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

Sequence

THE NEWSLETTER OF ALLIANCE FOR CANCER GENE THERAPY SPRING 2015



Immunotherapy recipient and cancer survivor, Emily Whitehead, (center) with her parents, Tom and Kari Whitehead



Barbara Netter presents Dr. Savio L.C. Woo with the *Innovative Leadership Award*



Immunotherapy recipient and cancer survivor, Bob Levis, (right) with his wife, Susan, and their sons, Jay and Adam

INNOVATIVE LEADERSHIP GALA SALUTES DR. WOO



"Savio wisely steered the Council to focus on young investigators and early clinical translation, which has resulted in multiple breakthroughs in cancer cell and gene therapy. Moving forward, we will focus more aggressively on later-stage research and trials, moving the science closer to application, which is very exciting."

– Joseph Glorioso, PhD,
University of Pittsburgh
Chairman, ACGT Scientific
Advisory Council

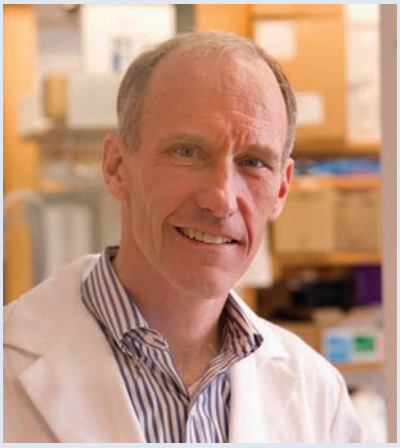
An enthusiastic crowd of scientists, patients, donors and friends celebrated the first *Innovative Leadership Award Gala* April 14 at the Harvard Club of New York City honoring Dr. Savio L.C. Woo, retiring Founding Chairman, now Chairman Emeritus, of the ACGT Scientific Advisory Council. Dr. Woo also recently retired as Founding Chair of the Department of Cell and Gene Medicine at Mt. Sinai School of Medicine, and professor of Hematology and Oncology at the prestigious Tisch Cancer Institute. The event raised over $750,000 for innovative cell and gene therapies for cancer.

Dr. Woo and ACGT co-founders Barbara and Edward Netter agreed nearly fifteen years ago to establish an organization devoted to the research and progress of cancer gene therapies, still in their infancy as medicine and proving rapidly to have the potential to revolutionize cancer care. The role of the Scientific Advisory Council has been integral to the foundation's mission to foster and fund the highest level of research, incorporating peer review and monitoring to ensure validity and reliability.

The gala featured two patients who survived because of clinical trials based on research first funded by ACGT. Tom Whitehead expressed his gratitude for the successful immunotherapy treatment that saved his daughter. Emily, who will turn 10 in May, was diagnosed with acute lymphoblastic leukemia [ALL] just after her 5th birthday. The Whiteheads were told at that time the cancer was the curable form, and after treatment, Emily enjoyed a 16-month remission; however, the relapse was severe, with "cancer spreading out of control." They were referred by the Children's Hospital in Philadelphia to (Continued on Page 3)

BREAKTHROUGHS AND NEWS

Interim results of a trial funded by the **2008 ACGT Joan Miller and Linda Bernstein Ovarian Cancer Grant** awarded to **Dr. Carl June**, University of Pennsylvania, were presented at the April annual meeting of the American Association for Cancer Research [AACR]. Patients in the Phase I trial at the Perelman School of Medicine had ceased responding to traditional therapies. They received genetically modified immune cells that successfully attacked pancreatic or ovarian cancers without adverse side effects. Additionally, the killer T-cells survived in the blood stream for up to 28 days. Patients will be studied for 15 years to monitor health and survival.

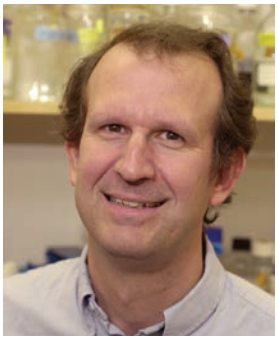


Dr. Carl June

Pharmaceutical giant Novartis announced intensified efforts to develop cancer drugs to harness the body`s immune system. The company entered into a multiyear alliance with Aduro Biotech to promote the discovery and development of next generation immunotherapies targeting the STING (Stimulator of Interferon Genes) pathway and launched a new immuno-oncology research group led by renowned cancer vaccine expert **Dr. Glenn Dranoff**, a 2010 ACGT Research Fellow.

Ken Burns' documentary on PBS based on the Pulitzer-Prize winning book, *The Emperor of All Maladies,* by Dr. Siddhartha Mukherjee, featured a breakthrough study conducted by ACGT Fellow and Scientific Advisory Council member **Dr. Carl June**, University of Pennsylvania, that yielded widespread remission in lymphoma/leukemia patients. Dr. June, who has earned many awards for his research, is the 2015 recipient of the Hubert J.P. Schoemaker Leadership award recognizing exemplary innovation by a member of the Pennsylvania life sciences community.





Dr. Glenn Dranoff

Dr. Harold Varmus, Nobel laureate and former director of the National Cancer Institute of the NIH, has taken on the future of targeted therapies at the New York Genome Center at Weill Cornell Medical Center. The center is one of several sprouting across the country in response to research outcomes and learning, and President Obama requested $215 million in his 2016 budget to launch a Precision Medicine Initiative to focus on genetically-based treatments.



Dr. Lieping Chen

Dr. Lieping Chen, founding member of our Scientific Advisory Council and the United Technologies Corporation Professor in Cancer Research at Yale University School of Medicine, is advancing treatments that deploy a new generation of agents known as PD-inhibitors, antibodies that block the pathway that prevents the immune system from destroying or protecting against cancer. This science is the basis for the drug Keytruda, which received FDA approval last September for use with advanced melanoma, and also Nivolumab, approved last July in Japan, which has successfully treated kidney and lung cancers.

Seeing Emily and Bob on stage brought tears to my eyes and made all the hard work in getting us from where we were to where we are now seem insignificant!

– Dr. Savio L.C. Woo
Chairman Emeritus,
ACGT Scientific
Advisory Council



Dr. Savio L.C. Woo, Barbara Netter, Dr. Joseph Glorioso, Emily Whitehead, Dr. Carl June and Bob Levis



ACGT Scientific Advisory Council member, Dr. Stephen Eck and ACGT Executive Director, Margaret Cianci

GALA (Continued from page 1)

Dr. Carl June's trial at the University of Pennsylvania, and after six weeks in isolation and a brief period of treatment, Emily woke up on her 7th birthday cancer free.

Bob Levis described his 10-year journey with chronic lymphocytic leukemia (CLL). After exhausting all treatments, he was advised to prepare for his death. Salvation came with eligibility for another study led by Dr. June. That trial used an HIV virus as the vector for life-saving T-cells, which multiplied dramatically after just one injection to tackle the disease. The only side effect was flu-like symptoms, which dissipated quickly, and Levis has been healthy and cancer free since 2012.

Earlier that day, members of ACGT's Scientific Advisory Council gathered together at a symposium to share the latest findings on cell and gene therapy research and to define the organization's strategic scientific direction. It was agreed that based on ACGT's tremendous track record, the focus will continue to be innovative basic cell and gene therapy research for cancer and clinical translation, including combination therapies, as well as seeking collaborations with biotechs and pharma. Breakthrough research and pioneering medical science have been and will continue to be the hallmark of ACGT's mission.

MAKE WAVES TO FIGHT CANCER: JUNE 27

SWIM ACROSS AMERICA
MAKING WAVES TO FIGHT CANCER



Greenwich-Stamford SAA Co-Chairs, Lorrie Lorenz and Michele Graham. Photo by ChiChi Ubiña

Swimmers, boaters, volunteers and spectators will gather for the 9th year on Saturday, June 27th to raise funds for cell and gene therapy research, and honor cancer victims and survivors. This year, the Greenwich-Stamford *Swim Across America* for ACGT promises an even greater splash with the heart-felt leadership of event co-chairs **Michele Graham** and **Lorrie Lorenz**, both mothers of teenage cancer survivors. Graham's daughter Nicole was the kick-off speaker at last year's event, and the Lorenz family has participated in the event for six years, since daughter Brooke survived lymphoma. Both girls are cancer free and eager to help others win their fight. Locals **Cindy** and **John Sites,** Olympic swimmer **Donna DeVarona** and her husband **John Pinto, Arlene** and **Reuben Mark, Mary Henry** and **Howard Rubin** will serve once again as honorary chairs. Last year, several hundred swimmers, kayakers and boaters made waves on the day of the swim across Long Island Sound as well as at swim clubs and private pools throughout the area. *Swim Across America,* founded in 1987 by Connecticut resident **Matt Vossler** and his college roommate **Jeff Keith**, organizes swimming events throughout the country to promote research, awareness and treatment for cancer. The first eight local events for ACGT raised $2.4 million for cancer cell and gene therapy research. For more about the swim, go to our events page at www.acgtfoundation.org.



Dr. Chris Niekrash, Dr. Bruce Koeppen, Judy Lahey, ACGT Board of Directors' members, Dr. John Lahey and Martin Winter

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Separate funding is provided to cover all administrative and fundraising expenses.

LEADERSHIP TIMES TWO: AYA JAKOBOVITS, PHD



Dr. Aya Jakobovits joins both the Board of Directors and the Scientific Advisory Council, bringing her scientific and corporate leadership experience to ACGT's governance and research. Dr. Jakobovits is a highly accomplished biotech executive, and a long-standing promoter of scientific innovation. Currently a Venture Partner with Orbimed Advisors, LLC., a healthcare-dedicated venture capital firm, Dr. Jakobovits was the President and founding CEO at Kite Pharma, Inc. and held executive positions at Agensys, Inc., a subsidiary of Astellas Pharma Inc., where she led research, clinical and corporate development activities. Prior to Agensys, she served as a director at Abgenix, Inc. and Cell Genesys, Inc., where she led the development of human antibody XenoMouse® technology. She earned a PhD in life sciences and MS.C in Chemistry at the Weizmann Institute of Science, Rehovot, Israel. Dr. Jakobovits also serves on the boards of cCAm Biotherapeutics Ltd. and Applied Immune Technologies, Ltd.



Meenakshi Hegde, MD

2015 YOUNG INVESTIGATOR GRANTS

Meenakshi Hegde, MD, Assistant Professor, Department of Pediatric Hematology, Texas Children's Cancer Center at Baylor College of Medicine, Houston, is researching immune-mediated treatments for melanoma. Melanoma has been steadily increasing in the last decade with over 76,000 new cases last year alone, and despite improvements in diagnosis and treatment in early stages, patients with late stage, metastatic disease have less than a 10% prognosis for survival. Dr. Hegde is aiming her study on the use of antigens, cancer cells that express tumor-specific molecules, which can be used to develop targeted therapies to attack the cancer without affecting healthy cells. The funded research explores two specific antigens: HER2 and GD2, and hopes to advance findings toward clinical trials. Dr. Hegde earned an MD at Rajiv Gandhi University of Health Sciences, India, served as pediatric resident at University of Illinois, Chicago, and was a post-doctoral fellow in pediatric hematology and oncology at Baylor College of Medicine, Houston, TX.



Christopher Jewell, PhD

"With ACGT support, we are using a multidisciplinary tool-kit from immunology, materials science, and bioengineering to study a new approach to DNA vaccination that will hopefully develop T-cells in a way that arm the immune system to combat relapse. I am so grateful for this transformative investment in breakthrough research."

– Dr. Christopher Jewell, University of Maryland, ACGT 2015 Research Fellow

Christopher Jewell, PhD, Assistant Professor, Department of Bioengineering, University of Maryland, College Park, is also researching immunology, with a focus on harnessing lymph nodes as therapeutic agents. The goal of this study is to engineer the lymph nodes, tissues that control the immune response, using controlled release vaccines with DNA-encoded molecules commonly found on cancer cells. In this way, the treatment can destroy tumors and establish long-lasting tumor resistance, becoming the basis for a new class of cancer vaccines. Dr. Jewell earned an MS and PhD in Chemical Engineering at University of Wisconsin, as well as a BS at Lehigh University, PA. He also held post-doctorate positions at MIT and Harvard University.

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.

The 2015 grant awards bring the total research funding since inception to...

over $25 million

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ACGT PRESIDENT WINS PRESTIGIOUS AWARD



photo by Jessica Lyon

Barbara Netter accepts the John H. Filer Award

Barbara Netter, President and Co-Founder of Alliance for Cancer Gene Therapy, has been awarded the John H. Filer Award from the Connecticut Council on Philanthropy, an honor bestowed annually on individuals who have made a significant impact on the public good statewide. Netter has a long history of philanthropy. She serves on the Board of the Barbara and Edward Netter Center for Community Partnerships at the University of Pennsylvania. Netter has been a facilitator for many years with the Family Centers' Den for Grieving Kids in Greenwich, CT. In 2011, Netter received an honorary doctorate of humane letters from Quinnipiac University, and in 2014 received the Ray of Hope award from Family Centers as well as the Distinguished Service Award from the American Society of Gene and Cell Therapies. In a professional capacity, she served for 20 years as a staff psychotherapist at Pelham Family Services, Pelham, NY. In her continuing leadership position with ACGT, Netter preserves the foundation's legacy as a tireless proponent for accelerated cancer research.