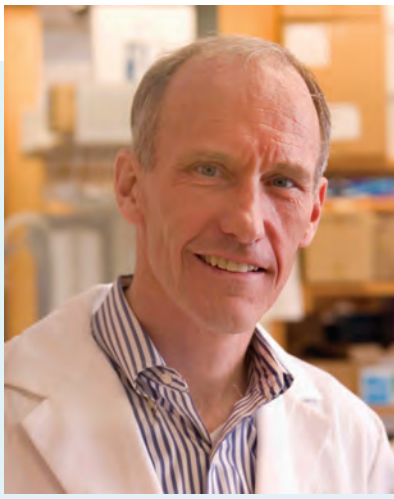
ACGT
ALLIANCE FOR
CANCER GENE THERAPY
Discovering new ways to treat and defeat cancer

Sequence

THE NEWSLETTER OF ALLIANCE FOR CANCER GENE THERAPY FALL 2014

IMMUNOTHERAPY TREATMENT NAMED BREAKTHROUGH THERAPY



> *We're at the dawn of a new industry. ACGT (funded research) has the potential to lead to less expensive cancer treatments – and, ultimately a cure.*
>
> – Dr. Carl June, University of Pennsylvania Abramson Family Cancer Research Institute and ACGT Research Fellow

An innovative gene-based treatment, seeded with early funding by ACGT, has proven so effective in trials for acute lymphoblastic leukemia (ALL) at University of Pennsylvania it has been designated by the FDA as a "Breakthrough Therapy" paving the way to an expedited (60 day) approvals process. ACGT Research Fellow Dr. Carl June spearheaded the research and is one of the leaders in the field. This is the first academic center and the first targeted cell treatment for cancer to achieve this special designation. To qualify, the treatment, on its own or in combination with other therapies, must demonstrate potentially lifesaving results. In the early stage clinical trials at the Perelman School of Medicine, 89 percent of trial patients who had not responded to conventional therapies went into complete remission after receiving CTL019, a drug that stimulates an immune system response and targets only the cancer, without toxicity to other parts of the body. The success of the treatment was also the basis for a recent agreement between the university and pharmaceutical firm Novartis to bring the drug to market sooner rather than later and the exclusive rights to develop and commercialize these therapies.



DISCOVER NEW WAYS TO TREAT AND DEFEAT CANCER

ACGT's website features reader-friendly information on the science of molecular medicine, research progress, clinical trials, and what you can do to help the fight against cancer. Sign on for postings on your computer, phone or tablet. More than 150 institutions throughout the United States and Canada, and thousands of scientists, are actively engaged in cell and gene-based therapies for cancer and we are closer every day to better treatments. You may want to like our Facebook page for regular updates and follow us on Twitter: @acgtfoundation.org.







> *Year after year, we are amazed by the heroic individuals and groups that swim in honor of a loved one or friend.*
>
> – Janel Jorgensen McArdle, *Swim Across America* President and 1988 Olympic silver-medalist

SWIM ACROSS AMERICA MAKES ANOTHER SPLASH

The 8th annual Greenwich-Stamford *Swim Across America* benefiting ACGT raised over $300,000 for cancer cell and gene therapy research, bringing the cumulative total of net proceeds to over $2 million. All funds raised support innovative therapeutic solutions by ACGT Research Fellows. The late June day was sunny and warm, the waters of Long Island Sound cool and crisp, and spirits high, as 175 swimmers and a throng of boaters glided through open water and in local pools, cheered on by a small army of volunteers. Seven swimmers have participated in all eight swims and everyone involved every year honors a cancer victim or survivor. 13 teams joined in this year as well, inspiring enthusiastic competitive fundraising, with *Team College* the winner, with a total of $50,000, closely followed by *Peter's Defeaters*. We are once again extremely grateful to event Chair Jacque Lang, Honorary Co-Chairs Cindy and John Sites, Mary Henry and Howard Rubin, Arlene and Reuben Mark, Donna deVarona and John Pinto, and Richards of Greenwich.

JUNE 27 2015

Save the Date for Next Year's Swim

RALLY *for* MEDICAL RESEARCH

ACGT is one of more than 100 partner organizations rallying at **Capitol Hill, September 18th** to call on our nation's policymakers to make funding for the National Institutes of Health (NIH) a federal priority and pay greater attention to the importance of medical research. You too can rally for the cause at any time. Tell your Congressional Representative that we cannot skimp on funding research.

STAND UP TO CANCER™

ACGT once again joined an impressive coalition of organizations supporting the national Stand Up To Cancer (SU2C) televised fundraising campaign on September 5th. SU2C is an initiative created to accelerate innovative cancer research and move new therapies to patients sooner rather than later. Emmy-winning producer Joel Gallen and Oscar-winning actress Gwyneth Paltrow were once again the moving forces behind the event, with performances by a stellar line-up of top recording artists and celebrities. Proceeds this year will fund fifteen teams of researchers exploring better options to treat a wide range of cancers.

DR. SAVIO WOO STEPS DOWN AS CHAIR OF SCIENTIFIC ADVISORY COUNCIL

Savio Woo, PhD, Professor of Hematology and Oncology at the Tisch Cancer Institute, Mount Sinai School of Medicine, NY, will step down at year-end from his post as founding Chairman of the ACGT Scientific Advisory Council. Dr. Woo was referred to Edward and Barbara Netter, ACGT Co-Founders, when they determined to discover better treatment options in the face of their daughter-in-law's death from breast cancer. Edward attended a lecture at Mt. Sinai on gene therapy and was immediately struck by the potential for molecular medicine. This was the beginning of a great friendship and the inception of ACGT. It was Dr. Woo who spearheaded the establishment of the peer review grants program and metrics for measuring progress, and he gathered together some of the most renowned scientists in the field to serve on the Council. Dr. Woo recently reminisced on the many highlights of his 13-year tenure. "When the first applications came in from Dr. Carl June and Dr. Michel Sadelain we thought, can you get something this complicated into the clinic and develop a drug that is more or less personalized?

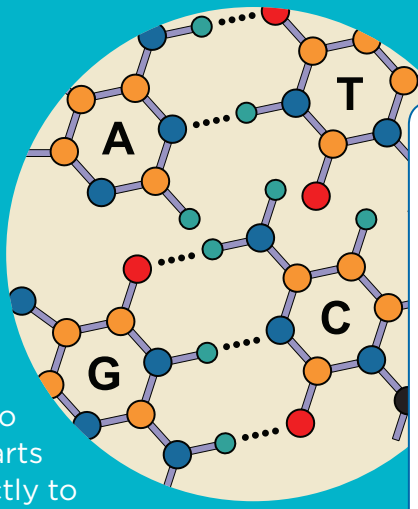


Back in those days, when Edward and Barbara started ACGT, we tried to go talk to the pharmaceutical industry to support gene therapy as the technology with enormous promise. Nobody wanted to talk to us. Gene therapy? Nice seeing you. In this last twelve months, $750 million in private money has been invested in gene therapy. This is a real sea change in terms of where we are today versus where we were before. Bodes well for the future.

– Dr. Savio Woo, Chair,
ACGT Scientific Advisory Council

And in those days, personalized was a dirty word. Today it is THE word. In the end, we said, you know, if we don't support innovative and risky projects why are we here? Risk taking and innovative groundbreaking research, that's what made ACGT so important from the beginning."

WELCOME TO THE WORLD OF MOLECULAR MEDICINE



Revolutionary science and modern technology is making it possible to decipher the genetic code, reset genes, awaken the immune system, shut down tumor life-support and deploy the body's own systems to reject cancer. Cell and gene-based therapies are targeted therapies, utilizing a patient's own cells to battle cancer, without damage to other parts of the body. Drugs can be delivered directly to the tumor to cause cancer cell death. Genes can be extracted, remodeled and returned to the body to awaken the immune system to destroy cancer cells and cancer stem cells, believed to be the root of many cancers. It is possible to strangulate a tumor by cutting off its blood supply. In the not so distant future, an inoculation may make it possible to reject cancers.

Many thanks to retiring Board members...

Edward E. Hartline, Esq, Oaks, Hartline & Daly, LLP, Houston, TX, and Mike Moen, CEO, Drive Power, LLC, St. Paul, MN, for your dedication to ACGT during your years of service and for your visionary support for molecular medicine.

A NEW GENERATION OF CANCER WARRIORS ON BOARD

Alexandra Landes, 28, of Pacific Palisades, CA, joins David M. Darst, 32, NYC, as the newest members of our Board of Directors. Both younger generation directors lost their mothers to cancer and both bring to ACGT a powerful blend of business acumen with a determination to put an end to cancer in the near future. Both also believe in the potential of cell and gene therapies as the new paradigm, targeted treatments that are proving effective with few if any side effects. David graduated Harvard Business School and Harvard College and has held several positions with biotechnology companies, and he currently serves as Chief Executive Officer of Rgenix, leading its cancer drug development. David is also a member of the Rockefeller University Council and co-founder and Director of Potentia Pharmaceuticals. Alexandra is one of the founders and is chief executive of Wendy Walk, a non-profit dedicated to raising funds and awareness for rare sarcomas. Named for her late mother, the organization in its first three years has raised $2 million to combat Liposarcoma. A graduate of Tufts University, Alexandra previously served in development and outreach roles with Bill de Blasio, New York City Public Advocate, the Met Council, and Seeds of Peace.

"ACGT's goal to make cancer a treatable, manageable disease is so compelling and so important. Our current options for battling this disease take a distressing toll on patients and it is encouraging to know less harrowing methods are in the pipeline."

– Alexandra Landes





"I was impressed by the quality of the science funded by ACGT and its positive impact on patients' lives. This is an organization I know well as my mother, Diane W. Darst, was a member of the Board. I look forward to continuing to help fund promising research by innovative young investigators."

– David M. Darst



"With the dearth of public funding, the importance of private philanthropy to support cancer research has never been more acute, and Josh is dedicated to expanding this funding stream. He has the experience and dedication to connect with donors keen to support the world's best cancer scientists and researchers. From the first, ACGT has benefited from the support and conviction of philanthropists and we look forward to continuing these crucial relationships."

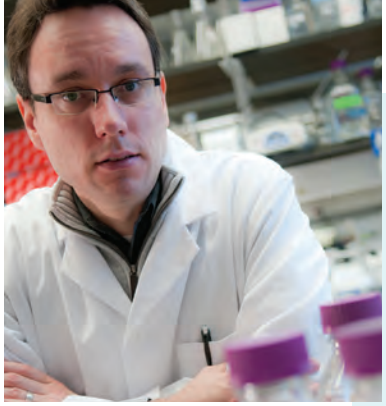
– Barbara Netter, Co-Founder and President of ACGT

JOSHUA CORDAY JOINS ACGT AS SENIOR DEVELOPMENT OFFICER

We are pleased to welcome Josh Corday to the leadership team. Josh is a veteran fundraising professional, with a fifteen-year history collaborating with donors and securing major gifts, as well as communications and event planning. Josh has served as Annual Giving Director for New York Hospital/Columbia University Medical Center, and previously held development positions with Rockland Family Shelter, United Ways of Essex and West Hudson, and Special Olympics NY. He earned a BS in Political Science at University of Utah.

PROGRESS REPORT

FIGHTING BRAIN CANCER



Malignant brain tumors are among the most deadly and despite advances in chemotherapy and surgical options, the worst of these, known as Glioblastoma (GBM), has one of the poorest survival rates, with an average prognosis of 14 - 16 months. An elusive and formidable receptor known as RTK, found in and around these tumors, has thus far made it impossible to penetrate and destroy cancer cells. However, Alexander H. Stegh, PhD, Assistant Professor at Northwestern University, the recipient of the ACGT 2012 Young Investigator grant, is half-way through a three year research project to explore treatment alternatives that break through these barriers. He has uncovered a natural survival protein (IDH1) that may be stimulated to counteract the blockade and in the first phase of the research has learned more about how IDH1 works. Going forward, Dr. Stegh and his team will complete molecular analysis of the protein and proceed to studies using laboratory mice. The ACGT grant has also made it possible for Dr. Stegh to win additional awards of almost $1 million from the American Cancer Society and the John McNicholas Foundation.

> *"An incomplete understanding of how catalogued genetic aberrations dictate intense therapy (apoptosis) resistance, combined with the lack of effective delivery of drugs bound for the central nervous system, have conspired to make GBM an enigmatic and incurable disease. We have sought to address these challenges, and have identified isocitrate dehyrdrogenase 1 (IDH1) as a critical oncogenic factor."*
>
> – Dr. Alexander H. Stegh, Northwestern University


THE CHRONICLE OF PHILANTHROPY

FACE OF PHILANTHROPY

Foundation Supports Cutting-Edge Findings for Cancer Treatment

IN THE NEWS

"Foundation Supports Cutting-Edge Findings for Cancer Treatment" in the August 4th *Chronicle of Philanthropy* featured ACGT as a driving force for revolutionary medicine and the best scientific research in the field.



RESEARCH FELLOW WINS SPECIAL AWARD

ACGT 2013 Young Investigator Dr. Fan Yang, who is developing new gene therapy treatments for pediatric brain tumors, is one of six researchers appointed a Faculty Scholar from the Child Health Research Institute at Stanford University where she is an Assistant Professor. The award pro-motes research involving the health of children.

LEAVE A LEGACY TO DEFEAT CANCER

What better way to help end cancer now and in the future than to stipulate in your estate plan that you wish to support ACGT research pioneers? Your children and grandchildren will be the beneficiaries of the breakthrough therapies being researched by ACGT investigators. Through Legacy Giving, you can help inspire a new generation of scientists and ensure the fulfillment of the mission established by Edward and Barbara Netter nearly fifteen years ago. You may designate gifts of cash, securities or annuities in your will, or establish a charitable trust. Gifts will reduce your estate tax and all donations go directly to cancer research.

Consult with your Estate Planner or contact Josh Corday, Senior Development Officer, at Development Officer, at 203.358.5055 for additional information.

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ACGT ALLIANCE FOR CANCER GENE THERAPY
Discovering new ways to treat and defeat cancer

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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.

100% OF DONATIONS go directly to research. Separate funding is provided to cover all administrative and fundraising expenses.

LEARN MORE AT www.acgtfoundation.org

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APRIL 14 2015

SAVE THE DATE

GALA HONORING DR. SAVIO WOO

Supporters, scientists, and ACGT leadership will celebrate Dr. Savio Woo's contributions to cell and gene therapies April 14, 2015 at the Harvard Club in New York City. Corporate and individual sponsorships and tables are available now. Contact Josh Corday, Senior Development Officer, at 203.358.5055.