

CR&T-FUNDED RESEARCH PRESENTED AT MAJOR HEMATOLOGY CONFERENCE

Two important studies examined the effectiveness of interferon-alpha in polycythemia vera.



(l-r) Drs. Ghaith Abu Zeinah, Richard T. Silver, and Joseph M. Scandura, Silver Center investigators

The Annual Meeting and Exposition of the American Society of Hematology (ASH) is the premier scientific conference in the field. At the 2019 meeting, held from December 7-10 in Orlando, FL, the Richard T. Silver, MD Myeloproliferative Neoplasms (MPN) Center at Weill Cornell Medicine was well represented, with an impressive 13 presentations and posters focusing on both basic and clinical research. Much of this work would not have been possible without the support of CR&T, which has committed a total of \$6.5 million for research at the Silver Center since 2011. Our goal is to sustain the Center's role as a world-class leader in research, treatment, and care for people with MPNs, a group of blood cancers. Below, we focus on key MPN studies presented by Silver Center investigators at the 2019 meeting and provide an update on an important study that was recently published in *Blood*, the leading ASH scientific journal.

Exploring the Role of Interferon

Interferon-alpha (IFN-alpha) is the only drug that can reverse the progression of MPNs. Research has shown that this drug can reduce fibrosis (scarring) in the bone marrow and alleviate symptoms. Richard T. Silver, MD, CR&T's Vice President and Medical Director, has conducted pioneering research into the use of interferon in the MPNs, and for many years, was one of the few investigators to focus on the drug. Today, Dr. Silver is joined by many other investigators worldwide who are

exploring the role of IFN-alpha in treating the MPNs. These include Andrew I. Schafer, MD, the Silver Center's Director; Joseph M. Scandura, MD, PhD, its Scientific Director; Ghaith Abu Zeinah, MD, Instructor in Medicine; and associated research assistants, technicians, and nurses.

Two important studies by these investigators and their colleagues focused on the effectiveness of IFN-alpha as a treatment for polycythemia vera (PV), one of the MPNs. One study showed, for the first time, that IFN-alpha lowers PV patients' risk of developing myelofibrosis, which causes scarring of the bone marrow and is the most serious form of MPN. Further, the drug enables these patients to live longer than those receiving other standard MPN therapies. Using computer-based information retrieval systems developed with Dr. Scandura's input, this study analyzed the records of 306 PV patients treated at Weill Cornell Medicine from 1974-2019. It is the largest study with the longest follow-up of PV patients treated with IFN-alpha.

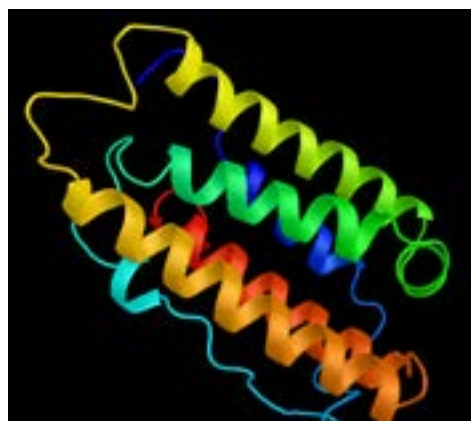
PV patients are also at risk for developing blood clots (thrombosis). This is because the disease causes the bone marrow to make too many red blood cells, which thickens the blood. Serious complications, such as splanchnic vein thrombosis (blood clots in the abdominal veins), stroke, and deep vein thrombosis (blood clots in the body's deep veins,

especially in the legs) can occur. Another large study by Silver Center researchers showed that interferon is more effective in reducing the risk of thrombosis than other standard treatments, such as chemotherapy and bloodletting (phlebotomy).

These studies would not have been possible without the Silver Center's extensive patient database, which was developed with CR&T funding. We are grateful to The Applebaum Foundation for its generous support of this research, including the development of the database.

Potential Treatment for Anemia in MPN Patients

Because of fibrosis (scarring) in the bone marrow, many people with MPNs suffer from chronic anemia, which is difficult to treat. This may be due to an excess of the protein TGF-beta in the marrow. Researchers believe that, in addition to suppressing red blood cell production, TGF-beta causes the scarring in myelofibrosis. Silver Center investigators, led by Dr. Scandura, were able to block the production of TGF-beta in mice. Next, they gave the mice a tiny dose of the hormone erythropoietin, which stimulated red blood cell production. Based on these findings, the researchers have received funding for a clinical trial that could lead to the development of a more effective treatment for anemia in MPN patients.



Ruxolitinib and Weight Gain

In the February 2020 issue of *Blood*, a paper written by Dr. Scandura and Silver Center colleagues was published, reporting on the results of an important study on the cause of weight gain in MPN patients who are taking the drug ruxolitinib.

Many people with PV or myelofibrosis have benefited from ruxolitinib. However, researchers found that some patients taking the drug gained considerable weight and that their

cholesterol was elevated – a serious concern in patients who are already at a higher risk for cardiological complications. The Silver Center team's goal was to identify those patients who were most likely to gain weight and to pinpoint the underlying cause of this weight gain.

Through their analysis of a mouse model, the researchers discovered that ruxolitinib blocks a hormone called leptin, which helps control appetite. Because their bodies don't signal that they are full, some patients are always hungry, which leads to significant weight gain. Based on their analysis of 179 patients, the investigators were able to predict which patients had the highest risk of gaining weight. They found that those who gained more than two percent of their pre-treatment weight within 60 days of starting

treatment with ruxolitinib were more likely to continue gaining weight, to increase their body mass index (BMI), and/or to become overweight. Armed with this information, physicians can provide dietary counseling and recommend lifestyle changes that will help these patients to control their weight early on.

This is just a sampling of the research that is underway at the Silver MPN Center, thanks to the continuing support of CR&T and donors like you. To learn more about the Silver Center, including information about clinical trials that are recruiting participants, visit <https://silvermpncenter.weill.cornell.edu>.

Some patients taking ruxolitinib gain weight because the drug blocks a hormone called leptin, which helps control appetite.



MESSAGE FROM THE PRESIDENT



“Somewhere, something incredible is waiting to be known,” Carl Sagan wrote. CR&T was founded to give talented investigators the means to make incredible discoveries – advances that transform cancer patients' lives. At the start of a new decade – and our 52nd year – this remains the heart of our mission.

As our cover story shows, our \$6.5 million investment in research at the Richard T. Silver, MD Myeloproliferative Neoplasms (MPN) Center at Weill Cornell Medicine is producing impressive results. Our commitment to the Center is a tribute to the man for whom it is named. This past year, after celebrating his 90th birthday, Dr. Silver – CR&T's founder – decided that it was time to move to Emeritus status, and to entrust the care of his patients to a gifted young colleague, Ghaith Abu Zeinah, MD. Dr. Silver continues to be a major force in the field: As Emeritus Director of the Center, he continues to conduct research, teach, speak at conferences in the U.S. and abroad, and consult with patients as needed. He also remains the guiding force behind CR&T, continuing to serve as Vice President and Medical Director.

This past December, during the Annual Meeting of the American Society of Hematology, Dr. Silver received the 2019 Career Achievement Award for Clinical Research in Hematology from Celgene Corporation. In recognition of his tremendous impact and significant contributions to the field, Celgene has made a grant to Weill Cornell Medicine to support additional research in hematology. Dr. Silver was nominated for the honor by Dr. Abu Zeinah, who was on hand to present the award.

Welcoming New Leadership

We are pleased to announce that Dr. Abu Zeinah and the Silver Center's Scientific Director, Joseph M. Scandura, MD, have joined CR&T's Medical Advisory Board (MAB). The MAB comprises prominent physician-researchers, such as John P. Leonard, MD, who was appointed Senior Associate Dean for Innovation and Initiatives at Weill Cornell Medicine in February. In this newly created role, Dr. Leonard will lead Weill Cornell Medicine's efforts to foster a vibrant culture of entrepreneurship and innovation, and to promote commercialization opportunities for inventions made by its scientists.

Our Board of Directors also welcomed two new members in 2019. Keith Civil taught in New York's Auburn Enlarged City School System for 33 years, retiring in 2005. Bharat Shah, PhD, was the Humanitarian Honoree at our 2017 Cancer

Survivors Hall of Fame Dinner. He is the Past President of Noble Investment Group, which has headquarters in Atlanta. Additionally, Jeff Puglisi, MD, an internist practicing in Greenwich, CT, and who has served as President of the Greenwich Medical Society since 2006, recently agreed to join the CR&T board and is expected to officially become a Director in mid-2020. Both Keith and Jeff initially became involved with CR&T when they attended our International Patient Symposium on MPNs. All three are dedicated supporters of CR&T, with the experience and skills to ensure our continued growth and contributions to cancer research.

The Year Ahead

Plans for our signature events are well underway. Because of popular demand, for the first time, we will present the International Patient Symposium on MPNs for two years in a row. This full-day education program, which features presentations by some of the world's leading MPN experts, enables patients and family members to learn about the latest developments in the field. This Symposium will be held at Weill Cornell's Belfer Research Building in New York City on October 28. We are grateful to the sponsors of our 2019 Symposium: Incyte (Presenting Sponsor), PharmaEssentia, The SASS Foundation for Medical Research, Sierra Oncology, and Laine and David Boule. From October 29-30, the 13th International Congress on MPNs, a professional educational program presented by Melton Medical Education, will be held at the Crowne Plaza Times Square. Drs. Richard T. Silver and Jerry Spivak, members of CR&T's MAB, are the co-chairs of this program, which features presentations by MPN specialists from around the world. Finally, our major fundraising event, the Cancer Survivors Hall of Fame Dinner, will take place at New York's elegant Essex House on November 18.

We hope that you'll join us for these exciting programs. We look forward to seeing you – and thank you for joining us on our voyage of discovery.

Thomas M. Silver, President

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2019 CANCER SURVIVORS HALL OF FAME DINNER

Commemorating 51 Years of Achievement



Hall of Fame 2019 Honorees and leadership (l-r): Dr. Ko-Chung Lin, Dave Boule, Tom Silver, Dr. Richard Silver, Dave Price, Dr. Dan Shapiro, Dr. Morton Coleman, and Dr. Ghaith Abu Zeinah

The Cancer Survivors Hall of Fame Dinner is CR&T's signature event — an annual celebration of courageous cancer survivors and the families, friends, and medical experts who are determined to find a cure. On November 12, nearly 200 guests joined us at New York City's elegant Essex House as we honored two remarkable individuals for their role in the fight against cancer.



CR&T Young Professionals led by AJ Shiekman and Tim Silver (first row, 4th from the left)

Ko-Chung Lin, PhD, Founder and CEO of PharmaEssentia Corp., received the 2019 Humanitarian Award for his commitment to developing new therapies and improving the quality of life for people with myeloproliferative neoplasms (MPNs) and other serious diseases. Our Cancer Survivor honoree was **Dan Shapiro, PhD**, Vice Dean for Faculty and Administrative Affairs at Penn State College of Medicine. A Hodgkin's lymphoma survivor, Dr. Shapiro has raised awareness through his books, articles, and talks to audiences throughout the U.S.

Dave Price, NBC 4 New York weather anchor, was an inspiring and entertaining master of ceremonies. **Anne Templeton** and **Barbara Silver** were the dedicated dinner committee co-chairs.

We are deeply grateful to the many friends and donors who make the dinner a great success, year after year.



Drs. Ghaith Abu Zeinah, Joseph Scandura, Agnes Viale and Neveen Alnajjar



Dr. Ko-Chung Lin and Dr. Manuel Hidalgo



Julia Johns (1st row, 2nd from the left) and Jennifer Johns Friel (2nd row, 2nd from the left) and guests

2019 CANCER SURVIVORS HALL OF FAME DINNER

Commemorating 51 Years of Achievement

(continued from page 5)



PharmaEssentia guests



Edie and Richard Rose (standing) and guests



Schuster Family



Dr. Ko-Chung Lin (first row, center) and PharmaEssentia guests

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CR&T's work would not be possible without the generous support of our donors and grantors. We are deeply grateful for their commitment to our mission. The list below comprises supporters who provided gifts of \$500 or more in 2019. For the complete list, please visit www.crt.org.

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SAVE THE DATE

Mark these exciting fall events on your calendar!

11th International Patient Symposium on Myeloproliferative Neoplasms

Wednesday, October 28, 2020

Belfer Research Building, Weill Cornell Medicine, New York City

CR&T is proud to sponsor this full-day event for patients, their families and friends. You'll learn about the latest developments in MPN research and treatment from distinguished physician-researchers.

To register, visit www.crt.org and click on the MPN Symposium tab.

2020 Cancer Survivors Hall of Fame Dinner

Wednesday, November 18, 2020

The Essex House, New York City

Join us for our signature fundraising event as we honor courageous cancer survivors, compassionate humanitarians, and distinguished physician-researchers for their achievements and commitment to eradicating cancer.

More details coming soon on our website, www.crt.org,
and in the next issue of this newsletter.

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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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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