



### 13TH INTERNATIONAL PATIENT SYMPOSIUM ON MYELOPROLIFERATIVE NEOPLASMS

Presented by Cancer Research & Treatment Fund

### **OCTOBER 23, 2024**

Ghaith Abu-Zeinah, MD Program Chair David Boule Patient Committee Chair

Belfer Research Building • Weill Cornell Medicine 413 East 69th Street • New York, NY 10021

### Welcome

**Cancer Research & Treatment Fund (CR&T)** welcomes you to its 13th International Patient Symposium on Myeloproliferative Neoplasms (MPNs). During this full-day educational event, patients, their families, and their friends will learn about the latest developments in the research and treatment of myeloproliferative neoplasms (MPNs).

You'll hear presentations by distinguished basic scientists and clinical research physicians from the world's most prestigious MPN centers. In the afternoon, you'll have the opportunity to speak with these experts during disease-specific Q&A sessions.

We are deeply grateful to our corporate and individual sponsors for their commitment to this event. Thanks to their generous support, we will be able to post the Symposium presentations on our website, www.crt.org, where they will be available to patients worldwide. We will notify you by email when these videos are available.

We also want to thank you for joining us today. We look forward to staying in touch as new information becomes available. Remember, we're always here to help! Follow us on our social media platforms, sign up for our email newsletter, and if you have any questions, please don't hesitate to contact us at 212-288-6604 or info@crt.org. We are truly grateful for your support.

Together, we can build a future without MPNs.

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**Ghaith Abu-Zeniah, MD** Symposium Chair Assistant Professor, Weill Cornell Medicine Medical Advisory Board, CR&T

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Dave Boule Patient Committee Chair Board of Directors, CR&T Retired Partner, Ernst & Young



# Program

8:00 AM Registration/Continental Breakfast/Exhibits 8:45 AM Welcome and Introduction: Ghaith Abu-Zeinah, MD - Symposium Chair Jedd Wolchok, MD, PhD, FASCO - Meyer Director of the Sandra and Edward Meyer Cancer Center at Weill Cornell Medicine Dave Boule - Patient Committee Chair 9:00 AM Origins and Genetics of MPNs - Linda Resar, MD 9:30 AM Inflammation in the MPNs - Hans Hasselbalch, MD 10:00 AM Cardiovascular Health in MPN Patients - Stephanie Feldman, MD 10:30 AM Break/Exhibits Symptom Relief, Diet, and Exercise in MPN Patients - Ruben Mesa, MD 11:00 AM 11:30 AM MPN Progression: Is it predictable? - Ghaith Abu-Zeinah, MD 12:00 PM Accelerated and Blast-Phase MPNs - Raajit Rampal, MD, PhD Lunch/Exhibits 12:30 PM 1.30 PM Drugs-Novel Agents for MPNs in 2024 - Naveen Pemmaraju, MD 2:00 PM Moderated O&A - Ghaith Abu-Zeinah, MD and entire panel 3:00 PM Break/Exhibits 3:30 PM BREAKOUT SESSIONS - Disease-Specific Groups Breakout Session - Polycythemia Vera (PV) Managing PV in 2024 - Richard T. Silver, MD 3:30 PM 4:00 PM Q&A - Drs. Abu-Zeinah, Hasselbalch, Silver Breakout Session - Essential Thrombocythemia (ET) 3:30 PM Managing ET in 2024 - Ellen Ritchie, MD Q&A - Drs. Mesa, Resar, Ritchie, Schafer 4:00 PM Breakout Session - Myelofibrosis (MF) 3:30 PM Managing MF in 2024 - Ronald Hoffman, MD 4:00 PM Q&A - Drs. Hoffman, Pemmaraju, Rampal 5:00 PM Wine Reception/Belfer Lab Tour 6:00 PM Adjournment



#### GHAITH ABU-ZEINAH, MD

Assistant Professor Assistant Attending Physician Weill Cornell Medical College/NewYork-Presbyterian Hospital

Ghaith Abu-Zeinah, MD, is Assistant Professor at Weill Cornell Medical College and an Assistant Attending Physician at the NewYork-Presbyterian Hospital. Dr. Abu-Zeinah earned his M.D. with academic distinction from the Weill Cornell Medical College in Qatar in 2013. He completed his

residency training in Internal Medicine (2016) and fellowship training in Hematology/Oncology (2019) at the NewYork-Presbyterian Hospital/Weill Cornell Medicine in New York City.

Dr. Abu-Zeinah is a Hematologist and Oncologist with a specialty practice in Myeloproliferative Neoplasms (MPN). His research is focused on identifying and developing therapies that target the malignant stem cells in patients with MPN. He received several awards for his work and presented at national conferences including the International Congress on Myeloproliferative Neoplasms and the American Society of Hematology. Dr. Abu-Zeinah is also the recipient of the 2022 MPN Hero Award. He has been the author or co-author of many scientific publications. He works at the Richard T Silver Myeloproliferative Neoplasms Center as a physician-scientist both treating patients and conducting research.

Dr. Abu-Zeinah is on the Medical Advisory Board of Cancer Research & Treatment Fund, Inc. (CR&T) and chair of CR&T's International Patient Symposium on Myeloproliferative Neoplasms (MPN).

#### STEPHANIE FELDMAN, MD

Assistant Professor of Clinical Medicine Director of the Cardio-Oncology Weill Cornell Medical College/New York Presbyterian Hospital

Dr. Stephanie Feldman is the Director of the Cardio-Oncology program at Weill Cornell Medical College/New York Presbyterian in Manhattan. She completed an Internal Medicine residency at Beth Israel Deaconess Medical Center/Harvard Medical School, fellowship in Cardiovascular



Disease at Boston Medical Center/Boston University School of Medicine, and an additional fellowship in Cardio-Oncology at Memorial Sloan Kettering Cancer Center. Prior to joining Weill Cornell in July of 2023, she founded and directed the Cardio-Oncology and Cardiac Amyloidosis programs at Rutgers New Jersey Medical School/University Hospital. Dr. Feldman has expertise in the management of cardiovascular disease in cancer patients before, during, and after cancer treatment. She has published on topics in cardio-oncology in JACC CardioOncology, the European Heart Journal, and the Journal of the American Heart Association. She has been featured on podcasts on topics in cardio-oncology including Cardio-Nerds and most recently New York Presbyterian's Advances in Care.



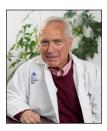
#### HANS HASSELBALCH, MD, PHD

Professor of Hematology Roskilde Hospital University of Copenhagen

Professor Hans Hasselbalch is Consultant Hematologist at the Department of Hematology, Roskilde Hospital, University of Copenhagen. For many years his professional interests have focused upon chronic myeloid neoplasms (MPNs), main research projects in MPNs being

integrated molecular (eg. gene expression profiling, epigenetics,SNPs), immune cell and microbiome studies in MPN patients before and during treatment with pegylated interferon-alpha2 (IFN)) and novel targeted therapies, including the JAK1-2 inhibitor ruxolitinib. Hasselbalch is principal investigator on the randomized Danish Multicenter Study on low-dose IFN (DALIAH). The results from the DALIAH-trial have contributed significantly to a paradigm shift in the treatment of MPNs , implying upfront treatment - sooner better than later – also to low-risk patients. In 2012, Hasselbalch published in Blood his hypothesis on chronic inflammation as the driver of clonal evolution, premature atherosclerosis and second cancers in MPNs and today chronic inflammation is considered a major driving force for disease progression, having likely great impact on the mutational landscape. His hypothesis has prompted several international studies on combination therapy with IFN and the JAK1-2 inhibitor ruxolitinib – a combination therapy which has been described as the most encouraging and promising treatment modality ever in MPNs. Hasselbalch has established epidemiological MPN-research on several cohorts. Most recently, these studies have unraveled MPNs in DK, equivalent to 550,000 in US.

It is envisaged that screening of "target populations " at risk of housing MPNs may unravel a large number of patients with MPNs, thereby integrating preventive MPN-medicine in the future care for MPN-patients, the ultimate goal being much earlier diagnosis and thereby institution of stem cell targeted IFN therapy much earlier than today and before they suffer life-threatening or life-invalidating thrombohemorrhagic events. Recently, Hasselbalch's hypothesis on some of the MPN mutations to be highly immunogenic has been confirmed and prompted the first MPN vaccination trials in the world in collaboration with National Center of Cancer Immune Therapy at Herlev Hospital. In recent years new Danish microbiome studies in MPNs have unravelled distinct signatures in MPNs and between MPN subtypes . Highly interestingly, IFN seems to normalize the abnormal microbiome signature in MPNs – an observation to be pursued in future "omics" studies.



#### RONALD H. HOFFMAN, MD, MSH

Albert A. and Vera G. List Professor of Medicine Director, MPN Research Program Icahn School of Medicine at Mount Sinai The Tisch Cancer Institute

Ronald Hoffman, MD, is the Albert A. and Vera G. List Professor of Medicine, Professor of Medicine (Hematology and Medical Oncology), and Director of the Myeloproliferative Disorders Research Program. He founded

this program in 2007 and has developed it into one of the most effective and well respected programs in the world dealing with myeloproliferative disorders. His interest in this group of blood cancers stemmed from his time as a fellow in Hematology at Mount Sinai. He continues to care for patients with myeloproliferative neoplasms on a daily basis. In 2003, Dr. Hoffman founded the Myeloproliferative Neoplasm Research Consortium, a multi-institutional program project grant funded by the National Cancer Institute, which is committed to research in and treatment of myeloproliferative neoplasms. Continuously funded by the National Cancer Institute for over a decade, the Consortium involves 12 institutions throughout the United States and Canada and is focused on identifying new therapies for patients with myeloproliferative neoplasms and evaluating their effectiveness in carefully constructed clinical trials

Dr. Hoffman is a leader in stem cell research. His studies of human hematopoietic stem cells and progenitor cells in myeloproliferative neoplasms have led to many therapeutic advances. He has also pioneered a unique method of increasing the number of cord blood stem cells used to treat patients with blood cancers and blood disorders. His extensive track record in flow cytometry, the performance of a variety of surrogate functional assays for hematopoietic stem and progenitor cells, immunohistochemistry and molecular biological based assay systems as well as expertise in clinical trials has allowed him to pursue hypothesis-based research both in the laboratory as well as the clinic. As a physician scientist who actively provides care to MPN patients, he's had the opportunity to observe the manifestations, sequelae and evolution of these diseases in patients which provides greater understanding of the biology of these chronic hematological malignancies.



#### RUBEN A MESA, MD, FACP

President, Enterprise Cancer Service Line Executive Director, Atrium Health Wake Forest Baptist Comprehensive Cancer Center Enterprise Senior Vice President, Atrium Health Vice Dean for Cancer Programs, Wake Forest University School of Medicine Professor of Medicine, Wake Forest University School of Medicine

Dr. Mesa recently began his tenure at Atrium Health and Atrium Health Wake Forest Baptist as President, Enterprise Cancer Service Line Atrium Health; Enterprise Senior Vice President for Atrium Health; Executive Director of the NCI Comprehensive Cancer based at Wake Forest Baptist; and Vice Dean for cancer programs at Wake Forest School of Medicine. From 2017-2023 Mesa was the Executive Director of the NCI Designated Mays Cancer Center at UT Health San Antonio MD Anderson where he developed and grew the cancer service line, co-led the development and construction of a new cancer focused hospital, grew cancer faculty, peer reviewed funded research and successfully renewed the NCI designation in 2020. Mesa practiced hematology at Mayo Clinic (MN 2002-2009, AZ 2009-2017)) and was Chair of Hematology & Medical Oncology and Deputy Director of the Mayo Clinic Comprehensive Cancer Center.

Mesa is an international expert in hematologic cancers, who has dedicated his life's work to research and drug development for myeloproliferative neoplasms and led/co-led the development of six drugs that have been FDA approved for myeloproliferative neoplasms and was the inaugural panel chair of the NCCN guideline panel for the first USA guidelines for MPNS. Mesa is passionate about advancing cancer health equity and increasing minority patients' participation in cancer clinical trials, implement a mandate that each new trial at the Mays Cancer Center had a minority accrual plan, and in March 2022, he testified on the importance of clinical trial diversity before the House Committee on Energy and Commerce. Mesa also co-led the Mays Cancer Center's biennial Advancing the Science of Cancer in Latinos Conference held in San Antonio.

#### NAVEEN PEMMARAJU, MD

Professor of Medicine

Director, Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN) Program Executive Director, Cancer Medicine for the MDACC Cancer Network The University of Texas MD Anderson Cancer Center



Naveen Permaraju, MD is Professor in the Department of Leukemia at MD Anderson Cancer Center (MDACC) in Houston, Texas. He is the inaugural Director of the Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN) Program

at MDACC and additionally serves as Executive Director for Cancer Medicine for the MDACC Cancer Network. Dr Pemmaraju is a world-leading clinical researcher in the area of rare myeloid malignancies including BPDCN, MPN, and AML and has served as lead national/international PI on numerous key clinical trials in the field. Dr Pemmaraju's leadership led to the approval of the first targeted therapy specifically approved in BPDCN in December 2018 (CD123-targeted therapy, tagraxofusp, Pemmaraju et al NEJM April 2019). He has authored/co-authored over 370+ papers in the medical literature and is currently the lead PI for 2 separate DOD grants. In addition, Dr Pemmaraju is a well-regarded clinician and medical educator, having been awarded the Gerald P. Bodey Excellence in Teaching Award at MDACC in 2020, the MPN Hero Award in 2022, the Faculty Educator of the Year in 2023 for MDACC and the MDACC Community Outreach Faculty Excellence Award in 2023.



#### RAAJIT RAMPAL, MD

Associate Member Director, MPN and Rare Hematologic Malignancies Program Director, Center for Hematologic Malignancies Memorial Sloan Kettering Cancer Center

Dr. Rampal is an Associate Member at Memorial Sloan-Kettering Cancer. He is Director of the MPN and Rare Hematologic Malignancies Program at MSK and also Director of the Center for Hematologic Malignancies at MSK.

The focus of his laboratory and clinical research has been elucidate mechanism of pathogenesis of the Philadelphia-chromosome negative Myeloproliferative Neoplasms (MPN) and to develop novel mechanism-based therapeutic approaches for MPNs.

#### LINDA RESAR, MD

Professor of Medicine (Hematology), Oncology, & Pathology, Co-Director, Bloodless Medicine & Surgery Program The Johns Hopkins University School of Medicine & the Johns Hopkins Hospital

Dr. Resar is a pediatric hematologist-oncologist and physician-scientist at the Johns Hopkins University School of Medicine. Her research program is directed at understanding how myeloproliferative neoplasms (MPN) develop

and why some patients progress to more advanced disease. She is particularly interested in preventing progression and optimizing therapy for children and young adults with MPN. Her team discovered that a protein, called HMGA1, acts as a molecular "key" that "unlocks" regions of the genome to drive MPN progression to myelofibrosis and leukemia. Dr. Resar and her team are now focusing on pathways triggered by HMGA1 to develop effective therapies to prevent progression.



#### ELLEN K. RITCHIE, MD

Associate Professor of Clinical Medicine Associate Attending Physician Member, Leukemia Program Weill Cornell Medical College/New York Presbyterian Hospital

Ellen K. Ritchie, MD is Associate Professor of Clinical Medicine, Associate Attending Physician, and a member of the Leukemia Program at the Weill Cornell Medical College of Cornell University and the New York

Presbyterian Hospital in New York City.

Dr. Ritchie graduated from Barnard College at Columbia University and received her medical degree from the College of Physicians and Surgeons at Columbia University in New York City. She was elected to the Alpha Omega Alpha Honor Society. She completed her internship and residency in internal medicine at New York Presbyterian Hospital, Columbia campus. Dr. Ritchie completed her fellowship in hematology and medical oncology at the New York Presbyterian Hospital, Columbia campus. Dr. Ritchie's research interests are in the treatment of older patients with anemia, cytopenias, myelodysplastic syndromes, myeloproliferative disorders and acute leukemia. She is interested in finding better therapies and supportive care strategies for older patients. Dr. Ritchie is the principal investigator on clinical trials investigating new diagnostic techniques, supportive care strategies and therapeutics aimed at the older patient. She collaborates with investigators in the Division of Geriatrics and Gerontology. She has been the author or co-author of many publications. She is interested in improving the medical care of older patients with hematologic malignancies.

#### ANDREW I. SCHAFER, MD

Professor of Medicine Director of the Richard T. Silver for Myeloproliferative Neoplasm Center Emeritus Physician-in-Chief Weill Cornell Medical College/New York Presbyterian Hospital



Dr. Andrew Schafer is Professor of Medicine at Weill Cornell, Director of the Silver MPN Center, and Emeritus Chair of Medicine and Emeritus Physicianin-Chief of Weill Cornell Medical College and the New York-Presbyterian

Hospital/Weill Cornell. Since his Hematology fellowship training at Harvard, he has continuously devoted much of his career to patient care and NIH-funded laboratory research in the MPNs. He previously served as Scientific Director of the MPN Research Foundation, and was most recently elected to be the first physician member of the Foundation's Board of Directors.

Trained at the University of Pennsylvania, University of Chicago, and Harvard (Brigham), he has made substantive contributions to our understanding of blood platelet biology, including in the MPNs, interactions between platelets with vascular endothelial and smooth muscle cells, and platelet activation under conditions of hemodynamic forces as well as in vivo. He has served as Chief of Medicine and Chair of Medicine at Baylor College of Medicine, Penn, and Weill Cornell. He was elected President of the American Society of Hematology, the Association of Professors of Medicine, and to membership in the National Academy of Medicine, and Master of the American College of Medicine. He has been Co-Editor of the last three editions of the Cecil Textbook of Medicine.



#### RICHARD T. SILVER, MD

Professor Emeritus of Medicine Director Emeritus of the Richard T Silver Center for Myeloproliferative Neoplasms *Weill Cornell Medicine* 

Dr. Silver is Professor Emeritus of Medicine and Emeritus Director, Richard T Silver Myeloproliferative Neoplasms Center, at Weill Cornell Medicine. He received undergraduate and medical degrees from Cornell University

and completed an internship and residency (hematology/oncology) at New York Hospital-Cornell Medical Center. He was a Clinical Associate at the National Cancer Institute, NIH. As a Visiting Fulbright Professor at the University of Bahia, Salvador, Brazil, Dr. Silver helped establish a residency program, supported by the Rockefeller Foundation. His research included the study of blood groups of indigenous people of the Upper Xingu River region, which led to his discovery of the third allele in the Kidd blood subgroup, among other findings.

Dr. Silver is an internationally recognized pioneer in clinical cancer chemotherapy and clinical and translational investigations in the myeloproliferative neoplasms (MPNs) and chronic myeloid leukemia. He is particularly known for the broad clinical application of the interferons for treating these diseases and is widely credited with developing and popularizing the use of the bone marrow biopsy technique used worldwide for diagnosing hematologic disorders.

He is a prolific author and lecturer at many national and international meetings and has held many distinguished visiting professorships. He has co-chaired 15 international congresses on MPNs. He holds several prestigious awards and honors from institutes worldwide. The Richard T Silver Distinguished Professor of Hematology and Oncology and the Richard T Silver MPN Center at Weill Cornell Medicine have been named in recognition of his contributions to the understanding and treatment of MPNs.

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