

# MPD FOUNDATION® UPDATE

A PERIODIC NEWSLETTER FOR  
THE MYELOPROLIFERATIVE DISORDERS COMMUNITY

VOLUME VI, NO. 2 FALL, 2007  
WWW.MPDFFOUNDATION.ORG

## OFFICERS AND DIRECTORS OF THE MPD FOUNDATION

---

**Robert Rosen**  
Chairman

**Barbara Van Husen**  
President

**Robert Pritzker**  
Vice President

**Felisse Sigurdson**  
Vice President

**Woody Woodruff**  
Secretary

**Sam Klepper**

**Celia Miltz**

**David Ricci**

**Robert Horwitz**

**Jeff Shier**

---

## SCIENTIFIC ADVISORY BOARD

**Ernest Beutler, MD**  
Chairman, Department of Molecular and  
Experimental Medicine  
The Scripps Research Institute, La Jolla, CA

**Martin S. Tallman, MD**  
Professor, Division of Hematology  
and Oncology  
Northwestern University, Chicago, IL

**Josef T. Prchal MD**  
Professor of Medicine and Genetics  
Hematology Division  
University of Utah, Salt Lake City, UT

**Hugh Rosen, PhD, MD**  
Professor, Department of Immunology  
The Scripps Research Institute, La Jolla, CA

**Connie Eaves, PhD**  
Professor, Medical Genetics, and Department  
Head, Terry Fox Laboratory  
BC Cancer Research Centre, Vancouver, BC

## FROM HYPE TO HOPE

by Robert Rosen

The MPD Foundation is run by hardworking volunteers and two paid staff. Working out of our subleased space in the Sears Tower in Chicago, we receive a steady stream of calls and emails from patients all over the world. Many are newly diagnosed and confused. Some want direction or advice; others just want to know that there is an organization that cares. We are always quick to disclaim any notion that we are medical doctors, but with our knowledge of the disorders and our familiarity with the state of research, we do what we can to help.

We marvel at how rapidly the research landscape has changed in the course of the last eight years in which the MPD Foundation has been raising money for MPD Research. We are now beginning to see the fruits of the remarkable discovery of the JAK2 V617F gene. The firestorm of interest that ensued has helped ignite a widespread quest to find small molecule targeted therapies that could fix the mutated gene. As more money has become available for research, new pathways are being explored. We feel comfortable that the MPD world is finally receiving the scientific interest and attention that it lacked for so long.

*The MPD world is finally receiving the scientific interest that it lacked for so long.*

As a patient I am impatient for results, and I assume that most other patients are also. Our organization is built around the needs of patients and the urgent desire for new and better treatments. In the post JAK2 world, the MPD Foundation's primary goal is to push this new research as fast and effectively as possible. In the past we made grants to highly qualified individual researchers, but now we have shifted to a collaborative model as a better way to accomplish our goals. That's why we started the MPD Research Alliance, a collaborative venture between Dr. Ayalew Tefferi at Mayo, Dr. Gary Gilliland at Harvard, and Dr. Ron Hoffman at Mt. Sinai.

*(continued on page 3)*

## RESEARCH ALLIANCE MEMBERS DELIVER YEAR 2 PROGRESS REPORTS

On Sunday, August 5, the semi-annual meeting of the MPD Research Alliance was held in Boston. Drs. Ron Hoffman, Gary Gilliland and Ayalew Tefferi met together with some of their colleagues to discuss progress against their Year 2 Research Alliance objectives. Following their scientific session, the researchers made presentations to the MPD Foundation Board and responded to questions.

All three researchers agreed that the extraordinary progress being made in their labs and at other institutions should be viewed as strongly encouraging to MPD patients.



*Dr. Gary Gilliland chats with Dr. Gerlinde Wernig at the morning researchers' session. She was a co-author of the Blood paper describing their mouse model.*

Dr. Hoffman reported on his studies of the stem cells that represent the origins of the disease for MPD patients. He has transplanted human stem cells with MF and PV into specially-conditioned mice, and is studying the effects of treatments in these animals. He is investigating and testing alternative drugs to the small molecule JAK2 inhibitors that are being tested in other laboratories. He is also participating in the current study being conducted by the Center for Disease Control (CDC) to determine whether environmental toxins may contribute to the incidence of the MPDs.

### A Unique Mouse Model

Dr. Gilliland continues to look for MPD markers beyond the JAK2 mutation, which accounts for many but not all cases of MPDs, in the hope that all causes for the disorders will someday be understood. His lab has developed a unique mouse-based platform for testing new drug compounds, and has identified several JAK2 inhibitors that are now moving to FDA-approved clinical testing in humans. Developing an accurate murine (mouse) model for the disease is a complex and time-consuming process, but provides a basis for identifying promising compounds and accelerating the drug development process.

Dr. Tefferi has built and is currently enhancing a robust MPD database that includes over 11,000 patient samples and corresponding clinical information from over 1,200 MPD patients. He is using this database to perform correlative studies that will further validate the effectiveness of compounds that have been identified as promising in Dr. Gilliland's mouse model.

All three researchers underscore the complexity of the drug development process. They emphasize the need to identify as many drug targets and test as many candidate compounds as possible to ensure that the safest and most effective treatments are developed. The three researchers will meet again in Chicago in February, 2008 along with the Scientific Advisory Board of the Research Alliance to mark the end of the second year of the Research Alliance and to present plans for moving their discoveries into Year 3 projects and initiatives.



*Lively discussions continued through lunch. Left to right, Dr. Ayalew Tefferi, Andy Selinger, Woody Woodruff, Rob Horwitz, Dr. Gary Gilliland.*

## TEAM WINDMULLER RAISES OVER \$50,000 FOR MPD RESEARCH ALLIANCE

On April 29, 2007, an MPD patient and several family members ran in the Big Sur International Marathon beginning in Big Sur, California, and ending in Carmel. The weather was perfect for the event and Team Windmuller raised over \$50,000 for the MPD Research Alliance.



The MPD Foundation congratulates and thanks Team Windmuller and all of their friends and family members for their generosity and support!

## 2ND ANNUAL AL BOLEA MEMORIAL BIKE RIDE SUPPORTS MPD RESEARCH ALLIANCE

Al Bolea was an avid cyclist who was known for his compassion and caring for others.

On August 12th, friends gathered for the second annual memorial bike ride to honor Al. Last year, they raised a substantial amount of money that was directed to the MPD Research Alliance for myelofibrosis research. Contributions to this event will again be directed to the Research Alliance.

Many thanks to this dedicated group!

## FROM HYPE TO HOPE (continued from page 1)

Although much new work is focused on JAK2 inhibitors, there are other genetically engineered drugs that are not JAK2 targeted. In addition our scientists tell us that there is evidence that a JAK2 inhibitor will be effective for JAK2-negative patients. More will be known when the results of the early clinical trials are available.

At the time of this writing, several clinical trials are in progress (see page 6). Others will follow. It makes us proud that our researchers have been directly or indirectly involved in much of the pre-clinical testing for some of these new compounds.

*We are confident we're on the right road.*

Although many patients have been able to live comfortably with their disorders, many others live with a wide range of symptoms that at best can impact quality of life, and at worst can threaten their lives. It is tantalizing to look at recent progress and think that we may be close to some answers. Our scientists warn us that the drug development is a long and uncertain process, but we are confident that we are on the right road.

## MPD Board Meets in Boston



The board session. Seated, left to right: Woody Woodruff, Barbara Van Husen, Jeff Shier, Fred Roy, Maria Roy (of Friends of ET Research), Robert Rosen. Standing, David Ricci, Felisse Sigurdson, Rob Horwitz, Celia Miltz.



## LARGE CLUSTER OF PV CASES REPORTED IN PENNSYLVANIA

### Is Toxic Waste the Culprit?

By Ann Brazeau

Recent news reports from Pennsylvania could have a significant impact on MPD patients, particularly those with polycythemia vera (PV).

The Pennsylvania Department of Health (PADOH) and the United States Toxic Substances and Disease Registry (ATSDR), an arm of the U.S. Department of Human Services, are investigating an alarming number of PV cases reported in three Pennsylvania counties.

MPD Research Alliance participants Dr. Ronald Hoffman and Dr. Ming Jiang Xu are assisting the ATSDR.

### PV Incidence Far Above the Norm

The area has long been under scrutiny by environmental groups and the residents of Luzerne, Carbon and Schuylkill counties. According to the Republican & Herald, concerns surfaced in June of 2004 when several people in Carbon county were diagnosed with PV. All of them lived along Ben Titus Road. There are now over 90 reported cases.

Newspapers report that between 1981 and 1982, 6,700 drums of hazardous waste were dumped into underground mine workings beneath the site. The Superfund site was on the EPA's list of the most serious uncontrolled hazardous waste sites in 2001.

PADOH and the ATSDR initiated an epidemiology investigation to better understand the PV cases and the potential residential and occupational environmental exposures. Federal authorities agreed to look into concerns related to polycythemia vera in September, 2006.

Additional concerns in the tri-county area include the Northwestern Power Company's waste-coal-fired power plant, which releases small quantities of radiation. One study has associated exposure to radiation with PV. Other studies point to the possible links between PV and exposure to benzene, dioxin and solvents – contaminants found at the Superfund site.

Investigators will confirm patients' PV diagnosis by testing for the presence of the JAK2 V617F mutation, which is present in most cases of PV.

---

## MPD FOUNDATION AND CR&T TO CO-HOST 4TH INTERNATIONAL PATIENT SYMPOSIUM IN NYC

By Ann Brazeau

On November 7th, 2007, MPD experts will be leading a daylong patient symposium in midtown Manhattan. This event will be a great opportunity for patients to hear the latest in MPD research as well as interact with other MPD patients

The MPD Foundation's Research Alliance investigators, Drs. Tefferi, Hoffman and Gilliland, will be presenting as well as Drs. Silver, Spivak, Mesa, and Barbui. Robert Rosen will also address the audience.

This year's topics include recent JAK2 developments, PV research, stem cell advancements, treatments for myelofibrosis and myeloid metaplasia, bone marrow transplantation, ET and exercise physiology.

If you have not registered for this event, you may do so by visiting our website at [www.mpdfoundation.org](http://www.mpdfoundation.org) and then selecting Upcoming Events. Or contact Ann Brazeau at 312-683-7226 or Keith Muhleman at 212-288-6604. Seating is limited, and registration is on a first-come, first-served basis.

We look forward to seeing you in NYC.

## MPD PATIENT SUPPORT GROUPS FLOURISH

by Ann Brazeau

A year ago, approximately seven MPD patient support groups were operating in the U.S. and abroad. Today, there are 21 support groups, and several new ones are planned over the next months. This demonstrates that patients want to be informed and educated about their disease, have access to other patients to share experiences, and be proactive in their approach to personal medical care now and in the future.

The MPD Foundation assists patient support group coordinators by providing patient brochures and newsletters, information on upcoming events and

access to MPD research experts. We also post information about support groups on our website, and hold conference calls with group coordinators to discuss their needs and to explore ways in which the Foundation can provide greater assistance.

We will hold our first annual in-person meeting with support group coordinators in 2008.

If you are interested in participating in a MPD Patient Support Group, contact the person listed below in your area:

**Atlanta, GA**–Mel Brantley  
mlbrsa@aol.com

**Australia**–Ken Young  
kyoung@yarranet.net.au

**Chicago, IL**–Sarah Dreller  
smdreller@hotmail.com

**Cincinnati, SW Ohio, NE Kentucky and SE Indiana**–Rebecca Lubitz  
rebeccalub@aol.com or 513-793-3179

**Denver, CO**–Dianne  
dianne@systemsaverinc.com

**Germany**–Email for group:  
mpd-netzwerk@yahoogroups.de

**Japan and Asia**–Kaori Taki  
patchleojp@yahoo.co.jp

**Knoxville, TN**–Linda Tunstall  
tunstalllinda@aol.com or 865-986-4565

**London, UK**–info@mpd-support.co.uk

**Los Angeles, CA**–Beatrice Larroque  
bealarroque@gmail.com

**Montclair, CA**–510-652-7000 or 510-654-5368

**NY, NJ & PA** (Meetings in New Brunswick)  
Matt Weismantel  
matt.weismantel@rutgers.edu  
or 732-932-9342 x 2609

**Northeastern U.S.** (NH, MA, RI, CT)–  
Jay Humphrey, wwhumphrey@aol.com  
or 401-640-1500

**Racine, WI** (Group for SE Wisconsin and NE Illinois)–Marge Blocks  
mblocks@wi.rr.com or 262-637-4338

**San Francisco Bay Area**–Susan Klepper  
susanklepper@hotmail.com

**Saskatoon, Saskatchewan, Canada**–Gordon Walz  
gwalz@sasktel.net

**Seattle, WA**–michele@anciauxinternational.com

**Toronto, Canada**–Kevin Brown  
kb@iconhomes.com or 416-420-2266

**Waco, TX**–Alan  
saalanc@hotmail.com

**Washington, DC**–Sarah Singer  
singers@dc-cap.leukemia-lymphoma.org or 703-960-1100 x 231

If you are interested in starting an MPD Patient Support Group, contact Ann Brazeau, Associate Director, at abrazeau@mpdfoundation.org, or by phone at 312-683-7226.

## CLINICAL TRIALS

### A MEASURE OF THE GROWING INTEREST IN MPDs

Since the JAK2 discovery, there has been a surge of interest in developing new compounds to treat MPDs. A quick visit to [www.clinicaltrials.gov](http://www.clinicaltrials.gov) shows 38 trials for PV, 36 for ET and 85 for myelofibrosis.

Here's a sampling of recent trials announced for MPD patients. For links to various lists of clinical trials, please visit [www.mpdfoundation.org/clinicaltrials.htm](http://www.mpdfoundation.org/clinicaltrials.htm)

- **Incyte Corporation–INCB018424.** A phase I/II, open-label study of the JAK2 inhibitor INCB018424 in patients with primary and secondary myelofibrosis. The sites are MD Anderson Cancer Center (Dr. Verstovsek) and Mayo Clinic (Dr. Pardanani).
- **Exelixis, Inc.–XL019.** A Phase I trial of XL019 is expected to begin in the second half of 2007. XL019 is a small molecule JAK2 inhibitor.
- **TargeGen–TG101348.** TargeGen plans to initiate a multi-center Phase I study with TG101348, a potent oral JAK2 inhibitor, in myelofibrosis patients in early 2008.
- **Cephalon–Cephalon 701.** Cephalon is conducting a Phase II open-label trial of the JAK2 inhibitor CEP-701 in patients with myelofibrosis. The principal investigator is Dr. Verstovsek at MD Anderson Cancer Center.
- **Celgene–Pomalidomide–CC4047.** This is a multi-site Phase II study in patients with primary and secondary myelofibrosis.

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

#### Update Editorial Staff

Woody Woodruff, Editor  
Amanda Friedeman, Layout Editor

## TANTALIZING CLUES, BUT WE STILL HAVEN'T FOUND THAT BREAKTHROUGH CURE

### Please Consider Making a Donation to the MPD Foundation

The MPD Foundation continues to fund cutting edge research that has ignited the interest of many in the world of MPDs. This is great news for MPD patients and their families as new drugs are introduced in Phase I clinical trials.

“We’ve seen a spectacular number of publications to date, over a broad scope, and we have even moved into clinical research,” Dr. Gary Gilliland told the Board in Boston. “That’s incredible progress. But there are many more compounds we need to evaluate. We still have a huge undertaking ahead of us.”

We were able to reach these milestones only because of your generous support. To keep this kind of momentum going, we need your help even more than before.

Consider making an investment in the MPD Foundation today by check or credit card. Please visit our website at [www.mpdfoundation.org](http://www.mpdfoundation.org) and click on How to Help, or mail us a check at the address below.

Invest in your health and your future.

For more information or to make a donation, contact the MPD Foundation at:

#### MPD Foundation

Sears Tower  
233 South Wacker Drive, Suite 375  
Chicago, IL 60606  
Tel (312) 683-7243 Fax (312) 332-0840  
[www.mpdfoundation.org](http://www.mpdfoundation.org)