

CLINICAL TRIALS AT THE SILVER MPN CENTER



Silver Center leadership, from left: Drs. Andrew I. Schafer, Richard T. Silver, Joseph M. Scandura, and Ellen K. Ritchie

*Leading the way
to advances
in treatment*

Since 2011, CR&T has contributed to the growth of the Richard T. Silver, MD Myeloproliferative Neoplasms (MPN) Center at Weill Cornell Medicine in New York City. Our goal is to ensure that the Center, named for CR&T's founder, will become the world's leading center dedicated to MPN research and patient care. To this end, the Silver Center's leadership has successfully worked to increase the number of clinical trials that could lead to new treatments for these blood cancers. Below are just a few examples of the interesting studies that are currently underway at the Silver MPN Center.

Project Title: A Double-Blind, Double-Dummy Phase II Randomized Study to Evaluate the Efficacy and Safety of Ruxolitinib Versus Anagrelide in Subjects with Essential Thrombocythemia Who Are Resistant to or Intolerant to Hydroxyurea (recruiting patients)

Essential thrombocythemia (ET) causes the bone marrow to make too many platelets, which can lead to blood clots and bleeding. Key goals of treatment are to manage symptoms and prevent the disease from progressing to the most severe type of MPN, myelofibrosis, which causes scarring of the bone marrow. The first-line treatment for ET is usually hydroxyurea, which decreases the patient's platelet count. However, some people do not respond to this treatment, or may not be able to tolerate the drug.

The purpose of this study is to evaluate the safety, effectiveness, and tolerability of ruxolitinib versus anagrelide, a platelet-reducing medication that is usually prescribed for ET patients who are unable to take hydroxyurea. Ruxolitinib (trade name: Jakafi) is an oral medication that curbs the activity of proteins called JAKs (short for Janus kinases), which cause the bone marrow to produce too many blood cells. The FDA has approved ruxolitinib for use in treating myelofibrosis (2011) and another MPN, polycythemia vera (2014), but the drug has not yet been approved for ET. This national study will help determine if ruxolitinib should be used as a second-line therapy for people with ET.

Project Title: Study Evaluating Tolerability and Efficacy of Navitoclax in Combination with Ruxolitinib in Subjects with Myelofibrosis (active; recruitment closed)

This national multicenter study is evaluating the effectiveness, safety, and tolerability of an experimental drug, navitoclax, in combination with ruxolitinib in people with myelofibrosis. As noted above, ruxolitinib targets JAK proteins. Navitoclax blocks the function of BCL2/BCXL proteins, which are found in myelofibrosis cells, thereby causing these cells to die.

Previous studies have shown that ruxolitinib can limit symptoms and help control blood counts in myelofibrosis patients. Although it is an important drug, its ability to

slow down the progression of the disease is limited. The investigators hope that combining these drugs will not only improve patients' quality of life, but extend their lives.

Project Title: A Phase 3, Multi-Center, Open-Label, Randomized Study of Oral ABL001 Versus Bosutinib in Patients with Chronic Myelogenous Leukemia in Chronic Phase (CML-CP), Previously Treated with Two or More Tyrosine Kinase Inhibitors (recruiting patients)

This study compares the benefits and side effects of ABL001, an experimental drug, to bosutinib (trade name: Bosulif) for the treatment of chronic myelogenous leukemia (CML).

CML starts in the bone marrow. Most patients have the "Philadelphia chromosome," an abnormal chromosome that creates an abnormal gene called BCR-ABL. This gene, in turn, releases a type of protein called a tyrosine kinase, which causes some white blood cells (leukemia cells) to grow.

Most people with CML are diagnosed when they are in the chronic phase – meaning that the disease is not advanced, symptoms are fairly mild, and patients generally respond to treatment. The standard drugs for chronic CML are tyrosine kinase inhibitors (TKIs), which target these harmful proteins and curtail their activity. There are various kinds of TKIs. Imatinib (Trade name: Gleevec), the first TKI to receive FDA approval, revolutionized the treatment of CML and remains the first-line therapy for the disease.

A number of other TKIs are available and are usually prescribed for patients who stop responding to imatinib. Both bosutinib and the study drug, ABL1001, belong to this class of therapy. If successful, this study will lead to the development of another therapeutic option for CML patients who do not respond to first-line treatment.

For a complete list of Silver Center studies that are currently recruiting patients, please visit <https://silvermpncenter.weill.cornell.edu/research-and-clinical-trials/myeloproliferative-clinical-trials>.

CLINICAL TRIAL RESOURCES



If you or someone you know is interested in learning more about clinical trials, the Internet offers a wealth of resources. Below are sites that provide trustworthy, accurate information. Of course, if you're interested in participating in a particular study, be sure to speak to your doctor about whether it might be right for you.

- **www.clinicaltrials.gov**, a service of the National Institutes of Health (NIH), is a registry and database of publicly and privately supported clinical studies conducted around the world. You'll also find information about how clinical trials are conducted and links to other trusted resources.
- **The NIH National Cancer Institute (www.cancer.gov/about-cancer/treatment/clinical-trials)** has comprehensive information about cancer clinical trials, benefits and risks, questions to ask, and patient safety. There's also a search function to help you find studies that may be of interest to you.
- **ResearchMatch (www.researchmatch.org)** is a free registry that connects volunteers with researchers who are searching for appropriate participants for their clinical trials and patient surveys. A collaborative project of academic institutions across the country, ResearchMatch is maintained by Vanderbilt University School of Medicine and overseen by the Vanderbilt Institutional Review Board.



MESSAGE FROM THE PRESIDENT

Few of us go through life without being touched by cancer in some way. All too many young people feel the impact of the disease when a parent or grandparent is diagnosed. But a recent study published in *Lancet Public Health* reported alarming results: It revealed that cancer rates were increasing in people between 25 and 49 years old.

Although the study didn't focus on the causes of the increase, the authors observed that most of these cancers—including kidney, gallbladder, and colorectal cancer—are linked to obesity. But they also found spikes in gastrointestinal cancer and leukemia, which are not. More research will be needed to learn exactly why these cancers are on the rise in younger adults.

With the incidence of cancer growing among young people, CR&T's mission is more urgent than ever. That is why we are delighted with the growth of our Young Professionals group, led by AJ Shiekman and Tim Silver, who recently joined our board of directors. Their commitment to CR&T is very personal. Tim has grown up with CR&T and is the third generation of our family to join the organization's leadership. AJ, inspired by his father's courageous battle against lung cancer, says that through his involvement with CR&T, he is "picking up the fight against cancer where my dad left off."

The Next Generation of Leaders

Our next generation of leaders is drawing on the knowledge and experience of our first group of Young Professionals,

including Amanda Johns Perez, Todd Shaw, and Adam Silver, who have served on our board of directors for years. Diagnosed with Hodgkin's lymphoma as a young man at the beginning of a career in finance, Todd participated in an early study that evaluated the effectiveness of an experimental therapy. Today, Hodgkin's lymphoma is considered one of the most treatable cancers, especially if found early. "My hope for the future of CR&T and cancer research is that I don't have a job here anymore," he says.

It's a hope that Tim, AJ, and their Young Professional friends and colleagues are working to fulfill. Over the past three years, the group has held two successful Halloween fundraisers and, in 2018, they had a significant presence at our 50th Anniversary Cancer Survivors Hall of Fame Dinner. In addition to exploring new ways to raise funds for research, they are planning to increase their support of the 2019 gala, which will be held at New York's Essex House on November 12.

If you or someone you know is a young professional who would like to become involved, please be sure to let us know. Simply contact our Director of Administration and Development, Barbara Rosenstein, at 212-288-6604 or brosenstein@crt.org, and she'll put you in touch with Tim or AJ. It's an opportunity to meet a dedicated group, have fun – and, most important of all, to give the gift of a healthy future to people of all ages who are living with cancer.

Thomas M. Silver
President

CR&T CO-SPONSORS PROSTATE SCREENING PROGRAM

Since 2011, CR&T has been proud to be a co-sponsor of the Annual Prostate Screening and Health Fair, held at First Presbyterian Church in Jamaica, Queens. Held on March 30, 2019, this community outreach event underlines the importance of early detection and education. The program is conducted under the leadership of the Rev. Patrick H. O'Connor, pastor of the church, and Douglas Scherr, MD, professor of urology and clinical director of urologic oncology at Weill Cornell Medicine.

In addition to the prostate screening – which includes a prostate-specific antigen blood test and a physical examination – this health fair offers free tests for men and women, including HIV, blood pressure, diabetes, and cholesterol tests, along with dental screening and nutritional counseling.



Reverend O'Connor (back row, 5th from the left) with health fair staff

Prostate cancer is the most commonly diagnosed nonskin cancer in America. If detected early, the disease has over a 90 percent survival rate. Events like this play a critical role by providing preventive services to underserved communities. In addition to supporting this life-saving program, CR&T continues to provide funding for prostate cancer research.

For more information about First Presbyterian Church and its community initiatives, please visit www.firstchurchjamaica.org.

MEDICAL ADVISORY BOARD NEWS

CR&T's research grants program is guided by its Medical Advisory Board (MAB), which comprises some of the world's leading physician-researchers. Our MAB members regularly speak at prestigious medical conferences, publish the results of important studies, and are honored for their contributions to research and patient care.



Andrew I. Schafer, MD

Two Silver MPN Center leaders (see cover story) who serve on our advisory board received significant recognition over the past few months. **Andrew I. Schafer, MD**, the Silver Center's Director, was elected an Honorary Member of the Interurban Clinical Club, which was founded by the great Canadian physician, Sir William Osler, often described as "the father of modern medicine," in 1905. Together with two other distinguished MPN experts, Drs. Ronald Hoffman and Ruben Mesa, he organized "New Insights into the Understanding of Thrombosis and Hemorrhage in the MPNs," a two-day conference that was held in Charleston, SC in June. The goal of this "first-ever" conference is to develop a position paper on consensus recommendations for prioritized research directions in this important area of MPN research.

This year, CR&T's founder, **Richard T. Silver, MD**, received many honors for his lifelong commitment to innovative cancer research. On February 12, he traveled to Mannheim, Germany, where he was honored with the European LeukemiaNet (ELN) Merit Award at the organization's 16th Symposium. The ELN's mission is to improve the care of leukemia patients in Europe and globally, and to focus on the clinical development of new strategies and drugs. Professor Rüdiger Hehlmann, Founder and Chair, noted that Dr. Silver was responsible for bringing Weill Cornell Medicine into the ELN as its first US institution. Since 2004, Dr. Silver also has served on the ELN's panel on chronic myeloid leukemia.

On April 3, Weill Cornell Medicine celebrated Dr. Silver's 90th birthday – and his long and distinguished career – by presenting a special academic symposium in his honor. The program began with grand rounds led by David P. Steensma, MD, Associate Professor of Medicine at Dana-Farber Cancer Institute, followed by a series of presentations by Dr. Silver's Weill Cornell colleagues. A highlight of the program was a video



From left: Drs. Ronald Hoffman, Vesna Najfeld, Richard T. Silver, and John Mascarhenas

tribute featuring Dr. Silver and two of his MAB colleagues, **Drs. Morton Coleman and John Leonard**.

In May, **Dave Boule**, CR&T's Treasurer and an MPN patient, joined him for his annual lecture to first-year medical students. While Dr. Silver focused on the basics of diagnosing and treating these blood cancers, Boule spoke about the impact of the MPNs on patients.

On May 16, Dr. Silver received the prestigious Wisch-Grinberg Award, presented by the Icahn School of Medicine at Mount Sinai in New York City. The recipient of this endowed visiting lectureship gives a grand rounds presentation on a hematology/oncology topic and is honored at a special dinner. As a pioneering researcher in the field of MPNs, Dr. Silver focused on the diagnosis and treatment of polycythemia vera.

We are proud to congratulate Dr. Silver for his many contributions to research, and for his compassionate care for patients and their families.



Dave Boule speaks to first-year medical students



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**10TH INTERNATIONAL PATIENT SYMPOSIUM
 ON MYELOPROLIFERATIVE NEOPLASMS**

Wednesday, October 23, 2019

**Belfer Research Building • Weill Cornell Medical College
 413 East 69th Street • New York, NY 10021**

CR&T is proud to sponsor this all-day event for patients, their families and friends. You'll learn about the latest developments in MPN research and treatment from internationally respected physician-scientists. You'll also participate in disease-specific Q&A sessions that allow you to speak one-on-one with these experts.

Sign up for the Symposium and receive a discount on registration for the 12th International Congress on Myeloproliferative Neoplasms, a two-day professional program that will be held from October 24-25 at the Westin New York-Times Square (www.12thMPNCongress.com).

To learn more and register, visit www.crt.org/10th-International-Patient-Symposium-on-Myeloproliferative-Neoplasms, call CR&T at 212-288-6604, or fill out the form below.



MPN Patient Symposium Registration Form

Registration is \$175 for patients, or for the first person; \$100 for your spouse and/or guest.

Please return to: CR&T, 500 East 77th St., Suite 1001, New York, NY 10162.

(Please print)

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22ND ANNUAL CANCER SURVIVORS HALL OF FAME DINNER

Tuesday, November 12, 2019

The Essex House | 160 Central Park South | New York, NY

Please join us for a very special evening commemorating 51 years of achievement. This annual dinner honors cancer survivors, humanitarians, and medical professionals for their courage and commitment to life-saving research. All proceeds benefit CR&T.

Humanitarian Honoree

Ko-Chung Lin

Founder and CEO
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Penn State College of Medicine
Hodgkin's Lymphoma Survivor

Master of Ceremonies

Raphael Miranda, Meteorologist, NBC 4 New York

Register 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.
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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.

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