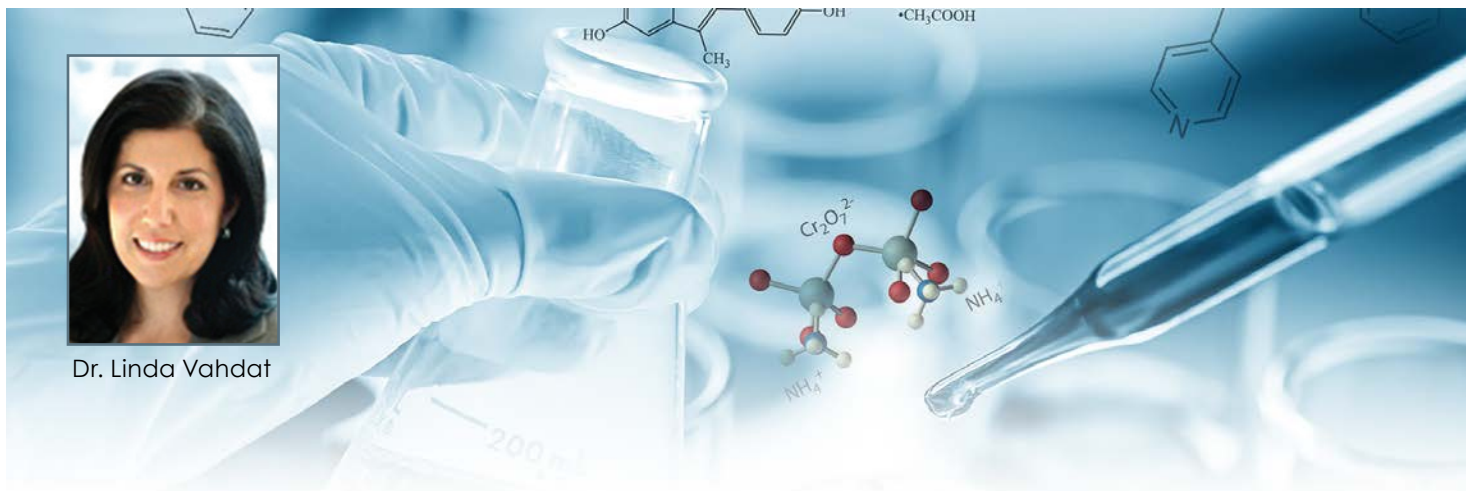


## CR&T SUPPORT ADVANCES PROMISING NEW TREATMENTS



Dr. Linda Vahdat

In laboratories worldwide, researchers are developing exciting new cancer treatments. Providing “seed money” for early studies that enable investigators to explore new ideas is one of the ways that CR&T contributes to this effort. Here is an update on CR&T-funded research that has recently been in the news.

### Preventing Breast Cancer Relapses

A drug that removes excess copper from the body may help protect breast cancer survivors who are at high risk of a relapse. In December 2016, the results of a phase 2 clinical trial of tetrathiomolybdate (TM for short) were reported in *Clinical Cancer Research*, a journal of the American Association of Cancer Research. The study, which was funded in part by CR&T, was conducted by a team led by Dr. Linda Vahdat, Director of the Breast Cancer Research Program at New York Presbyterian/Weill Cornell Medicine (New York, NY) and a member of CR&T’s Medical Advisory Board.

Over two years, TM was administered in pill form to 75 women who had previously undergone standard treatments for breast cancer, including surgery and chemotherapy. Although the women did not show evidence of the disease when they enrolled in the study, all faced high odds of a relapse. For some, a recurrence could mean that they would have only months to live.

The group included women who had been treated for breast cancer that had spread to other organs, as well as patients with triple negative breast cancer (TNBC). In TNBC, the tumor tests negative for the three elements that usually cause

tumor growth: estrogen receptors, progesterone receptors, and the HER2 protein. Since TNBC does not respond to therapies that target these three culprits, this form of breast cancer is especially difficult to treat.

The results of the copper depletion study were very promising, especially for women with TNBC. After approximately six years of follow-up, overall survival was 84 percent, and event-free survival – meaning that patients did not have a relapse or develop a new primary cancer – was 72 percent. No further signs of cancer were detected in 90 percent of the participants with TNBC.

Importantly, the researchers also studied the science behind TM’s effectiveness by testing the drug in mice. As Dr. Vahdat explained in an interview, copper “facilitates tumors doing their bad things.” The mineral is involved in a number of biologic processes that play an important role in tumor growth, both in the tumor itself and in the environment around it. Simply put, copper depletion therapy renders the tumor dormant by starving it of a mineral that is essential for its growth. “The effect is similar to having a car just run out of gas,” says Dr. Vahdat.

Although the results of this clinical trial are exciting, further studies are needed in order to prove the treatment’s effectiveness in large numbers of patients and to understand exactly why it works. Dr. Vahdat and her team are planning additional TM research.

Continued from page 1

## New Treatments Highlighted at Hematology Annual Meeting

The annual meeting of the American Society of Hematology attracts experts from around the world. CR&T-funded research was the focus of two presentations at the Society's 58th annual meeting, which was held in San Diego in December 2016.

Mantle cell leukemia (MCL) is a rare and often aggressive form of blood cancer that most commonly occurs in men over 60. In 2013, the FDA approved the drug ibrutinib, which is prescribed for MCL patients who have previously received other treatments. Unfortunately, although many people initially respond well to ibrutinib, their symptoms often return after a year or so – a condition known as “ibrutinib resistance.”

At the ASH annual meeting, Dr. Peter Martin (Assistant Professor of Medicine, Weill Cornell Medical College) presented the results of a phase 1 clinical trial, which showed that combining ibrutinib with another oral drug, palbociclib, benefits patients who become resistant to ibrutinib treatment. Palbociclib has already been approved for the treatment of certain forms of breast cancer, and researchers are exploring its use in other cancers as well. Twenty patients were enrolled in the MCL study, whose primary objective was to determine a safe and effective dosage for use in additional clinical trials. However, the results of this early trial were promising. Approximately 70 percent of patients responded to the therapy, and 45 percent went into remission. Only one patient experienced lymphoma progression.

This study, which was supported by



Dr. Selina Chen-Kiang



Dr. John Leonard

the National Cancer Institute, builds on the work of Dr. Selina Chen-Kiang, Professor of Pathology and Laboratory Medicine at Weill Cornell. In 2014, Dr. Chen-Kiang and her colleagues, including Dr. John Leonard, a member of CR&T's Medical Advisory Board, published a paper showing that palbociclib could prevent MCL cells from growing and dividing. CR&T provided partial funding for this important work, which showed that palbociclib could play a role in blocking ibrutinib resistance. Now, a large, multi-center clinical trial of the combination therapy is in the planning stage. Researchers hope to launch the study later this year.



Dr. Richard Silver

on alpha, an antiviral agent, in treating early-stage myelofibrosis. This disease is one of three rare blood cancers that are known collectively as myeloproliferative neoplasms (MPNs). Myelofibrosis is characterized by scarring of the bone marrow. In some patients, it progresses to a more

severe disease, acute myeloid leukemia. The goal of this phase 1 clinical trial was to establish a baseline that would help physicians identify patients who are most likely to benefit from interferon alpha. The researchers obtained molecular profiles of 30 patients with early myelofibrosis. Molecular profiling is a sophisticated form of testing that pinpoints the specific characteristics of an individual's cancer cells or tumor, such as gene mutations and biomarkers. This powerful tool is used to select the best existing treatment for a particular patient, or to develop new therapies that are targeted to the needs of a specific group of patients.

After receiving a course of low-dose interferon alpha, 73 percent of the study participants improved or remained stable. Molecular profiling showed that the patients who did not respond well to treatment had “high molecular risk” (HMR) gene mutations, which are associated with more aggressive disease. Based on these findings, the researchers concluded that “a molecular profile should be obtained initially to identify HMR mutations, which negatively affect response to treatment including [interferon alpha].” Patients without HMR mutations are the best candidates for interferon alpha therapy. In this group, early treatment may prevent the progression of the disease. This important study has been selected for publication and an editorial in *Cancer*, a journal of the American Cancer Society.

Please be sure to look for more updates on the work of CR&T-funded investigators in future issues of *CR&T News*. We are deeply grateful for your support, which makes this life-saving research possible.

## SAVE THE DATE

Wednesday  
November 1, 2017

Weill Cornell  
Medical College  
New York, NY

## 9th INTERNATIONAL PATIENT SYMPOSIUM ON MYELOPROLIFERATIVE NEOPLASMS

CR&T is proud to sponsor this full-day event for patients, their families and their friends. Learn about the latest developments in MPN research and treatment from distinguished basic scientists and clinical research physicians.

Online registration coming soon. For more information, please contact  
Barbara Rosenstein at 212-288-6604 or brosenstein@crt.org.

## MESSAGE FROM THE PRESIDENT

Thanks to your support, 2016 was a highly successful year for CR&T, and 2017 is off to a great start! Our Hall of Fame Dinner surpassed its revenue goals, and our new Young Professionals group was energized by the enthusiastic response to its first event, the Halloween Bash. We were busy investing funds too. During the past year alone, we committed nearly \$860,000 to crucial research initiatives.

### Progress at the Silver MPN Center

Our lead story includes an update on a study of the role of interferon alpha in treating myelofibrosis (page 2). The study was conducted by researchers at the Silver Myeloproliferative Neoplasm (MPN) Center at Weill Cornell Medical College, as well as other national and international investigators. In 2017, we will invest \$833,000 in the Silver Center, part of a multi-year, \$4.7 million commitment to building world's leading center dedicated to MPN research and patient care.

Silver Center investigators are conducting basic and clinical studies, presenting their findings at key professional conferences, and publishing the results of their research in peer-reviewed medical journals. Initiatives that are currently underway include:

- **A consortium that brings together MPN and cardiology experts from Weill Cornell and Memorial Sloan Kettering Cancer Center to study pulmonary arterial hypertension (PAH).** This common – but little understood – complication can lead to heart failure. The consortium seeks to identify the causes of PAH in MPN patients, which will lead to the development of new therapies.
- **Research into the relationship between the drug ruxolitinib and weight gain.** Although this drug helps prevent blood clots from forming in MPN patients' veins, it may also cause significant weight gain. This can increase the risk of cardiovascular problems in some patients. This study aims to identify patients who are at risk, enabling physicians to provide counseling and treatment.
- **A comprehensive evaluation of the benefits of interferon alpha therapy for patients with polycythemia vera (PV), an MPN that causes bone marrow scarring.** This study will provide extensive data on the safety and effectiveness of interferon alpha in comparison to the standard PV therapies. The results will help assure physicians nationwide that the drug is a valuable treatment option. CR&T is grateful to the Applebaum Foundation, which has provided a generous grant in support of this research.

### Commitment to Education

In 2017, we will continue our efforts to advance patient and professional education. Two members of our Medical Advisory Board, Drs. Richard Silver (Weill Cornell) and Jerry L. Spivak (Johns Hopkins University School of Medicine), will chair the 9th International Congress on MPNs, Myelodysplasia and Chronic Myeloid Leukemia, which will be held in New York City from November 2-3, 2017. This biennial conference draws outstanding medical professionals from around the world.

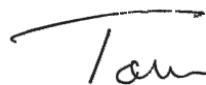
Faculty members from the Congress will join us for the 9th International Patient Symposium on Myeloproliferative Neoplasms, which will be chaired by our Immediate Past President, Dave Boule, and Dr. Silver. CR&T is proud to sponsor this full-day event, which will take place in New York on November 1. For more information, please see page 2.

All of these programs are made possible by the dedication of our volunteers and donors, including the members of our Medical Advisory Board. One of these prominent physician-scientists, Dr. John P. Leonard, recently was appointed to serve as the Interim Chair of the Weill Department of Medicine (WDOM). We are proud to offer our congratulations to Dr. Leonard.

We also want to acknowledge the ongoing support of Incyte Corporation and *CURE* magazine, sponsors of the annual MPN Heroes Recognition Program. CR&T has received a generous contribution through this program, "in recognition of dedication to patient care." You can learn more about the MPN Heroes program, which honors people and organizations that have dedicated themselves to improving patients' lives, by visiting [www.voicesofmpn.com](http://www.voicesofmpn.com).

In 2018, we will mark our 50th anniversary. You'll be hearing much more about our plans for celebrating this milestone in upcoming issues of *CR&T News*. For now, please accept our heartfelt thanks for your loyal support. Together, we are building a world without cancer.

Sincerely,



Thomas M. Silver  
President



SAVE THE DATE  
Tuesday, November 14  
The Essex House · NYC

2017 HALL OF FAME DINNER  
More information coming soon!

## 2016 Hall of Fame Dinner: Celebrating Courage and Research Excellence

**T**he Cancer Survivors Hall of Fame Dinner is CR&T's signature event – an annual celebration of courageous cancer survivors and the family, friends, and medical experts who are determined to find a cure. On November 15, more than 200 guests joined us at New York City's elegant Essex House as we honored two extraordinary individuals who have played a role in the fight against cancer.

Jennifer Arnold, MD, MSc, a neonatologist and co-star of TLC's popular docudrama, "The Little Couple," was our 2016 Cancer Survivor honoree. Our Lifetime Achievement Award was presented to Nicholas J. Sarlis, MD, PhD, Chief Medical Officer and Senior Vice President at Sellas Life Sciences Group, in recognition of his outstanding contributions to cancer research. We also presented a token of our appreciation to Raphael Miranda, the Emmy Award-winning meteorologist for NBC 4 New York, who serves as our master of ceremonies every year.

CR&T's President, Thomas Silver, welcomed and thanked the honorees and guests for their support. "Whether you're an old friend or new to our CR&T family, you're part of a small, close-knit organization that has a history of big accomplishments," he said. "We've contributed to the development of new treatments for blood cancers, breast cancer, and lung cancer." He also acknowledged the dedication and hard work of the dinner committee members, Diane Sapega, Barbara Silver, and Anne Templeton. Finally, he thanked CR&T's Medical Advisory Board for its commitment to our mission.

### Inspiring Honorees

Dr. Richard Silver, CR&T's Vice President and Medical Director, introduced Dr. Sarlis and spoke about his distinguished career. Dr. Sarlis is an experienced oncology clinician and a recognized expert on the global pharmaceutical market, with a focus on both blood cancer and solid organ tumors. His many accomplishments include an instrumental role in developing Jakafi, the first FDA-approved therapy for the blood cancers known as myeloproliferative neoplasms (MPNs).

Frank LaFerlita, an MPN patient and longtime friend of CR&T, joined Dr. Silver in presenting the award to Dr. Sarlis. "I'm alive today because of my doctor, Richard Silver, and our honoree, Dr. Nicholas Sarlis," said LaFerlita. "My family and I can't thank Dr. Sarlis enough for helping to make this drug available to patients like me." Dr. Sarlis's acceptance speech conveyed his passionate pursuit of scientific knowledge and his commitment to making a difference in the lives of people who are bravely coping with cancer.

Dave Boule, CR&T's Immediate Past President, introduced Dr. Jennifer Arnold, who stands at 3 foot 2 inches and has a rare type of dwarfism, which has led to more than 30 surgeries. More recently, Dr. Arnold faced a new medical challenge when she underwent surgery and chemotherapy for a rare type of cancer. Millions of viewers followed her cancer journey on "The Little Couple," which has helped to break down barriers and educate Americans about individuals with disabilities.

"Dr. Arnold has met every challenge with grace, courage, and a fantastic sense of humor," said Boule. "As an MPN patient, I am personally grateful for her commitment to raising awareness and building a supportive community for people with rare cancers." The audience – which included many cancer survivors and their families – responded enthusiastically to Dr. Arnold's inspiring talk.

Guests also had the opportunity to bid on a wide range of silent auction items and to enter a raffle drawing for a luxury vacation. Thanks to the generosity of our supporters, the event raised nearly \$400,000 for CR&T and its cutting-edge research initiatives.



Front row: Dr. Jennifer Arnold and her husband, Bill Klein  
Back row (l-r): Dave Boule, Tom Silver, Dr. Richard Silver, Frank LaFerlita, and Dr. Nicholas Sarlis

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Our work would not be possible without the generous support of our donors and grantors. We are deeply grateful for their commitment to CR&T's mission. The list below comprises supporters who provided gifts of \$500 or more in 2016.

For the complete list, please visit [www.crt.org](http://www.crt.org).

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## MEET CR&T's LEADERSHIP: MICHAEL C. KEMPNER



*This is the first of series of brief profiles that will introduce you to members of CR&T's Board of Directors, Medical Advisory Board, and other key volunteers. We are grateful to these friends for the generous gift of their time, expertise, and resources.*

Michael Kempner brings outstanding professional experience and a strong commitment to philanthropy to CR&T. Mike, who serves on our Executive Committee and heads our Investment Committee, is a co-founder and Chief Operating Officer of KS Management Corporation, a New York-based investment company. He was recruited to CR&T in 2008 by our founder, Dr. Richard Silver, while both were serving on the board of Connecticut's Rectory School.

Mike's expertise and counsel are key to ensuring that CR&T has the resources needed to fulfill its mission. "This is a very exciting time for cancer research, with so many sophisticated new treatments in the pipeline," he says. "We all know someone whose life has been touched by cancer, whether it's a family member, friend, or co-worker. It's enormously rewarding to be a part of an organization that has helped so many patients to lead healthier, longer lives."

Mike began his career at Bear Stearns, followed by positions with Kaufman Alsberg and Prudential-Bache Securities, where he was a risk arbitrage analyst. He earned an AB from Harvard College and received an MBA from Columbia Business School. In 2008, he received his designation as a Chartered Alternative Investment Analyst (CAIA).

Mike is a trustee and treasurer of the De La Salle Academy, a New York school for academically talented and economically less advantaged boys and girls. He also serves on committees of the Harvard Alumni Association and the Harvard Art Museum. He lives in New York City with his family. We are honored to have him as a member of the CR&T family, and thank him for his service to our organization.



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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.

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