

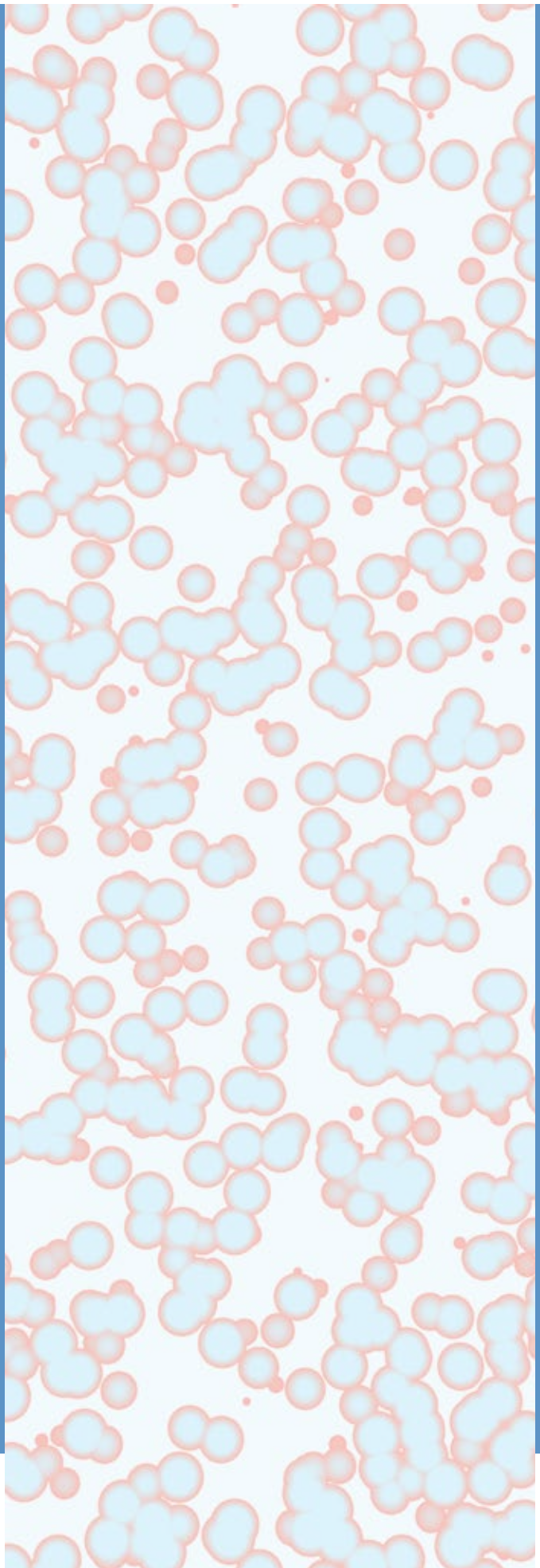
ACGT®

ALLIANCE FOR
CANCER GENE THERAPY

2014

Breakthroughs

Treating and Defeating Cancer



ALLIANCE FOR CANCER GENE THERAPY is the only charitable organization in the nation dedicated exclusively to funding and fostering research into molecular medicine for cancers.

OUR MISSION is to seek out and support revolutionary scientific research into the causes, treatment and prevention of all types of cancer using cells and genes as medicine.

TO THIS END we fund and monitor studies and trials that meet the highest possible standards, educate the public on the potential, and facilitate greater collaboration among the scientific and philanthropic communities to promote progress.

ACGT® ALLIANCE FOR CANCER GENE THERAPY

Discovering new ways to treat and defeat cancer



Since the beginning nearly fifteen years ago, our organization has steadfastly stepped into the future of molecular medicine.

Dear friends,

More than ever, the door is open to a new realm for medical science. Reports of progress inspire us every day and the world has taken note. Our research fellows are proving the promise of cell and gene therapies, pharmaceutical companies are forging partnerships with research institutions and devoting greater capital to bringing innovations to the marketplace and, most importantly, thousands of patients have participated in clinical trials with many lives extended or saved.

We are particularly excited that the FDA has designated a gene therapy for cancer with breakthrough status, which will further accelerate progress. The media reports regularly on groundbreaking treatments and genetic medicines. ACGT has new board members, new management staff, new collaborators, and new Research Fellows destined to revolutionize treatments for some of the deadliest cancers.

Since the beginning nearly fifteen years ago, our organization has steadfastly stepped into the future of molecular medicine. Now we march, with great optimism, and we are more determined than ever to expedite progress.

This year, we rallied on Capitol Hill for public funding. We benefited from the eighth annual *Swim Across America* fundraiser and joined with *Stand Up To Cancer.* We rededicated our mission, revamped our website, allocated resources to more effective social media, awarded three major grants, and claimed a new tag line to define our goal: *Discovering new ways to treat and defeat cancer.*

Our mentor and guide, Dr. Savio Woo, will step down next year as Founding Chair of the Scientific Advisory Council, at the same time he retires from academia. Savio has been integral to our work from day one and the Council is the embodiment of ACGT's resolve to seek out and support the revolutionary science of cell and gene therapies for cancer. We are forever grateful for his singular contributions.

None of this would be possible without you: donors, volunteers, scientists. As tremendous as this year has been, I expect next year will be even better. Join us for a healthier future.

Thank you.

Barbara Netter

Barbara Netter
Co-Founder and President

BREAKTHROUGHS: REVOLUTIONARY RESEARCH



ACGT LEADER PAVES THE WAY

Scientific Advisory Council member, Lieping Chen, MD, PhD, at Yale Cancer Center, received the 2014 William B. Coley award for breakthrough cancer research. Dr. Chen has been studying an experimental drug used to disable the shield that repels the immune system, which has resulted in dramatic shrinkage of lung, skin and kidney cancers that had previously defied treatment. Drug company Merck received the go-ahead to market the first of this class of drug for advanced melanoma. The treatment blocks a protein called PD-1 (Programmed Death 1) first identified in 1992 in Japan and one of the reasons the immune system fails to attack cancer.

ACGT FELLOW LEADS THE CHARGE

Scientists at Massachusetts General Hospital, led by ACGT Fellow Khalid Shah, PhD, MSc, Harvard Medical School, have successfully applied cancer-killing viruses to glioblastoma brain tumors in laboratory study. Dr. Shah, recipient of the 2007 *Swim Across America* Young Investigator award, has made considerable progress since his early laboratory research using neural stem cells to deliver therapeutic drugs to stop cancer stem cells from proliferating.



Khalid Shah, PhD, MSc

Photo by Deepak Bhere

2014 GRANTS FORGE NEW GROUND

Arnob Banerjee, MD, PhD, University of Maryland School of Medicine, was awarded a Young Investigator grant for research into immunotherapy. Dr. Banerjee will use the grant to study methods to optimize immunology primarily for blood cancers. Immune-mediated therapies are the most advanced form of gene therapy, activating the body's natural defenses to reject and/or eradicate tumor cells. This study aims to defuse obstacles to treatment by opening T-cell pathways. The grant will finance further development of his laboratory research program to understand and optimize the processes governing T-cell differentiation during anti-cancer immune response. Dr. Banerjee earned his MD/PhD at Columbia University and was a resident, fellow and instructor at the University of Pennsylvania.

Fan Yang, PhD, Assistant Professor of Medicine at Stanford University School of Medicine, will focus her grant on research into non-viral vectors for pediatric brain cancer. These tumors are the leading fatality of childhood cancers because they are highly invasive, penetrating the delicate brain stem and making them especially difficult to treat. The study will deploy stem cells targeted to the tumor in conjunction with chemotherapy. Funding



Lieping Chen, MD, PhD

Photo by Peter Baker



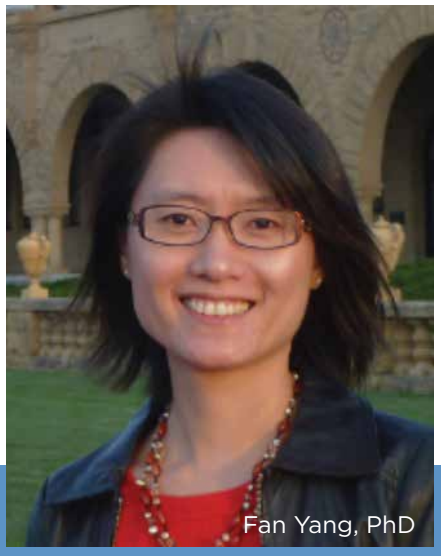
John Bell, PhD

Photo courtesy of *Ottawa Life Magazine*. Photo by Mark Holleron



Arnob Banerjee, MD, PhD

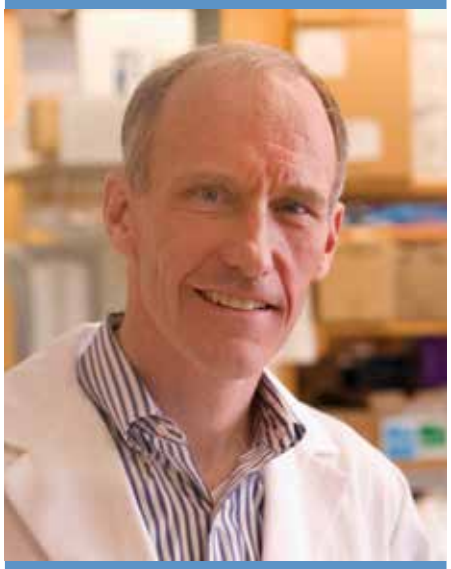
Photo by Kavitha Sadashivaiah



Fan Yang, PhD

will support proposed collaborative work to develop innovative solutions for advanced care. Yang earned a PhD and completed her residency at Johns Hopkins University and was a post-doctoral fellow at MIT.

John Bell, PhD, Senior Research Scientist and Professor of Medicine at Ottawa Hospital Research Institute, Canada, will focus his Investigator grant on the use of oncolytic viruses (OV) in the treatment of glioblastomas, brain cancers among the most difficult to treat. OV have proven effective in late-stage clinical trials for other forms of cancer and these viruses carry genes able to selectively replicate and kill tumor cells without harm to normal cells. However, the treatment has been less safe for the brain and more difficult to implement on a large scale. Dr. Bell and his team have discovered a new variant, a Chimeric Maraba virus, less toxic and more effective in rodent models with human glioblastoma, which has the potential to treat primary, metastatic and recurrent brain cancer. The grant will enable the design of a GMP-manufacturing process of this virus and the initiation of a Phase I human trial to establish safety and efficacy. Dr. Bell earned a BS and PhD in biology at McMaster University, Ontario, Canada.



"It's a cellular war: T-cells become 'hunter' cells, attacking one tumor cell after another."

– Carl June, MD, Abramson Cancer Center, University of Pennsylvania

BREAKTHROUGHS: SAVING LIVES

SAVING MORE LIVES SOONER

The FDA named a revolutionary gene-based treatment, seeded with early funding by ACGT, as a "Breakthrough Therapy" paving the way to an expedited approvals process.

Translational research at the University of Pennsylvania, spear-headed by ACGT Research Fellow Dr. Carl June, proved exceptionally effective in attacking acute lymphoblastic leukemia (ALL). In early stage clinical trials at the Abramson Cancer Center, 89 percent of patients who had not responded to conventional therapies went into complete remission after receiving the drug that stimulates an immune response that targets the cancer, without toxicity to other parts of the body. This is the first academic center and the first targeted cell treatment for cancer to achieve the prestigious designation.

To qualify for breakthrough status, the treatment, on its own or in combination with other therapies, must demonstrate potentially life-saving results. We fully expect other cell and gene therapies currently in or moving towards clinical translation will earn the same designation, accelerating progress from the bench to bedside. More and more, treatments like these will fulfill the possibilities of molecular medicine and, once on a faster track to commercial development, will treat many more patients and save many more lives sooner.





By January 2013, he was living from one blood transfusion to the next...



Within two months of the trial, he said he felt like himself again and he is back on the golf course.

BACK ON PAR

In 2012, Bob Levis, an Allentown, Pennsylvania resident, believed he had come to the end of his life. Diagnosed in 2002 with chronic lymphocytic leukemia, the cancer had resisted every possible treatment and had infiltrated his bone marrow, paralyzing his immune system and stifling the production of life-saving blood cells. By January 2013, he was living from one blood transfusion to the next with hardly the energy to breathe and was denied further treatment at a drug trial because his blood counts were so low.

After a long painful journey, he had exhausted all options, so he thought. No more bicycling, no golf, and little time left with family.

Salvation came in response to a plea to Dr. Carl June at the University of Pennsylvania, whose life-saving immunotherapy treatment, based on research first funded by ACGT, had made headlines throughout the world. Dr. June was inundated with requests and his trial was reserved for patients who had no options and no hope. Levis' blood markers confirmed that he was a perfect candidate for the revolutionary trial and he was enrolled as patient number 15.

The trial used a disabled version of an HIV virus as the vector for patients' own genes to turn on the immune system. After just one injection, 'hunter' T-cells multiplied dramatically. The only side effect was flu-like symptoms that depleted his already weakened frame, but he rallied quickly and, miraculously, Bob Levis was free of cancer. Within two months of the trial, he said he felt like himself again and he is back on the golf course. He returns to the lab three times a year for blood work and the T-cells produced in the trial continue to stand guard against recurrence.

"My latest biopsy and cat scans are clean, blood work is normal, and I'm back to my life." This is the promise of gene therapy.

HAPPENINGS 2014

STAND UP TO CANCER

ACGT once again joined an impressive coalition of organizations supporting the national *Stand Up To Cancer* prime-time fundraising campaign September 5th, broadcast this year via 31 channels. Since the first, *Stand Up To Cancer* has brought together more than 750 researchers from 112 institutions in six countries, and has launched or completed more than 140 clinical trials involving roughly 5,700 patients. Their mission, which dovetails our own, is to get new therapies to patients quickly and save lives now.



RALLY FOR RESEARCH

ACGT was one of more than 100 partner organizations rallying at Capitol Hill in September to urge the nation's policymakers to make funding for the National Institutes of Health (NIH) a federal priority and pay greater attention to the criticality of medical research. Progress made in recent years has been too valuable to ease the focus on public funding, and private funding continues to be crucial to ensure breakthrough studies.

FOUNDERS RECEIVE SPECIAL AWARD

At the American Society for Gene and Cell Therapy (ASGCT) annual meeting in Washington DC in May, ACGT founders Edward (posthumously) and Barbara Netter received the Distinguished Service Award for launching the only not-for-profit organization solely devoted to cell and gene therapeutics for cancer. It was noted that under the Netters' leadership, ACGT has become a major source of research funding and, by virtue of educational forums and communications, has also raised awareness for cell and gene therapies. 1700 scientists and physicians attended the conference this year and there were many exciting presentations on the progress of molecular medicine. Mrs. Netter also sponsored a New Members reception with subsidies to ten young scientists who would not otherwise have been able to attend the conference and each thanked her for the inspiration as well as generosity.





We are grateful to Swim Across America for its support and for the constant commitment of 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.



Photo by Katie Farro

A NOBLE COMPETITOR

In the eight years of *Swim Across America* for ACGT, more than $2 million has been raised for research. More importantly, many participants have been able to honor their loved ones and their own cancer journey; notably, 18-year old Nicole Graham, who, as an intern for ACGT, organized a swim team to celebrate her personal triumph over cancer. As a three-sport athlete, she knows something of competition; however, she never imagined she would have to compete against such a vicious opponent. Not only did she battle acute lymphocytic leukemia, she had to conquer terrible treatment setbacks, including septic shock, two strokes and the loss of 60% of her muscle mass. In June this year, Nicole graduated with her Greenwich High School class of 2014 and volunteered at the *Swim Across America* Greenwich-Stamford event. Someday, patients like Nicole will be treated for cancer with cell and/or gene therapies without fear of debilitating and traumatic side effects.

ACGT FINANCIAL REPORT
FOR YEARS ENDING APRIL 30, 2014 AND 2013

CONDENSED STATEMENTS OF ACTIVITIES	2014	2013
Support and revenue:		
Contributions	$785,961	$712,207
Contributions for administrative expenses (specially designated)	279,100	146,000
Contributed services	265,789	322,709
Interest and dividend income	87,795	74,468
Realized and unrealized gains on investments, net	216,559	265,637
TOTAL SUPPORT AND REVENUE	1,635,204	1,521,021
Expenses:		
Program services:		
Research grants and awards	794,126	832,668
Management and general	188,233	255,167
Fundraising	56,246	49,876
TOTAL EXPENSES	1,038,605	1,137,711
Increase in total net assets	596,599	383,310
Total net assets at beginning of year	3,386,103	3,002,793
TOTAL NET ASSETS AT END OF YEAR	$3,982,702	$3,386,103

CONDENSED STATEMENTS OF FINANCIAL POSITION	2014	2013
Assets:		
Cash and cash equivalents	471,784	1,004,966
Contributions receivable, net	25,000	25,000
Investments, at fair value	4,227,234	3,388,234
Accounts receivable and prepaid expenses	8,430	26,338
TOTAL ASSETS	$4,732,448	$4,444,538
Liabilities and net assets:		
Liabilities:		
Grants payable	$728,762	$1,025,279
Accounts payable and accrued expenses	20,984	33,156
TOTAL LIABILITIES	749,746	1,058,435
TOTAL NET ASSETS	3,982,702	3,386,103
TOTAL LIABILITIES AND NET ASSETS	$4,732,448	$4,444,538

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





Three people every minute of each day

hear these words: *You have cancer.*

190

people are diagnosed with cancer every hour.

1,600

people die of cancer every day.

14.5 million

Americans alive (1 in 22) have overcome and/or live with cancer.

1 of 4

of us will develop cancer at some point in time.

50%

of new cancers attack those 65 and older.

Source: *National Cancer Institute* (U.S. statistics)

ACGT RESEARCH FELLOWS

Young Investigator grants award $250,000 for discovery research and Investigator grants award $500,000 - $1,000,000 to support translational research.

Applications are peer-reviewed and progress monitored by ACGT's Scientific Advisory Council.

2013-2014

Arnob Banerjee, MD, PhD
Institution: University of Maryland School of Medicine
Focus: Blood Cancer
Research: Immunotherapy
Award: *Swim Across America Award*

Fan Yang, PhD
Institution: Stanford University School of Medicine
Focus: Brain Cancer
Research: Non-viral Vectors
Award: *Swim Across America Award*

John Bell, PhD
Institution: Ottawa Hospital Research Institute
Focus: Brain Cancer
Research: Oncolytic Viruses
Award: *Swim Across America* Award

2012-2013

Douglas Mahoney, PhD
Institution: University of Calgary
Focus: Breast Cancer
Research: Tumor Specific Viruses & Bacteria

Alexander Stegh, PhD
Institution: Northwestern University
Focus: Brain Cancer
Research: Gene/Cell Mediated Therapies

Herbert J. Zeh, III, MD, FACS
Institution: University of Pittsburgh
Focus: Pancreatic Cancer
Research: Immunotherapy

2011

Hui Hu, PhD
Institution: University of Alabama
Focus: Ovarian Cancer
Research: Immunotherapy

2010

Nabil Ahmed, MD, MPH
Institution: Baylor College of Medicine
Focus: Brain Cancer
Research: Stem Cell Directed Therapy

Glenn Dranoff, MD
Institution: Dana Farber/Harvard Cancer Center
Focus: Leukemia
Research: Immunotherapy

Thomas Kipps, MD, PhD
Institution: Moores Cancer Center, University of California, San Diego
Focus: Lymphoma/Leukemia
Research: Immunotherapy
Award: *Swim Across America* Award

Michael Z. Lin, MD, PhD
Institution: Stanford University
Focus: Brain & Breast Cancer
Research: Tumor Specific Replicating Viruses & Bacteria

2009

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

2008

Carl June, MD
Institution: University of Pennsylvania Abramson Cancer Center
Focus: Ovarian Cancer
Research: Immune-mediated Gene Therapy
Award: The Joan Miller & Linda Bernstein Ovarian Cancer Award

2007

Antonio E. Chiocca, MD, PhD
Institution: Harvard Medical School
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, MSc
Institution: Harvard Medical School, Massachusetts General Hospital
Focus: Brain Cancer
Research: Tumor Targeting
Award: *Swim Across America* 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: The Dr. Judah Folkman Anti-angiogenesis Award

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: Multiple 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

2005

Biao He, PhD
Institution: University of Georgia
Focus: Breast Cancer
Research: Tumor Specific Replicating Viruses & Bacteria

Thomas J. Kipps, MD, PhD
Institution: Moores Cancer Center, University of California, San Diego
Focus: Lymphoma/Leukemia
Research: Immune-mediated Gene Therapy
Award: *Swim Across America* Award

Hyam I. Levitsky, MD
Institution: Johns Hopkins University School of Medicine
Focus: Lymphoma/Leukemia
Research: Immune-mediated Gene Therapy

Miguel Sena-Esteves, PhD
Institution: University of Massachusetts Medical School
Focus: Brain Cancer
Research: Oncogenesis

Koji Tamada, MD, PhD
Institution: University of Maryland
Focus: Lymphatic Systems
Research: Immune-mediated Gene Therapy

Yiping Yang, MD, PhD
Institution: Duke University Medical Center
Focus: Lymphoma/Leukemia
Research: Immunotherapy

Xianzheng Zhou, MD, PhD
Institution: New York Medical College
Focus: Blood, Lymphoma/ Leukemia
Research: Immune-mediated Gene Therapy

2004

Chien-Fu Hung, PhD
Institution: Johns Hopkins University School of Medicine
Focus: Ovarian Cancer
Research: Immunotherapy

Mukesh Jain, MD, FAHA
Institution: Case Western Reserve University
Focus: Metastatic Cancer
Research: Anti-angiogenesis

Carl H. June, MD
Institution: University of Pennsylvania Abramson Cancer Center
Focus: Lymphoma/Leukemia
Research: Immune-mediated Gene Therapy

Suzie Pun, PhD
Institution: University of Washington
Focus: Metastatic Cancer
Research: Targeted Non-Viral Delivery
Award: Patricia Zoch Tate Award

Michel Sadelain, MD, PhD
Institution: Memorial Sloan-Kettering Cancer Center
Focus: Lymphoma/Leukemia
Research: Immune-mediated Gene Therapy

Jian Yu, PhD
Institution: University of Pittsburgh School of Medicine, Hillman Cancer Center
Focus: Lung Cancer
Research: Oncogenesis

2003

Laurence Cooper, MD, PhD
Institution: University of Texas, MD Anderson Cancer Center
Focus: Lymphoma/Leukemia
Research: Immune-mediated Gene Therapy

Timothy Lane, PhD
Institution: University of California, Los Angeles
Focus: Breast Cancer
Research: Anti-angiogenesis
Award: The Kimberly Lawrence Netter Breast Cancer Award

Todd R. Reilly, PhD
Institution: Johns Hopkins University (through 2006)
Focus: Breast Cancer
Research: Immune-mediated Gene Therapy

Katherine Ryman, PhD
Institution: University of Pittsburgh, Center for Vaccine Research
Focus: Prostate Cancer
Research: Tumor Specific Replicating Viruses

Robert Vonderheide, MD, DPhil
Institution: University of Pennsylvania Abramson Cancer Center
Focus: Neuroblastoma
Research: Immune-mediated Gene Therapy

2002

Jeffrey S. Bartlett, PhD
Institution: The Research Institute at the National Children's Hospital (through 2012)
Focus: Ovarian Cancer
Research: Vector Development

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

Thomas S. Griffith, PhD
Institution: University of Minnesota
Focus: Prostate Cancer
Research: Immune-mediated Gene Therapy

WE ARE GRATEFUL TO ALL OF OUR SUPPORTERS

Your gifts have been crucial to funding the science of cell and gene therapies for cancer. Incredible progress has been made, and with your continued support, there is more to come.

THANK YOU.

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In memory of B.J. Abney

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In honor of Anna Stern

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In memory of Inge L. & Heinz Annus

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Mr. & Mrs. Alan Englander

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Mr. Christopher Meyering

Mr. John Miller
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Cliff & Joyce Millian
In memory of Dick Ryczek

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- decipher an individual's biological code
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- Cancer stem cells, believed to be the root of many cancers, can be destroyed using targeted therapeutics.
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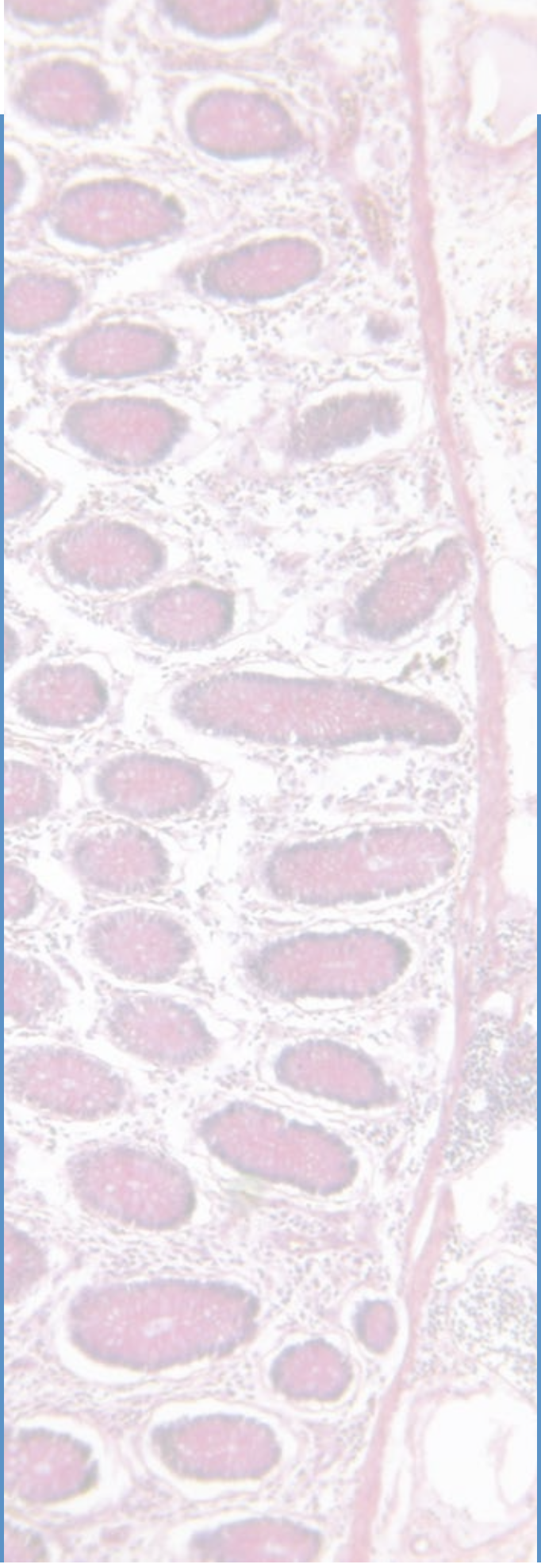
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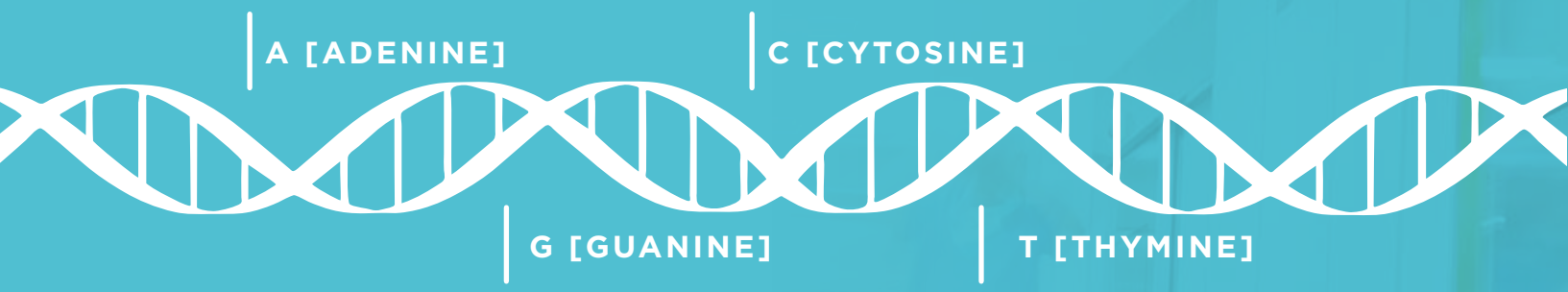
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