CR&T News
Cancer Research & Treatment Fund, Inc.
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Impact of Anticompetitive Practices on Prescription Drug Prices

About 77 percent of Americans believe that prescription drug prices are too high, according to a poll from the Kaiser Family Foundation. This belief has been fueled by recent controversies, such as Turing Pharmaceuticals' 5,000-percent price hike for Daraprim, a life-saving AIDS drug that has no generic equivalent. Because the burden on cancer patients is especially high, with many new drugs priced at $100,000 a year, this is an issue of great concern to CR&T's leadership. To learn more about the factors that contribute to high drug prices, we invited Michael Carrier, Distinguished Professor at Rutgers Law School, to speak at the June 15th meeting of our Board of Directors and Medical Advisory Board.

A leading authority in antitrust and intellectual property law with expertise in the pharmaceutical industry, Prof. Carrier is the author of books and articles in leading journals, and is frequently interviewed by major media outlets. At the CR&T meeting, he focused on the strategies that brand-name drug companies use to delay or prevent the availability of generic drugs. Prof. Carrier's comments on anticompetitive practices are the basis for this article.

A 1984 federal law, the Hatch-Waxman Act, was designed to strike a balance between encouraging industry to develop innovative new drugs and accelerating the availability of lower-cost generic therapies. The average cost to successfully develop a new prescription drug is nearly $2.6 billion. In recognition of this major investment and the risks involved in drug development, the patents on brand-name drugs in the U.S. typically remain in force for 20 years. Once a patent expires, however, other companies can manufacture the same drug and market it as a generic, thereby increasing competition and lowering costs.

Hatch-Waxman sped up the approval process for generic drugs by allowing manufacturers to file an Abbreviated New Drug Application (ANDA) with the Food and Drug Administration. Since the FDA has already approved the

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Dr. Richard Silver

New Study Supports Interferon Treatment for Early Myelofibrosis

Myelofibrosis is a chronic blood cancer that causes scar tissue to form in the bone marrow, impeding the formation of normal blood cells. Over time, the disease progresses, causing a range of symptoms, from anemia and fatigue to thrombosis. Although researchers from the U.S., Scandinavia, and France have been evaluating the use of interferon alpha in myelofibrosis for many years, the standard treatment has been to "watch and wait" until a patient develops symptoms. The results of a new CR&T-funded study, published in May 2017 in *Cancer,* a journal of the American Cancer Society, support the treatment of early myelofibrosis with interferon alpha.

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Michael Carrier

brand-name drug, no additional clinical trials are required. Instead, the generic manufacturer must run tests to prove that its product is identical to the original drug.

Unfortunately, many health advocates believe that pharmaceutical companies have found loopholes in the Hatch-Waxman Act that allow them to prevent generic drugs from entering the market. These anticompetitive practices increase the drug prices paid by patients, governments, and insurance companies.

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To address this issue, the FDA is working on a Drug Action Plan. This past July, as part of this effort, the agency held a public meeting to solicit input on the Hatch-Waxman Act. Prof. Carrier was one of the experts who spoke at the meeting.

Anticompetitive practices include:
Pay-for-Delay. Under Hatch-Waxman, the first generic manufacturer to file an ANDA before a branded drug's patent expires gets 180 days of exclusive rights to market the generic product. The ANDA must include a patent challenge, which claims that the patent is not valid or that it would not be infringed by the generic drug.

This provision of the law was intended to encourage competition by bringing generic drugs to the market as quickly as possible. Unfortunately, in practice, it often results in "pay for delay" settlements, in which the company holding the patent pays the generic manufacturer to withdraw the challenge and delay entering the market. Both parties benefit from this arrangement: The company that produces the branded drug maintains its exclusivity, and the generic manufacturer receives a significant financial settlement for keeping the product off the market.

Product Hopping. State laws allow pharmacists to recommend that a generic drug be substituted for a brand-name product if the two drugs are the same: if they are absorbed into the body at the same rate (i.e., are bioequivalent), have the same dosage, and are equally safe and effective. The manufacturer of a branded drug can work around this by pulling its product off the market shortly before the patent expires, and then introducing a new formulation with minor changes. For example, the dosage may be changed from twice a day to once a day, or the drug may be administered as a tablet instead of a capsule. While these changes are not therapeutically significant, they may prevent pharmacists from substituting the generic product.

Citizen Petitions enable individuals and community organizations to bring their concerns about a product's safety to the FDA. However, this process also has become a way for pharmaceutical companies to block generic drug approvals. In 2016, Dr. Carrier and colleagues published a study showing that, between 2011 and 2015, 92 percent of FDA citizen petitions were filed by brand-name drug manufacturers. Although the FDA ultimately denied most of these petitions, this time-consuming process proved to be an effective delaying tactic. Under a new rule issued in November of 2016, the FDA has no more than 150 days to take action on any petition that might delay the approval of a pending new drug application. The only exception would be the agency's determination that a delay is necessary to protect the public health.

Preventing Access to Drug Samples. Without test samples of the brand-name drug, a generic manufacturer is unable to prove that its product is identical. Usually, about 1,500 to 3,000 doses of the original product are needed. Sometimes the brand company seeks to delay the approval process by refusing to sell samples to the generic company, on the grounds that the request violates FDA safety requirements. As a basis for denying access, the patent holder may point to the FDA guidelines known as REMS (Risk Evaluation and Mitigation Strategies), which include guidance on implementing secure distribution practices for risky drug products.

What can be done to prevent anticompetitive behavior? This is a complex issue, since federal patent and antitrust laws, the Hatch-Waxman Act, and state legislation all come into play. Lawsuits charging violation of antitrust laws may be brought against companies on a case-by-case basis. In addition to examining the need to take more forceful measures that lie within its own scope, the FDA has indicated that it may need to collaborate with the Federal Trade Commission, whose mission is to prevent anticompetitive business practices. In addition to addressing the issues discussed above, this would help prevent companies from purchasing the distribution rights to a drug and then raising prices simply because they can – as was the case with Daraprim. Other solutions – which would not only pertain to generics – include allowing Medicare to negotiate drug prices; establishing a review process to ensure that drugs are priced fairly, based on the value of the treatment to patients; and allowing drugs to be imported across U.S. borders for personal use. (Identical drugs sometimes cost half the price in Canada.)

CR&T will continue to provide updates on this important topic as new information becomes available.

MESSAGE FROM THE PRESIDENT

At this year's Cancer Survivors Hall of Fame Dinner, we will launch a year-long celebration of CR&T's 50th Anniversary. Since 1968, we have been committed to advancing research that promises new treatments and cures for cancer. Thanks to your generous support, CR&T-funded investigators have:

- Conducted the initial clinical studies leading to the FDA's approval of imatinib (Gleevec®), a revolutionary drug used to treat chronic myeloid leukemia;
- Participated in the development of new therapies for Hodgkins' and non-Hodgkin's lymphoma;
- Contributed to the understanding of how and why breast cancers spread, and to the development of new breast cancer treatments;
- Identified the processes of blood cell formation, which led to new anti-antiogenesis treatments for blood diseases and solid tumor cancers;
- Discovered the molecular mechanism that causes some patients to be resistant to ibrutinib, an important drug used to treat mantle cell leukemia; and
- Contributed to the use of interferon alpha and other biologics in treating MPNs.

Since 2011, we have focused on the expansion of the Richard T. Silver, MD Myeloproliferative Neoplasm (MPN) Center at Weill Cornell Medical College. By 2019, we will have committed a total of $4.7 million to building the world's leading center dedicated to MPN research and treatment.

This support is generating important contributions to the field. Of particular note, in May 2017, the results of two key studies conducted by Silver Center investigators appeared in highly respected medical journals. *Cancer* published a paper by CR&T's founder, Dr. Richard Silver, and colleagues, which demonstrated the effectiveness of the drug interferon in early myelofibrosis (page 1). On the basic research front, Drs. Joseph Scandura, the Silver Center's Scientific Director, and Shahin Rafii, a member of our Medical Advisory Board, were part of a team that has discovered a way to create an unlimited supply of healthy stem cells by reprogramming cells that line the blood-vessels. This "first," which was featured in *Nature*, could lead to the development of new therapies for the MPNs, leukemia, and genetic blood diseases such as sickle cell anemia.

We look forward to keeping you up-to-date on the many other exciting projects underway at the Silver Center, as well as the progress of two current CR&T-funded studies that focus on new approaches for diagnosing and treating prostate cancer.

This will be the final issue of *CR&T News* for 2017, so I'd like to close by asking a favor: Please consider making a year-end gift to CR&T. There are so many ways to help us fulfill our mission: Join us for our Hall of Fame Dinner on November 14 (back cover) or donate via our website (www.crt.org) or by using the enclosed envelope. At this time of year, a tribute or memorial gift would be an especially meaningful way to acknowledge or remember a special person in your life.

On behalf of all of us at CR&T, thank you for your support over the years – and warmest wishes to you and yours for a joyous holiday season and a happy and healthy New Year.

Sincerely,

Tom

Thomas M. Silver, President

Interferon for Early Myelofibrosis (Continued from page 1)

This clinical trial, which was led by CR&T's founder, Richard T. Silver, MD, and colleagues, followed 30 low- and medium-risk patients with early myelofibrosis who were treated with low-dose interferon. Of this group, 73 percent improved or remained stable, and 37 percent achieved complete or partial remission. The median duration of treatment was 5.6 years, and the longest-living patient has been treated with interferon for 25 years.

This research is exciting for two reasons. It is the first study to systematically demonstrate the effectiveness of interferon in early myelofibrosis, employing a multi-specialty international group of experts that included clinicians, molecular biologists, hematopathologists, and statisticians. In addition, the study underscores the importance of molecular profiling, also known as personalized medicine. This helps physicians to predict how a patient will respond to therapy by pinpointing the specific gene mutations or biomarkers found in that person's cancer cells or tumor at the time of diagnosis. To read an abstract of the study, visit **http:/onlinelibrary.wiley.com/doi/10.1002/cncr.30679/full**.

CR&T is deeply grateful to the Johns Family Fund and the William and Judy Higgins Trust for their support of this important research.

RESOURCES FOR PATIENTS AND CAREGIVERS

The Internet offers a wealth of information on every aspect of cancer treatment and care. Below is a list of trusted resources that can help you.

General Information

- The National Cancer Institute (www.cancer.gov) is the federal government's principal agency for cancer research and training. This site provides comprehensive information about all types of cancer.
- The American Cancer Society (www.cancer.org) offers detailed information, as well as online community resources to connect with other people affected by cancer.
- Disease-specific Organizations, such as the MPN Research Foundation (www.mpnrf.org), the Leukemia & Lymphoma Foundation (www.lls.org), and the Prostate Cancer Foundation (www.pcf.org) are great sources of information and support. Professional associations such as the American Society of Hematology (www.hematology.org) and the American Society of Clinical Oncology (www.asco.org) feature resources for patients on their websites.

Financial Assistance

- Cancer Care (www.cancercare.org): Professional oncology social workers provide free services for people with cancer, caregivers, loved ones and the bereaved. Services include counseling; in-person, online, and telephone workshops; and financial and co-pay assistance.
- Cancer.Net (http://www.cancer.net/navigating-cancer-care/financial-considerations/financial-resources): This page provides a comprehensive list of national and local organizations that provide help with transportation, housing, and medical treatment. Especially helpful is an extensive list of drugs, with contact information for the manufacturers' financial assistance programs.

Clinical Trials

- www.clinicaltrials.gov, a service of the National Institutes of Health, is a registry and database of publicly and privately supported clinical studies conducted around the world. You'll also information about how clinical trials are conducted, and links to other resources.
- 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.

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

LIMITED SEATING AVAILABLE – REGISTER TODAY!

9TH INTERNATIONAL PATIENT SYMPOSIUM ON MYELOPROLIFERATIVE NEOPLASMS

Wednesday, November 1, 2017

Griffis Faculty Club, Weill Cornell Medical College
521 East 68th Street • New York, NY 10065

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 internationally respected basic scientists and clinical research physicians. Topics include current and new therapies, clinical trials, developments in stem cell transplantation, and natural history and heredity. You'll also participate in disease-specific Q&A groups that allow you to speak one-on-one with the experts.

Sign up for the Symposium and receive **complimentary** registration for the 10th International Congress on Myeloproliferative Neoplasms, a two-day professional program that will be held from November 2-3, at the Intercontinental New York Times Square (www.10thIntMPNcongress.com).

To learn more and register, visit www.crt.org/9th-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 $150 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)

Name _____

Address _____

City _____ State _____ Zip _____

Phone _____

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Guest: _____

Guest: _____

Please Select Breakout Group:

___Polycythemia Vera ___Myelofibrosis ___Essential Thrombocythemia

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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.
50 Years of Achievement | 1968-2018

CR&T INVITES YOU TO THE

CANCER SURVIVORS HALL OF FAME DINNER

Tuesday, November 14, 2017

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

Please join us as we launch our 50th Anniversary year and honor outstanding individuals for their dedication and accomplishments. All proceeds benefit CR&T, enabling us to accelerate the discovery of life-saving treatments and cures for cancer.

Bharat Shah, PhD
Philanthropist and author of
America My Destiny
Humanitarian Honoree

Mack Dryden
Award-winning writer, comedian, actor
Two-time cancer survivor
Cancer Survivor Honoree

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

Cancer Research & Treatment Fund, Inc.
Established 1968

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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.