

MAJOR ADVANCES SINCE 2000, THANKS TO YOUR SUPPORT

By Andrew Schafer, MD

Dr. Schafer is Chairman, MPN Research Foundation Scientific Advisory Board; Chairman of Medicine, Weill Cornell Medical College; E. Hugh Luckey Distinguished Professor of Medicine, Weill Cornell Medical College; and a true friend of the MPN community.



Since 2000, the MPN Research Foundation has provided nearly \$9 million in research funding to advance the treatment of the myeloproliferative

**The MPN Research Foundation
has provided key support for
virtually every major advance in
MPN research since 2000.**

— Andrew Schafer, MD

neoplasms (essential thrombocythemia, polycythemia vera and myelofibrosis.) It is a modest amount relative to the magnitude of the problem and the scale of research needed to tackle it. But the Foundation has had a remarkable track record of success in optimally allocating these limited funds to achieve the highest impact for MPNs.

(continued on page 4)

REPORT FROM ASCO, JUNE 2013

The American Society of Clinical Oncologists recently wrapped up its annual meeting in Chicago. The meeting brought together over 25,000 oncology professionals, drug companies, advocacy folks and journalists striving to understand what's new in cancer research and treatment. We were there, too.

The MPN Research Foundation met with about 9 different pharmaceutical companies working on drugs in our space.

Significant major news relating to the MPNs included the announcement by Incyte that Jakafi is showing a clear positive impact on fibrosis in many MF patients. Bone marrow biopsies from patients who have been on this drug for up to 48 months have in some cases shown a reversal of progression and in other cases a stabilization of fibrosis.

Our field remains crowded with pharmaceutical firms vying to provide improved versions of JAK inhibitors. Other more novel mechanisms of action are either in early stage clinical trials or headed toward trials later this year.

Sanofi hopes to apply for FDA approval later this year for its JAK2 inhibiting drug, and Promedior hopes to start trials soon for a novel drug that has shown efficacy in halting and perhaps reversing the fibrosis process in Pulmonary fibrosis. There is reason to believe – but no proof so far – that it will be effective in bone marrow fibrosis.

The next conference to produce major MPN news will be ASH, in December 2013.

MISSION

The primary mission of the MPN Research Foundation is to stimulate original research in pursuit of new treatments — and eventually a cure — for myeloproliferative neoplasms (MPNs). In addition, the MPN Research Foundation promotes collaboration in the scientific community to accelerate research, and serves as a powerful patient advocacy group for patients and their families.

OFFICERS AND DIRECTORS OF
THE MPN RESEARCH FOUNDATION

Robert Rosen

Chairman

Barbara Van Husen

President

Felisse Sigurdson

Vice President

Celia Miltz

Secretary

David Boule

Treasurer

Jen Bealer

Stephanie Cindric

Molly Guy

Robert Horwitz

Sam Klepper

JoAnn Mason

Ed Ogunro

David Ricci

Jeff Shier

SCIENTIFIC ADVISOR

John Crispino, PhD

Northwestern University Feinberg School
of Medicine

SCIENTIFIC ADVISORY BOARD

Andrew Schafer, MD

Weill Cornell Medical College

Connie Eaves, PhD

BC Cancer Research Center

Jonathan Licht, MD

Northwestern University Medical School

Hal Broxmeyer, PhD

Indiana University School of Medicine

Hugh Rosen, PhD

The Scripps Research Institute

MORE PROGRESS THAN YOU MIGHT THINK, ON THE LONG ROAD TO A CURE

By Robert Rosen

Sometimes I lie awake at night worrying about whether the MPN Research Foundation is actually making a difference. Yes, we have made research grants of almost \$9 million to both up-and-coming researchers and leading authorities in myeloproliferative neoplasms. But despite all the effort, we still seem to be a long and frustrating way from a cure.



In the course of a conversation with our distinguished friend Dr. Andy Schafer, I mentioned that sometimes I don't really know how much we are accomplishing with the grants that the foundation awards. He gave me a funny look, and promptly offered to write up an unbiased review of our achievements. It certainly made me feel better, and I hope it will remind all our donors that their money is being invested well and is genuinely benefiting MPN patients, their families and caretakers – even though we still have a long way to go. Dr. Schafer's article begins on page 1.

And soon we'll be able to share even more good news about the achievements of the researchers we support. In the last month or so, we have received final reports from the grants we funded in 2011, for projects conducted in 2012. Andy Schafer and our Scientific Advisor, John Crispino evaluated the results, and they are pretty terrific.

I'd say that five out of the six projects have made valuable contributions to our MPN knowledge, and two compounds may soon start clinical trials.

None of the results have been published yet, but we will give you the details as soon as they are released. And remember – none of it would have happened without your support.

WITH A LITTLE HELP FROM HER FRIENDS

By Raquel Nuñez

Annette De Bow has done it again! Her 2013 Trek For A Cure sets a new third party event donation record for the MPN Research Foundation.



Annette De Bow

It means a lot. It means a lot for a person to donate his or her own time. It means a lot to demonstrate dedication, determination, and strength. It means a lot to take on a challenge that not many others have conquered in the name of finding a cure for MPNs. That's why Annette De Bow means a lot to the MPN Research Foundation and the entire MPN community.

We would like to thank Annette and everyone who participated in the 2013 Trek For A Cure for raising over \$30,000 to support the MPN Research Foundation's initiative in finding a cure for MPNs.

I was lucky enough to participate in the 2013 Trek For A Cure, and I witnessed a truly inspirational group comprised of entire families, friends, artists, doctors, teachers, musicians, children, trekkers, and MPN patients. They gave selflessly of their time and passion to further the Foundation's mission of finding a cure for all Myeloproliferative Neoplasms. As a first line of defense against these diseases, the Foundation relies heavily on individual donations and fundraising events like Annette De Bow's Trek For A Cure. These events raise awareness

about MPNs by building unity and strengthening community from the ground up.

As a community, each of you can give your voice, your talents, your skills, and advocacy to support the MPN Research Foundation's mission of promoting, funding, and supporting the most innovative and effective research into the causes and treatments of these diseases, with the ultimate goal of accelerating the discovery of a cure. Not only does it mean a lot to everyone here at the Foundation, it means a lot to the entire MPN community. If you are interested in organizing an event that supports the MPN Research Foundation, please contact William Crowley at 312-683-7226 or via e-mail at wcrowley@mpnresearchfoundation.org.



Participants rally for a three-day, 24 mile segment of the trek.

MAJOR ADVANCES

(continued from page 1)

It has done so by funding groundbreaking projects that are now receiving an enormous amount of additional support from other private and federal sources, such as NIH. It has also helped launch the MPN research careers of our currently most productive investigators in the field, as well as incentivizing already successful scientists in related areas to turn their attention to curing the MPNs.

In other words, the return on investment and the multiplier effect of the Foundation's research grant program have been extraordinary. Some of the following examples are illustrative. (The years in parentheses following an individual researcher's name represent the period of their support from the MPN Research Foundation.)

Dr. Josef Prchal (2000-2002) used Foundation support to become the first to discover a loss-of-heterozygosity of chromosome 9q in polycythemia vera. This led to the subsequent discovery of JAK2-V617F, the diagnostic mutation which is now known to be located on chromosome 9q.

Dr. Robert Kralovics (2011-2013), a student of Dr. Prchal, subsequently became himself a major innovator in MPN research and has received independent Foundation support to advance the genomics of MPN. Dr. Kralovics was among the first to identify the JAK2-V617F mutation in a widely cited, first-authored article in the *New England Journal of Medicine*.

Dr. Gary Gilliland (2006-2008) made the discovery of JAK2-V617F simultaneously and used Foundation support to pioneer our understanding of the genetic basis of MPNs. Dr. Gilliland, in turn, trained **Dr. Ross Levine** (2011-2013), discoverer of the MPLW515 mutation of the thrombopoietin receptor, as well as **Dr. Ben Ebert** (2011-2013), and **Dr. Ann Mullally** (2012-2013).

Dr. Francois Delhommeau (2009-2011) worked in Dr. William P. Vainchenker's lab, where he was involved in developing a novel liquid culture system to grow polycythemia vera erythroid cells in the absence of added growth factors; this was the technical advance that allowed the development of JAK2 inhibitors. Dr. Delhommeau has been

searching for the "driver mutations" of MPNs and reported in *The New England Journal of Medicine* during his Foundation funding period that TET2 mutations in the stem cells of MPN patients precede the JAK2-V617F mutation.

Dr. Ronald Hoffman (2003-2011) made several key discoveries during his Foundation funding period in defining the molecular underpinnings of the MPNs and then using this basic knowledge to develop and test novel agents in preclinical studies.

Dr. Ayalew Tefferi (2006-2011) established the leading biobank for peripheral blood and bone marrow samples from consenting MPN patients, which has been used as the indispensable core facility for genetic studies of MPNs by investigators throughout the world.

Dr. Ruben Mesa (2003-2005), who was trained by Dr. Tefferi, is making the first breakthroughs in the treatment of myelofibrosis, including leading the investigation of the clinically effective JAK1 and JAK2 inhibitor, ruxolitinib.

During the past two years, the Foundation has partnered with the **Leukemia and Lymphoma Society** to tackle head-on, for the first time, the problem of myelofibrosis (MF), perhaps the most poorly understood and treatment-refractory of all the MPNs. We have been frustrated by the lack of substantive progress in even understanding this disease. Therefore, two successive grant funding opportunities that we named the "MF Challenge" solicited the exploration of entirely new ideas about the cause and treatment of myelofibrosis. We attracted dozens of outstanding grant applications, and some potentially groundbreaking proposals, from both established and young scientists.

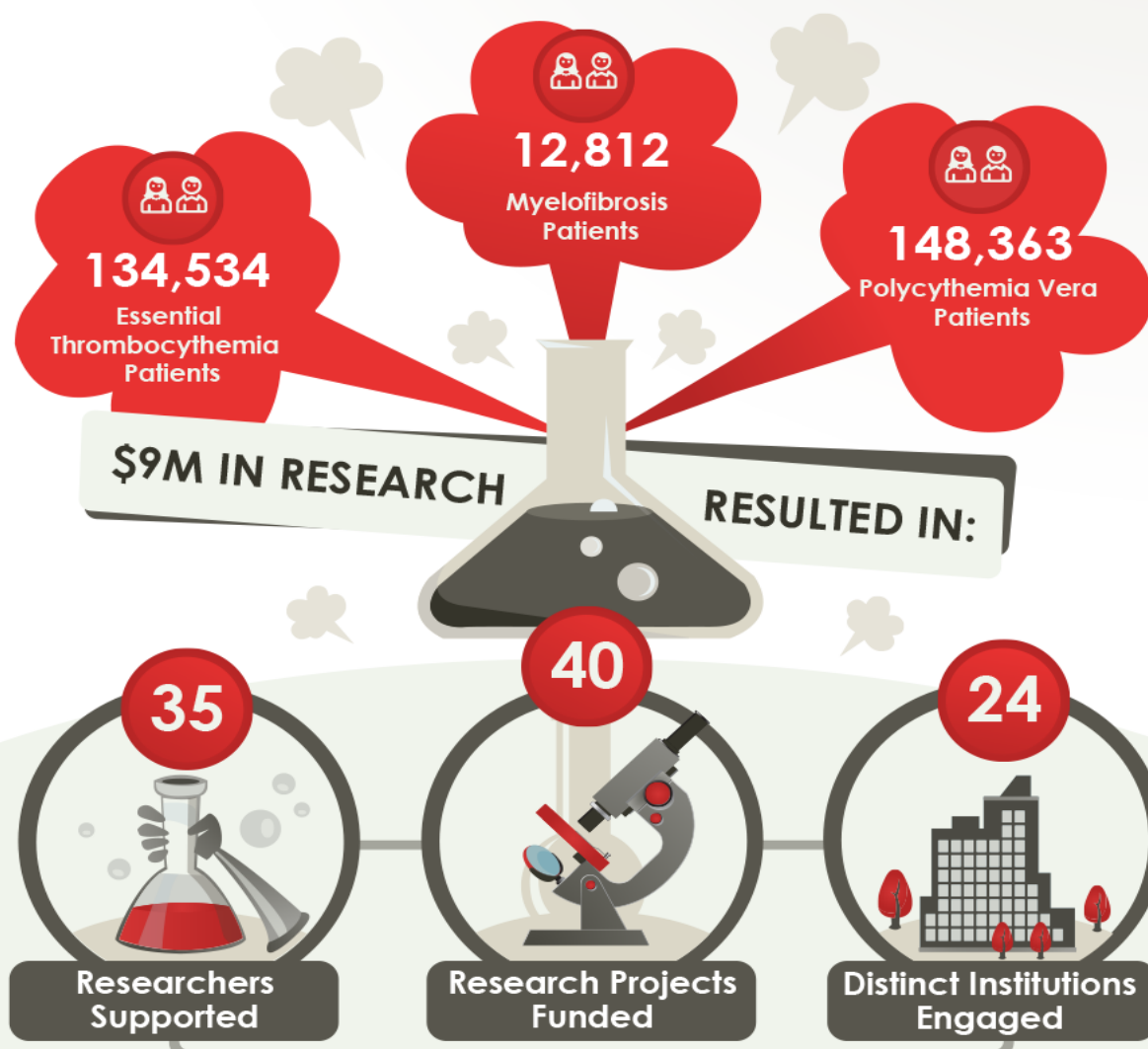
These examples also highlight the multiplier effect of senior investigators superbly training the next generation of MPN scientists, who have themselves already begun to make their independent contributions and have begun to win competitive Foundation grants.

Plain and simple, the MPN community needs more of what the MPN Research Foundation and its supporters are doing so well.

MPN BY THE NUMBERS

Channeling the strength of patients

In 2000, the MPN Research Foundation was founded on the idea that people living with Myelofibrosis, Polycythemia Vera, and Essential Thrombocythemia COULD and SHOULD do something to drive research towards these rare blood cancers. The Foundation directs their funds raised by patients, their friends, and families, towards the projects that offered promise of getting closer to a cure. This is an unprecedented time of interest from academia and bio-pharma, but we have far to go until the last patient is cured. We want all patients to stand up today and join us.



We've had 12,000 donations from 7,000 donors since inception.
Want to help us grow that number?



Learn how at mpnresearchfoundation.org

AN MPN ALL-STAR: JEN BEALER'S BIG BIKE TOUR

In 2001, Jen Bealer lost her Grandma to a Myeloproliferative Neoplasm. Nearly five years ago, her mom had a platelet count over four times higher than normal, and she has since been diagnosed with an MPN.

Jen's grandma had only one treatment option, which had severe complications. Now, 12 years later, Jen's mom is being successfully treated. So the Bealers know first hand how important it is to find better treatments for MPNs, and they support the MPN Research Foundation in every way they can.

Jen and her brother, along with their awesome friends, rode 40 miles for a cure in the Five Boro Bike Tour in New York City on May 5, just in time for Mother's Day! They raised over \$13,000 for MPN research.

Join Jen and Become an MPN All-Star

Challenge yourself to help find better treatments and a cure for MPNs by signing up to be an All-Star. Go the distance in the fight against these devastating diseases by training for and completing an endurance challenge while raising funds to support the MPN Research Foundation. Contact William Crowley for help planning your next event: wcrowley@mpnresearchfoundation.org

As an All-Star, you will have access to fundraising support and, most importantly, you will join a community determined to leave its mark in the fight against MPNs.

Seven Ways to Help If You Are Not Interested in an Athletic Challenge

Pledge your next birthday. It's simple. Go to www.mpnresearchfoundation.org/birthdays. When your birthday is near, we will remind you to start a fundraising campaign. You will ask your friends and family to donate to find a cure for MPNs in honor of your big day. The money raised will fund cutting-edge research projects.



Jen Bealer and her brother – both of them clearly winners for the MPN community.

Dinner party. Ask your guests to donate what they would have spent on a meal and drinks at a restaurant.

Golf. Ask your foursome to donate to the MPN Research Foundation. By giving what they would already spend for a day on the links, they'll enjoy each other's company – and learn more about how they can become MPN All-Stars.

Neighborhood block party. Set up games like cornhole, badminton or volleyball. Collect donations to play or entry fees for teams.

Movie night. Charge "admission" or pass around an empty popcorn tub for donations.

Garage sale. Donate half the proceeds – and let your buyers know it!

Poker or bridge. Split the winning pot with the MPN Research Foundation. Maybe the winner will donate back to the cause!

HOW MANY OF US ARE THERE?

Researchers from Mayo Clinic, Boston University and the pharmaceutical company Sanofi recently concluded a study of the prevalence (the total number of patients) and incidence (the number of new diagnoses) of the myeloproliferative neoplasms. They reported their findings at the American Society of Hematology annual meeting in 2012 and placed the total number of MPN patients in the United States at almost 300,000.

They used two major US health insurance claims databases (with over 70m enrollees/year) to retrospectively identify unique patients with primary myelofibrosis, secondary myelofibrosis, polycythemia vera and essential thrombocythemia between 1/1/08 and 12/31/10. Using the Marketscan estimates from 2010, they estimated the prevalence of MPNs in the US at:

- MF – 12,812
- PV – 148,363
- ET – 134,534

Additional research using other national databases and/or study designs is needed to substantiate these findings. But the data provide compelling evidence that the prevalence of MPNs is higher than has been reported in the past.

Even so, each of the MPNs (taken separately) can still be classified as an orphan disease, which is defined as a disease that affects fewer than 200,000 patients in the US.

This is important to drug developers – and patients – because the government goes out of its way to encourage the development of orphan drugs by offering an accelerated approval process, extended patent exclusivity and other financial incentives.

MPN Research Foundation Update

is a periodic newsletter published by the MPN Research Foundation to provide members of the MPN community with information on current research and the Foundation's activities.

2013 MF CHALLENGE GRANTS AWARDED

The MPN Research Foundation and the Leukemia & Lymphoma Society are proud to announce the winners of the second round of the MF Challenge grant program. Winners of these one-year concept grants focusing on fibrosis and myelofibrosis are:

- John Varga and Jonathan Licht (Northwestern University)
- Golam Mohi (State University of New York)
- Emmanuele Passegue (University of California at San Francisco)
- Xiaoli Wang (Mt. Sinai School of Medicine)

Concept grants such as these are intended to identify innovative and novel approaches to an issue. As in the first round of MF Challenge grants, each researcher will receive \$100,000 over one year to try to develop a proof of concept for ideas on how to stop or reverse fibrosis. The grant review was conducted by a multi-disciplinary group overseen by the MPN Research Foundation's Scientific Advisory Board Chair, Dr. Andrew Schafer, from Weil Cornell.

The first round of MF Challenge concept grants was funded in July 2012. We'll update you on the progress of these four projects toward the end of the year. Projects with potential to go further will be candidates for longer term funding.

The potential of the MF Challenge is its ability to stimulate research in areas that are currently untested in fibrosis and myelofibrosis, offering hope to patients struggling with a relative lack of treatment options and understanding about their disease.

Update Editorial Staff

Woody Woodruff, Editor
Susan E Rinehart, Layout Editor

MPN Research Foundation

180 N. Michigan Avenue, Suite 1870
Chicago IL 60601
Tel (312) 683-7249 • Fax: (312) 332-0840
www.mpnresearchfoundation.org

BLOOD CANCER AWARENESS MONTH AND OTHER AUTUMN BEGINNINGS

By Molly Rosen Guy

Hi! Molly here, Bob's daughter, writing from Brooklyn, New York, where a long, humid summer is slowly coming to an end. As I'm writing this, I can feel a teeny, imperceptible Autumn chill in the air this morning.

It's always a little melancholy saying goodbye to the sunshine, but truthfully I am super excited for cozy fires and hot cider and wool sweaters.

When I was younger, Autumn meant returning to school and all the great and terrible things that came with it: new pencil cases, fresh shoes, field hockey, try-outs for the play and more. It was a scary, hopeful time of possibility and new beginnings. Now, years later, as a working Mom, it's easy to forget the importance of starting over: In fact, if it weren't for you guys, September would have been just be another month in a string of months. But now that I am on the board of the MPN Research Foundation, it's taken on a whole new meaning altogether.

September was Blood Cancer Awareness Month, emphasis on the word "awareness" which, among other things, is defined as "having knowledge." And knowledge is power. Power and knowledge is a pretty awesome combo if you ask me. It's what we need collectively to take our "Little Engine That Could" foundation to the next level. We've already funded 9 million dollars in select research projects in the thirteen years since our inception. Now it's time to fund \$9 million more.

Let's make a pact – to let our communities know who we are and how the MPNs have affected our lives.

Even though September is over, ask your friends over for drinks and talk to them about the foundation. Create a FirstGiving (www.firstgiving.com) or Crowdrise (www.crowdrise.com) page in honor of someone you know who has been impacted by the

HOW TO LEAVE A LEGACY THAT COULD HELP LEAD TO A CURE

Alec Pauluck passed away in 2011. In his will, he provided a gift to fund the MPN Research Foundation.

Thanks to Mr. Pauluck's deep commitment, his generosity is helping all of us find a cure for MPNs.

The bequest of Mr. Pauluck demonstrates the importance of planning ahead to ensure you have a real impact on the programs you feel strongly about.

Remembering the MPN Research Foundation in your estate plans is a wonderful way to help find a cure for MPNs. Everything you do to help can make a significant impact.

We can provide bequest language to include the Foundation in your Will, or talk with you or your financial advisor about a wide variety of planned giving options.

To establish your personal legacy, please contact William Crowley at 312/683-7226 or wcrowley@mpnresearchfoundation.org.

(continuing from left column)

disease. Create a social media platform and spread the word. Don't be scared to inform your friends and families about who we are. Don't be afraid to ask for their help.

It's a cliché, but in this case it's true. Every little bit does count. Every dime really does get us closer to a cure.

Happy Autumn to all of you, and on behalf of the Rosen Guy family, I salute you in whatever leg of the MPN journey you're on. Here's to health, to the Fall and to beautiful new beginnings.

xx

Molly Rosen Guy