

MPN RESEARCH FOUNDATION

CHANGE YOUR PROGNOSIS UPDATE



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NEVER ALONE**

VOLUME XIX, NO. 2 FALL 2020

PERIODIC NEWSLETTER FOR THE MYELOPROLIFERATIVE NEOPLASMS COMMUNITY

MPNRF WELCOMES NEW EXECUTIVE DIRECTOR KAPILA VIGES

Barbara Van Husen
Board President



Executive Director,
Kapila Vigés

Towards the end of 2019, our Executive Director Michelle Woehrle made the difficult decision to step back from her all-consuming roll at MPNRF to spend time with her young family. With regret but also determination, the Foundation embarked on an executive search for her replacement, a difficult task under any circumstances. Little did we know *how* difficult this search would be.

We had recruited a short but excellent group of candidates when a certain

virus caused a certain national shutdown, and all of us retreated to our ZOOM-enabled home offices. With the assistance of Kittleman & Associates, a national search firm, we discussed requirements, screened resumes, and finally conducted virtual interviews via ZOOM. Both candidates and the members of the Foundation search committee became ZOOM experts. I am pleased and excited to announce that the search for a new Executive Director to replace Michelle has come to a successful conclusion. With the enthusiastic approval of our entire search committee, we have identified an individual we believe will help the Foundation surge to a new level of effectiveness.

Kapila Vigés comes to us with a long and strong background in supporting cancer research, biopharma interactions, and development of creative partnerships to accelerate new therapy development.

Although we spoke to a number of very qualified candidates, our goal was to find someone special—someone with Michelle's leadership

skills and sense of strategy, but who would also be able to propel the Foundation to a new level of research funding and innovation. Kapila meets this criteria with flying colors. Her experience includes management and strategy consulting in biomedical industry, biomedical startup incubation and seed investing of translational research, and public-private-partnerships in economic development. Kapila joined us this fall.

"IT HAS BEEN AN ABSOLUTE PLEASURE GETTING TO KNOW THE MPNRF BOARD AND TEAM. I AM GRATEFUL TO MICHELLE FOR THE SOLID FOUNDATION SHE HAS BUILT AND HOPE TO CARRY ON THE SPIRIT AND PASSION IN THIS NEXT CHAPTER. I LOOK FORWARD TO LEARNING AND GROWING TOGETHER WITH THE TEAM TO FURTHER MEANINGFUL IMPACT ON PATIENTS." — KAPILA VIGES

This search has been a true team effort, and I want to thank everyone who contributed their time and brainpower. It's been hard, as you can imagine, to find someone in this strange environment who can even come close to the leadership we have been blessed with from Michelle. Fortunately, Michelle, with the help of Rick Winneker, has kept the Foundation boat afloat and steering well during this difficult time.

We welcome Kapila with open arms, and send Michelle off with our best wishes and the hope we will be seeing her again in a new MPNRF role soon. ■

Many thanks to our sponsors AbbVie and Bristol Myers Squibb.

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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.

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MPN RESEARCH FOUNDATION UPDATE

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.

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MESSAGE FROM THE BOARD OF DIRECTORS

To the MPN community of patients, clinicians, nurses, caregivers, researchers: We know that so much has changed in this world over the last several months. Despite the pandemic, our own organization has remained active and continues to change and evolve to meet the new requirements of doing business. We resolutely remain an organization focused on patients. We are here to help change your prognosis – for the better. We, the board members of MPN Research Foundation, represent people living with PV, ET or MF, their friends and family members and remain committed to **you**.

We are excited to continue our venture into new discoveries and have renewed our dedication in our search for new and varied sources in the field to provide relief and possible cure. Thank you for your support through the years and investing your hope and faith in us. Together, we are confident we will meet the moment. ■

ANSWERING THE HARD QUESTIONS WITH HELP FROM OUR PARTNERS

Rick Winneker, PhD
Director, Strategies and Research Operations

In 2017 MPNRF began funding the MPN Interferon Initiative – four projects that explore how Interferon works in achieving remissions in some MPN patients, and why it doesn't work as well in others or loses its effectiveness over time. The answers to these questions are essential to designing new and improved ways to enhance the activity of Interferon and increase the patient population that will respond to this therapy, which is alone in its ability to reduce the mutated JAK2 in MPN for some patients.

It is important to note that the Interferon Initiative was made possible not only by our donors, but also with support from PharmaEssentia, The MPN Alliance Australia and The Cancer Treatment & Research Fund. We have ensured the research team (located at six different institutions in the US, France, Germany and Australia) that we need to continue to support this project to its successful conclusion.

With the completion of this important 3-year endeavor on the horizon, we will be reviewing results of all projects as well as determining what, if any, our next steps should be. We encourage all participants to continue to publish their findings and present them publicly so that they will be discoverable by researchers for years to come. Through this research and others, clinicians will have better information about 1) who are ideal candidates for Interferon-based therapy and why, and, 2) which patients will respond better or longer to Interferon alone or when combined with other therapies. As with all medicines, the more we know about what makes it work and why, the better able we are to maximize the effectiveness of each decision made by a clinician with his or her patients thereby potentially prolonging life and increasing quality of life.

We look forward to reporting the findings of the Interferon Initiative team to you in our next newsletter. And we would love to hear from you: what do you think about the Interferon Initiative? Send your thoughts to info@mpnrf.org. ■



YOU ARE A PARTNER, AN ADVOCATE, A FRIEND... *We Need Your Help Now!*

Pamela Soto
Director of Individual & Major Giving



As long as MPNRF exists, there will be a funding stream for a MPN clinical researcher community to support those living with the disease. This year, our Foundation marks over two decades for working for the fight against MPNs. Amid the Covid-19 pandemic, we are doing all we can to keep our momentum going and fighting on behalf of

those with MPNs. Continuity is so important to furthering research goals, so we are regularly in touch with our grant recipients, as well as maintaining outreach to experts about the impact of Covid-19 on our patient population. We'll provide current information on our website, communications, and patient support groups.

Our dedication to the MPN Community has never been stronger, but we need your help. There is strength when people come together. You can make a difference in advancing the growth of our mission and the fight against MPNs. We were founded *by patients, for patients*. You are a partner. You are an advocate. You are a friend. Reflecting on our 20 years, we are proud of what we have accomplished, but our programs are evolving and reflect the need for increased funding for more research.

We urge you to support the mission of the MPN Research Foundation today with a gift. Especially now, during these uncertain times, you can make a difference in our research!

Thank you for your ongoing support and confidence. Reach out to me at psoto@mpnrf.org or donate at mpnresearchfoundation.org/Donate-to-MPN-Research. ■



CHANGE YOUR PROGNOSIS

PROVIDING EDUCATION AND RESOURCES FOR A BETTER TODAY. INVESTING IN RESEARCH FOR A BETTER TOMORROW.

Partner. Advocate. Friend. At the MPN Research Foundation, we're committed to standing with you in the fight against polycythemia vera (PV), essential thrombocythemia (ET) and myelofibrosis (MF) – the group of blood cancers collectively known as myeloproliferative neoplasms.

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MYMPN – DATA PRESENTED DURING LIVE WEBINAR

Lindsey Whyte
Longitudinal Research Project Manager

Many thanks to all who joined us for the June 19th webinar featuring Dr. Alison Moliterno, who presented myMPN research titled "Characteristics and Features of Non-Caucasian Myeloproliferative Neoplasm Patients in the myMPN Registry." Following the presentation, Dr. Moliterno was joined by Drs. Ruben Mesa, Srdan Verstovsek and Amylou Dueck, who answered questions submitted in advance and by webinar participants. If you missed it, you can visit our website at www.mpnrf.org/myMPN-poster-webinar to see a replay.

We look forward to presenting myMPN data in this format again in the near future and are open to topics of interest to the MPN community! MPNRF staff are also available to speak to local support groups virtually. Please contact us at myMPN@mpnrf.org if you have questions or topics that interest you relating to patient registry data, or you would like to schedule a support group presentation. ■



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THE MPN PATIENT VOICE AND THE FDA



Michelle Woehrle

A year ago, we hosted an externally-led Patient Focused Drug Development (PFDD) meeting. The purpose of this meeting was to elevate the voices of the patient population for the benefit of improving drug development for people with Polycythemia Vera (PV), Essential Thrombocythemia (ET) and Myelofibrosis (MF). We were thrilled to have partnership with our patient advocate allies and support from industry for this full day meeting in Maryland. Dr. Ann Farrell, Division Director of the Division of Hematology Products participated in the meeting during which a diverse group of people shared their experiences living with an MPN. They touched on what it was like to have medicine after medicine fail, to rely on blood transfusions for their anemia, to deal with symptoms of the disease (or side effects from medicines) while trying to maintain their employment, social lives, and more.

Our goal was to ensure that future MPN drug development is centered on the factors that matter to the patients: improving quality of life and halting disease progression. Drugs that bring relief to some symptoms have been life changing for some with an MPN, and stem cell transplant remains an option for those who qualify and get it in time. However, there is more to be accomplished where impact to the underlying disease is concerned.

The videos of the meeting are available publicly on our YouTube channel and transcripts are being synthesized into a *Voice of the*



Patient Focused Drug Development (PFDD) Meeting, 2019

Patient report, which we anticipate to be completed Fall 2020. We are working with the firm Evidera on the report, which will be an ongoing reference for FDA regulators, researchers and others, posted on the FDA website and distributed to the MPN community as soon as it is available. That report will provide us additional opportunities to identify areas for future patient advocacy, working closely with regulators and the researcher-clinician community to validate new clinical end points that are meaningful to the patients so that even better therapies for PV, ET and MF are discovered. ■

CLINICAL TRIAL HIGHLIGHTS



Research for Myeloproliferative Neoplasms has picked up heavily over the last few years. There are many MPN Clinical Trials underway. A complete list can be found on our website at:

mpnresearchfoundation.org/Clinical-Trials.

The trials listed below maybe of interest as they are currently enrolling patients:

PTG-300

Sponsor: Protagonist Therapeutics, Inc
Contact: 1-888-899-1543
ptgxcntrials@ptgx-inc.com
Diagnosis: Polycythemia Vera

IMG-7289

Sponsor: Imago Biosciences
Contact: 415-529-5055 info@imagobio.com
Diagnosis: Essential Thrombocythemia

9-ING-41

Sponsor: Actuate Therapeutics Inc.
Contact: Francis J. Giles, MD
fgiles@actuatetherapeutics.com
Diagnosis: Myelofibrosis

Momelotinib

Sponsor: Sierra Oncology, Inc.
Contact: Ashwin Swami, MD
MBA aswami@sierraoncology.com
Diagnosis: Myelofibrosis

Navitoclax

Sponsor: AbbVie
Contact: AbbVie Clinical Trials
abbvieclinicaltrials@abbvie.com
Diagnosis: Myelofibrosis (MF),
Primary MF, Secondary-MF,
Post-polycythemia vera (PPV) MF,
Post-essential thrombocytopenia (PET) MF

Pacritinib

Sponsor: CTI BioPharma
Contact: Sirin Artan Kahrs, MD
sartankahrs@ctibiopharma.com
Diagnosis: Myelofibrosis ■

TRACKING PROGRESS IN MPN RESEARCH THROUGH CLINICAL TRIAL UPDATES

Rick Winneker, PhD
Director, Strategies & Research Operations

Tracking progress in MPN research and development is an important part of our mission. We do this through regular interactions with clinician/researchers and biopharma companies developing new therapies. Another source of great information are the updates on clinical trials presented at international meetings. Below are a few trial updates recently reported at this year's European Hematology Association meeting.

CPI-0610 / "MANIFEST" STUDY

This is a phase II clinical trial of CPI-0610, a BET inhibitor, in patients with myelofibrosis (MF). This study has 3 treatment arms to capture different patient populations and treatment strategies. CPI-0610 given alone or in combination with ruxolitinib was generally well tolerated and continues to show promise. Data is particularly promising for the first line CPI-0610 plus ruxolitinib treatment cohort, converting some patients to transfusion independence and improving hemoglobin levels. Based on these results, a phase III trial focusing on this treatment arm is planned for later this year. The other two arms will be expanded to include more patients.

ROPEGINTERFERON IN LOW RISK PV / "LOW PV" TRIAL

Patients with polycythemia vera (PV) should keep their hematocrit (HCT) level at or below 45% to reduce major thrombotic events. The Low PV trial addresses the question of whether phlebotomy alone or the addition ropeginterferon alfa-2b (Ropeg), can lead to improved outcomes for those patients defined as "low risk" (< 60 years of age and no history of thrombosis). The interim analysis presented indicated that Ropeg in addition to phlebotomy is more effective than phlebotomy alone to maintain HCT at the desired level. Patients will be followed for an additional year.

MOMELOTINIB LONG-TERM SAFETY

Data were presented from more than 90 patients who participated in previous phase III trials but continued on momelotinib for 3.5 years or longer. The results were consistent with the data from the previous trials in that there remained a sustained increase in hemoglobin levels and platelet counts without significant rates of high-grade hematological toxicities. Long-term tolerability was favorable with no new safety signals or evidence of cumulative toxicity. Momelotinib is now being compared with danazol in a phase III MOMENTUM clinical trial in patients with symptomatic and anemic myelofibrosis who have been treated previously with a JAK inhibitor.

IMETELSTAT / "IMBARK" TRIAL

Data were presented from the "IMbark" phase 2 clinical trial evaluating 2 doses of imetelstat in relapsed/refractory MF patients. Of note, improvements were seen in overall survival with imetelstat and this potential survival benefit was supported by the trend of correlation with other clinical benefits such as symptom response and spleen volume reduction. Furthermore, it appears that triple negative patients, i.e., lacking JAK2, CALR, or MPL mutations, treated with the high dose of imetelstat had better clinical outcomes when compared to non-triple negative patients suggesting that this drug may be helpful for this high-risk group of patients.

RUXOLITINIB / REAL WORLD DATA IN PATIENTS WITH PV

Interim results were presented for a phase IV observational study to monitor patients with PV treated with ruxolitinib. The study follows patients from 88 hospitals across Europe who are being treated with ruxolitinib over the course of 24 months. Results were presented for 60 patients followed for 1 year. Overall, patients achieved hematocrit control, reduced dependence on phlebotomies, and maintained or reduced spleen size during the first year of this study. Data collection will continue on all 352 patients over the course of 2 years.

NAVITOCCLAX

An update of the phase II study of the combination of navitoclax with ruxolitinib in MF patients was presented. Data were presented for 34 patients following 24 weeks of treatment. Overall, meaningful spleen reductions and improvements in symptoms were seen along with some reduction in bone marrow fibrosis. The drug combination was well tolerated. ■



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MEET MARY GRANT: SUPPORT GROUP LEADER AND MF PATIENT

Jennifer Acker
Content Writer for MPN Research Foundation

In 2012, Mary Grant joined a support group in Boise to help her navigate a primary myelofibrosis diagnosis. Today she leads that group, meeting with other patients for lunches and holiday get-togethers. Over the years, Mary has formed close friendships, grieved the losses of members, and welcomed new members. One patient is an SCT survivor, others are in clinical trials, but this group shares in common their mutual support and diverse knowledge of MPNs.

Mary considers herself a rare patient in that with minimal intervention, she is doing better today than when she was first diagnosed with PMF.

"The original diagnosis I received was not kind or encouraging. They made me feel like I was going to die, and it would be soon. When doctors said, 'The only cure is an SCT, and you're too old and have no siblings' and 'There's a drug we can give you, but it makes you feel like you have the flu,'... it felt like a death sentence."



Mary Grant (front left, in black sweater) with her MPNRF support group

Mary's diagnosis began with a startling phone call at midnight. She had been to the urgent care that day because she was having difficulty catching her breath, but everything checked out fine until her labs were finally read. The doctor on the other end of the line told her she was high risk for having a heart attack because her hemoglobin was dangerously low (6.1). She was advised to get to the hospital immediately. When she arrived, she was given three units of blood and underwent a colonoscopy and endoscopy to determine if she had internal bleeding; however, everything checked out fine. But

it wasn't long before her hemoglobin started dropping again. After meeting with an oncologist, Mary was told she had PMF and would be transfusion-dependent (she would need blood every 10-14 days.)

However, Mary sought out a second opinion at the Mayo Clinic in Scottsdale, Arizona.

"That is where I was given hope," she says. "They said I could get a transplant into my early 70's if I didn't have other issues. I had just turned 65, but they wouldn't do a transplant unless I were actually dying, so I called it my 'Hail Mary pass'". The Mayo Clinic also advised Mary to start with Procrit injections.

Slowly Mary's mindset shifted. She had spent forty years taking care of other family members, and realized it was time to take care of herself.

"When I was diagnosed, I had so much stress from caring for my mother and, ultimately, from her death. I tell people to be sure to take care of themselves when caring for others or they lose themselves, and that is what happened to me."

From September 2012 to September 2013, she had received 52 pints of blood, but then something unexpected happened. Mary's hemoglobin slowly went up, so much so that she wasn't transfusion dependent.

So, what was it? In talking about her progress, Mary says, "I believe I am in remission, but the doctors say there is no such thing as remission. The only cure is an SCT. They say I'm stable."

Currently, Mary gets her blood checked every six months, and her hemoglobin is usually around 14. Mary adheres to 10mg of prednisone and has no other symptoms related to MF. Her doctor had tried to wean her off of prednisone five years ago, but her numbers plummeted. Mary is also a firm believer in the collective power of positive energy from her support group.

"I continue to learn about MPNs, along with everyone in my group. We have had Zoom calls for the last two months, and it's so nice to see how everyone is doing and what we learn from one another. I think it's important to have fun. We laugh a lot and we're always there for each other. When I first started, there wasn't much information or help for us. Now there are so many resources that it is almost overwhelming.

"Today, I deal with things as they come up. I have many signs in my house that say things like 'Joy.' And I do choose joy now, every day." ■