicians and Surgeons. Following widely-recognized work in the field of molecular immunology at Columbia, Dr. Yancopoulos left academia in 1989 as founding scientist for Regeneron Pharmaceuticals, where he continues to serve as Chief Scientific Officer. He is also adjunct professor at Columbia University and was recently awarded the Stevens Triennial Prize for Research and the University Medal of Excellence for Distinguished Achievement. Dr. Yancopoulos is widely regarded as a world leader in several fields of biology and has authored more than 300 scientific manuscripts. In 2004, he was elected to both the National Academy of Sciences and the American Academy of Sciences. Dr. Yancopoulos' scientific efforts have focused on the receptors and pathways of growth factors, as well as on developing new platforms for target and drug discovery such as VelociGene and VelocImmune. His research has led to unifying models of molecular and biologic function, as well as new approaches to treating disease. Dr. Yancopoulos' efforts have resulted in progression of numerous drug candidates to human trials, including the VEGF Trap for cancer and blinding eye disease and the IL1-Trap which has recently been approved for treatment of an orphan inflammatory disease.

2007 HIGHLIGHTS



Honorary SAA Co-Chairs John Sites, ACGT Board Member, left, and Cindy Sites, far right, with ACGT Co-Founders Barbara and Edward Netter



Swimmers enter the water at Greenwich Point to make waves to fight cancer

"Our beneficiary, ACGT, currently funds the research of preeminent scientists in premier institutions nationwide. We are proud to be raising money to increase this number and further expand the reach of cancer gene therapy research."

Janel Jorgensen
Swim Across America Executive Director and 1988 Olympic Silver Medalist

SWIM ACROSS AMERICA LAUNCHES ANNUAL EVENT

On a chilly June day, 70 cancer survivors, friends of cancer victims, and those who simply hope to eradicate cancer in our lifetimes swam or boated the 1.5 mile stretch of Long Island Sound from Tod's Point beach in Greenwich, CT to ACGT headquarters in Stamford. They did this not only to raise funds for cancer gene therapy research, but to show their support for the important studies underway by researchers across the country.

Founded in 1987 by Jeff Keith and Matt Vossler, Swim Across America builds support for cancer research, prevention and treatment, through swimming-related events and fund-raisers, literally from Boston Harbor to San Francisco and cities in-between. To date over $16 million has been raised through multiple events, with proceeds benefiting cancer research and treatment at hospitals and research institutions designated as the beneficiary of each annual event.

In 2007, Swim Across America chose ACGT as its partner in its inaugural Greenwich-Stamford event, which will be repeated yearly. Children from the community were treated the day before the event to a clinic at the Greenwich Boys & Girls Club pool, under the tutelage of Olympians Rowdy Gaines, Eric Wunderlich, Craig Beardsley and Janel Jorgensen, who joined the swimmers the next day in the swim. Honorary Co-Chairs, Board member John Sites and wife Cindy, and Reuben and Arlene Mark, captained the event that raised over $200,000 to support a Young Investigator grant to Assistant Professor Dr. Khalid Shah, who is researching new therapies for brain cancers at Harvard Medical School, Massachusetts General Hospital.

ACCT YOUNG INVESTIGATORS PRESENT FINDINGS

ACGT Research Fellows reported on their culminating research projects to the Board of Directors and special guests at a luncheon November 29th. The occasion provided the opportunity to hear firsthand from these 2003 Young Investigators their challenges, disappointments and successes during three years of scientific investigation on projects sponsored by the ACGT Fund for Discovery.



Laurence Cooper, MD, PhD, *Children's Cancer Hospital, MD Anderson Cancer Center, Houston, TX,* has been developing immune-mediated gene therapy and cancer vaccines to fight leukemia and lymphoma in children. Dr. Cooper's lab focuses on using the immune system's T-cells as a weapon against relapsed or drug-resistant cancers. "This therapy harnesses the power of T-cells, a type of immune cell, and uses gene therapy to redirect the function of the cell so that it can target a patient's leukemia or lymphoma," Cooper said. Experiments initially funded by ACGT have led to new clinical trials that will facilitate the implementation of the next generation of immunotherapy protocols.

Timothy Lane, PhD, *Assistant Professor, Jonsson Comprehensive Cancer Care, University of California, Los Angeles, recipient of the Kimberly Lawrence Netter Gene Therapy Breast Cancer Award,* studies anti-angiogenic therapies as a potential complement to more traditional cancer treatments for breast cancer. Since most human tumors recruit new blood vessels in order to grow, anti-angiogenesis is viewed as a promising treatment that strangulates the tumor by denying its blood supply. Dr. Lane's lab has identified a genetic element that allows genes to be expressed in a group of bone marrow-derived cells that contribute to cancer-related blood vessels. Called "endothelial cell precursors [ECP's]" this type of stem cell plays a role in the formation of new blood vessels. A simple virus was designed to carry genes to the ECP's to prevent tumor growth, and thus far, research studies are proving the hypotheses.





Katherine Ryman, PhD, *Assistant Professor, Louisiana State University Medical Center, Shreveport, LA,* has explored the use of tumor-killing viruses to destroy prostate cancer cells. The use of these killer cells, in an approach known as "virotherapy," exploits viruses that are able to target and destroy cancer cells, while sparing the surrounding normal tissue. Dr. Ryman's lab employs a virotherapy approach based on infection with Sindbis virus, which travels rapidly in the bloodstream through the body to improve access to metastasized cancer cells. Positive results have led to using these agents in cultured cells to optimize the ability of the virus to cure this potentially deadly cancer.

Robert Vonderheide, MD, D. Phil, *Assistant Professor of Medicine, University of Pennsylvania, Philadelphia, PA,* was unable to attend, so his colleague, Dr. Bruce Levine, spoke on behalf of his research into immunotherapy for neuroblastoma. Dr. Vonderheide's lab is developing a vaccine that will awaken an immune response to destroy the cancer cells. One strategy under investigation has been to use the patient's own immune cells, removed from the body and re-engineered before re-injection, which Dr. Vonderheide is deploying for children with cancer. This work has established the scientific and manufacturing rationale for the translation of gene therapy technology for clinical application, and, as a result of this study, a trial was launched for canines with lymphoma, which frequently mimics the human experience, with great success.





George Coukos, MD, PhD, left, with ACGT Executive Director, Margaret Cianci, and Stephen Eck, MD, PhD, member Scientific Advisory Council.

ACGT RESEARCH FELLOW SPREADS THE WORD

ACGT 2006 Investigator, Dr. George Coukos, recipient of the $1 million Dr. Judah Folkman Angiogenesis Award for Cancer Gene Therapy, presented in April 2007 a synopsis of his research using anti-angiogenic gene therapy for ovarian cancer. The study tests the hypothesis that T-body cell therapy is the only form of anti-angiogenetic gene therapy immediately translatable clinically that can deliver sustained VDA-type effect which is tumor-specific, self-amplifying in vivo, and endowed with memory (sustainable). Coupled with the genetic stability of tumor epithelium and the catastrophic consequences that vascular damage has on the tumor, T-body immune-gene therapy could potentially achieve tumor eradication. In the initial stages of a five year study, in vitro human lymphocytes were engineered in the lab to recognize and attack tumor blood vessels.

"Our translational team of basic scientists and clinicians will provide the first comprehensive evaluation of the use of this redirected T-cell concept to implement anti-angiograph immune gene therapy in cancer patients. Currently, we are the only group worldwide with the combined expertise to test this hypothesis."

Coukos reports that preliminary results are encouraging, and that the lab is well into the planning and development of parallel human and murine immortalized endothelial cells expressing the target TEMI [tumor endothelial marker I.] Development of specialized models of mice that can be repopulated with the human blood vessels is proceeding as planned and will lead to a Phase I trial. The Coukos lab seeks a new and effective treatment for this deadly cancer, a fatal diagnosis for 60% of patients within five years.

"By altering the genes expressed in a tumor (e.g., expressing new genes or inhibiting the expression of existing genes) one can change the biology of the tumor and alter its fate. Moreover, gene therapy is not a single therapy but a platform for many different therapies, whereby the genes expressed (or inhibited) can be selected to achieve optimal results."

Stephen Eck, MD, PhD
Vice President, Translational Medicine and Pharmacogenics, Eli Lilly and Co.
ACGT Scientific Advisory Council Member

ACGT RESEARCH FELLOWS 2002 – 2007

2007

Antonio E. Chiocca, MD, PhD
Ohio State University Research Foundation
Glioma
Tumor Specific Replicating Viruses and Bacteria

Ronald Levy, MD
Stanford University
Lymphoma
Immune-Medicated Gene Therapy and Cancer Vaccines

Clodagh O'Shea, PhD
Salk Institute for Biological Studies
Breast Cancer
Tumor Specific Replicating Viruses and Bacteria

Khalid Shah, PhD
Harvard Medical School, Massachusetts General Hospital
Brain Cancer
Tumor Targeting
Recipient of the Swim Across America Young Investigator Award

2006

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

George Coukos, MD, PhD
University of Pennsylvania Abramson Family Cancer Research Institute
Ovarian and Peritoneal Cancer
Anti-angiogenesis
Recipient of the Dr. Judah Folkman Angiogenesis Award for Cancer Gene Therapy

Maciej S. Lesniak, MD
University of Chicago
Brain Cancer
Tumor Targeting and Vector Development

Kah-Whye Peng, PhD
Mayo Clinic Cancer Center
Myeloma
Tumor Specific Replicating Viruses and Bacteria

Eckhard Podack, MD, PhD
University of Miami Miller School of Medicine
Lung Cancer
Immune-mediated gene therapy

Harald Sauthoff, MD
New York University
Breast/Lung Cancer
Tumor Targeting and Vector Development

2005

Biao He, PhD
Pennsylvania State University
Breast Cancer
Tumor Specific Replicating Virus

Thomas J. Kipps, MD, PhD
University of California, San Diego, Moores Cancer Center
Lymphoma/Leukemia
Immune-mediated gene therapy

Hyam I. Levitsky, MD
Johns Hopkins University School of Medicine
Lymphoma/Leukemia
Immune-mediated gene therapy

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

Koji Tamada, MD, PhD
University of Maryland
Lymphatic systems
Immune-mediated gene therapy

Xianzheng Zhou, MD, PhD
University of Minnesota – Twin Cities
Blood, Lymphoma/Leukemia
Immune-mediated gene therapy

Yiping Yang, MD, PhD
Duke University
Lymphoma/Leukemia
Immunotherapy

2004

Chien-Fu Hung, PhD
Johns Hopkins University School of Medicine
Ovarian Cancer
Immunotherapy

Mukesh Jain, MD, FAHA
Case Western Reserve University
Metastatic Cancer
Anti-angiogenesis

Carl H. June, MD
University of Pennsylvania Abramson Family Cancer Research Institute
Lymphoma/Leukemia
Immune-mediated gene therapy

Suzie Pun, PhD
University of Washington
Metastatic Cancer
Targeted non-viral delivery
Recipient of Patricia Zoch Tate Gene Therapy Award

Michel Sadelain, MD, PhD
Memorial Sloan Kettering Cancer Center
Lymphoma/Leukemia
Immune-mediated gene therapy

Jian Yu, PhD
University of Pittsburgh School of Medicine, Hillman Cancer Center
Lung Cancer
Oncogenesis

2003

Laurence Cooper, MD, PhD
University of Texas, MD Anderson Cancer Center
Lymphoma/Leukemia
Immune-mediated gene therapy

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

Todd R. Reilly, PhD
Johns Hopkins University [thru 2006]
Breast Cancer
Immune-mediated gene therapy

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

Robert Vonderheide, MD, D.Phil.
University of Pennsylvania Abramson Family Cancer Research Institute
Neuroblastoma
Immune-mediated gene therapy

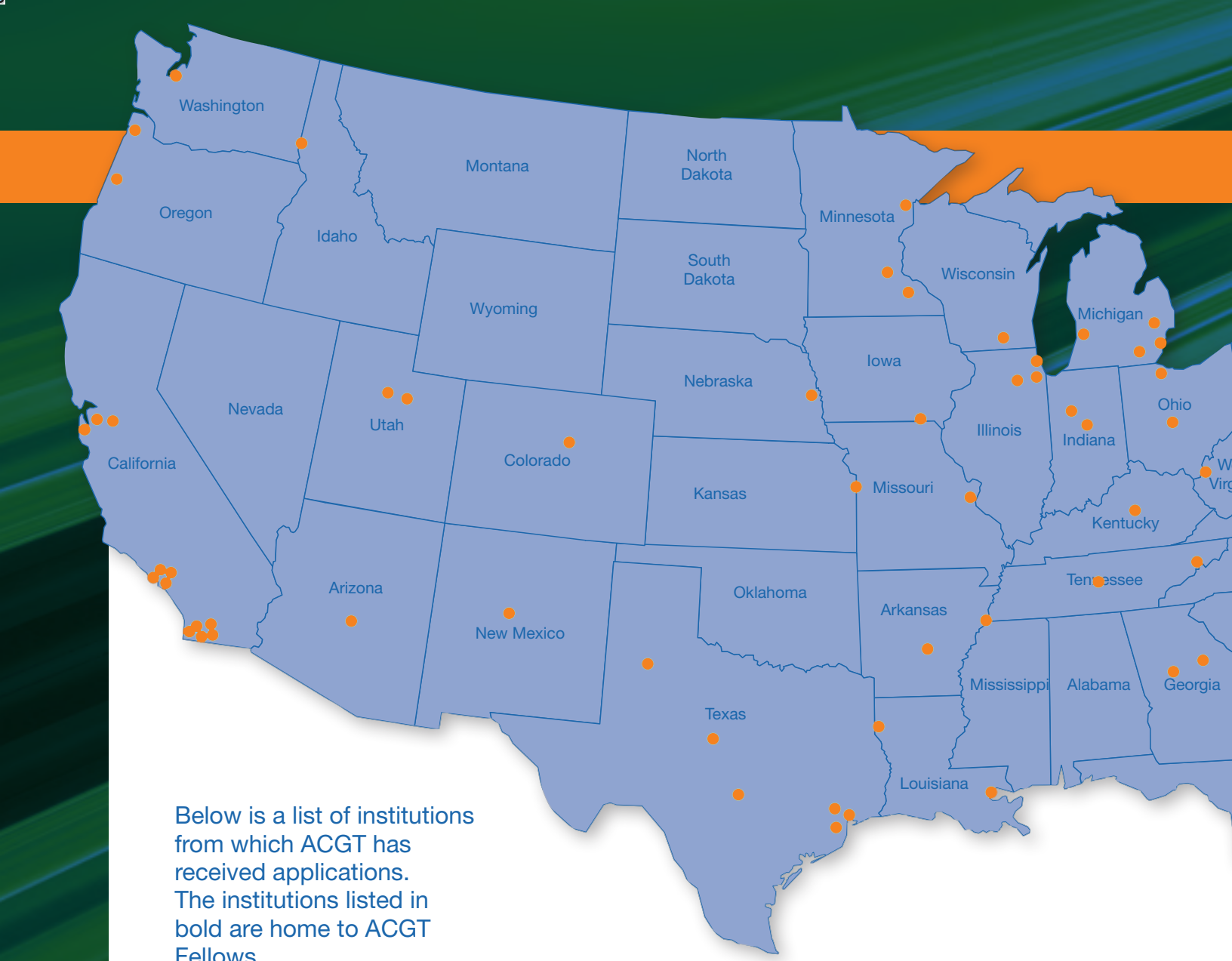
2002

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

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

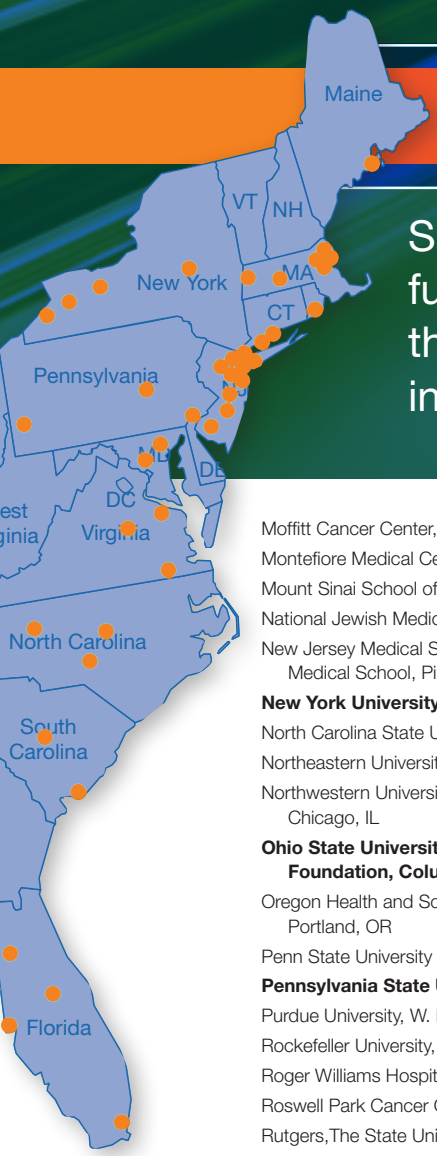
Thomas S. Griffith, PhD
University of Iowa
Prostate Cancer
Immune-mediated gene therapy

"ACGT has created an army of investigators."
Dr. George Coukos, MD, PhD, *University of Pennsylvania, Abramson Family Cancer Research Institute*



Below is a list of institutions from which ACGT has received applications. The institutions listed in bold are home to ACGT Fellows.

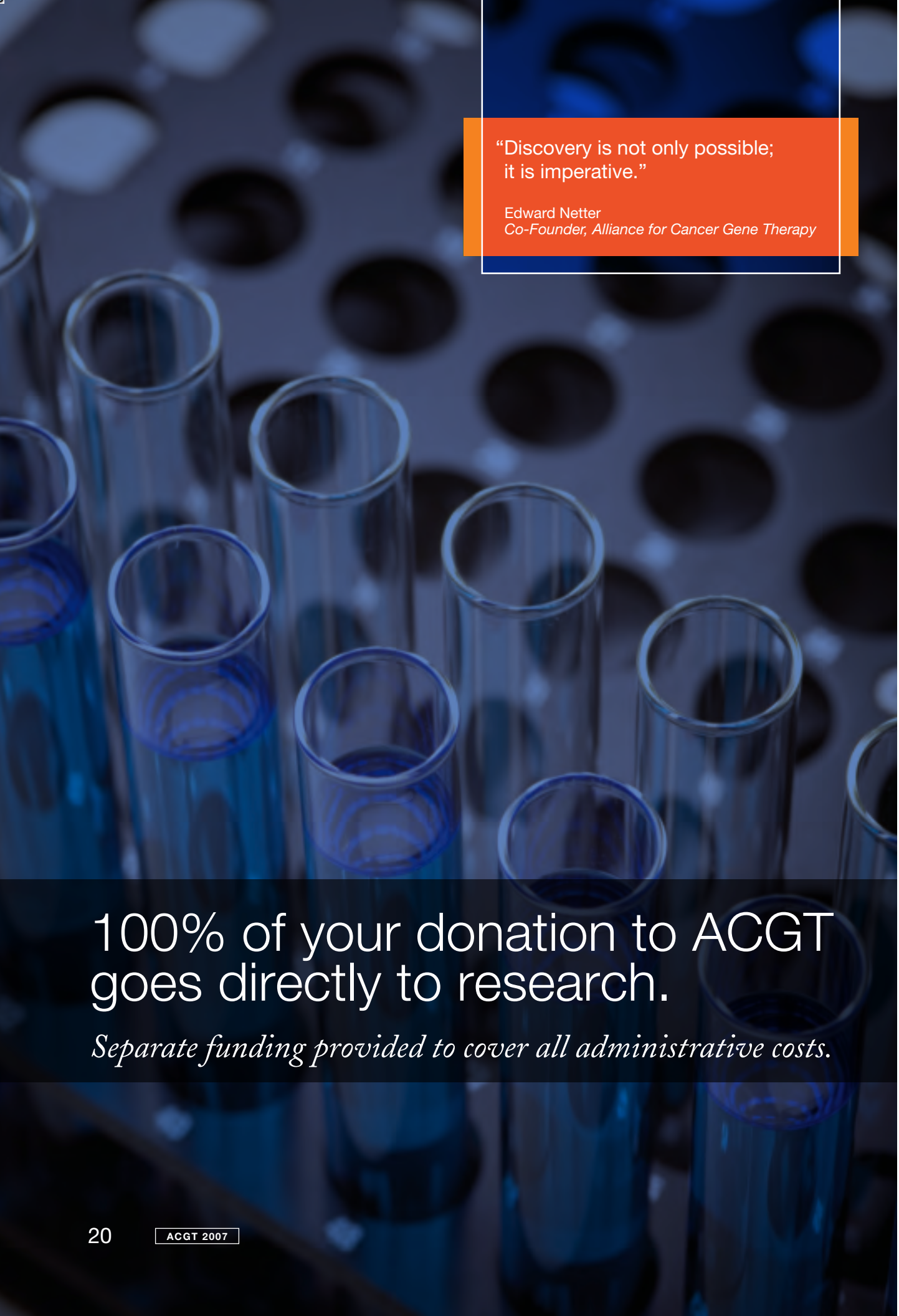
Albany Medical Center, Albany, NY
Albert Einstein School of Medicine, Bronx, NY
Arizona State University, Phoenix, AZ
Auburn University, Auburn, AL
Aurora Healthcare, Inc., Milwaukee, WI
Baylor College of Medicine, Houston, TX
Baystate Medical Center, Springfield, MA
Beth Israel Deaconess Medical Center, Boston, MA
Boston University, Medical Campus, Boston, MA
Brigham and Women's Hospital, Inc./Harvard University Medical School, Boston, MA
Case Western Reserve University, Cleveland, OH
Cedars-Sinai Medical Center, Los Angeles, CA
Children's Hospital Boston, Boston, MA
Children's Hospital, Los Angeles, Los Angeles, CA
Children's Hospital, Philadelphia, Philadelphia, PA
City of Hope National Medical Center, Duarte, CA
Cold Spring Harbor Laboratory, Cold Spring Harbor, NY
Columbus Children's Research Institute, Columbus, OH
Dana-Farber Cancer Institute, Boston, MA
Dartmouth College, Hanover, NH
Duke University School of Medicine, Durham, NC
East Tennessee State University, Johnson City,TN
Eastern Virginia Medical School, Norfolk, VA
Emory University, Atlanta, GA
Father Flanagan's Boy's Home, Boys Town, NE
Fred Hutchinson Cancer Research Center, Seattle, WA
Georgetown University Medical Center, Washington, DC
Henry Ford Health System, Detroit, MI
Illinois Institute of Technology, Chicago, IL
Indiana University School of Medicine, Indianapolis, IN
Jackson Laboratory, Bar Harbor, ME
Johns Hopkins University School of Medicine/Sidney Kimmel Comprehensive Cancer Center, Baltimore, MD
La Jolla Institute for Allergy & Immunology, San Diego, CA
Louisiana State Health & Sciences Center-Shreveport, Shreveport, LA
Lovelace Respiratory Research Institute, Albuquerque, NM
M.D. Anderson Cancer Center, University of Texas, Houston, TX
Marshall University Research Corporation, Huntington, WV
Mary Crowley Medical Research Center, Dallas, TX
Massachusetts General Hospital/Harvard University Medical School, Boston, MA
Mayo Clinic/Foundation, Rochester, MN
Memorial Sloan Kettering Cancer Center, New York, NY
Methodist Hospital Research Institute, Houston, TX



THE REACH OF ACGT

Since 2001, ACGT has received 428 applications for funding from 117 prestigious institutions throughout the country investigating cancer gene therapies as indicated on this map.

Moffitt Cancer Center, Tampa, FL
Montefiore Medical Center, Bronx, NY
Mount Sinai School of Medicine, New York, NY
National Jewish Medical & Research Center, Denver, CO
New Jersey Medical School (UMDNJ), Robert Wood Johnson Medical School, Piscataway, NJ
New York University School of Medicine, New York, NY
North Carolina State University, Raleigh, NC
Northeastern University, Boston, MA
Northwestern University Feinberg School of Medicine, Chicago, IL
Ohio State University College of Medicine/OSU Research Foundation, Columbus, OH
Oregon Health and Science University School of Medicine, Portland, OR
Penn State University College of Medicine, Hershey, PA
Pennsylvania State University, University Park, PA
Purdue University, W. Lafayette, IN
Rockefeller University, New York, NY
Roger Williams Hospital, Providence, RI
Roswell Park Cancer Center, Buffalo, NY
Rutgers,The State University of New Jersey, Piscataway, NJ
Salk Institute for Biological Studies, San Diego, CA
Southern Methodist University, Dallas, TX
South Carolina Medical University of Charleston, SC
St. Louis University Medical Center, St. Louis, MO
St. Jude Children's Research Hospital, Memphis, TN
Stanford University, Stanford, CA
SUNY, State University of New York, Buffalo School of Medicine, Buffalo, NY
SUNY, State University of New York, Downstate, Brooklyn, NY
SUNY, State University of New York, Stony Brook School of Medicine, Stony Brook, NY
Texas Tech University, Lubbock, TX
Thomas Jefferson University, Kimmel Cancer Center, Philadelphia, PA
Torrey Pines Institute for Molecular Studies, San Diego, CA
Tufts University, Medford, MA
Tulane University, New Orleans, LA
University of Alabama, Birmingham School of Medicine, Birmingham, AL
University of Arkansas for Medical Sciences, Little Rock, AR
University of California, Berkeley School of Medicine, Berkeley, CA
University of California, Davis School of Medicine, Sacramento, CA
University of California, Irvine, Irvine, CA
University of California, Los Angeles/Brentwood Biomedical Research Institute, Los Angeles, CA
University of California, Los Angeles/Jonsson Comprehensive Cancer Center, Los Angeles, CA
University of California, San Diego School of Medicine/ Moores Cancer Center, La Jolla, CA
University of California, San Francisco, San Francisco, CA
University of Central Florida Burnett College of Biomedical Sciences, Orlando, FL
University of Chicago, Chicago, IL
University of Delaware, Newark, DE
University of Florida, Gainesville College of Medicine, Gainesville, FL
University of Georgia Biomedical and Health Sciences Institute, Athens, GA
University of Illinois Chicago College of Medicine, Chicago, IL
University of Iowa College of Medicine, Iowa City, IA
University of Kansas Medical Center Research Institute, Kansas City, KS
University of Kentucky College of Medicine, Lexington, KY
University of Maryland, Baltimore, MD
University of Massachusetts Medical Center, Worcester, MA
University of Miami - Miller School of Medicine, Miami, FL
University of Michigan Medical School, Ann Arbor, MI
University of Minnesota, Twin Cities, Duluth, MN
University of Missouri, Columbia, Kansas City, MO
University of North Carolina at Chapel Hill, Chapel Hill, NC
University of Pennsylvania School of Medicine/Abramson Family Cancer Research Institute, Philadelphia, PA
University of Pittsburgh School of Medicine, Pittsburgh, PA
University of Rochester Medical Center, Rochester, NY
University of South Carolina Research Foundation, Columbia, SC
University of Southern California, Keck School of Medicine, Los Angeles, CA
University of Texas, Austin, Austin, TX
University of Toledo, Ohio, Toledo, OH
University of Utah School of Medicine, Salt Lake City, UT
University of Washington, Seattle, WA
University of Wisconsin Medical School, Madison, WI
Van Andel Research Institute, Grand Rapids, MI
Vanderbilt University Medical Center, Nashville, TN
Virginia Commonwealth University, Richmond, VA
Wake Forest University School of Medicine, Winston-Salem, NC
Washington State University, Pullman, WA
Washington University in St. Louis, St. Louis, MO
Wayne State University School of Medicine, Detroit, MI
Yale University School of Medicine, New Haven, CT



"Discovery is not only possible;
it is imperative."

Edward Netter
Co-Founder, Alliance for Cancer Gene Therapy

100% of your donation to ACGT goes directly to research.

Separate funding provided to cover all administrative costs.

FINANCIAL REPORT

APRIL 30, 2007 AND 2006

CONDENSED STATEMENTS OF ACTIVITIES	2007	2006
Support and revenue:		
Contributions	$3,312,876	$4,176,363
Contributions for administrative expenses (specially designated)	217,100	92,404
Contributed services	264,392	181,057
Interest and dividend income	180,995	82,782
Realized and unrealized gains on investments, net	308,741	218,159
TOTAL SUPPORT AND REVENUE	**4,284,104**	**4,750,765**
Expenses:		
Program services:		
Research grants and awards	3,522,732	3,946,262
Supporting services	424,253	213,852
TOTAL EXPENSES	**3,946,985**	**4,160,114**
Increase in total net assets	337,119	590,651
Total net assets at beginning of year	1,932,156	1,341,505
TOTAL NET ASSETS AT END OF YEAR	**$2,269,275**	**$1,932,156**

CONDENSED STATEMENTS OF FINANCIAL POSITION	2007	2006
Assets:		
Cash and cash equivalents	$3,115,698	$1,344,978
Contributions receivable, net	1,974,315	3,197,082
Investments, at fair value	3,126,407	2,891,197
TOTAL ASSETS	**8,216,420**	**7,433,257**
Liabilities and net assets:		
Liabilities:		
Grants payable	5,941,775	5,486,635
Accounts payable and accrued expenses	5,370	14,466
TOTAL LIABILITIES	**5,947,145**	**5,501,101**
TOTAL NET ASSETS	**2,269,275**	**1,932,156**
TOTAL LIABILITIES AND NET ASSETS	**$8,216,420**	**$7,433,257**

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

DONORS ARE CATALYTIC AGENTS



In six short years, the Alliance for Cancer Gene Therapy has achieved a leadership position as a non-profit organization demonstrating the highest standards of governance, effectiveness, public communications and financial management. Our commitment to investing 100% of all contributed funds has resulted in the selection of thirty-one outstanding ACGT Research Fellows, several of whom are profiled in this report. Neither what we have accomplished to date ($18.8 million) nor our goal to reach a total investment of $38 million in a decade could possibly be realized without the support and in-kind effort of the individuals and organizations listed below. To each of you, we extend our heartfelt thanks for your commitment to ACGT and for the affirmation of our mission that it represents.

Virginia V. Boldt
Director of Development & Strategic Planning

We have made every effort to ensure the accuracy of our donor listing. If your name has been omitted or misprinted, please accept our sincere apologies and notify us by email at fyoung@acgtfoundation.org or call Francine Young at 203.358.8000 extension 349.

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Dr. Jason Faller and Mrs. Karen Wagner
Mr. & Mrs. William Farrell
Ms. Patricia N. Fast
Mr. & Mrs. Conner Fay
Mr. Stephan Feder
Mr. Marc A. Feigen
Mr. & Mrs. Joseph Feldman
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Mr. Alexander Floyd
Mr. & Mrs. Brian Forrow
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Mr. & Mrs. William Fox, Jr.
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Mr. William Golden
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In Honor of Robert Rosenberg
In Honor of Edward Greenberg
In Honor of Toby Ritter
In Honor of Rita and Philip Rosen
Mr. Harold Goldman
Mr. & Mrs. Peter Goldsmith
Mr. & Mrs. James Golub
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Ms. Jodie Green
In Memory of Marion Gould
Mr. Todd W. Green
Mr. Edwin Greenhaus
Ms. Kathleen Gregar
Mr. Michael Gregory
Mr. & Mrs. James Griffin
Mr. John Griswold
Ms. Ann Hagmann
Mr. Robert Hahn
Mr. & Mrs. Barry G. Haimes
Dr. Christine Hamilton and Mr. Malcolm Hall
Ms. Sarah Wolf and Mr. Charles Hallac
Ms. Diana Hamilton
Mr. & Mrs. John W. Hanauer



Board member, Eric Rothfeld, who established the $1 million *Joan Miller and Linda Bernstein Gene Therapy for Ovarian Cancer Award*

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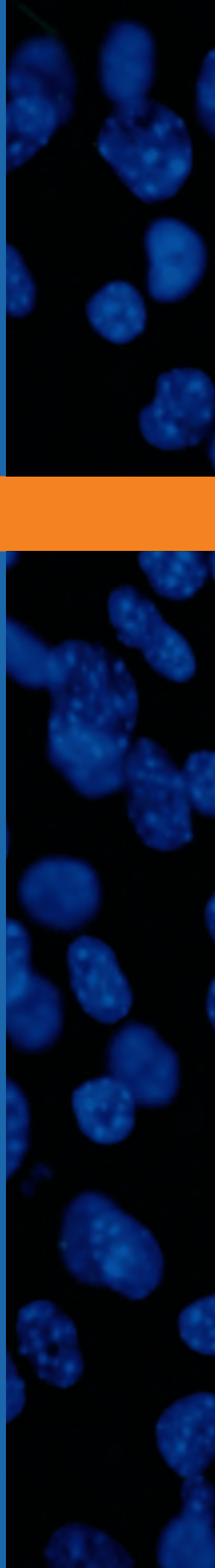
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