

OFFICERS AND DIRECTORS OF  
THE MPD FOUNDATION

---

**Robert Rosen**  
Chairman

**Robert Pritzker**  
Vice President

**Felisse Sigurdson**  
Executive Director

**Amy McMurray**  
Treasurer

**Sam Klepper**

**Celia Miltz**

**David Ricci**

**Barbara Van Husen**

**Woody Woodruff**

MEDICAL ADVISORY BOARD

---

**Ernest Beutler, MD**  
Professor and Chairman, Department of  
Molecular and Experimental Medicine, The  
Scripps Research Institute, La Jolla, CA  
Senior Consultant, Division of Hematology  
and Oncology, Scripps Clinic Medical Group,  
La Jolla, CA

**John K. Camoriano, MD**  
Department of Hematology and Oncology,  
Mayo Clinic, Scottsdale AZ

**Gary Gilliland, Ph.D., MD**  
Associate Professor of Medicine, Harvard  
Medical School. Associate Investigator,  
Howard Hughes Medical Institute, Boston MA

**Ron Hoffman, MD**  
Director, Cancer Center  
Eileen Heidrick Professor of Oncology  
University of Illinois at Chicago

**Josef T. Prchal M.D.**  
Professor of Medicine, Pediatrics and Cell  
Biology, Department of  
Hematology/Oncology, Baylor College of  
Medicine, Houston, TX

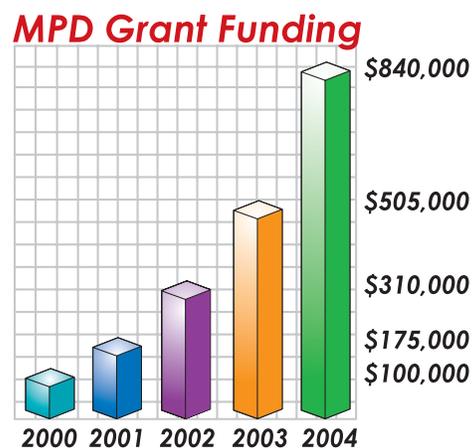
LETTER FROM THE PRESIDENT

It has now been five years since we started the MPD Foundation. Lately I have been reflecting on all we have done since our inception: raising money, building a volunteer staff, funding scientific research, and building a nationwide and worldwide community of people with similar interests. The questions in all of our minds now are where we go from here, and how we get there.

**We started with hope and not much else**

We started the MPD Foundation toward the end of 1999 with several donations by board members, one research project, no public credibility, and lots of hope and hard work. We were uncertain about our ability to make meaningful alliances, the possibility of developing a website, finding an executive director, and ever raising enough money to sustain and grow our organization in a meaningful way. It is now 2004 and together we have leapfrogged far ahead of all of these concerns.

*(continued on page 7)*



## LOOKING BACK: MY FIRST YEAR AS EXECUTIVE DIRECTOR OF THE MPD FOUNDATION

By Felisse Sigurdson

I was diagnosed with Polycythemia Vera (PV) in August 2002, at age 40 and the mother of twin 6 year-olds. That first year was filled with both emotional and physical adjustment to being diagnosed with a rare disease, the treatment, and the symptoms that remain on a daily basis. My husband and I spent a great deal of time educating ourselves on the treatment options and locating the most knowledgeable hematologist in the Chicago area. During my research, I discovered the MPD Foundation, also based in Chicago. I met with several board members and was energized by their commitment to MPD research. I was also very impressed by their incredible record of annual funding growth and the fact that 95% of the funds raised go to medical research.

In September 2003, I decided to donate my time to the MPD Foundation working part-time as the Executive Director. For me, it's a great opportunity to give back to the world, and some day it may also benefit my own health and well being. Prior to joining the MPD Foundation, I spent 20 years at IBM in various sales and marketing roles. While the world of medical research is new to me, I find my sales and marketing background a great asset in creating awareness for MPDs and stimulating investments in MPD research.

At my first year anniversary with the MPD Foundation, I thought I would review some of things we accomplished this year and the exciting plans we have for the coming year in support of MPD research.

### **MPD Foundation Website**

We have just completed a major update of the MPD Foundation Website. Most noticeable is the change to the site's look and feel. But we also added new content with MPD News, new links in MPD Resources, the addition of the grants process, and a full listing of past research awards. We also added a Registration Function

that makes it easy for you to join our mailing list, and we have expanded the online donation capability to capture donations in honor or memory of another person. You can see all these changes by visiting the site at [www.mpdfoundation.org](http://www.mpdfoundation.org)

### **MPD Prevalence and Incidence**

There is very little information on how many people have an MPD, and what information does exist, is based on studies outside the US. This limits our fundraising

*"The data show a much greater prevalence of MPDs than the current medical literature reports."*

potential in the US and the attention that public sources such as the federal government and pharmaceutical companies pay to MPD research.

In 2004, we started to gather data on MPD prevalence. The data show a much greater prevalence of MPDs than the current medical literature reports. In 2005, we plan to sponsor an epidemiology study to identify MPD prevalence in the United States. This information has the potential to be enormously beneficial in reshaping public policy relative to MPDs.

### **US Federal Government and MPD research**

We have become painfully aware that the federal government hardly knows the MPDs exist. This is an important issue for us since the federal government is the largest source of medical research funding in the United States. In 2004, we started to address this issue and plan to expand our efforts in 2005.

*(continued on page 6)*

## ALLOGENIC BONE MARROW TRANSPLANT WITH REDUCED INTENSITY CONDITIONING: ONE PATIENT'S EXPERIENCE

By Paul Sassano

Myelofibrosis, Allogenic Stem Cell Transplant, Graft vs. Host. In June, 1999 I had no idea what those words meant. Six months later, I was diagnosed with Myelofibrosis at the age of 47.

I was given the name of the disease, the fact that it was “idiopathic,” and one doctor’s opinion that there was no cure. With the exception of shortness of breath and an enlarged spleen, which I discovered while swimming the backstroke, I had no sense of being “sick.” I felt some apprehension and a little fear but mostly a sense of challenge to learn more about the disease: To learn more about the way it might affect my life, to learn more about the people who knew more about Myelofibrosis. Books that I had access to had no information, the Web had only slightly more.

In January, 2000, through an optometrist friend of my brother-in-law, I was given the name of Dr. Hoffman at University of Illinois Hospital and was told he was a leader in research on a class of disorders called MPD, of which Myelofibrosis is one. He agreed to see me quickly, so by February, 2000 I felt I was moving forward. After a first and second visit to Dr. Hoffman and his review of my bone marrow biopsy to confirm the diagnosis, he suggested the possibility of an allogeneic stem cell transplant with one of two “conditioning regimens” performed before the transplant. Dr Hoffman introduced me to Dr. Devine who along with Dr. Van Besian was doing research on the efficacy of two different types of “conditioning” prior to transplant. The conditioning would reduce my white cell count, so my body would more easily accept the new stem cells, as well as help to clear out the “malignancy” in my bone marrow so that new marrow could be grown. Four months after being diagnosed with a disease I had never heard of, I was being allowed to participate in a relatively new study of a procedure that had a chance of curing a previously “incurable” disease (my opinion only, the term “cure” was not used).

By August, 2000 my sister had donated stem cells, and I was embarking on an experience that was new to the medical community and me. Dr. Devine and his staff had prepared me with large doses of compassion and as much knowledge as they could impart in layman’s terms, as well as a game plan for my wife and family. I was to spend the next 4 weeks in the hospital and the next year at home with quick access to everyone on his staff (my home is 30 minutes from the hospital). I still had very little sense of being ill, my head was clear, I was in no pain, and I felt I had a tremendous support group in my family and the doctors involved in my case.

During my first week in the hospital I was given doses of fludarabine and melphalan. This regimen did not make me nauseous but I quickly lost all my hair and energy. At the end of the week, over a one-hour period, my sister’s stem cells were introduced into my body. I quickly lost thirty pounds from my 155 pound frame.

*“Today, more than four years after the transplant, I am a healthy and active brother, father, and husband...”*

While the next two weeks are now a blur in my mind, I do remember that I was extremely well cared for, saw my wife every day, my children once, and experienced some problems with my lungs and bladder that landed me in the ICU for several days. Week four brought a marked return of all cell counts and my strength and memory as well. At no time during my four-week stay did I feel that I was in a state that was intolerable or a position that I couldn’t fight back from. Achy, shaky, klutzy, and homesick, but never defeated.

Finally, I was able to go home. Isolated from the rest of the house, but home. Back to the clinic every three days and some bouts with flu like symptoms, maybe graft vs. host, but mostly comfortable and with my wife’s help

*(continued on page 6)*

## Proteomic Approach to the Diagnosis of Chronic MPDs

*Alison Moliterno, MD*



*Dr. Alison Moliterno in her lab at Johns Hopkins University Medical Center.*

The chronic myeloproliferative disorders have been the focus of both my clinical practice and my translational research program since completing my clinical training in hematology. For the past ten years I have been immersed in translational research projects in the chronic myeloproliferative disorders. While many facets of these disorders have fascinated me, their obscure molecular basis and the long term clinical relationships that I have developed with MPD patients form the basis of my commitment to understanding these disorders.

The goal of the project funded by the MPD Foundation is to define the protein signature of platelets in patients with chronic myeloproliferative disorders. The chronic myeloproliferative disorders, polythemia vera, essential thrombocytosis and idiopathic myelofibrosis, are diseases of unknown cause and therefore specific diagnoses are defined by very broad clinical and laboratory criteria. While patients may fulfill these broad diagnostic criteria, they differ dramatically in terms of survival, clotting complications, and development into other, more aggressive blood disorders.

We hypothesize that different MPD patients will express unique platelet protein profiles. By comparing these

different profiles, we hope to identify characteristics associated with each particular disease, clotting risk or disease transformation. As platelet proteins are easily obtained from small amounts of blood, the goal of this project is to develop the platelet protein signature assay as a practical clinical tool that will be useful both for more accurate diagnosis and prognosis. This project will advance our understanding of the biologic heterogeneity of the MPDs and has the potential to offer more accurate diagnostic and prognostic classification of patients with myeloproliferative disorders.

## Exploration of a Unique Phosphatase as a Potential Therapeutic Target for the Treatment of Polycythemia Vera

*Mingjiang Xu, MD, Ph.D., Edward Bruno, MD, and Ronald Hoffman, MD*

Hematopoietic stem cells (HSC) are rare cells which act as parent cells, giving rise to all the different types of cells in the blood, including red and white cells and platelet-producing cells called megakaryocytes. These HSC are normally present in the bone marrow (BM) and send normal cells into the blood circulating throughout the body. In polycythemia vera (PV), the normal HSC in the BM become abnormal and produce excessive numbers of red cells, white cells and megakaryocytes into the blood. PV is, therefore, a type of blood cancer in which normal stem cells are transformed into abnormal or malignant stem cells which subsequently increase in number and replace the normal stem cells.

*(continued on next page)*



*Drs. Mingjiang Xu and Edward Bruno in their lab at The University of Illinois at Chicago.*

## 5TH ANNUAL FRIENDS OF ET RESEARCH GOLF CLASSIC RAISES NEARLY \$80,000!

Friends, family and loyal supporters gathered once again in the foothills of the Catskill Mountains at Centennial Golf Club in Carmel, NY for a day of golf, fun and entertainment on September 14, 2004. More than 165 people were in attendance playing golf, volunteering, or attending the dinner and evening festivities which included a raffle and a silent auction. New York Knicks All-Star basketball player Allan Houston was a special guest. Also attending this year were Irene McDonnell, Paige Nesis and Mike Hvizdo, all of whom have ET.

The primary purpose of this annual event is to raise funds for medical research for the myeloproliferative disorders including Essential Thrombocythemia (ET.) Celia and Don Miltz organized Friends of ET Research in 1999 to raise funds and public awareness, and to help support their daughter Stephanie, who was diagnosed with ET in 1997. In five years, they have raised \$400,000, all of which has gone to support medical research for ET, PV and MF.

Several years ago, Friends of ET Research formed a partnership with The MPD Foundation that allows both organizations to support more research grants by commingling their funds.



Stephanie Miltz with special guest, New York Knicks star Allan Houston.

## UPDATE: MPD RESEARCH CONSORTIUM

The Summer edition of Update introduced you to a consortium of 21 researchers and clinicians at 14 institutions to work collaboratively on MPDs. Organized by Dr. Ronald Hoffman at the University of Illinois at Chicago Cancer Center, the Consortium resubmitted its proposal for a grant from NCI on June 1, 2004.

The site visit for the MPD Consortium Grant was held on Thursday September 23. The participating researchers are hopeful but reserved in their expectations because of the budget pressures at the NCI. They expect to receive notification from the NCI by year end.

## MPD FOUNDATION GRANT UPDATES

*(continued from page 4)*

An important finding in the study of PV is that the HSC in PV patients demonstrate a high response to a group of substances, called growth factors, which help control the production of all of the cells circulating in the blood. This increased sensitivity to these substances has been shown to be due to a group of proteins called phosphatases (PTPs), which help to control the effects of these growth factors on the HSC. We have identified a specific PTP, PTP-MEG2, which is elevated in PV.

Our study on the role of PTP-MEG2 has helped to further understand how PV develops, while providing a potential target for the development of new tools to diagnose PV and new therapeutic agents for the treatment of PV. Our final goal is to develop a specific inhibitor(s) of PTP-MEG2 which will only decrease the numbers of abnormal but not normal HSC, which will hopefully be used to treat patients with PV.

## ALLOGENIC BONE MARROW TRANSPLANT WITH REDUCED INTENSITY CONDITIONING: ONE PATIENT'S EXPERIENCE

*(continued from page 3)*

self-sufficient. I spent a fast year in home recovery keeping my “pick” clean, eating well, and taking a riot of pills. On September 14, 2001, three days after the World Trade Towers were destroyed, I went back to work. “Life goes on within you and without you.”

Today, more than four years after the transplant, I am a healthy and active brother, father, and husband, visiting the Oncology clinic every six months with a good looking “CBC” and just the regular aches of a 52 year old, although many physical changes have occurred following the transplant:

Prior to transplant I had curly hair. It is now wavy at best. Prior to transplant I was diagnosed with heart disease and was being treated with blood pressure and cholesterol lowering medicines. Now, both the high

blood pressure and high cholesterol are gone. I am taking no medicine of any kind today. NONE!

Prior to transplant I had very bad gum disease that had sent me through several gum “shaving” operations. My dentist now tells me my gums are in excellent health.

I can not explain, nor have I asked, why or how these changes took place. They are part of my life today.

I am told, and believe, that my positive state of mind, the tremendous amount of support, both spiritual and physical, around me, and my inclusion in the University of Illinois Hospital study have allowed me to continue to live an active and fulfilling life, albeit with “wavy” hair.

## LOOKING BACK: MY FIRST YEAR AS EXECUTIVE DIRECTOR OF THE MPD FOUNDATION

*(continued from page 2)*

In 2004, the National Institutes of Health (NIH) budget for medical research grants was \$15 billion. In addition to the NIH, the Department of Defense (DoD) has a significant medical research budget. To date, an insignificant amount of these funds has been allocated to MPD research. Thanks to the efforts of Congress, MPD researchers, ASH and the MPD Foundation, we’ve started to see some small but positive steps by the federal government in support of MPDs. Some examples include a Congressional mandate directing the NIH to expand research into MPDs, new NIH grants targeted at MPD research, and the DoD

announcement of \$4.25 million to support MPD research. All a start in the right direction.

### **Partnering**

We continued to work with the Leukemia & Lymphoma Society to direct more LLS funds toward MPD research and help them focus on basic MPD research in addition to translational research. Next year we hope to explore relationships with other well-funded cancer organizations such as the American Cancer Society and private foundations.

## **We've done a lot, with a lot of help**

Every year donations have increased. We are now responsible for 9 research grants and two organizational grants with a total dollar amount in play of approximately \$3,000,000. Thanks to the critical evaluations of our esteemed medical advisory board, the grants we give are serious grants to serious researchers. We have relationships with hematologists in America and elsewhere in the world. We work closely with some of the best MPD labs and professionals at highly regarded centers which include Mayo, John Hopkins, Baylor, and University of Illinois at Chicago.

In addition, we have been instrumental in providing support money and energy toward the formation of the International MPD Research Consortium, which is currently waiting for approval from the NCI for a \$25 million MPD grant.

We have assembled a remarkable volunteer board of directors where every member makes tangible contributions to our continuing success. The board has offered advice and consultation, provided gifts of money without which we cannot sustain our mission, and invested time and energy in a multitude of projects. Amy McMurray, our treasurer handles all the bookkeeping and correspondence, Woody Woodruff, with the assistance of Amanda Friedeman, makes our newsletters sleek and readable. Bob, Barbara, Celia, Sam, and David all contribute in meaningful ways throughout the year.

I don't know how we could have managed the last year without Felisse Sigurdson, our executive director. Her tireless energy and incisive mind have exponentially increased our ability to function effectively.

We receive many hundreds of individual donations every year, some of them quite substantial. The grants we award are costly, and we cannot grow without this help.

*"Although many of us who are patients do well for long periods of time with these disorders, there is urgency for others and a great deal of uncertainty for all."*

At last, for the first time, the federal government is taking an interest in the MPDs; in 2004, both the NIH and the Department of Defense opened grants targeted at MPDs.

## **Many questions remain unanswered**

However, our good news cannot make us complacent about the daunting task in front of us. Although many of us who are patients do well for long periods of time with these disorders, there is urgency for others and a great deal of uncertainty for all.

There are issues that we can all relate to, and some issues that are specific only to certain patients. Blood clots remain a concern for almost everyone. Two people on our board have suffered serious, potentially life threatening thrombotic events. After all these years the medical community still does not know why MPD patients are prone to clotting, nor can anyone predict who is at the greatest of developing clots.

There are no significant biomarkers to help understand who is at greatest risk and how to estimate longevity or disease progression. Without good biomarkers, treatments tend to be given without knowing which ones will work best for which patients. The doctors don't agree on what drug treatments are appropriate for which disorders, and when and how to differentiate the needs between patients.

There are newer medications in use; interferon, peg-interferon and Gleevec. We know these are active in many or most patients yet we do not have good enough information to determine long-term consequences, impact on longevity or why they help some patients and not others.

Some of us have a disease called myelofibrosis which can be as urgent and acute as any malignancy anywhere, yet it is the focus of far too little primary research. We can hardly get decent research proposals on MF, yet I

*(continued on page 8)*

LETTER FROM THE PRESIDENT  
*(continued from page 7)*

get calls at my office from recently diagnosed patients who want to know where to turn for help.

Our patients see hematologists who misdiagnose them, and who are not familiar with the MPDs. We worry that there are not enough young researchers coming through the ranks to ensure the best future scientific research and care.

There has not been a large scale study group since the Wasserman study over 30 years ago. Enormous progress was made as a result of that work, and we need to do it again in the light of recent scientific progress in our understanding of the human genome. In spite of the efforts of Dr. Prchal and others, we do not yet have a conclusive handle on the genetic defects and molecular characteristics that cause MF, PV or ET. Without these we cannot have targeted therapies.

Because the NIH does not track incidence and prevalence data for the MPDs, there is no credible data from which to establish priorities with potential funding sources to compete for public and private research dollars.

We get donations from only a small minority of people compared to the number of patients, relatives, and friends associated with these diseases.

### Looking forward

I was diagnosed with PV in the fall of 1997 after experiencing unexplained neuropathies in my toes and fingers for almost a year. At the time of diagnosis my hematocrit was 60 and the doctor asked if I had any pains in my chest or other heart symptoms. I was emotionally devastated for many months, and my family had difficulty coping with the fact of my illness. I couldn't believe that in this day and age, there were no advocacy groups acting effectively to promote medical research in the MPD's.

With the passage of time I was able to regain my balance and direct my frustration into something constructive. With the help of many good people we are moving forward towards a time of better understanding and inevitably better treatments for our conditions.

We are grateful for your support.



Robert Rosen

---

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

**MPD Foundation**  
Box 273  
858 West Armitage Avenue  
Chicago, IL 60614  
(312) 683-7247  
[www.mpdfoundation.org](http://www.mpdfoundation.org)