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The Hanns A. Pielenz Clinical Research Center for Myeloproliferative Neoplasia Newsletter

MPN Focus

THE UNIVERSITY OF TEXAS MD Anderson Cancer Center

Making Cancer History®

The Role of the Research Nurse in Clinical Trials for Patients with Myeloproliferative Neoplasms

By Mary Ann Richie, RN, BSN, MA, CCRP
Research Nurse Specialist

The research nurse plays a vital role in managing clinical trials for patients with myeloproliferative neoplasms (MPNs). It is the responsibility of the research nurse to educate the MPN patient and monitor their health while they participate in a clinical trial. A research nurse acts as a liaison between the patient and the physician to ensure the best possible experience and outcome for their patient. Prior to managing a clinical trial for MPNs, a research nurse must know the nature of these diseases to be able to truly understand this unique group of patients. MPN symptoms can be painful, disfiguring, and life-threatening if not monitored and treated on an ongoing basis. Patients are faced with fear of their disease transforming into acute leukemia, uncertainty about making plans for their future, the expense of caring for a chronic condition, and the large amount of time spent on managing their disease, which often leads to major disruptions to their lifestyle and home life. The research nurse must understand these concerns and use his/her knowledge of MPNs and experience with patients to prepare for the first step in the process: The Patient Interview.

How the research nurse prepares for the patient interview

Preparation is the foundation for communicating with a patient about possible participation in a clinic trial. Prior to discussing a clinical trial with a patient, the research nurse must familiarize themselves with the details of the patient's case by reviewing their medical record– especially notes from treating physicians– as well as results from laboratory and other tests. A review of personal information such as where the patient lives, what language they speak, and other specifics is also done to determine if the patient needs an Interpreter or has other special needs. Next the research nurse reviews the eligibility criteria for the clinical study to confirm whether the patient is eligible to participate.

Communication with the patient

From the initial patient interview to the time that the patient is taken off the study, communication with the patient is the most important responsibility of the research nurse. At the first patient

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See Inside:

Letter from the Director

As 2015 draws to a close we reflect on the past year and the progress that has been made. In myelofibrosis, clinical trials combining ruxolitinib with other novel drugs are now enrolling patients with the hope for bringing additional benefits to patients, to improve anemia or bone marrow fibrosis, along with good control of splenomegaly and systemic symptoms. New approaches are being explored, like anti-fibrotic medications and immune checkpoint inhibitors. Clinical studies for atypical MPNs are becoming available. In addition, with the availability of genomic information and our large MPN sample bank, we are poised to be able to identify new gene mutations and their effects on patient outcomes.

For the third year in a row, we co-hosted a patient meeting with Patient Power in October. The event was another great success, with patients and their families attending in person as well as for those who could not travel to MD Anderson and could join us live online. The fourth annual event is already scheduled for October 1, 2016, so mark your calendars!

Dr. Srdan Verstovsek, Professor, Department of Leukemia, MD Anderson Cancer Center serves as Director of the Hanns A. Pielenz Clinical Research Center for Myeloproliferative Neoplasia. Dr. Verstovsek is an internationally recognized physician scientist dedicated to understanding the biology of and developing new therapies for MPNs.

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The Role of the Research Nurse in Clinical Trials

interview, the research nurse must obtain written and informed consent from the patient to participate in the study. First, the research nurse introduces him/herself and confirms the patient's name. In addition, the research nurse must confirm that the treating physician has spoken to the patient about the nature of the investigational treatment as well as other treatment options for their disease. A brief overview of the study drug is given along with an explanation of the purpose of the study. A patient must be informed about the investigational nature of the drug(s) and their responsibilities as a patient. The must be given adequate time to ask any questions they feel are necessary to make an informed decision about participation in the clinical trial. Once the specifics of the study have been reviewed with the patient, the research nurse will ask the patient what they have heard and understood and whether they have any questions. Once the patient has had all his/her questions answered and has had adequate time to think about what has been discussed, they are asked if they are interested in participating in the clinical trial. If the patient agrees they must sign the informed consent form, and the research nurse will give them the tools to successfully fulfill their responsibilities on the study.

Patient Handouts

Patients are presented with a great deal of unfamiliar information at their initial interview. Therefore, a written reinforcement of the vast and detailed information is greatly appreciated by patients when they are enrolled in a clinical trial. The information can be overwhelming and many patients feel desperate to write down everything for fear that they won't be able to remember it. In most cases there is a handout for patients that documents the information being discussed so that they can focus on listening to what is being discussed with them in the interview rather than taking notes. In addition to discussing the details of their responsibilities when on the study, the research nurse provides patients with the information and tools needed to complete any required assessments during the course of the study. A study calendar is often used to summarize and reinforce the information in the patient handout. The calendar should show the specific dates they need to return to the clinic for evaluation and the assessments that will be performed at each clinic visit *(Table 1)*. Another variant on the study calendar for the tech-savvy patient is an electronic Excel file that automatically calculates and updates the time points at which a patient will need to have a particular assessment done. The advantage of this tool is that it automatically updates the time points when there is any variation in the visit dates.

Patient Contact

The research nurse will remain in contact with the patient for the duration of the clinical trial and the follow-up period. This is accomplished through phone calls, emails, and/or clinic visits. Patients may not be aware, but they are being followed carefully by the research nurse to ensure their safety, and therefore they need to immediately report any serious or unusual side effect. Regular contact and open communication encourages patients to report information on their condition that they may or may not consider relevant. Some patients do not want to complain or feel too embarrassed to ask questions. However, all questions are important to ensure the patient's care and safety. Patients should also document all of their symptoms so that future patients can benefit from their candor. The research nurse provides multiple forms of contact information including office, pager, and fax numbers, as well as email information in case patients have questions or need

Table 1 January 2015 – (Cycle 1)

SUNDAY	MONDAY	TUESDAY	WEDNESDAY	THURSDAY	FRIDAY	SATURDAY
				1 New Year's Day	2	3
4	5	6 BL BMA	7 C1 D1 Research Labs Screen+C1 D1	8 C1 D2 Research Labs	9	10 C1 D4 Research Labs
11	12 BMA	13	14 C1 D8 Research Labs	15	16 FT Labs Review	17
18	19 Martin Luther King, Jr. Day	20 FT Labs Review	21	22 C1 D15 Research Labs PFT	23	24
25	26 BMA FT Labs Review	27	28 Clinic Visit C1 D21 Research Labs	29 Cycle 2 Day 1 Drug Research Labs	30 Research Labs Review	31 Cycle 1 Day 4 Research Labs

Research Labs – means that all labs will be drawn by the Leukemia Research Lab – on research visit days
FT Labs & Review (only) days – FT Lab will draw the routine labs on days that you don't have research scheduled.
BMA = Bone Marrow Aspirate **PFT** – Pulmonary Function Test **C** = Cycle **D** = Day **BL** = Baseline
FT = Leukemia Fast Track Lab

to report a side effect or hospitalization. Through written request, family members can be included in the communications.

Monitoring Patients

The research nurse is the primary member of the study team who monitors patients in between clinic visits with their physicians. Patients have assessments per protocol requirements or more frequently if the physician determines it is necessary and in their best interest. The research nurse assesses the results of laboratory and other tests, such as chest x-rays, electrocardiograms, and pathology reports and documents this information. If there is an unusual finding or the patient is hospitalized, the research nurse conveys this to the physician immediately. Otherwise, regular reports on patients are given by physician preference.

Communication with the Physician

The research nurse must act as a liaison between the patient and physician. While the research nurse is often the one who communicates directly with the patient, the treating physician is kept updated with the patient's status and needs. This is usually accomplished in the form of emails that can be used for source documentation or reference later if needed. Meetings between the research nurse and physician can be arranged–but a dictation to the patient's medical record will be made to document what was discussed about the patient–especially if a treatment decision was made.

Patient Participation in their Treatment

A responsible and caring research nurse will encourage patients to actively participate in their treatment decisions.

All patients react differently to receiving an MPN diagnosis. Some patients become actively involved by educating themselves about their disease, asking questions, and making informed treatment decisions. Other patients may feel like they have lost control of their life and health and may withdraw from involvement in their treatment. They may make statements like "I don't have a choice" or "just tell me where to go and what to do". This is a something that needs to be discussed with the patient's physician who can determine whether the patient is at risk for depression. Everyone is unique–but generally, patients are encouraged to learn more about their disease, so that they can make informed decisions about their treatment, and, most importantly, they should ask questions.

Current Research and Continuing Education

Research for MPNs is becoming more widespread and information is constantly evolving. Therefore, the research nurse must keep up with the current research and new discoveries so that he/she is better able to understand the disease and the rationale for new clinical trials.

Research nurses play an important role in the care of patients with MPNs who are participating in a clinical trial. Each patient is unique and deserves understanding, care, support, and competence from a research nurse who can help them with their individual needs. Through current knowledge of MPNs, preparation through review of the protocol and patient information, creation of patient study tools, vigilant monitoring of the patient's status, and effective communication, the research nurse ensures that MPN patients have a safer, more successful, and less stressful clinical trial experience.

MPN Clinical Trials

Listed below are all open clinical trials enrolling patients with MPNs at MD Anderson as of November 15, 2015. For more information on these clinical trials, call the information line toll-free at 1-800-392-1611. For information on other clinical trials in MPN go to www.clinicaltrials.gov.

Phase 2 Study of Nivolumab in Patients with Myelofibrosis

2014-0962 (NCT No: NCT02421354)

Principal Investigator: Srdan Verstovsek
Study Description: The goal of this study is to determine the effectiveness of nivolumab in patients with myelofibrosis. The safety of this drug will also be tested. Nivolumab is a treatment that uses your immune system to treat disease. Patients will receive nivolumab intravenously every 2 weeks for 8 doses and then every 3 months thereafter.

Phase 3 Randomized Study of Oral Pacritinib vs. Best Available Therapy in Patients with Thrombocytopenia and Myelofibrosis

2013-1001 (clinicaltrials.gov NCT No: NCT02055781)

Principal Investigator: Srdan Verstovsek
Study Description: The goal of this study is to compare the effectiveness of 2 different dose schedules of pacritinib to standard treatments in patients with myelofibrosis (MF). Pacritinib is an oral drug that inhibits the activity of JAK2, but does not worsen thrombocytopenia, suggesting it may be a better alternative for treating patients with low platelet counts. Study visits will be every week for the first month and then once per month up to week 24. After 24 weeks, patients receiving best available therapy will receive pacritini

Phase 2 Study of Ruxolitinib and Pracinostat in Patients with Myelofibrosis

2014-0445 (clinicaltrails.gov NCT No: NCT02267278)

Principal Investigator: Srdan Verstovsek
Study Description: The goal of this study is to determine the effectiveness of the combination of ruxolitinib and pracinostat in patients with MF. The safety of this drug combination will also be studied. Pracinostat is a histone deacetylase inhibitor. Patients will receive ruxolitinib orally as a single agent for the first 3 months, after which point oral pracinostat will be added. This study is accepting patients with MF who have not been previously treated with a JAK inhibitor.

Phase 2 Clinical Trial to Evaluate Pegylated Interferon Alfa-2a in Patients with High-Risk Essential Thrombocythemia or Polycythemia Vera

2015-0307 (clinicaltrials.gov NCT No: NCT01259817)

Principal Investigator: Srdan Verstovsek
Study Description: The goal of the study is to evaluate the effectiveness of pegylated interferon alfa-2a in patients high-risk polycythemia vera or high-risk essential thrombocythemia who are either refractory or intolerant to hydroxyurea or who have suffered a splanchnic vein thrombosis. The safety of the drug will also be studied. Patients will self-administer pegylated interferon alfa-2a as an injection under the skin once per week. Will open in early 2016.

Phase 3 Randomized, Double-Blind Study of Momelotinib vs Best Available Therapy in Patients with Anemia or Thrombocytopenia and Myelofibrosis Who Have Been Previously Treated with Ruxolitinib

2014-0258 (clinicaltrials.gov NCT No: NCT02101268)

Principal Investigator: Srdan Verstovsek
Study Description: The goal of this study is to compare the effectiveness of momelotinib to standard treatments in patients with myelofibrosis. Momelotinib is an oral JAK2 inhibitor. Patients will be randomized to receive either momelotinib orally once daily or best available therapy. Study visits will be every 2 weeks for at least 24 weeks. After 24 weeks, patients receiving best available therapy will receive momelotinib.

Phase 2 Prospective, Open-Label Study of Sotatercept (ACE-011) in Patients with Myelofibrosis and Significant Anemia

2012-0534 (clinicaltrials.gov NCT No: NCT01712308)

Principal Investigator: Srdan Verstovsek
Study Description: The goal of this study is to learn if sotatercept can help to control MF and anemia. The safety of this drug will also be studied. Sotatercept (ACE-011) may increase the growth and development of red blood cells. Patients will be given subcutaneous injections once every 3 weeks for at least 6 months. Study visits will be once per week for at least 4 months. This study is accepting patients with myelofibrosis and significant anemia.

Phase 2 Open-Label, Dose-Escalation Study of NS-018, a JAK2 Inhibitor, in Patients with Myelofibrosis Previously Treated with Ruxolitinib

2011-0090 (clinicaltrials.gov NCT No: NCT01423851)

Principal Investigator: Srdan Verstovsek
Study Description: The goal of this clinical research study is to find the highest tolerable dose of NS-018 that can be given to patients with MF. The safety and efficacy of this drug will also be studied. NS-018 is a drug that blocks the JAK2 protein, similar to ruxolitinib. Patients will receive NS-018 orally once daily. Study visits will weekly the first month, monthly for months 2-4, and then every 3 months thereafter. Only patients previously treated with a JAK2 inhibitor are eligible to enroll.

Phase 2 Study of LCL-161 in Patients with Myelofibrosis

2013-0612 (clinicaltrials.gov NCT No: NCT02098161)

Principal Investigator: Naveen Pemmaraju
Study Description: The goal of this clinical research study is to learn if LCL-161 can help to control myelofibrosis. The safety of this drug will also be studied. LCL-161 is an oral drug that activates a signaling pathway that promotes cancer cell death. Patients will receive LCL-161 orally every 7 days. Study visits will be weekly during the first month and then monthly thereafter.

Phase 2 Study of Ruxolitinib and 5-Azacytidine (hypomethylating agent) in Patients with Myelodysplastic Syndrome/Myeloproliferative Neoplasm or Myelofibrosis

2012-0737 (clinicaltrials.gov NCT No: NCT01787487)

Principal Investigator: Naval Daver
Study Description: This goal of this study is to learn if the combination of ruxolitinib and azacytidine can help to control disease in patients with myelodysplastic syndrome (MDS)/MPN or myelofibrosis. The combination of ruxolitinib and azacytidine may improve the overall effectiveness of each drug. Ruxolitinib will be taken orally twice per day for the first 3 months, after which time low-dose azacytidine will be added. Azacytidine will be given intravenously daily for the first 5 days of each 28-day cycle.

Phase 1/2 Study of SL-401 in Patients with Advanced, High-Risk MPNs, Including MF, CMML, and HES/CEL Principal Investigator: Naveen Pemmaraju

2014-0976 (clinicaltrials.gov NCT No: NCT02268253)

Study Description: The goal of this study is to study the safety and efficacy of SL401 in patients with high-risk MPNs. SL401 is a biological agent that binds to cells that cause MPNs. Patients with symptomatic myelofibrosis who are not candidates for, are intolerant of or have failed therapy with ruxolitinib are eligible. Patients with chronic myelomonocytic leukemia (CMML) or primary eosinophilic disorders who are not candidates for therapy with imatinib are also eligible. SL401 will be given intravenously daily for the first 3 days of each 28-day cycle.

Phase 2 Study of Brentuximab Vedotin (SGN-35) in Patients with CD30-Positive Aggressive Systemic Mastocytosis with or without an Associated Hematological Clonal Non-Mast Cell Lineage Disease

2012-0734 (clinicaltrials.gov NCT No: NCT01807598)

Principal Investigator: Srdan Verstovsek
Study Description: The purpose of this study is to determine if the drug brentuximab vedotin (Adcetris) can help control systemic mastocytosis. Brentuximab vedotin is a biological therapeutic designed to bind to a certain protein (CD30) on cancer cells and kill them. Patients will receive brentuximab vedotin intravenously once every 21 days for up to 8 cycles. Study visits will be weekly during the first month and then twice a month thereafter.

Prospective Evaluation of Ruxolitinib Efficacy for Chronic Neutrophilic Leukemia/Atypical Chronic Myeloid Leukemia Patients with Mutation of CSF3R

2014-0764 (clinicaltrials.gov NCT No: NCT02092324)

Principal Investigator: Jorge Cortes
Study Description: The goal of this study is to learn about the effects ruxolitinib has on patients with chronic neutrophilic leukemia or atypical chronic myeloid leukemia. The safety of this drug will also be studied. Ruxolitinib is drug that blocks the activity of JAK2. Patients will receive ruxolitinib orally twice daily for 24 months.

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To Schedule an Appointment

Call **1-85-LEUKEMIA** (toll-free)
or **713-563-2000**

Forum Brings Together Patients and Physicians

Expanding Research and Treatment Options for Myeloproliferative Neoplasms

Hosted by Patient Power in partnership with MD Anderson Cancer Center

Patient Power

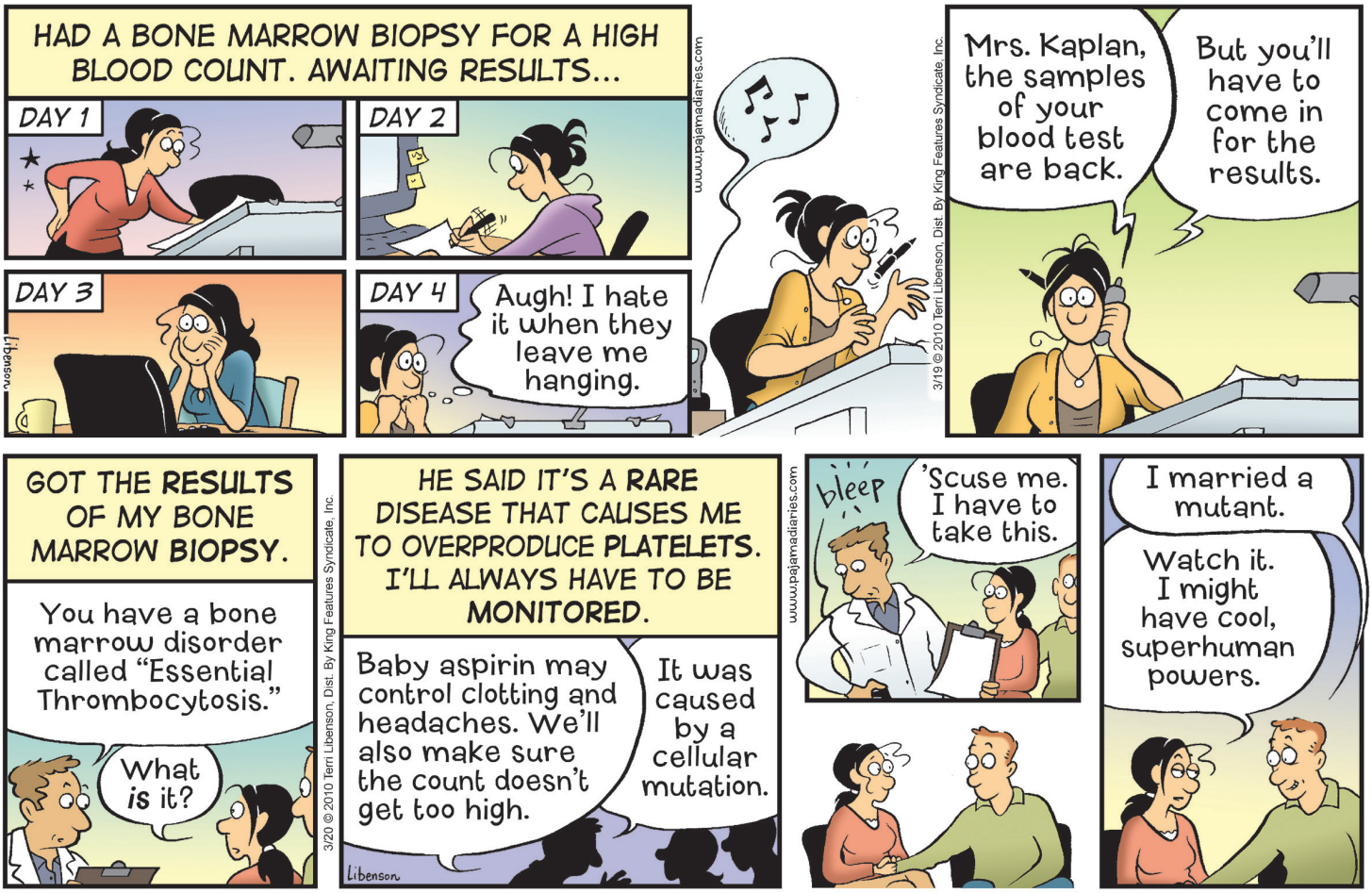
On October 24, 2015 135 MPN patients and caregivers attended a live forum held at MD Anderson Cancer Center. The meeting featured an expert panel that included Dr. Laura Michaelis from Medical College of Wisconsin, Dr. Rami Komrokji from Moffitt Cancer Center in Florida, Dr. Srdan Verstovsek and Dr. Naval Daver, from the Leukemia Department at MD Anderson.

Patients attended the meeting both in-person as well as online. The meeting was presented in a town meeting format, with much of the time reserved for attendees to ask questions of the experts. The physicians shared the latest news in MPN research and treatment and also provided helpful strategies for coping with disease symptoms and treatment side effects. They also provided advice on communicating with your medical team. In addition, three patient advocates, Voncille Fryou, Irene Badal, and Sandra Johnson also joined the discussion to tell their stories and share their advice for living well with an MPN.

Save the Date!!
This event will be held for the 4th year at MD Anderson Cancer Center on Saturday, October 1, 2016. Registration will be free for patients, their family members and caregivers.

For more information on Patient Power and for updates and additional information on the next meeting please visit www.patientpower.info.

Photos courtesy of John Everett



Terri Libenson is the cartoonist of the internationally syndicated comic strip, *The Pajama Diaries.* She was also an award-winning humorous card writer for American Greetings.

Terri graduated from Washington University in St. Louis in 1992 with a BFA in illustration. She developed her first professional comic strip, *Got a Life,* in 2000, which was distributed by King Features Weekly Service.

In 2006, Terri's daily strip, *The Pajama Diaries,* launched. Inspired by her early motherhood days of juggling jobs and family, it centers on the outlook and challenges of a multitasking mom. *The Pajama Diaries* currently runs in hundreds of newspapers throughout the country and abroad. In 2014, it was nominated by The National Cartoonists Society for "Best Newspaper Strip."

Terri has three *Pajama Diaries* book collections: *Déjà To-Do, Having it all…and no time to do it,* and *Bat-Zilla.*

Born and raised in northeast PA, Terri lives with her husband and two daughters in Cleveland, OH. You can find her work online at www.pajamadiaries.com

MPN Research: YOU Can Make a Difference

Gifts provide critical support needed to conduct innovative MPN research. Our MPN clinical and laboratory research team is dedicated to improving treatment outcomes for patients with MPNs.

To make a donation by mail, please send gifts to The University of Texas MD Anderson Cancer Center and specify "MPN Clinical Research Center" in the memo line using the attached envelope.

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for **Myeloproliferative Neoplasia** Newsletter

MPN Focus

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Editor: Kate J. Newberry, Ph.D.
For questions, comments or to subscribe, please contact Kate Newberry at **kjnewber@mdanderson.org**

Resources for Patients

2016 Patient Education Symposia hosted by MPN Advocacy & Education International

MPN ADVOCACY & EDUCATION INTERNATIONAL

- **January 28, 2016** in Seattle, Washington
- **February 25, 2016** in Baltimore, Maryland
- **March 17, 2016** in San Mateo, California

For more information visit:For more information visit: **www.mpnadvocacy.com** or contact **Ann Brazeau** at **517-889-6889** or **abrazeau@mpnadvocacy.com**

Founded by Ann Brazeau, former vice president of development at **MPN Research Foundation, MPN Advocacy & Education International** (MPN AEI) provides educational programs, materials, and resources for patients, caregivers, physicians, and entire healthcare teams to improve their understanding of MF, PV, and ET.

Other Online Resources:

MPNforum Monthly…the MPN community's hometown paper

MPNforum Monthly is a not for profit online magazine founded by MPN patient Zhenya Senyak. MPNforum monthly (mpnforum.com) publishes stories, features and columns that impact the lives of patients suffering from an MPN.

MPDSUPPORT.ORG

Founded in 1994 by patient advocate, Robert Tollen, the **MPD-SUPPORT** website and email list has offered interesting information on MPNs. Anyone is welcome to subscribe and all archives are available. Robert, who was diagnosed with PV in 1990 has also created a closed Facebook group with more than 1500 members. For more information or to join the list serve go to MPNSUPPORT.ORG or email listserv@listserv.icors.org with "subscribe mpdsupport" in the body of the email. To join the Facebook group go to: https://www.facebook.com/groups/375525335856981/

MPN EDUCATION FOUNDATION

MPN-NET

MPN Education Foundation

Formed in 2004, the **MPN Education Foundation** aims to bring information, reassurance and support to MPN patients and their loved ones all over the world via a website (www.mpninfo.org), by convening a patient conference every 2 years, and via the email-based support group **MPN-NET.**

MPN-NET is an email-based support group formed in 1994 by patient Joyce Niblack. In May of 1996 the group became a member of the Association of Cancer Online Resources, distributing email via a listserv platform. Although MPN-NET remains a US-centric organization, the group has nearly 2900 members from around the globe. All discussions since its inception in May 1996 are archived and available to all members. You can subscribe to MPN-NET on Foundation's homepage at www.mpninfo.org.

Apfed
American Partnership for Eosinophilic Disorders

APFED is a non-profit patient advocacy organization established to assist and support patients and their families coping with eosinophil associated diseases (EADs), including eosinophil-associated gastrointestinal disorders, hypereosinophilic syndrome, and Churg-Strauss Syndrome. For more information go to www.apfed.org.

The Mastocytosis Society

The Mastocytosis Society, Inc. is a non-profit organization dedicated to supporting patients with mastocytosis and mast cell activation disorders, as well as their families, caregivers and physicians through research, education and advocacy. www.tmsforacure.org.

Support for Patients in Texas

Founded by MPN patient and advocate Charlie Nielsen, the South Texas support group meets several times a year to discuss issues associated with living with an MPN.

The North Texas support group led by Karen Stern meets quarterly.

Both groups provide an opportunity to meet and share with others with a similar diagnosis.

To find out more information or join either group, please contact them either by e-mail or through our Facebook page:

North Texas, Dallas/Ft. Worth - Karen-Stern@sbcglobal.net

South Texas, Houston – CharlieNielsen@aol.com

Facebook: https://www.facebook.com/groups MPNSupportTX/