Externally-Led Patient-Focused Drug Development Meeting

FOR MYELOPROLIFERATIVE NEOPLASMS

SEPTEMBER 16, 2019
HYATTSVILLE, MD
Dear Myeloproliferative Neoplasms community member:

On behalf of the MPN Research Foundation, I want to welcome you to the Externally-Led Patient-Focused Drug Development Meeting for the MPNs! We are so pleased that you have been able to join us in person for this event and taking part via the webinar. We and our partners - Leukemia & Lymphoma Society, MPN Advocacy & Education International, MPN Cancer Connection, and MPN Education Foundation – are eager to put a spotlight on the stories of people living with Polycythemia Vera, Essential Thrombocythemia and Myelofibrosis.

We know that it will be impossible to bring to bear the individual experiences of the hundreds of thousands of people living with an MPN in the U.S. alone, but we want to ensure that the professionals in the Food and Drug Administration have the benefit of what we as advocates hear daily.

People with PV, ET, and MF are living with this chronic blood cancer. They often suffer in silence. They live with a variety of symptoms and health risks as well as side effects of their treatments. Finally, they are eager for more therapeutic options and, eventually, a cure.

By being here today or taking part in the webinar, you are helping every one of the people living with an MPN. Thank you for making a difference.

Sincerely,

Michelle Woehrle, MPA
MPN Research Foundation
# AGENDA

<table>
<thead>
<tr>
<th>Time</th>
<th>Session Details</th>
</tr>
</thead>
<tbody>
<tr>
<td>8:30 am</td>
<td>Registration</td>
</tr>
</tbody>
</table>
| 9:30 am | Opening Remarks  
Michelle Woehrle, Executive Director, MPN Research Foundation |
| 9:40 am | Clinical Overview of MPNs  
Robyn Scherber, MD, MPH |
| 9:55 am | Introduction and Meeting Overview  
James E. Valentine, J.D., M.H.S., Meeting Moderator |
| 10:05 am | Audience & Remote Demographic Polling |

**Patient Panels and Audience Discussion**

| Time  | Topic 1: Living with an MPN |-| Panel Discussions |
|-------|-----------------------------|---|
| 10:15 am | • Living with Polycythemia Vera |
| 10:15 am | • Living with Essential Thrombocytethmia |
| 10:15 am | • Living with Myelofibrosis |

<table>
<thead>
<tr>
<th>Time</th>
<th>Audience and Remote Polling</th>
</tr>
</thead>
<tbody>
<tr>
<td>10:45 am</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Time</th>
<th>Moderated Audience Discussion</th>
</tr>
</thead>
<tbody>
<tr>
<td>11:00 am</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Time</th>
<th>Lunch</th>
</tr>
</thead>
<tbody>
<tr>
<td>12:00 pm</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Time</th>
<th>Living with MPN Video</th>
</tr>
</thead>
<tbody>
<tr>
<td>12:50 pm</td>
<td></td>
</tr>
</tbody>
</table>

| Time  | Meghana Chalasani  
Center for Drug Evaluation and Research, U.S. Food and Drug Administration |
<table>
<thead>
<tr>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>1:00 pm</td>
<td></td>
</tr>
</tbody>
</table>

| Time  | Topic 2: Current & Future Treatments |-| Panel Discussion |-| Audience & remote polling |
|-------|-------------------------------------|---|-----------------|---|
| 1:05 pm | |

<table>
<thead>
<tr>
<th>Time</th>
<th>Moderated audience discussion</th>
</tr>
</thead>
<tbody>
<tr>
<td>1:40 pm</td>
<td></td>
</tr>
</tbody>
</table>

| Time  | Recap and Closing Remarks  
Ruben Mesa, MD, FACP and John Mascarenhas, MD |
<table>
<thead>
<tr>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>3:00 pm</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Time</th>
<th>Farewell Remarks</th>
</tr>
</thead>
<tbody>
<tr>
<td>3:20 pm</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Time</th>
<th>Meeting concludes</th>
</tr>
</thead>
<tbody>
<tr>
<td>3:30 pm</td>
<td></td>
</tr>
</tbody>
</table>
Our Mission
The mission of MPNRF is to stimulate original research in pursuit of new treatments - and eventually a cure - for the blood cancers polycythemia vera, essential thrombocythemia, and myelofibrosis, known collectively as myeloproliferative neoplasms (MPN).

Through a combination of MPN cancer research, advocacy, and education, we bring together patients, researchers, and clinicians around the common goal of realizing new treatment options and a cure for MPNs.

Our History
Diagnosed with polycythemia vera in 1997, Chicago businessman Robert Rosen was shocked to discover that little research was being conducted on PV, ET, and MF and that there were no advocacy groups working to assist people with these rare blood cancers.

Our history as an organization that was founded by patients for patients continues to influence everything we do. In addition to funding promising MPN research, we work to educate and empower patients, family members, doctors, and researchers across the MPN community. Together, we’re committed to change the prognosis for people living with an MPN.

Our Research Impact
With more than $13 million in funding and over 60 funded research projects, we have made a significant difference in advancing our understanding of the causes of MPNs, the development of new drug therapies and in cutting edge research that has been published in leading scientific journals.

Trusted Resource for Patients
Founded by patients for patients, the MPN Research Foundation works every day to educate and empower patients, caregivers, doctors, and researchers throughout the world. MPNRF strives to provide the MPN community with the latest news in MPN research, treatments, drug discovery, community events, support groups, and educational resources.

Study Locations: Global (including US, EU, Canada and Asia-Pacific).

For symptomatic patients (N = 180) experiencing anemia with Primary Myelofibrosis, Post-Polycythemia Vera Myelofibrosis or Post-Essential Thrombocythemia Myelofibrosis who were previously treated with JAK inhibitor therapy.

For further information, healthcare providers may contact info@momentumtrial.com.

Study activation planned for Q4 2019.
MPN Advocacy and Education International is dedicated to providing the knowledge, support, and resources patients will need as they adjust to living with an MPN through educational symposia in several cities each year, website access, free webcasts of each program, collateral materials, and direction to people, resources and other organizations that can help. MPN Advocacy and Education International identifies unmet needs in the MPN Community and provides advocacy support to individuals and groups unable to represent themselves.

The MPN Education Foundation is a non-profit organization run by volunteer MPN patients. It was incorporated in early 2004 at the urging of Dr. Ayalew Tefferi of Mayo Clinic (Rochester). The initial thrust of the Foundation was to continue the information/education/support goals of Dr. Harriet Gilbert through her MPN Research Center, which unfortunately died when she did in 2003. Our co-founder, Joyce Niblack (who passed in 2009), was a patient volunteer for Dr. Gilbert’s center and Editor of the MPD VOICE newsletter.

MPN Cancer Connection (MPN-CC) was created by David Wallace. He is an outspoken “patient advocate” who has Polycythemia Vera, an uncommon MPN blood cancer. Speaking from the patient point of view, he experiences areas that need improvement and acts upon those needs. MPN-CC partners with organizations who provide valuable resources to fellow patients. Funds raised are used to create more educational programming and resources for patients.

The Leukemia & Lymphoma Society (LLS) is the world’s largest voluntary health agency dedicated to blood cancer. The LLS mission: Cure leukemia, lymphoma, Hodgkin’s disease and myeloma, and improve the quality of life of patients and their families. LLS funds lifesaving blood cancer research around the world and provides free information and support services for patients and caregivers.
Michelle Woehrle, MPA
Michelle Woehrle has been working with the MPN Research Foundation since 2007 in a variety of roles, most recently as Executive Director. PV patient and founder Bob Rosen was Michelle’s mentor and was the first person with an MPN she met (that she knew of). Her passion is executing the mission of MPNRF in a way that is true to the greatest unmet needs of the people living with PV, ET and MF. Michelle has a Master’s in Public Administration with a focus in nonprofit management. She lives in the suburbs of Chicago with her husband and two little boys.

James Valentine, JD, MHS
Mr. Valentine assists medical product industry and patient advocacy organization clients in a wide range of regulatory matters, including new drug and biologic development and approval issues. Before joining his current firm in 2014, Mr. Valentine worked in FDA’s Office of Health and Constituent Affairs where he facilitated patient input in benefit-risk decision-making and served as a liaison to stakeholders on a wide range of regulatory policy issues. There, Mr. Valentine administered the FDA Patient Representative Program, launched the Patient-Focused Drug Development program, and developed the FDA Patient Network.
Ruben Mesa, MD, FACP

Dr. Ruben Mesa is the director of UT Health San Antonio MD Anderson Cancer Center. He has been involved in MPN research for more than 20 years. He led the development of National Comprehensive Cancer Network’s panel guidelines, the first U.S. guidelines on the diagnosis and treatment of MF, PV and ET. Dr. Mesa has been the principal investigator or co-principal investigator of more than 70 clinical trials. He co-led the research team leading to the FDA’s approval of fedratinib and ruxolitinib for PV and MF. Dr. Mesa was elected to sit on the National Board of Directors for the Leukemia and Lymphoma Society and is Chair on the Steering Committee for myMPN, the MPN Research Foundation’s online patient registry.

Robyn Scherber, MD, MPH

Dr. Robyn Scherber is an active clinician and researcher focusing on chronic myeloid blood cancers, specifically the myeloproliferative neoplasms. She currently sees a variety of patients with myeloid disease at UT Health San Antonio MD Anderson Cancer Center, including myelodysplastic syndrome and acute leukemia. She has a broad portfolio of research experience that has included the investigation of novel treatment strategies to improve quality of life. These efforts include conducting investigational trials that evaluate the impact of pharmacologic and/or integrative care approaches to therapy.

John Mascarenhas, M.D.

Dr. John Mascarenhas is an Associate Professor of Medicine at the Icahn School of Medicine at Mount Sinai (ISMMS) and a member of the Tisch Cancer Institute. Dr. Mascarenhas is the Director of the Adult Leukemia Program and Leader of Clinical Investigation within the Myeloproliferative Disorders Program at Mount Sinai, under the direction of Dr. Ronald Hoffman. As a clinical investigator in malignant hematology with a focus in translational research involving myeloproliferative neoplasms (MPNs) and evolution to acute myeloid leukemia (AML), he is focused on the evaluation of rationale based novel therapies for patients with MPNs and AML.
David K - PV Patient

David is a 54-year-old marketing and technology business executive living in Colorado. After losing significant weight and several years of experiencing visual disturbances, he was eventually diagnosed with JAK2 positive PV in June 2019 and started treatment with phlebotomies and a compound to control thrombotic complications. He had been working in a demanding field but had to stop work due to the mental fog caused by fatigue and visual disturbances. Even now with the symptoms mostly alleviated, he is concerned his life will never get back to normal.

Vicki B - ET Patient

Vicki is a medical journalist living in New York. She was diagnosed with Essential Thrombocytthemia 8 years ago. Her professional skills help her to investigate every angle of her diagnosis with ET, and seek information to help her understand how to manage her disease best and what her future may hold, especially as she fears she may be progressing to Myelofibrosis.

Vivienne W - PV Patient

Vivienne has been living with PV for 20 years. She was put on a JAK2 inhibitor four years ago and it’s done a great job controlling her spleen and blood counts, but she now struggles with skin lesions, GI problems and other quality of life challenges. She is thankful for the years that she has survived and the wonderful people that she has met on her PV journey, including MPNRF, her support group, her doctor and his office staff, the scientists, the FDA and pharmaceutical companies, all who have made her journey possible. She is grateful to her husband Richard and her family, who have always understood her limitations.

Bridget B - ET Patient

Bridget works in financial services operations and lives in East St. Louis. She was diagnosed with ET in 2015 and has experienced discordance between doctors’ opinions on how to best treat her disease and symptoms. She’s been stable on a platelet reducing drug along with an aspirin regimen but still experiences clotting issues, and her platelets are not well controlled. Her frequent symptoms include fatigue, itching, numbness in her hands and legs, joint pain, and problems with concentration.
PATIENT PANELISTS

Ruth Fein R - MF Patient
Ruth has lived with an MPN for 23 years. She was first diagnosed with ET, which transitioned to PV, and recently to MF. She has two adult sons living in Manhattan and she lives with her husband in Saratoga Springs, NY. Her MPN experience includes a misdiagnosis from bone pain and migraines, a major thrombotic event, and a serious GI hemorrhage following surgery for colon cancer last year. Still, she lives her day-to-day life, “primarily symptom free.” She is a freelance writer and communications consultant specializing in health and renewable energy. Recently, she began lobbying for rare diseases and exploring ways to help others living with MPNs.

Karrie S - ET Patient
Karrie is 48 years old and was diagnosed with ET when she was pregnant with her first child at age 37. Two years later, her diagnosis changed to PV. Her MPN posed significant hurdles for her in achieving her goal to have more children. She then found a drug that helped control her disease and allowed her to carry her second child to term. She’s now focusing on making the most out of life and bringing awareness to patients like her who may not realize that there are therapeutic options available.

David A - PV Patient
Living with diagnosed PV since 2005, but neurocognitive issues for 15+ preceding years. A regular blood donor since age 18, he always felt better after donation, same after starting therapeutic phlebotomies. Started a JAK2 inhibitor in 2016 against intractable pruritus but also had a 70% spleen response, although he had no spleen symptoms. Had to discontinue the JAK2 inhibitor in 2019 due to life threatening cryptococcal infection. He has remained on a phlebotomy regime without problems since diagnosis. David is President of the all-patient/volunteer MPN Education Foundation, and is an online and DC local support group leader.

Diane R - PV Patient
Diane was diagnosed with an MPN 14 years ago and has seen it change from one form to another. She has some success with medication controlling her blood counts but not her symptoms. Her biggest concerns are the possible negative side effects of current treatment options and that those same treatments don’t alleviate her ongoing MPN symptoms.
PATIENT PANELISTS

**Nancy D - MF Patient**

Nancy is 52 years old, is JAK2 positive, and has been living with an MPN for 33 years. She was diagnosed with PV in 1986 during a routine blood test for mono. She then progressed to MF 26 years later. After many different therapies, including two clinical trials, nothing had been able to shrink her spleen until her current treatment, the recently approved JAK2 inhibitor. She was recently diagnosed with the ASXL1 gene and is contemplating a stem cell transplant, but does not currently qualify due to her massive spleen and among other complications. Her other MPN symptoms have been manageable with each therapy along the way.

**Ned W - MF Patient**

Ned, trained as a chemist, is semi-retired as an executive in the pharmaceutical and medical device industry. He is 76 and was diagnosed with PMF in November 2018. He entered a clinical trial for a new experimental drug for MF in late March 2019. This has required travel from Northern Utah to UCLA every 3 weeks. He feels his constitutional symptoms have been significantly reduced and his spleen volume has also decreased, however he has had to begin red cell transfusions. Overall he is currently living an almost normal life with the disease.

**Morgan G - MF Patient**

Morgan is a 52 year old married mother of three. She lives in the Chicagoland area and works as a Project Manager for a meetings and event company. She was diagnosed in January 2019 and takes a compound to control thrombotic complications and aspirin daily. Her MPN experience includes living with daily symptoms of bone pain, night sweats, fatigue, joint pain, numbness/tingling in hands and feet, and issues with concentration. Her biggest concern is living with the unknown progression of an MPN.
myMPN PATIENT REGISTRY
www.MPNRF.org/mympn-register

About myMPN
myMPN empowers patients to change their prognosis by sharing their experience of living with an MPN. The development of targeted disease therapies will be expedited when firsthand information about disease symptoms and progression are available from patients.

What is myMPN?
- It’s a digital hub for patients to record and anonymously share their unique MPN journey with the research community.
- Participants can access a secure online portal with a personalized dashboard. As they complete surveys listed on the dashboard, the registry will provide insights into how the user’s MPN experience compares to other registry users.
- All patient data is protected and only shared according to individual user privacy settings.
- myMPN is a place for eligible patients to connect with upcoming drug trials and observational studies that will help increase our knowledge about PV, ET, and MF.
- Participation is currently limited to those inside the U.S. and over the age of 18.
- myMPN is not yet mobile-friendly, so please use a laptop or desktop to access your account.

What are the benefits of joining myMPN?
- myMPN functions as a repository for each participant’s experience with an MPN. Each completed survey is accessible by the user going forward. Participants can download their symptom, event and other data to share with their doctor or for other purposes.
- The registry provides participants with the opportunity to inform the research and drug development community with data they need to move better MPN treatments (and, potentially, cures) through the discovery pipeline more quickly.
- The platform offers the research community the ability to follow-up on specific topics of interest. For example, a researcher may request additional information on the experience of a group of patients who have used a particular therapy.
- Participants will learn about clinical trials that may be of interest to them.

myMPN PATIENT REGISTRY

Committed to Improving the Lives of Patients Worldwide®

www.celgene.com © 2019 Celgene Corporation

Patient Focused Drug Development Meeting for Myeloproliferative Neoplasms
THANK YOU TO OUR SPONSORS

GOLD

Incyte

PharmaEssentia

SILVER

SIERRA ONCOLOGY

Constellation

PHARMACEUTICALS

BRONZE

geron

Genentech

A Member of the Roche Group

cti

BIOPHARMA

KARTOS THERAPEUTICS