CR&T News
Cancer Research & Treatment Fund, Inc.
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IMMUNOTHERAPY: THE NEXT GENERATION OF CANCER TREATMENTS

This revolutionary new class of treatment "promises to transform cancer care."

– American Society of Clinical Oncology

In March of 2016, former President Jimmy Carter made a startling announcement: Only months after doctors found that melanoma had spread to his brain, they declared that he was cancer-free. By August, the 91-year-old, who initially believed that he had only "a few weeks left," was in Memphis, helping to build houses as part of an annual Habitat for Humanity work project. Carter's remarkable recovery was attributed to pembrolizumab, an immunotherapy drug, which was combined with radiation and surgery. His is just one of many immunotherapy success stories that have been documented in the scientific literature and the mainstream media. It's no wonder that the American Society of Clinical Oncology (ASCO) has hailed immunotherapy as its 2016 Advance of the Year, stating that this revolutionary new class of treatment "promises to transform cancer care."

Simply put, immunotherapy trains the body's immune system to attack and destroy cancer cells. In 2011, the FDA approved the first immunotherapy drug for melanoma, a deadly cancer that is notoriously tough to treat. A number of other cancer immunotherapies are now on the market, and studies are evaluating promising treatments for solid-tumor cancers (including lung, kidney, liver, bladder, and head and neck cancer), as well as some blood cancers. Clinical trials are also underway to determine the effectiveness of combining immunotherapy with standard cancer treatments.

"Immunotherapy" actually is a broad term that covers several different types of cancer treatments. This article provides an overview of these therapies and the current status of research in this field.

Checkpoint Inhibitors

This treatment blocks the activity of proteins known as checkpoints. As their name suggests, checkpoints normally help to regulate the immune system, preventing it from going into overdrive and destroying healthy tissue. They do this by helping T cells – whose job is to identify and attack harmful invaders – to distinguish between healthy cells and cancerous mutations. But cancer cells can be deceptive. Sometimes they are able to hide behind the checkpoints. As a result, the T cells fail to detect the cancer cells, which continue to grow. Administered intravenously, checkpoint inhibitors "turn off" the checkpoint proteins, enabling the T cells to find and destroy hidden cancer cells.

To date, four checkpoint inhibitors have received FDA approval for use in specific cancers. They are also under study for their potential to treat other cancers.

- *Pembrolizumab (Keytruda)* and nivolumab (Opdivo) block a checkpoint protein called PD1. Both drugs can be used to treat melanoma and non-small cell lung cancer. Pembrolizumab also is approved for head and neck cancer, and nivolumab is approved for renal cell cancer and Hodgkin lymphoma.
- *Atezolizumab (Tecentriq)*, which targets a checkpoint protein called PD-L1, is used to treat bladder cancer.
- *Ipilimumab (Yervoy)* is a melanoma therapy.

Adaptive Cell Therapy (ACT)

Still in the early stages, this experimental treatment uses a patient's own immune cells to fight cancer. In one type of ACT, T cells are collected from the patient's blood, then genetically modified to produce proteins called chimeric antigen receptors (CARs). These specially engineered T cells are now able to recognize a specific antigen on the surface of the cancer cells. After large quantities of the CAR T cells are grown in the laboratory, they are given to the patient by intravenous infusion. Once inside the body, the T cells multiply, then home in on the cancer cells and destroy them.

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ACT has only been studied in a small number of subjects, and more clinical trials must be conducted to see if it is effective in large numbers of patients. So far, however, the study results have been highly promising, especially in people with acute lymphoblastic leukemia (ALL) and lymphoma. Some patients who had not responded to standard treatment have gone into remission, sometimes for years.

Monoclonal Antibodies

Monoclonal antibodies (mAbs) are cancer-fighting molecules that are grown in the laboratory. Over the past 20 years, numerous mAbs have been approved for the treatment of cancer. These antibodies, which bind to cancer cells, can perform many different functions. For example, some mAbs alter the immune response that causes cancerous tumors to grow. Others deliver chemotherapy or radiation directly to a tumor. A recent development is the bispecific monoclonal antibody, which binds to a T cell and a cancer cell at the same time. Now, the T cell should be able to close in on the cancer cell and destroy it. In 2014, the FDA approved blinatumomab (Blincyto) for the treatment of acute lymphoblastic leukemia. Other bispecific mAbs are in development.

Vaccines

Vaccines marshal the body's natural immune defenses to prevent or treat disease. A vaccination introduces your immune system to killed or weakened viruses or bacteria. If you are exposed to the disease in the future, your immune cells will "remember" how to fight the foreign invader.

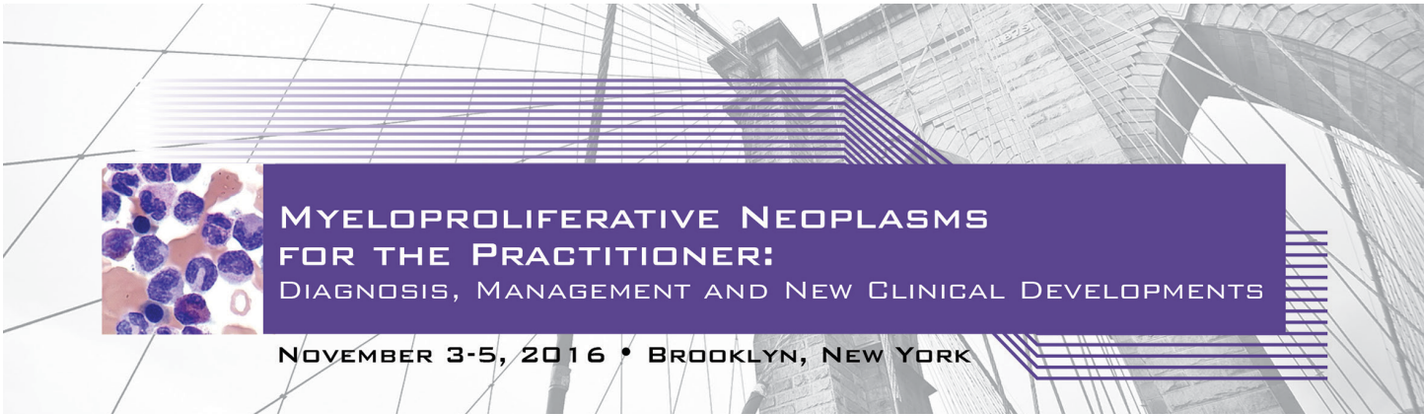
Cancer vaccines are designed to activate "killer T cells," which are programmed to destroy specific cancer cells. Most cancer vaccines are still in development. In 2010, the FDA approved sipuleucel-T (Provenge) for advanced prostate cancer.

Building Healthy Lives

While researchers are making great progress in developing immunotherapy, they caution that much work remains to be done. Although immunotherapy has produced extraordinary results in some critically ill patients who didn't respond to standard treatment, it doesn't work for everybody. In fact, only about 10 to 30 percent of patients respond to immunotherapy. Researchers are not only working to develop new immunotherapies, but to answer crucial questions, including: Why is a given treatment effective in some patients, but not in others? What are the correct dosages? What side effects do these drugs cause, and how can we prevent or minimize them? Beyond the medical questions, key issues revolve around the high cost of developing and producing these drugs and how it will affect health insurance coverage.

Still, there is no question that this new class of therapy will have a major impact on the future of cancer treatment – and on the life expectancy and quality of life of cancer patients. "Now that we have better surgery, less severe and more targeted radiation and chemotherapy, plus immune therapies, we are beginning to attack cancer in a scientific way rather than an empirical way," Dr. Lewis Cantley, the Meyer Director of Weill Cornell Medical College's Meyer Cancer Center, said in a recent interview. "Our goal is to figure out how to combine these therapies for each and every patient so that we can control their disease and help them live a healthy, normal life."

— Barbara Rosenstein



PROFESSIONAL MPN CONFERENCE WELCOMES PATIENTS

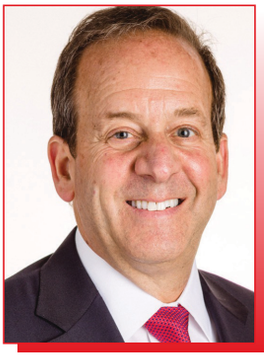
Patients and family members are welcome at this three-day professional conference, which will focus on the practical aspects of the diagnosis and treatment of the myeloproliferative neoplasms (MPNs). The world-class faculty is led by Drs. Richard T. Silver (New York-Presbyterian Weill Cornell Medical Medical College) and Jerry L. Spivak (Johns Hopkins University School of Medicine).

Register today and receive a special patient discount of $50 off the standard rate of $249!
Just select the "Fellow/Resident/PA/Nurse/Other" category when you register online and use the coupon code **MPN50** at checkout. (Hurry – late registration fees go into effect on 10/28!)

Learn more and register at www.mpnforthepractitioner2016.com

New York Marriott at the Brooklyn Bridge • 333 Adams Street, Brooklyn, NY 11201

MESSAGE FROM THE PRESIDENT



Throughout the year, CR&T-funded researchers are racing to find new treatments and cures for cancer. As the end of 2016 approaches, we're also racing to meet our fundraising goal for the year: $850,000, our investment in the future of the Richard T. Silver, MD Myeloproliferative Neoplasm (MPN) Center at Weill Cornell Medical College.

As we reported in the last issue of *CR&T News*, these funds are part of a multi-year, $4.7 million commitment to building the world's leading center dedicated to MPN research and patient care. CR&T's support will enable the Silver Center to:

- Expand the number of basic and clinical studies conducted at the Center;
- Fill critical staff positions, including hiring a Clinical Director; and
- Continue to build the infrastructure needed to accelerate the pace of research, including an extensive patient database and a biorepository of patient specimens.

We want to thank you, our friends, for your very generous, ongoing support of this initiative, which is the heart of CR&T's research strategy. We know that we can count on you to ensure the success of our upcoming events: our annual Cancer Survivors Hall of Fame Dinner (page 5) and our first Young Professionals Halloween Bash (page 4). We also wanted to take this opportunity to mention other ways that you can help CR&T fulfill our mission. As you make your plans for year-end giving, we hope you'll consider the following:

- **Gifts of Securities**: Your gift of appreciated securities – stocks, bonds, or mutual funds – can provide significant tax savings while helping to support CR&T's search for cures.
- **Memorial or Tribute Gifts**: Commemorate a milestone or special occasion, or honor the memory of a friend, loved one or colleague with a gift that will have a lasting impact on the lives of patients and families.
- **Planned Giving**: Help us build a cancer-free future by making CR&T part of your estate plans.
- **Employee Giving**: Many companies offer these programs, which allow you to deduct the amount you specify from your paycheck. Be sure to ask if your employer also offers matching gifts, which will increase the value of your donation.
- **Shop Online**: Give a gift to CR&T every time you shop – without spending another penny! With the holidays approaching, it's a great time to take advantage of the AmazonSmile program. Just name CR&T as your charity of choice, and Amazon will donate 0.5% of the price of your purchases every time you start your shopping at www.smileamazon.com.
- **Donate a Silent Auction Prize**: The Silent Auction is always a highlight of our Hall of Fame Dinner. All of the prizes we offer are donated by our friends and their contacts, ensuring that every penny raised benefits CR&T. Whether it's dinner at a favorite restaurant, theater tickets, an elegant accessory from a local boutique, or a great travel experience – if you're able to secure an auction item, we'd love to hear from you!

As always, we welcome your questions and suggestions about our programs, plans, and giving opportunities. Please don't hesitate to contact Barbara Rosenstein, Director of Administration and Development, at 212-288-6604 or brosenstein@crt.org.

Most important of all, we want to wish you and yours a joyous holiday season, and a very happy and healthy New Year. We look forward to sharing more news and information in our next issue, which will be published early in 2017.

Sincerely,

Thomas M. Silver
President

News Briefs

Members of our Medical Advisory Board (MAB) and Board of Directors have recently been recognized for their achievements or appointed to prestigious positions. **Morton Coleman, MD**, was granted honorary membership by the Weill Cornell Medical College Alumni Association. Each year, this honor goes to two Weill Cornell faculty members and/or clinicians who are not alumni of the Medical College. This special honor recognizes outstanding scholarship, dedication, and leadership. **John Leonard, MD,** was named Corporate Chair of the Leukemia & Lymphoma Society's 2016 Manhattan Light The Night Walk, which took place on October 6. **Andrew Schafer, MD**, Director of the Richard T. Silver MPN Center at Weill Cornell, has been named president-elect of the American Clinical and Climatological Association for 2016-2017. He was also appointed to the scientific advisory council of the Doris Duke Charitable Association. **Tom Silver**, the president of CR&T's Board of Directors, was elected President of the Mu Chapter of the Sigma Pi Educational Foundation at Cornell University. This 501(c)(3) nonprofit organization funds educational programs for members of the Mu Chapter and supports education-related programming that benefits the youth of the greater Ithaca community.

Congratulations to all!

CLINICAL TRIAL RESOURCES

Are you or a loved one thinking about volunteering for a clinical trial? As a study participant, you may benefit from a promising new treatment or receive care from leading cancer specialists. What's more, you'll have the satisfaction of contributing to the progress of cancer research.

While the advantages of enrolling in a trial can be significant, it's vital that you make an informed decision. That means speaking with your doctor, sharing information with your family, and understanding the benefits and risks of any study you're considering. This list of helpful resources will help you get started.

- www.clinicaltrials.gov
  A service of the U.S. National Institutes of Health (NIH), this website is a registry and database of publicly and privately supported clinical studies conducted around the world. You'll also find in-depth information about how clinical trials are conducted, a glossary of terms, and links to valuable resources across the Web.
- National Cancer Institute (www.cancer.gov/about-cancer/treatment/clinical-trials)
  Here, you'll find a database of clinical trials sponsored by the National Cancer Institute, a division of the NIH, along with a wealth of educational information.
- CenterWatch (www.centerwatch.com)
  Founded in 1994, CenterWatch is a trusted source for clinical trials information for both professionals and patients. In addition to educational information, the site offers an extensive database of studies that are enrolling patients.
- ResearchMatch (www.researchmatch.org)

  This free registry connects volunteers with researchers who are searching for appropriate participants for their clinical trials and other kinds of research studies, such as surveys. A collaborative project led by the Vanderbilt Institute for Clinical & Translational Research, ResearchMatch involves a wide network of medical institutions and non profit partners.
- ACT (www.learnaboutclinicaltrials.org)

  Sponsored by Genentech, a pharmaceutical company, in collaboration with the American Cancer Society (ACS), this site includes links to resources like the ACS's clinical trials matching service, along with useful information, videos, patient stories, and materials, such as a discussion guide that you can print out and take with you when you meet with your doctor.

For more helpful information and resources, be sure to visit our website at www.crt.org.

2016 HALLOWEEN BASH!

Hosted by CR&T Young Professionals

JOIN US FOR A GREAT NIGHT AND HELP FIGHT CANCER!

Saturday, October 29
Open Bar and Live DJ
$100 per person

Proper West
54 West 39th Street, New York City

Space is limited • Reservations required!
Dress Code: Costumes
Register online at www.crt.org/2016-Halloween-Bash or contact Barbara Rosenstein at 212-288-6604 or brosenstein@crt.org

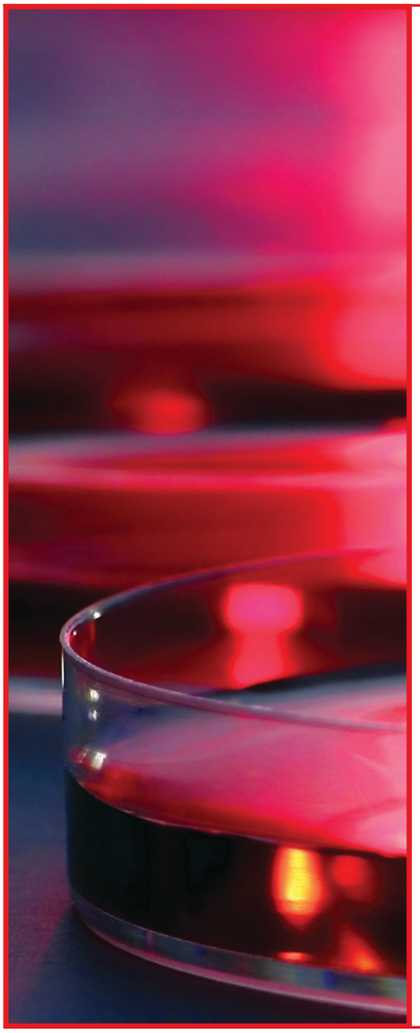
MEET OUR 2016 CANCER SURVIVOR HONOREE: DR. JENNIFER ARNOLD

We are proud to announce that Jennifer Arnold, MD, MSc, FAAP, will be honored at CR&T's 2016 Cancer Survivors Hall of Fame Dinner on November 15. Dr. Arnold, the co-star of TLC's popular docudrama, "The Little Couple," underwent surgery and chemotherapy for gestational trophoblastic neoplasm, a rare type of cancer stemming from an abnormal pregnancy. Now in remission, she is dedicated to raising awareness and building a supportive community for people with rare diseases. At the dinner, which will take place at the elegant Essex House in New York City, we will also present our Lifetime Achievement Award to Dr. Nicholas J. Sarlis, a distinguished investigator and industry leader.

Dr. Arnold is the medical director of a state-of-the-art simulation center at Texas Children's Hospital. For the last seven years, she has been involved in simulation education, which uses sophisticated technology to replicate complex medical scenarios. A neonatologist, she is also a practicing physician at the hospital's neonatal intensive care unit (NICU), the largest in the country. She has spoken both nationally and internationally on healthcare simulation education and has received numerous professional awards.

Dr. Arnold stands at 3 foot 2 inches and has a rare type of dwarfism called Spondyloepiphyseal Dysplasia Type Strudwick, which has led to more than 30 surgeries. Now in its 8th season, "The Little Couple" follows the personal and professional lives of Dr. Arnold, her husband, Bill, and their children, Will and Zoey. The show has helped to break down barriers and educate people around the country about people with disabilities. Dr. Arnold has also appeared on many high-profile TV shows, including "Oprah," "The Today Show," "Good Morning America," and "Anderson Cooper 360."

"Dr. Arnold is an inspiration to people who are coping with cancer and other serious health challenges," said Tom Silver, CR&T's president. "We are thrilled to have this opportunity to recognize her courage, compassion, and accomplishments."



PLEASE JOIN US FOR THE

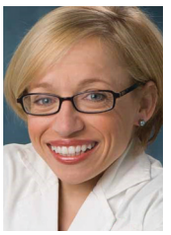
2016 CANCER SURVIVORS HALL OF FAME DINNER

Tuesday, November 15, 2016
The Essex House • 160 Central Park South • New York, NY

CANCER SURVIVORS HALL OF FAME™

Join us as we honor a prominent investigator and a courageous cancer survivor for their dedication and accomplishments. All proceeds benefit CR&T's efforts to discover life-saving treatments and cures for cancer.

Nicholas J. Sarlis, MD, MPH
Distinguished cancer investigator
and industry leader
Lifetime Achievement Award



Jennifer Arnold, MD, MSc, FAAP
Physician and co-star of
TLC's "The Little Couple"
Cancer Survivor Honoree

Master of Ceremonies
Raphael Miranda, Meteorologist, NBC 4 New York

RSVP online at www.crt.org/Hall-of-Fame-Event
or contact Barbara Rosenstein at 212-288-6604 or brosenstein@crt.org

Cancer Research & Treatment Fund, Inc.
Established 1968

CR&T NEWS
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ABOUT CR&T

Since 1968, CR&T has funded the world's most promising physician-scientists, equipping them with the resources to advance the treatment of various types of blood cancers, including myeloproliferative neoplasms (MPNs), leukemia, non-Hodgkin's lymphoma, Hodgkin's disease and multiple myeloma, as well as other common cancers, such as breast and lung cancer.