



National Patient & Family Conference

July 17 - 19, 2020

*The Power of Patients
Driving New and Better Treatments*

Conference Agenda

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FRIDAY, JULY 17, 2020 AGENDA & SESSION DESCRIPTIONS

- 12:00pm **Welcome & Opening Remarks**
- 🔴 Kevin Lyons-Tarr, AAMDSIF Board Chair
 - 🔴 Connie Britton, Actress
- 12:15pm **Concurrent Sessions**
- 🔴 **Blood Transfusions, Iron Overload and Treatment Options**
Andrew Brunner, MD will discuss the most common treatment option for bone marrow failure patients – blood transfusions. Long-term blood transfusion patients have a lot of information that they need to know, and this is a great session to ask those questions you might have from both Dr. Brunner and other patients like you. This session will include images of the blood transfusion process which might not be suitable for all participants.
 - 🔴 **Should I Enroll in a Clinical Trial? Stories from Participants**
Olga Rios, RN, BSN coordinates clinical trials for the Bone Marrow Failure Team at the National Heart, Lung and Blood Institute (NHLBI) at the National Institutes of Health and will lead a discussion about how patients can select which clinical trial is right for them, what is the role of patients in finding trials and what it's really like to be in one. Theresa Elgin, a bone marrow failure disease patient, and clinical trial participant will share her experience as well.
 - 🔴 **Pediatric Bone Marrow Failure Diseases: A Special Session for Kids (and their Families)**
David Margolis, MD will lead an informal session for pediatric patients and their family members, covering many of the issues that matter most to kids living with bone marrow failure disease. Dr. Dave will present information in language appropriate for grade school and older children.
- 1:15pm **Visit the Exhibit Hall and Lounge**
- 1:30pm **Concurrent Sessions**
- 🔴 **Managing Fatigue and Other Side Effects**
Christina Cline, RN, BSN, CCRC will lead this important session on managing bone marrow failure diseases. Fatigue is a symptom across all bone marrow failure diseases and our patients report that it continues even post-treatment. This session will offer a practical approach to managing fatigue before, during and after treatment and is suitable for patients and caregivers.

🔥 **Role of Complementary and Alternative Medicine in Bone Marrow Failure Disease Treatment**

Mikhail Kogan, MD will present an informative session on complementary and alternative medicine. These treatments have been used for centuries and can include acupuncture, dietary supplements, massage therapy, hypnosis, and meditation. In this session, you will learn about what aspects of these practices can benefit bone marrow failure disease patients and you can ask questions about your own health situation. As always, please consult your own health care provider before undertaking any complementary or alternative medicine practice.

🔥 **Social Media Fun (in the Lounge)**

Join AAMDSIF staff, members of the Patient Education Council and some friends of the Foundation in the Lounge for some fun with social media. This kid-focused gathering will be brief so bring your smart phone and your smarts!

2:30pm **Visit the Exhibit Hall and Lounge**

2:45pm **Closing Plenary Session**
“When Blood Breaks Down”
Mikkael Sekeres, MD
Taussig Cancer Center, Cleveland Clinic

4:00pm **Patient Support Group - Aplastic Anemia**
Please use the link provided on the agenda in the Theater or in the email sent to you to join this session.

SATURDAY, JULY 18, 2020 AGENDA & SESSION DESCRIPTIONS

- 12:00pm **Opening Keynote: National Advocacy to Find a Cure**
🔴 Debby Cook, AAMDSIF Board Member and aplastic anemia patient
🔴 Congresswoman Doris Matsui
- 12:15pm **A Survivor's Journey: A Conversation with Robin Roberts and Gail Roboz, MD**
- 12:45pm **Concurrent Sessions**
- 🔴 **Aplastic Anemia: A Patient-Centered Approach to Diagnosis and Treatment**
Phil Scheinberg, M.D. will provide an accessible overview of aplastic anemia diagnosis and treatment including the latest developments and thinking including potential new drugs and protocols.
 - 🔴 **PNH: A Patient-Centered Approach to Diagnosis and Treatment**
Jamile Shammo, MD, will provide an overview of the diagnosis and treatment options for PNH patients including a review of what is in the development pipeline.
 - 🔴 **Living with Lower Risk MDS: What Are Your Options?**
Joshua Zeidner, MD, will discuss the most recent treatment options for lower risk MDS patients as well as what is in the development pipeline. Dr. Zeidner will also discuss the changing perspective on transplants for older patients.
- 1:45pm **Visit the Exhibit Hall and Lounge**
- 2:00pm **Keynote Speaker: Chasing My Cure**
David Fajgenbaum, MD, MBA, MSc, FCPP
Castleman Disease Collaborative Network
- 2:45pm **Concurrent Sessions**
- 🔴 **Aplastic Anemia and Overlapping Diseases: Understanding the Risks and Treatment Options**
Bart Scott, MD, will discuss the increased risk that aplastic anemia patients have for developing other bone marrow failure diseases including MDS and PNH and will cover the potential treatment options for these overlapping conditions.
 - 🔴 **The Changing Landscape for PNH Patients: Selecting the Right Treatment Option**
Jorge Leguizamo, MD, will focus on helping PNH patients understand the newest treatment options (including those in clinical trial) and how to make an informed and empowered choice.

- **Moderate and High Risk MDS: A Patient Centered Approach to Diagnosis and Treatment**
David Sallman, MD, will provide a thorough review of the diagnostic process used to determine the risk levels for MDS patients, explain the various mutations in MDS and how MDS can transition to AML.

3:45pm Visit Exhibit Hall and Lounge

4:00pm **Patient Support Group – MDS**
Please use the link provided on the agenda in the Theater or in the email sent to you to join this session.

SUNDAY, JULY 19, 2020 AGENDA & SESSION DESCRIPTIONS

- 12:00pm **Concurrent Sessions**
- 🔴 **Long Term Survivors and Bone Marrow Transplants: The Risks, the Rewards and What You Should Know**
Jim Gajewski, MD and John DiPersio, MD will provide a “big picture” view of the important things patients who are considering or who have already had a transplant should know. Frank Schiro and Kyle Rocheleau are long-term transplant patients who will share their experiences and answer questions from the audience.
 - 🔴 **Financing Your Rare Disease: Navigating Insurance and Patient Assistance Programs**
Melissa Dornan (CCCS Maryland), Amy Niles (PAN Foundation) and Leigh Clark (AAMDSIF) will help patients and families understand the financial implications of being diagnosed with bone marrow failure disease, what programs are available to support patients, how to navigate insurance and assistance programs and how to protect your family’s assets.
- 1:00pm **Visit Exhibit Hall and Lounge**
- 1:15pm **Concurrent Sessions**
- 🔴 **Caregivers: Taking Care of Yourself While Caring for Others**
Isabel Schuermeyer, MD, will lead this session on the unsung heroes of the bone marrow failure world, sharing important information on preventing caregiver burnout, self-care techniques for caregivers and much more.
 - 🔴 **How Long Do I Have to Live? Prognosis for Bone Marrow Failure Patients**
Sandrine Niyongere, MD will present on the most frequently asked question asked by bone marrow failure patients and their families. The variables are complex, and this session will delve into how hematologists and oncologists answer this question.
- 2:15pm **Visit Exhibit Hall and Lounge**
- 2:30pm **AAMDSIF Leadership Awards**
- 3:30pm **Patient Support Group - PNH**
Please use the link provided on the agenda in the Theater or in the email sent to you to join this session.

SPEAKER BIOGRAPHIES

Andrew Brunner, MD is an Associate Professor of Medicine at Harvard Medical School and Assistant in Medicine, Center for Leukemia, at Massachusetts General Hospital. He earned his medical degree at Brown University School of Medicine. Dr. Brunner's clinical area of focus and research projects are focused toward understanding treatment patterns and patient outcomes in MDS and other advanced hematologic malignancies, and to develop new treatments for these patients. He is involved in clinical trials for all stages of MDS with an overall goal of tailoring treatment to improve the outcomes of these patients.

Leigh Clark is the Patient Educator with the Aplastic Anemia and MDS International Foundation. For the last 13 years, she has provided direct support to thousands of patients and their families. Leigh is a proud soccer parent and lives with her family in rural Maryland.

Christina Cline, RN, BSN, CCRC is the Manager of Clinical Trials at University of Florida Health Cancer Center's Clinical Trials Office for Malignant Hematology. She earned an Associate of Arts and Sciences in Registered Nursing from Santa Fe College and earned a Bachelor of Science in Registered Nursing at University of Central Florida. Along with her registered nursing degrees, Christina is also a Certified Clinical Research Coordinator (CCRC).

John DiPersio, MD, PhD is the Chief of the Division of Oncology, Deputy Director of the Siteman Cancer Center at Washington University School of Medicine in St. Louis, and the Virginia E. and Samuel J. Golman Professor of Medicine. Dr. DiPersio's clinical research focuses on fundamental and translational aspects of leukemia and stem cell biology. These studies include identification of genetic abnormalities in human leukemias, understanding processes involving stem cell and leukemia cell trafficking, and clinical and translational programs in both leukemia/myelodysplastic syndrome and transplantation immunology. Dr. DiPersio earned an M.D. and a Ph.D. from the University of Rochester and a B.A. in Biology (Magna Cum Laude) from Williams College. He completed his subspecialty training in Hematology-Oncology and his post-doctoral work at UCLA.

Melissa Dornan, MBA, is the Outreach Coordinator with Consumer Credit Counseling Services of MD and DE, a nonprofit organization providing financial counseling and education where she provided financial education and counseling. She is National Board Member and past Chapter President of the Society of Financial Service Professionals, a Premier Graduate of Leadership Howard County, and past Advisory Board Chair of Urban Ed. She received her Master of Business Administration from The George Washington School of Business.

David Fajgenbaum, MD, MBA, MSc, FCPP, is the co-founder and Executive Director of the Castleman Disease Collaborative Network (CDCN), Assistant Professor of Medicine in Translational Medicine & Human Genetics at the University of Pennsylvania, Associate Director, Patient Impact for the Penn Orphan Disease Center, and author of the national bestselling book,

Chasing My Cure: A Doctor's Race to Turn Hope Into Action. Dr. Fajgenbaum is also a patient battling idiopathic multicentric Castleman disease (iMCD). He became ill during his third year of medical school in 2010, had his last rites read, and had four deadly iMCD relapses. In 2012, Dr. Fajgenbaum co-founded the CDCN, through which he has spearheaded the "Collaborative Network Approach." He currently leads 18 translational research studies, including an international natural history study, the first-ever NIH R01 grant studying iMCD, and a clinical trial of sirolimus in iMCD.

James Gajewski, MD received his A.B. degree in Government and International Studies from the University of Notre Dame, and his M.D. degree from Temple University School of Medicine. He subsequently completed his residency training in Internal Medicine at the University of Cincinnati Hospital and fellowship in Hematology and Oncology at UCLA Hospital and Clinics. His early medical career at UCLA focused on scientific and clinical research in unrelated donor hematopoietic stem cell transplantation. Many of the unrelated marrow donor transplantations he performed in 1988 and 1989 became the world's longest survivors. Dr. Gajewski is one of the pioneers in graft-versus-host disease (GvHD), the need for better immunosuppression after unrelated donor transplant, as well as the need for use of molecular HLA typing to select for appropriate unrelated marrow donors. He further ventured into haploidentical transplantation and contributed to important clinical data with the Center for International Blood and Marrow Transplant Research (CIBMTR) on transplant outcomes. Dr. Gajewski was founding committee member of both the Foundation for Accreditation of Cellular Therapy (FACT) and National Comprehensive Cancer Center Network's leukemia and lymphoma clinical guidelines committee. He also served as the American Society for Bone Marrow Transplantation expert liaison to the FDA cellular therapeutics advisory committee. Dr. Gajewski serves on the Board of Directors with the Aplastic Anemia and MDS International Foundation.

Mikhail Kogan, MD, ABIOM, RCST is the Medical Director of GW Center for Integrative Medicine and in addition to multiple research and teaching responsibilities, Dr. Kogan serves as an associate director of the Geriatrics Fellowship Program and provides geriatric consults at George Washington University Hospital. Dr. Kogan also serves as associate director of newly formed GW Interdisciplinary Integrative Medicine Fellowship. Dr. Kogan obtained his medical degree from the Drexel University College of Medicine in Philadelphia, Pennsylvania. He completed his residency in Internal Medicine at Montefiore Medical Center in New York City and a fellowship in Geriatric Medicine at The George Washington University Medical Center.

Jorge Leguizamo, MD with Georgia Cancer Specialists is board certified medical oncology and hematology. He received his medical degree from Columbia University. Dr. Leguizamo is a member of the American Society of Hematology, the Georgia Society of Clinical Oncology, the American Society of Clinical Oncology, and the International PNH interest group. He is also a Fellow of the American College of Physicians. He has special interest in Hematological Malignancies, Breast Cancer, PNH And Atypical HUS. Dr. Leguizamo serves on the medical advisory board of the International PNH Interest Group.

David Margolis, MD is currently the Interim Chair of the Department of Pediatrics at MCW/Children's Wisconsin. He received his medical degree from the University of Wisconsin-Madison in 1989. His clinical and research interests include taking care of children and young adults with aplastic anemia as well as alternative donor blood and bone marrow transplants for children and young adults with aplastic anemia, sickle cell anemia and leukemia. Dr. Margolis is a member of the Aplastic Anemia and MDS International Foundation medical advisory board.

Doris Matsui is a member of the United States House of Representatives representing the city of Sacramento and its surrounding areas since 2005. Congresswoman Matsui's late husband, Congressman Bob Matsui, was an MDS patient.

Amy Niles, MBA, serves as Vice President, External Affairs at the Patient Access Network (PAN) Foundation, a leading national independent organization providing copay financial assistance to people who are underinsured. In this capacity, she oversees alliance development with patient and professional groups and the Foundation's advocacy and public policy initiatives. Before joining the PAN Foundation, Ms. Niles served for eight years as Chair, Medical Relations and Advocacy for the Together Rx Access program, a prescription savings program for the uninsured. Prior to that, she was President and CEO of the National Women's Health Resource Center, now known as Healthy Women, for more than a decade. She began her career in hospital administration. Amy has a Master of Business Administration degree from Baruch College, City University of New York, and an undergraduate degree in Biology from the University of Rochester.

Sandrine Niyongere, MD is an Assistant Professor of Medicine with the University of Maryland School of Medicine and Marlene and Stewart Greenebaum Comprehensive Cancer Center. She received her medical degree from University of Alabama School of Medicine. Her clinical focus is in acute leukemias, myelodysplastic syndromes, myeloproliferative neoplasms, and bone marrow failure syndromes. Dr. Niyongere's primary research interests are in finding new therapeutic targets by understanding oncogenic signaling pathways involved in AML and MDS as well as studying the role of the tumor microenvironment in the development of chemoresistance in leukemia. Dr. Niyongere also serves as an attending physician on the inpatient leukemia service training fellows, internal medicine residents and medical students.

Olga Rios, RN, BSN is a Research Nurse Specialist with the Bone Marrow Failure Team at the National Heart, Lung and Blood Institute (NHLBI). Olga received her Registered Nursing degree from the University of Puerto Rico in 1981. She had been a member of the staff at the National Institute of Health since 1986. Olga has participated in numerous medical and scientific articles published by the NHLBI team.

Kyle Rocheleau is a 30+ year allogeneic unrelated donor transplant survivor who lives in Utah.

David Sallman, MD is an Assistant Member in the Department of Malignant Hematology at Moffitt Cancer Center. Dr. Sallman's clinical interests are myelodysplastic syndromes (MDS), acute

myeloid leukemia (AML) and myeloproliferative neoplasms (MPN). Dr. Sallman earned his MD degree from the University of South Florida College of Medicine. He completed an Internal Medicine Residency at Massachusetts General Hospital and, most recently, a Hematology/Oncology Fellowship at Moffitt Cancer Center. His research interests focus on the development of novel, targeted therapeutic strategies (Phase I and II clinical trials) for patients with myelodysplastic syndromes (MDS) and acute myeloid leukemia (AML), based on the underlying mutational drivers of each disease.

Phillip Scheinberg, MD is Chief of Clinical Hematology at the Hospital Sao Jose, Beneficencia Portuguesa de Sao Paulo in Brazil. He received his medical degree in 1995 at the University of Santo Amaro in Sao Paulo, Brazil and completed his residency (1997-2001) at Mount Sinai Medical Center in Miami Beach, FL where he became Medical Chief Resident. Dr. Scheinberg held a Hematology/Oncology Fellowship (2001-2005) before becoming a staff clinician at the Hematology Branch of the National Heart, Lung, and Blood Institute (NHLBI) at the National Institutes of Health (NIH) in Bethesda, MD. During his time at NHLBI, he was involved in clinical protocols in bone marrow failure syndromes such as aplastic anemia, myelodysplastic syndromes, and large granular lymphocytic leukemia. The focus of these protocols was to investigate novel immunosuppressive regimens that will improve the response rate in untreated severe aplastic anemia (SAA) and prevent relapses and clonal evolution to myelodysplastic syndromes. Dr. Scheinberg is a member of the Aplastic Anemia and MDS International Foundation medical advisory board.

Frank Schiro is a 70-year-old retired teacher and football coach from Central California. He underwent an allogeneic BMT for CML in 1991 and received a Donor Leukocyte Infusion (DLI) in 2011 when he came out of remission 20 years after his BMT in 1991. He is now in remission again. He and his wife, Kathy, have a blended family of 6 children and 30 grandchildren and great grandchildren, and live in Visalia, California.

Bart L. Scott, MD is the Clinical Director, MDS/MPN and Myeloid Malignancies at Seattle Cancer Care Alliance, Associate Professor, Division of Medical Oncology at University of Washington School of Medicine and Associate Professor, Clinical Research Division at Fred Hutchinson Cancer Research Center. He earned a Bachelor of Science in Biology and Chemistry from University of Alabama at Birmingham and his medical degree from University of South Alabama. Dr. Bart Scott specializes in treating patients with myelodysplastic syndromes, or MDS, and myeloproliferative neoplasms, or MPN. Dr. Scott studies ways to improve treatment strategies for these patients, new combinations of conditioning regimens prior to blood stem cell transplant.

Mikael A. Sekeres, MD, MS has been Medical Advisory Board Co-Chair since 2004. As a professor of medicine and director of the leukemia program, Dr. Sekeres is also Vice Chair for Clinical Research at the Cleveland Clinic Taussig Cancer Institute in Ohio. He earned his medical degree and a master's degree in clinical epidemiology from the University of Pennsylvania School of Medicine. Dr. Sekeres completed his postgraduate training at Harvard University, finishing an

internal medicine residency at Massachusetts General Hospital and a fellowship in hematology-oncology at the Dana-Farber Cancer Institute in Boston. He chaired the Oncologic Drugs Advisory Committee of the FDA.

Jamile Shammo, MD, FACP, FASCP is an associate professor of medicine and pathology, Section of Hematology and Stem Cell Transplantation, Division of Hematology/Oncology, at Rush University Medical Center in Chicago where she spearheads the MDS/MPN/Bone marrow failure program. She is also director of the Hematology/Oncology Fellowship Program and CME Course Director in the Division of Hematology/Oncology. After earning a medical degree with honors from Aleppo Medical School in Syria, Dr. Shammo completed internships and residencies in the departments of pathology and internal medicine at McGaw Medical Center of Northwestern University, in Evanston, and a 3-year fellowship in the Division of Hematology/Oncology at University of Chicago. She is recognized nationally for her expertise in bone marrow failure syndromes/PNH and was chosen to serve in the role of a national coordinator for the U.S. PNH registry.

Isabel Schuermeyer, MD is a psychiatrist who is board certified in Consultation-Liaison psychiatry (previously psychosomatic medicine) and has worked at the Cleveland Clinic since completing fellowship in 2005. She graduated from the University of Cincinnati College of Medicine in 2000. While in fellowship she started working in the field of Psychosocial Oncology and helped develop the Interdisciplinary Psychosocial Oncology team at the Taussig Cancer Center. Dr. Schuermeyer is a clinician who treats patients with cancer along with either new onset psychiatric symptoms or exacerbation of an underlying psychiatric illness. Her focus has been on a team-based approach, including the oncology team, given the risk of interactions between psychiatric medicines and cancer treatments. Her research interests are end of life decisions, determining capacity and working within an interdisciplinary team. She is a past-president of American Psychosocial Oncology Society and has been involved with the American Psychiatric Association. Dr. Schuermeyer serves as the chair of the Patient Education Council with the Aplastic Anemia and MDS International Foundation.

Joshua F Zeidner, MD is Assistant Professor of Medicine in the Division of Hematology/Oncology at The University of North Carolina at Chapel Hill School of Medicine. He earned a Bachelor of Arts in Biology and Psychology from Boston University and earned his medical degree from New York Medical College. Dr Zeidner's research interests include discovering innovative methods to improve outcomes, drug discovery and development, and designing clinical trials in acute myeloid leukemia (AML), myelodysplastic syndromes (MDS), and myeloproliferative neoplasms (MPN). He leads the Leukemia Clinical Trials Research Protocol Office Disease (POD) Group, overseeing a multitude of cutting-edge clinical trials for acute leukemia, MDS, and MPN. He serves as the Principal Investigator of various industry-sponsored, investigator-initiated, and academic collaborative clinical trials evaluating novel agents in MDS and AML.

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Children’s programming is supported by a generous donation from Westside Donut Ventures in memory of Drew Stadlen.

Conference music is provided by MDS patient, George Winston, “in memory of my dear friend David Fleck, an incredible person and music lover who passed on at a young age of cancer in 1982”.

PLANNING COMMITTEE

The 2020 AAMDSIF Virtual National Patient & Family Conference would not be possible without the tireless support of our Board of Directors, our Medical Advisory Board, and our Patient Education Council.

AAMDSIF Patient Education Council

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Thank you for joining us and for making AAMDSIF your resource of choice!

