CR&T news
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MPN Advocates Meet with FDA Representatives

CR&T Board Member Dave Boule Reports on His Visit to the FDA

The federal Food & Drug Administration (FDA) is working hard to incorporate the patient voice in their mission of approving safe and effective new drugs. The Office of Patient Outcomes, part of the agency's new Oncology Center of Excellence, asked three MPN patient advocacy groups to send representatives to an FDA mini-symposium, "Myeloproliferative Neoplasms (MPNs): A Conversation with Patient Advocates," which was held in September 2017.

As a member of the boards of CR&T and the MPN Research Foundation (MPNRF), I was asked to attend, along with Michelle Woehrle, MPNRF's executive director. I was one of three MPN patients. Dr. Paul Kleutz, Acting Director of Patient Outcomes, led the FDA contingent. Seven other FDA speakers included the Director of Orphan Products Development and several medical officers who are hematologist/oncologists in the Center for Drug Evaluation and Research. Additionally, two physicians who had worked extensively on approving Jakafi, the one new drug approved for MPNs since the discovery of the JAK2 mutation in 2005, were also at the table.

The program, which was essentially "FDA 101" as it relates to the MPNs, was four hours long and highly interactive. We learned about the regular and accelerated drug approval processes. Importantly for the MPN community, we also learned about the Orphan Drug Designation, which provides enormous financial incentives to pharmaceutical companies that develop drugs for diseases that affect 200,000 or fewer U.S. patients. The MPNs (polycythemia vera, essential thrombocythemia, and myelofibrosis), which are the primary focus of CR&T and the Silver MPN Center, fall into this category. We also had presentations on patient access to drugs that are undergoing clinical trials but have not yet been approved, as well as personal importation of drugs that are approved for use outside the U.S.

Advocating for Interferon Approval

While the opportunity to learn these fundamentals was extremely important, Michelle and I wanted to promote the interests of the MPN patient community in two specific ways: 1) to determine how patient advocates can help to get the drug interferon approved as a front-line therapy for MPN patients; and 2) to determine how the data being gathered by the MPNRF's brand-new MyMPN Patient Registry might be helpful to the FDA.

In pursuit of the first goal, I had a long conversation before the meeting with our own Dr.

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Highlights from the American Society of Hematology Annual Meeting

CR&T-Funded Researchers Present Their Studies

Clinicians and scientists from around the world attend the annual meeting of the American Society of Hematology (ASH), the premier conference featuring the latest developments in the field. Six papers by CR&T-funded researchers were presented at the 2017 conference, which was held in Atlanta this past December; two of these are discussed below. In addition, three papers were accepted at other important conferences in 2017. This is a testament to the quality of work conducted by both senior investigators and fellows at the Richard T. Silver Myeloproliferative Neoplasm(MPN) Center at Weill Cornell Medicine.

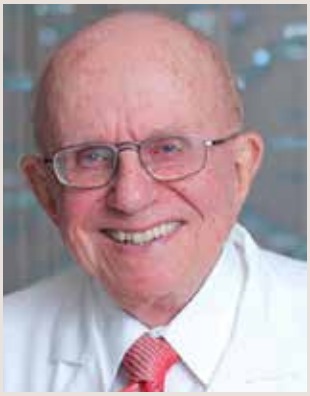
Focus on Interferon

Interferon is the single drug that provides disease-altering effects for MPN patients. The ASH meeting offered a number of presentations that focused on this therapy, which is often used to treat the MPNs, although it has not yet received FDA approval for use in these diseases.

Two Silver Center researchers, Dr. Richard Silver, a pioneer in the use of interferon in the MPNs, and Dr. Ellen Ritchie, were among the investigators who participated in an international multi-center clinical trial that examined the impact of pegylated interferon on MPN patients' symptoms and quality of life.

Between 2012 and 2015, the study enrolled 115 high-risk patients, aged 20-85, with either essential thrombocythemia or polycythemia vera. These patients had previously been treated with hydroxyurea, the drug most commonly used to treat the MPNs. However, they were unable to tolerate this therapy. Studies have also shown that hydroxyurea may cause the disease to progress to acute myeloid leukemia (AML).

The researchers found that interferon treatment improved MPN-related symptoms, including fatigue, dizziness, numbness/tingling, and weight loss. However, some patients experienced drug-related side effects, such as flu-like symptoms, injection-site irritation, and vision problems. Since symptoms improved and quality of life was not decreased for patients who were able to tolerate interferon, the investigators concluded that further research is needed to determine which patients are most likely to tolerate treatment with pegylated interferon.



Richard T. Silver, MD

Ellen Ritchie, MD

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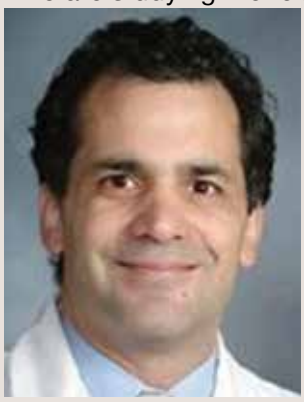
Pulmonary Hypertension in MPN Patients

As previously reported in *CR&T News*, Joseph Scandura, MD, Scientific Director of the Silver MPN Center, has assembled a consortium of investigators who are studying the relationship between the MPNs and pulmonary hypertension (PH) – high blood pressure in the arteries of the lungs. Little is known about why and how some MPN patients develop this cardiovascular complication, which can lead to heart failure.



Joseph Scandura, MD, PhD

At the ASH meeting, these researchers reported on a study of 300 MPN patients who had undergone echocardiograms. Fifty-six percent of these patients had PH; in 20 percent, the disease was advanced. MPN patients with PH are more likely to experience fatigue. Advanced PH is associated with a higher mortality risk, independent of the MPN diagnosis. The investigators concluded that since PH is very common in people with MPNs, all MPN patients should undergo cardiac screening so that they can receive appropriate treatment.

CR&T will continue to report on new studies in these and other key areas.

We are grateful to David Alexander for providing source information for this report.

MPN Advocates meet with FDA

Continued from page 1

Richard T. Silver, the pioneer in the use of interferon in the MPNs. This is the only drug on the market that can reverse the progression of MPNs. Interferon has been shown to reduce fibrosis in the bone marrow; normalize blood counts, including eliminating anemia in early-stage myelofibrosis; reduce mutated JAK2; and alleviate symptoms, including shrinking an enlarged spleen.

Yet, while interferon is approved for use in hepatitis C, the pharmaceutical companies that manufacture the drug have never asked the FDA to approve it as an MPN therapy. Dr. Silver noted that their perception is that the MPN market is too small to warrant the expense of a Phase III clinical trial — the final and very expensive phase in the FDA's drug approval process.

The safety and effectiveness of interferon in the MPNs has been confirmed by many peer-reviewed and published clinical studies conducted in both the U.S. and Europe, as well as by decades of clinical use "off label." I asked if the FDA could approve its use in the MPNs based on this data. FDA approval would have two important benefits. First, it would encourage wider use of the drug by giving interferon greater visibility among hematologists who do not treat a large number of MPN patients. Second, it would simplify the process of reimbursement from patients' insurance companies.

We were disappointed in the answer, which can be summed up in Dr. Kleutz's words: "It's not our drug." Essentially, the FDA can't approve a drug without an application for approval. So our next question was: "Could the FDA help us as advocates to develop a roadmap for cost-effective approval that we could discuss with the manufacturers?" That's pretty tricky from the FDA point of view, so they are thinking about it. They did note that one barrier has been removed: The $2 million fee previously imposed on pharmaceutical companies entering into the FDA approval process has been eliminated for orphan drugs.

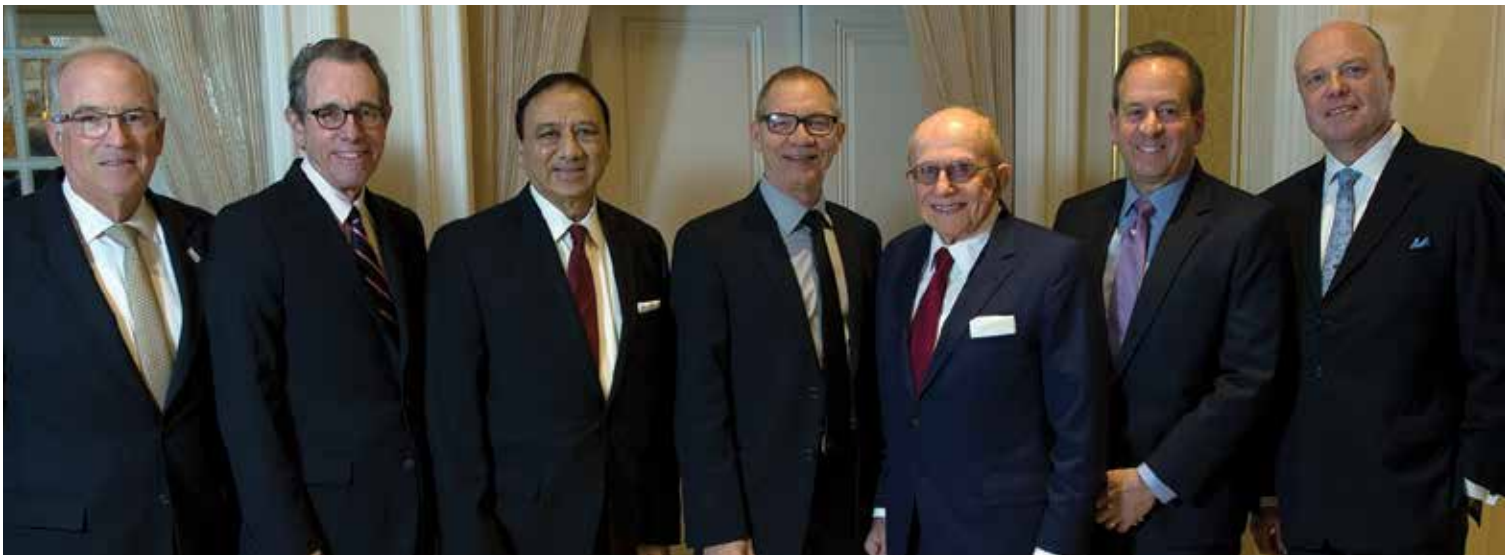
The FDA's answer on using data gathered through MyMPN was an unequivocal yes.

The FDA's answer on using data gathered through MyMPN was an unequivocal yes. The agency has already started a process of gathering and analyzing patient-reported outcomes and other patient data to determine if they can use it productively.

All agreed that the meeting was a valuable event for both the FDA and the patient advocates, and expressed a desire to have another mini-symposium where we could do a deeper dive on a couple of key topics. We will be sure to keep you up to date about these important conversations.

2017 Hall of Fame Dinner

A Celebration of Courage and Philanthropy



L-R: Richard Rose; David Boule; Bharat Shah, Humanitarian Honoree; Mack Dryden, Cancer Survivor Honoree; Dr. Richard Silver; Thomas Silver; and Dr. Richard Woodman of Novartis Pharmaceuticals.

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 14, nearly 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. **Raphael Miranda**, the Emmy Award-winning meteorologist for NBC 4 New York, again served as our master of ceremonies.

This year's dinner launched a year-long commemoration of CR&T's 50th Anniversary, which will take place in 2018.

David Boule, Immediate Past President, encouraged CRT's friends to support our 50th Anniversary Campaign (see page 6).

Bharat Shah, **PhD**, philanthropist and author of *America My Destiny,* received CR&T's Humanitarian Award. In 2000, he was told that he had chronic myeloid leukemia and that he had six months to three years to live. His family's research led them to CR&T's founder, **Dr. Richard Silver**, who was conducting a clinical trial of a new CML drug. This drug, known as imatinib, has since revolutionized CML treatment. Today, Bharat is leading a healthy, active life and supports many charities in the U.S. and India. Every year, he joins Dr. Silver at Weill Cornell, where he speaks to medical students about his cancer journey. **Dr. Richard Woodman**, SVP and Head, US Clinical Development and Medical Affairs at Novartis Pharmaceuticals, which produced imatinib, congratulated Bharat and spoke about the impact of the drug on the cancer community.

Richard Rose, former CR&T President and current board member, introduced **Mack Dryden**, our 2017 Cancer Survivor honoree. A speaker, comedian and author, Mack won two Associated Press writing awards as a reporter in his home state, Mississippi. He then became a professional comedian, and has appeared in several movies and hundreds of TV shows, including "The Tonight Show" with both Johnny Carson and Jay Leno. A hilarious motivational speaker who has survived both testicular and eye cancer, Mack now brings his message of hope and humor to thousands of people each year.

CR&T's President, **Thomas Silver**, thanked the dinner committee, led by **Barbara Silver**, for their hard work, and acknowledged the commitment of our board of directors and Medical Advisory Board. "Most importantly," he told the audience, "I want to thank you – our friends and supporters – for being part of the CR&T family and for your incredible generosity."

All proceeds from the Hall of Fame Dinner benefit CR&T's support of cutting-edge research that seeks effective treatments and cures for cancer.

From left: Milan and Bharat Shah, surrounded by their family.

All proceeds from the dinner benefit CR&T and its mission

CR&T's Young Professionals group

Mack Dryden and Tom Silver

Raphael Miranda

MESSAGE FROM THE PRESIDENT

Dear Friends,

This past November, at our Hall of Fame Dinner, we launched a year-long commemoration of CR&T's 50th Anniversary. Dr. Richard T. Silver, founded our organization in 1968, one of the most turbulent years in American history. But it was also a year of life-changing technological and medical advances. Apollo 8 became the first manned spacecraft to orbit the moon, and the 911 Emergency Phone Service was launched. The year also saw two medical breakthroughs: the first successful heart transplant, and the first bone marrow transplant to treat severe immunodeficiencies.

Although cancer researchers had made some landmark discoveries, a host of major milestones lay in the future – and CR&T was to be a vital part of that future. For nearly five decades, we have played a key role in advancing research into blood, breast and lung cancer, and we will not stop until cures have been found.

I hope you will consider supporting our 50th Anniversary Campaign

50th Anniversary Campaign

None of our achievements would have been possible without the generous support of friends like you. Now, I hope you will consider supporting our 50th Anniversary Campaign, which will fund the next phase of our most important initiative: building the world's leading center dedicated to the study and treatment of the myeloproliferative neoplasms (MPNs).

In 2011, CR&T made a multi-year, multi-million-dollar pledge to expand the Richard T. Silver, MD MPN Center at Weill Cornell Medicine. The Silver Center's vision is to integrate:

- Outstanding medical care for MPN patients,
- Clinical trials to test new drugs, and
- Cutting-edge laboratory research to discover new approaches to control and cure these blood cancers.

Led by Dr. Andrew Schafer, the Director; Dr. Joseph Scandura, the Scientific Director; and Dr. Silver, the Director Emeritus, the Center has become a hub for innovative research collaborations. Over the past few years, CR&T has provided nearly $1 million annually to the Center. Now, we plan to increase our funding to support existing staff and laboratory research, and a new Clinical Director, who will lead clinical research and expand patient care capability.

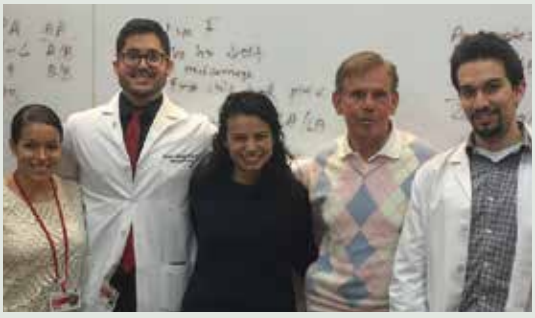
Our support will allow Dr. Scandura and his team to widen the scope of their research, including participation in an unprecedented new collaboration, the MPN Interferon Initiative. As noted in our cover story, interferon is the only drug that has disease-altering effects in the MPNs. Pioneering research into the use of this drug has been one of Dr. Silver's major achievements. However, we still have much to learn about how and why the drug works. This initiative brings together world experts in interferon, MPNs, and solid tumors, who will generate a broad and deep understanding of this issue. The expert Advisory Group that will guide this effort includes Drs. Schafer and Silver.

As we move forward during our 50th Anniversary year, you'll learn more about these and other crucial projects. On behalf of everyone at CR&T, thank you for being part of our long history of achievement – and for helping us build a brighter future for cancer patients and their families.

Thomas M. Silver, President

CR&T Supports Professional Education

Teaching rounds enable specialists to present case studies and treatment options to residents, fellows, and medical students. With partial support from CR&T, two distinguished experts gave lectures and then conducted teaching rounds at Weill Cornell Medicine in late 2017.



Dr. Gale (2nd from the right) with attendees

On November 14, Animesh Pardanani, MBBS, PhD, discussed treatment options after JAK inhibitor failure in myelofibrosis. Dr. Pardanani is a hematology consultant and professor of medicine at the Mayo Clinic College of Medicine. His research explores the application of new diagnostic and treatment approaches in hematological cancers.

On December 5, Robert Peter Gale, MD, PhD, Celgene Corporation's Executive Director, Clinical Research-Hematology/Oncology, lectured on precision oncology. This rapidly developing field looks at the molecular and biologic characteristics of each cancer patient, and tailors treatment accordingly.

CR&T is proud to support programs that further the education of medical professionals at every stage of their career.

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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 2017. For the complete list, please visit www.crt.org.

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NOW ONLINE!

2017 MPN PATIENT SYMPOSIUM VIDEOS

CR&T was proud to host the 9th International Patient Symposium on Myeloproliferative Neoplasms on Tuesday, November 1, 2017, at Weill Cornell Medicine in New York City. This full-day educational event allows patients and caregivers to learn about the latest developments in MPN research and treatment.

The program featured eight presentations by distinguished basic scientists and clinical research physicians from the world's leading MPN centers. Now, you can view these videos online at: **www.crt.org//9th-International-Patient-Symposium-on-Myeloproliferative-Neoplasms**.

Front row (l-r): Drs. Richard T. Silver, Alison Moliterno, and Jason Gotlib with David Boule, Patient Committee Chair; Back row (l-r): Drs. Carlos Besses, Koen van Besien, Andrew Schafer, Jerry Spivak, and Ronald Hoffman. Not pictured: Dr. Ruben Mesa.

This program was made possible by the generous support of corporate and individual sponsors:

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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.