A new decade
a new era
for molecular medicine

Believing that medical science can do better
Envisioning the future

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 cell and gene therapies, in the hopes of realizing an effective alternative to the treatment and management of cancer.

Our mission

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 cell and gene therapy and facilitate greater collaboration among the scientific and philanthropic communities to foster the best possible research and ultimately the best treatment.
1 of 3 Americans will be diagnosed with cancer at some point. One half will die from their cancer.

According to the World Health Organization, cancer will be the leading worldwide cause of death in 2010.

One of every five deaths is due to cancer.

Nearly 1.5 million new cancers will be diagnosed in 2010.

More than one-half million succumb to cancer every year. 1,500 deaths every day.

Over 11 million people are living with cancer.

Facing the reality of cancer in America

Traditional treatments are still the only options.

Surgery is dependent on location, size and stage, and is an option in only 1 of 20 cancers.

Radiation depends on location and may endanger healthy body tissue.

Chemotherapy attacks the entire body to treat a cancer.

According to the World Health Organization, cancer will be the leading worldwide cause of death in 2010.

“Eight years is a nano-second in research terms, yet we have accomplished a great deal in this short time. Well over one hundred academic medical centers throughout the country now sponsor research into cancer cell and gene therapies, clinical trials of novel therapeutics continue to be launched, and new biologic drugs will be coming to market. Many great minds are devoted to seeking solutions to this dreadful disease, if only they can secure the funding and the support they need.”

Savio Woo, PhD, Mount Sinai School of Medicine Chairman, ACGT Scientific Advisory Council

“The cancer fatality rate is the equivalent of three jumbo jets crashing every day.”

Dr. Francis Collins, Director, National Institutes of Health
Alliance for Cancer Gene Therapy is the only non-profit in the nation dedicated exclusively to funding and fostering research into cell and gene therapies for cancer.

The FUND FOR ADVANCEMENT supports established investigators moving successful research from the laboratory to clinical translation and trials.

Grants up to $1 million awarded to move the science forward
11 Clinical Translational Awards
$10.5 Million awarded

The FUND FOR DISCOVERY encourages innovation among tenure track assistant professors seeking new treatments using cell and gene therapies.

Grants up to $500,000 awarded for innovative research
22 Discovery Awards
$10.45 Million awarded
ACGT grant applicants represent 145 prestigious research institutions
ACGT Research Fellows conduct studies at 25 institutions
ACGT research implemented at 10 of the top 12 NCI designated cancer centers
33 Grants awarded in eight years
New grants to be announced in 2010
Grant results are enhanced by further funding, research and trials
- 16 human clinical trials approved
- 2 patents pending
- 107 publications in leading professional journals cite ACGT as a facilitator of important research

More than 150 universities and research institutions are now engaged in the investigation of cell and gene therapies for cancer.

Funding and fostering the most promising research

“We know that literally hundreds of genes might be mutated or deleted in any cancer cell. You might ask, how can we replace all of those genes and get their function going again in the cancer cell? The interesting observation that we made almost 15 years ago was that it’s not necessary to completely normalize the cancer cell genome. All that’s necessary is to put back one critical gene, and that may be enough to trigger the cell death program in the cancer cell.”

Jack Roth, MD, Director, W. M. Keck Center for Cancer Gene Therapy, M. D. Anderson Cancer Center
ACGT Scientific Advisory Council

“We private funding is crucial to fulfill the promise of cell and gene therapies and to move the science from the laboratory to practice.”
Margaret C. Cianci Executive Director, ACGT
CLINICAL TRANSLATION is the process that transforms research evidence into clinical guidelines and policy. The aim of translation is to provide useful, useable, and relevant packages of summarized evidence to clinicians.

CLINICAL TRIAL is a biomedical experiment with patients that follows very precise rules so that treatment may be evaluated for safety, toxicity and efficacy. Participants, who continue to work with their primary care physician during the trial, are among the first beneficiaries of new treatments, and also contribute to future alternatives for cancer patients of all types.

It is anticipated that 1 in 50 cancer patients will enter a clinical trial in the next 5 years.

“Clinical trials offer patients promising new therapies and high quality care. We need to reduce the unnecessary barriers so that doctors can enroll patients and patients have the information and coverage they need to participate.”

Julie Gralow, MD, Executive Editor, Clinical Cancer Advances, 2008

For advanced cancer patients, early phase clinical trials are often the only life-changing, therapeutic approach.

PHASE I AND II TRIALS test an experimental treatment with first a small, then a larger group of patients, to determine the safest dosage and efficacy.

PHASE III AND IV trials are conducted with much larger groups to confirm results and lay the groundwork for regulatory approval including a drug’s risks, benefits and optimal use.
We now know that all cancer evolves from damaged, missing or mutated genes: a biological misfire. Every tumor has a blueprint that we will be able to identify and alter, with little or no effect on other parts of the body.

- Gene drugs that are delivered directly to the tumor to facilitate cancer cell death.
- Cell and gene vaccines that boost the immune system to reject and destroy cancers.
- Targeted cell and gene therapeutics to destroy cancer stem cells, which are believed to be the root of many kinds of cancers.
- Gene medications that will strangulate cancer cells by cutting off their blood supply (anti-angiogenesis).

Imagine gene therapy as a molecular Trojan horse, with the potential to neutralize and or destroy the enemy.

“DNA in our cells is the blueprints of our life. If there is any mistake or changes in that blueprint, that can result in diseases. And so our goal is to actually be able to go back and correct those blueprints back to their original form.”
Suzie Pun, PhD, University of Washington ACGT Research Fellow 2004

“The problem is that you’re just not treating one simple abnormality in the cancer cell, but you have many things to take care of. There probably won’t be one single form of gene therapy – one magic bullet if you will. There will be several avenues of attack. And as opposed to saying someone has breast cancer, you will define five or six different kinds of breast cancer with different gene abnormalities, each treated in a slightly different way.”
Robert Martuza, MD, Massachusetts General Hospital, Harvard Medical School ACGT Scientific Advisory Council
### 2009

**Steve Thorne, PhD**  
**Institution:** University of Pittsburgh  
**Focus:** Breast & Ovarian Cancers  
**Research:** Tumor Specific Replicating Viruses & Bacteria

### 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:** Brain Cancer  
**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 Viruses & Bacteria

**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

“ACGT has created an army of investigators.” Dr. George Coukos, MD, PhD, University of Pennsylvania,
Abramson Family Cancer Research Institute, ACGT Research Fellow 2006
Extending our reach

Since 2001, ACGT has received 629 applications for funding from 145 different institutions throughout the U.S., Canada and Puerto Rico investigating cancer cell and gene therapies.

- Institutions from which ACGT has received applications
- Locations of ACGT Fellows
- Locations of multiple ACGT Fellows
Accelerating research into trials

16 of 33 ACGT-funded research projects have been approved for clinical trials, of which 7 were implemented or completed in 2009.

Thomas Griffith, PhD
University of Iowa
ACGT Research Fellow 2002
Focus: Prostate Cancer

This trial employs a unique agent that induces apoptosis (cell death) without typical side-effects. The agent is also unique in its ability to influence a wide range of tumor cells, without harm to normal cells or tissue. Safety and efficacy was demonstrated in previous research and further studies indicated the impact is enhanced if used in combination with chemotherapy and/or radiation. This trial has further demonstrated a positive effect on larger more established tumors, as well as small.

“The research has yielded a profound effect! We have produced a clinical-grade vector that facilitates cancer cell suicide. We have also been able to learn how to combine this treatment with other compounds to enhance the effect.”

Laurence Cooper, MD, PhD
University of Texas
M.D. Anderson Cancer Center
ACGT Research Fellow 2003
Focus: Lymphoma/Leukemia

Because many gene therapies are dependent on genetically altered cells, increasing the speed of production is crucial. The cells for this trial were developed using a new device that significantly reduces the time needed for manufacture. Set up largely as a laboratory within a laboratory, manufacture has been established in compliance with best practices in the field. The new process manufactures cells 100 times faster than standard technologies. Once re-engineered, cells are reintroduced into the patient to attack and kill tumors, with minimum risk to the patient’s health.

“Our goal is to provide therapy to patients closer to their time of need, which we also hope will translate to better treatment opportunities for relapsed patients.”
Accelerating research into trials (continued)

Michel Sadelain, MD, PhD
Memorial Sloan-Kettering Cancer Center
ACGT Scientific Advisory Council
ACGT Research Fellow 2004
Focus: Lymphoma/Leukemia

Cell engineering is a promising approach for establishing potent and durable immunity against cancer. This trial is based on previous research that consistently demonstrated that the transfer of genes encoded with anti-tumor receptors will stimulate the rejection of tumor cells and the prevention of cell malfunctions in cancer patients. Cells can also be engineered to express biological markers used to track their locations throughout the body and monitor the patient’s response to therapy through non-invasive imaging technologies.

“I am hopeful that the future development of this and other gene therapies will become easier as we fulfill the promise of providing effective gene therapies for patients with illnesses that cannot be cured through other means.”

Hyam Levitsky, MD
Johns Hopkins School of Medicine
ACGT Research Fellow 2005
Focus: Lymphoma/Leukemia

This trial targets a particularly virulent form of leukemia that frequently results in bone marrow failure. A vaccine has been developed that alerts the immune system to the tumor. The research is based on pre-clinical models that have demonstrated unequivocal progress in generating an immune system response without any unfavorable reactions. Continued trials have been designed to evaluate the potential to integrate use of the vaccination with broader therapies.

Thomas Kipps, MD, PhD
University of California, San Diego
Moores Cancer Center
ACGT Research Fellow 2005
Focus: Lymphoma/Leukemia

Using a direct injection of a virus into the lymph nodes of patients with chronic lymphocytic leukemia (CLL) this study is based on previous research which demonstrated that treatment is safe and has systemic anti-tumor effects. Patients experienced no long-term side-effects and an optimum dosage was derived, to be tested further in Phase II clinical trials. These results will also make possible similar studies in patients with lymphoma.

“Our goal is to engineer ‘super T cells’ that will track down tumor cells in any location and persist as long as there are tumor cells to be found.”
Eckhard Podack, MD, PhD
University of Miami
Miller School of Medicine
ACGT Research Fellow 2006
Focus: Lung Cancer

This Phase I proof of principle trial tested the safety and impact of a vaccine in patients with non-small cell lung cancer. The vaccine utilizes lung tumor cell secretions that facilitate the generation of killer cells. Three dose combinations were alternated, with each third of patients receiving injections at 2 week intervals, weekly or twice per week, without any toxicity. Trial findings suggest that more frequent injections increase efficacy. This form of lung cancer is especially virulent; however tumors have ceased to progress in some trial patients as long as 104 weeks thus far.

“Killer cells are a very important component of the immune system. And it is by now accepted by a large number in the community that killer cells are really the primary weapon against cancer if the immune system is involved.”

Carl June, MD
University of Pennsylvania Abramson Family Cancer Research Institute
ACGT Scientific Advisory Council
ACGT Research Fellow 2004/2008
The Joan Miller and Linda Bernstein Gene Therapy Award
Focus: Ovarian Cancer

Dr. June, a two-time grant award recipient, continues his groundbreaking research to harness the power of the immune system to recognize and destroy tumor cells. The technique was first researched for leukemia and expanded into this trial for ovarian cancer. Cells are removed from cancer patients, manipulated in the laboratory to add engineered genes, and transferred back to the patient, triggering an immune system response which results in the eradication of tumors.

“I wake up every morning wondering how to basically ‘put the immune system on steroids’. A number of approaches currently under development using cancer gene therapy have the potential for enhancing treatment outcomes while minimizing side effects.”

“Most commonly used chemotherapies are little more than DNA poisons that slow down tumor growth but do not ultimately cure patients. There is a desperate need to identify new drugs and therapeutic modalities that ablate cancer cells while leaving normal cells unharmed.”

Clodagh O’Shea, PhD
Salk Institute for Biological Studies
ACGT Research Fellow 2007

“Although studies are still experimental, we 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 stem cells will be introduced near the remaining tumor cells, eliminating the risk of recurrence.”

Khalid Shah, PhD
Harvard Medical School
Massachusetts General Hospital
ACGT Research Fellow 2007
A spring 2009 article entitled “Cancer in 2009: What Needs to be Done” published in Promise magazine, by Dr. John Mendelsohn, President of the University of Texas M.D. Anderson Cancer Center, outlines ten steps to increase the ranks of survivors, with #1 being a research focus on human genetics and the regulation of gene expression.

“Most cancers involve several abnormally functioning genes – not just one – which makes understanding and treating cancer terribly complex. The good news is that screening for genes and their products can be done with new techniques that accomplish in days what once took years.”

In the September 3rd, 2009 issue of Time magazine, Alice Park’s report, “A Shot at Cancer” highlights the National Cancer Institute’s growing embrace of the prospect of a cancer vaccine.

“An immune system trained to recognize the first signs of new or recurrent growth can begin to attack malignances far earlier than the best scans can detect them.”

In the September 2009 issue of Discover magazine, Journalist Jill Neimark, in “The Second Coming of Gene Therapy” cites the American Society of Gene and Cell Therapy’s conclusion that successful gene therapy depends as much on manipulating cells, including immune system cells, as the genes within.

“It was this new perspective more than anything else that turned gene therapy from a simple but failed and frustrated hope into, once again, medicine’s next big thing – a stunning spectacle of hubris, ignominy, and redemption on the scientific stage.”

An October 2009 editorial in Nature magazine, “Gene therapy deserves a fresh chance” suggests that the science of molecular medicine has moved beyond its difficult beginnings and deserves appropriate recognition as a serious option for the future of cancer treatment.

“As a result, both scientists and clinicians now have a battery of extraordinarily refined tools for preclinical and clinical studies of gene therapy. The field is ripe for further successes.”

Gene therapy investigations are underway for many types of disease and each research finding provides new learning to combat all diseases and cancers.

On October 25, 2009, the Los Angeles Times article, “Gene therapy transforms eyesight of 12 people with rare visual defect” reported on the remarkable restitution of vision in 12 patients, including a legally blind boy, using gene therapy to replace a faulty gene in the retina. The new gene enters the cell nucleus where it makes the healthy enzymes required to see.
Believing that medical science can do better

<table>
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<th>Year</th>
<th>Events</th>
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| 2001 | - Alliance for Cancer Gene Therapy founded by Barbara and Edward Netter  
- Scientific Advisory Council formed with Dr. Savio Woo as Chair |
| 2002 | - First three grant awards focus on discovery in the fields of ovarian and prostate cancers and neuroblastoma  
- Educational program launched by co-founder Barbara Netter  
- www.acgtfoundation.org goes live |
| 2003 | - Five grants awarded from Fund for Discovery to encourage innovative research |
| 2004 | - Six grants awarded including 2 for Clinical Translation of Lymphoma/Leukemia  
- ACGT presents to members of the media at “An Editor’s Roundtable” in NYC |
| 2005 | - Seven grants awarded |
| 2006 | - Six grants awarded including the Judah Folkman Angiogenesis Award  
- Fifth anniversary dinner presents Lifetime Achievement Award to Dr. Judah Folkman, Children’s Hospital, Boston  
- Better Business Bureau awards seal of approval from Wise Giving Alliance |
| 2007 | - Four grants awarded including 2 for Clinical Translation  
- Swim Across America launches first annual Greenwich/Stamford Event for ACGT  
- Educational forums expanded to include researchers and practitioners throughout the country |
| 2008 | - Carl June, MD, receives the Joan Miller & Linda Bernstein Gene Therapy Ovarian Cancer Award for immune mediated gene therapy research |
| 2009 | - One Young Investigator grant awarded  
- 3 Educational Forums presented |
| 2010 | - ACGT cited frequently in the media as a facilitator for gene therapy research  
- Special forum on Brain Cancer hosts best researchers in the field  
- April 21, 2010 Gala Event Honors ACGT Research Scientists |

“The discovery and perfection of new treatments for cancer has been our target since the founding of the Alliance for Cancer Gene Therapy in 2001. We have never wavered from our belief that cell and gene therapies will become the new medical paradigm for treating and someday eradicating cancer. The promise is even more credible to us now, as research results continue to prove the potential of this extraordinary medical science.”

Barbara and Edward Netter, Founders, Alliance for Cancer Gene Therapy
The remarkable progress that has been made in a fairly short time is possible only because of the support of those who believe that medical science can, and must, do better. Donations and grants fund the best research and move the science forward toward clinical practice. Thank you for your support – past, present and future.
ACGT Leadership

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Progress Report created by Randy Kraft/Writer4Hire and Tara Framer Design.
“Cell and gene therapies attack cancer, and only the cancer, and offer great hope to make cancer a manageable disease.”

Savio L.C. Woo, Ph.D.
Professor and Chairman,
Department of Cell and Gene Medicine,
Mount Sinai School of Medicine
Chair, ACGT Scientific Advisory Council

www.acgtfoundation.org – 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.

- Learn more about our mission, vision, governance and alliances across the country
- Discover the definition and types of cancer cell and gene therapies and current research results
- Find active clinical trials and patient resources
- Access grant qualifications and applications
- Request digital recordings (DVDs) of educational events

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.