

# *The National Academies of* **SCIENCES • ENGINEERING • MEDICINE**

The State of the Science of Hematopoietic Stem Cell Treatment and Disability:  
A Workshop

## **Speaker and Moderator Biosketches**

**Frederick Appelbaum, M.D.** is Executive Senior Vice President & Deputy Director, Fred Hutchinson Cancer Research Center and Professor, Division of Medical Oncology, University of Washington in Seattle, Washington. Dr. Appelbaum studies the biology and treatment of leukemias, lymphomas and other blood cancers and sees patients with these disorders. He has been involved in laboratory and clinical research involving hematological malignancies for over four decades. He described the first use of autologous transplantation as curative therapy for malignant lymphoma, a therapy now used to treat tens of thousands of patients annually and helped develop the first antibody-drug conjugate ever approved by the FDA. Dr. Appelbaum has received continuous peer-reviewed support from the NIH for 40 years and is the author or co-author of more than 800 peer-reviewed articles. In addition to caring for patients and conducting clinical trials, as the executive vice president and deputy director of Fred Hutchinson Cancer Research Center, he leads strategic research partnerships with the National Institutes of Health and other federal agencies and helps to ensure that Fred Hutch advances are shared with the wider community. Dr. Appelbaum has made numerous landmark contributions to his field. Notably, he helped develop Fred Hutch's renowned program in blood stem cell transplantation. He also participated in the Hutch's pioneering work to develop targeted antibody-based cancer therapies. Today, Dr. Appelbaum leads Fred Hutch's Adult Leukemia Research Center and the Hematologic Malignancy Program of the Fred Hutchinson/University of Washington Cancer Consortium. These efforts are aimed at enhancing the understanding of these diseases and improving treatments like blood stem cell transplantation. In 2013, Dr. Appelbaum was elected as member of the National Academy of Medicine. In 2019, he was one of 22 fellows elected to the American Association for Cancer Research (AACR) Academy.

**Staci D. Arnold, M.D., M.B.A., M.P.H.** is Associate Professor of Pediatrics at Emory University School of Medicine. She is also Program Director of General Hematology, and Director of the Comprehensive Bone Marrow Failure Clinic, Aflac Cancer & Blood Disorders Center at Children's Healthcare of Atlanta. She completed both her MD and Pediatric residency at Duke University. She specialized in pediatric hematology after completing a fellowship and a Master of Public Health at Columbia University. She has particular expertise in the care of children undergoing treatments including stem cell transplantation for noncancerous disorders such as sickle cell anemia, aplastic anemia, and bone marrow failure syndromes. In addition to caring for patients, Dr. Arnold conducts clinical research. She uses her training in business and medicine to conduct outcomes research to investigate patient reported outcomes and the cost-benefit of novel and curative pediatric therapies.

**Margaret Bevans, Ph.D., R.N., F.A.A.N.** is the Director at the Office of Research Nursing, NHLBI Office of the Clinical Director and CAPT, United States Public Health Service. As the Director for the Office of Research Nursing, Dr. Margaret Bevans leads a team of research nurses and patient care coordinators who are committed to quality research and compassionate care through excellence in leadership, interdisciplinary collaboration, customer service, and clinical expertise. She provides organizational leadership for the NHLBI Office of the Clinical Director and advises on strategic initiatives and process improvement. Dr. Bevans is a well-established clinician, investigator, and leader in the field. Previously, Dr. Bevans was the Program Director and a Clinical Nurse Scientist in the NIH Clinical Center studying the outcomes of cancer patients, specifically hematopoietic stem cell transplantation recipients and their caregivers. She is a member of the Oncology Nursing Society, the American Psychosocial Oncology Society and served as a co-chair for the NIH Hematopoietic Stem Cell Transplant (HSCT) Late Effects Initiative Patient-Centered Outcomes Working Group. In addition, she served as an advisor for the National Marrow Donor Program (Be the Match®) for a PCORI Patient and Caregiver Engagement Grant and Housing and Caregiver Needs study. Her expertise includes patient-reported outcome methodology, including health-related quality of life and symptom assessment. She has served as Principal and Associate Investigator for multiple NIH protocols and was inducted as a fellow into the American Academy of Nursing in 2014. Dr. Bevans received her bachelor's degree from Johns Hopkins University, and her master's and doctorate degrees from the University of Maryland. She also holds assistant/adjunct faculty positions at the Uniformed Services University of the Health Sciences Graduate School of Nursing and the University of Maryland School of Nursing.

**Stella Davies, MBBS, Ph.D., MRCP** is a professor of pediatrics, holder of the Jacob G Schmidlapp endowed chair in pediatrics and director of the Division of Bone Marrow Transplantation at Cincinnati Children's Hospital. Dr. Davies is current president of the American Society of Transplantation and Cellular Therapy and is the PI of 2 RO1 grants focused on complications of transplantation

**Steven Devine M.D.** is Chief Medical Officer at the National Marrow Donor Program (NMDP)/Be The Match and Associate Scientific Director at the Center for International Blood and Marrow Transplant Research (CIBMTR). Prior to joining the NMDP, he was the Director of the BMT Program at The Ohio State University Comprehensive Cancer Center. He served for eight years as Chair of the National Cancer Institute funded Alliance Transplant Committee as well as a 2-year term as Chair of the NIH-funded Blood and Marrow Transplant Clinical Trials Network (BMT CTN) Steering Committee. He is currently one of the three Co-PIs for the BMT CTN Data Coordinating Center. He has a major research interest in the application of stem cell transplantation for patients with acute leukemia and myelodysplastic syndrome. He has authored more than 200 peer-reviewed papers and more than 400 abstracts as well as several reviews and book chapters in the field of stem cell transplantation, leukemia, and hematology. He is an Associate Editor for the ASTCT journal Transplantation and Cellular Therapy and a member of the Journal of Clinical Oncology editorial board.

**Areej El-Jawahri, M.D.** serves as the Director of the Blood and Marrow Transplant Survivorship Program, the Associate Director of the Cancer Outcomes Research and Education Program, and the Director of Digital Health at Massachusetts General Hospital. Dr. El-Jawahri is an oncologist specializing in the care of patients with hematologic malignancies and those undergoing hematopoietic stem cell transplantation and adoptive cellular therapy. Her goal is to improve the quality of life and care for patients with hematologic malignancies and their families. Her research interests include investigating patient-reported outcomes, developing interventions to enhance patient-centered decision-making, and designing supportive, palliative, and digital health interventions to improve the care of patients with hematologic malignancies and their families. Dr. El-Jawahri graduated from Harvard Medical School and completed her residency training at Massachusetts General Hospital. She subsequently completed her hematology-oncology fellowship at the Dana Farber Harvard Cancer Center Fellowship Program.

**Betty K. Hamilton, M.D.** is the Interim Director of the Blood and Marrow Transplant Program at the Cleveland Clinic Taussig Cancer Institute and Associate Professor of the Cleveland Clinic Lerner College of Medicine of Case Western Reserve University. Dr. Hamilton's clinical and research interests focus on the treatment of hematologic malignancies with allogeneic hematopoietic cell transplantation; and improving outcomes

post-transplant, specifically in graft-versus-host disease (GVHD), survivorship, and late effects. Her research interests are in novel ways to prevent and treat GVHD, and patient-centered outcomes in GVHD and associated late effects. Dr. Hamilton established and leads the Victor Fazio MD Cancer Survivorship Clinic for transplant recipients at the Taussig Cancer Institute. Dr. Hamilton is also currently the Director for Community/Clinical Practice of the American Society of Transplant and Cellular Therapy (ASTCT). She serves as co-chair of the Late Effects and Quality of Life Working Committee of the Center for International Blood and Marrow Transplant Research (CIBMTR). She recently chaired the Late Effects, Quality of Life and Economics Committee for the Blood and Marrow Transplant Clinical Trials Network (BMT CTN) State of the Science Symposium. Dr. Hamilton is an active investigator with the BMT CTN and site principal investigator on many GVHD prevention and therapeutic clinical trials, including active involvement in the National Institutes of Health (NIH) Chronic GVHD Consortium and participation in the 2020 NIH Chronic GVHD Consensus project.

**Mary M. Horowitz, M.D., M.S.** is the Robert A. Uihlein Professor of Hematologic Research and Deputy Cancer Director at the Medical College of Wisconsin in Milwaukee. Dr. Horowitz has been involved in clinical research in hematopoietic stem cell transplantation (HCT) for more than 35 years. She served as Chief Scientific Director of the Center for International Blood and Marrow Transplant Research (CIBMTR) for 30 years and is now Scientific Director Emeritus. The CIBMTR is a network of more than 350 centers worldwide who conduct clinical and translational studies focused on improving access to and outcomes of blood and marrow transplantation. Its more than 1500 published studies include seminal articles defining optimal strategies for using unrelated donor and cord blood transplants, providing insight into the potent anti-cancer effects of transplantation and describing both malignant and non-malignant late effects. Dr. Horowitz is Principal Investigator for the Coordinating Center of the U.S. Blood and Marrow Clinical Trials Network, a multicenter group funded by the National Institutes of Health (NIH) to test new therapies to improve the safety and effectiveness of transplantation and cellular therapy. Dr. Horowitz has received numerous awards for her work to advance HCT, including the American Society of Blood and Marrow Transplantation's Lifetime Achievement Award in 2014, the 2019 American College of Physicians Harriet P. Dustan award for outstanding science related to medicine and the 2019 Aplastic Anemia and Myelodysplastic Syndrome Foundation's Lifetime Achievement in Science Award. She is also dedicated to fostering the next generation of clinical scientists and received the American Society of Hematology's Mentor Award for Clinical Science in 2010 for her work guiding, supporting, and promoting the training and career development of young investigators. She has served on numerous NIH advisory panels, coauthored more than 400 publications addressing diverse issues in HCT BMT and been continuously funded by the NIH since 1990.

**Margaret Jurocko** has been navigating the healthcare of her immune-comprised sons for over 24 years while working in a variety of different industries ranging from pharmaceuticals, corporate production to managing her own private Pilates business. Her passion and determination to get a diagnosis for her sons led her to the National Institute of Health where in 2015 both young men were diagnosed with XMEN. Unfortunately, people with XMEN have an increased risk of developing Epstein Barr Virus- (EBV) driven lymphoma. Both of her sons contracted EBV within days of one another and, as a result, her youngest needed a bone marrow transplant to re-set and replenish his immune system in 2016. Her oldest has managed to maintain his disease on Intravenous Immune Globulin Therapy ("IVIG"). Five years later, Margaret continues to work faithfully to advocate and volunteer with Be The Match to support other caregivers, raise awareness of patient needs and to enrich her own life by giving others the love and respect that they need to live a purposeful life.

**Stephanie J. Lee, M.D., M.P.H.** is a Member of the Fred Hutchinson Cancer Research Center and Professor at the University of Washington. She holds the David and Patricia Giuliani/Oliver Press Endowed Chair in Cancer Research. She is also the Associate Director of the Clinical Research Division and the Research Director of the Long-Term Follow-Up Program at Fred Hutchinson. Her research focuses on outcomes after hematopoietic cell transplantation, with a particular emphasis on chronic graft-versus-host disease. She is the past 2020 President of the American Society of Hematology.

**Suzanne McCarroll** is a Stanford alum and a long time CBS News journalist. She loved her career choice. When she was diagnosed with lymphoma in 2011, she was able to juggle treatments and career. She went through RCHOP therapy and was in remission for four and a half years. Since then had two rounds of immunotherapy at the Hutch in Seattle and two transplants (one at Dana Farber in Boston, the other in Denver). She has struggled with GVHD in her stomach and mouth. Suzanne is currently being treated at City of Hope in Los Angeles. Three years ago she had to quit her job, as television news reporting is not compatible with a compromised immune system. She often says that managing insurance claims and medicine is a full time job. She is lucky to have wonderful friends and family who have helped and nurtured me through all this. Other than having annoying mouth issues, she feels high energy and well. Suzanne feels very lucky and blessed every day.

**Miguel-Angel Perales, M.D.** is the Chief of the Adult Bone Marrow Transplantation Service at Memorial Sloan Kettering Cancer Center in New York, as well as an Associate Professor of Medicine at Weill Cornell Medical College. He is an internationally recognized leader in the field of hematopoietic stem cell transplantation and cellular therapy, and serves as Chair of the Alliance for Clinical Trials in Oncology Transplantation and Cell Based Therapies Committee, Vice-Chair of the Board of Directors of Be The Match (National Marrow Donor Program - NMDP), and Vice-President of the American Society for Transplantation and Cellular Therapy (ASTCT, former ASBMT). He also holds leadership positions and has been extensively involved in activities of the Center for International Blood and Marrow Transplant Research (CIBMTR) and Blood and Marrow Transplantation Clinical Trials Network (BMT CTN). Dr. Perales has over 300 publications including peer-reviewed articles, position papers, guidelines, book chapters, editorials and invited reviews, and has been an invited speaker at numerous national and international meetings over the past twenty years. Dr. Perales received his MD from the Free University of Brussels, and completed postdoctoral training at the Tufts Medical Center and Memorial Sloan Kettering Cancer Center.

**David L. Porter, M.D.** is the Jodi Fisher-Horowitz Professor of Leukemia Care Excellence at the Perelman School of Medicine and Abramson Cancer Center, and Director of Cell Therapy and Transplant at the University of Pennsylvania. He graduated the University of Rochester and earned a medical degree at Brown University. He completed internship and residency at Boston University Hospital, and fellowship training at Brigham and Women's Hospital and Harvard Medical School. Dr. Porter chairs or serves on numerous local, national and international committees focused on hematologic malignancies, stem cell transplantation and cell therapy. He is the immediate past Chair and current member of the Board of Directors of the National Marrow Donor Program and serves on a number of cell therapy advisory boards, monitoring boards, and steering committees for several organizations including the Foundation for the Accreditation of Cell Therapy, the Association of American Cancer Institutes, the Society for Immunotherapy of Cancer and the Center for International Blood and Marrow Transplant Research. He is the recipient of several prestigious awards from local and national organizations that include recognition for Service, Professionalism and Mentorship. He has an extensive publication record and has been the primary or co-author of over 200 research and review articles. He currently serves as the Deputy Editor of Transplantation and Cell Therapy (formerly Biology of Blood and Marrow Transplantation), and is an Associate Editor for the American Journal of Hematology. He has worked with colleagues at the University of Pennsylvania to pioneer successful development of CAR T cells. Other research highlights include development of novel methods to improve safety and outcomes after allogeneic stem cell transplantation.

**J. Douglas Rizzo, M.D., M.S.** is the Associate Director of Clinical Operations, Senior Scientific Director at the Center of International Bone Marrow Transplant Research (CIBMTR), Professor of Medicine/Hematology and Oncology, and Project Director Stem Cell Therapeutic Outcomes Database. The Associate Director of Clinical Operations (ADCO) for the MCW Cancer Center provides direction for cancer clinical operations while overseeing the multidisciplinary clinics and Cancer Service Line. Dr. Rizzo fosters a climate of multidisciplinary cancer care with a visible emphasis on research-driven patient care. He works closely with Froedtert administrative leadership and Medical College clinical leadership to ensure that MCW clinicians deliver top quality cancer care and create an environment that is structured for and supports clinical research. He assists with implementation of community engagement strategies, and integration of care across all Cancer Network locations. He is responsible for design and implementation of value-based care strategies – including CMS' Oncology Care Model. Dr. Rizzo coordinates with counterparts at Children's Hospital of Wisconsin to share knowledge and apply research-driven cancer care best practices. He is also the Project Director of the Stem Cell Therapeutic Outcomes Database (SCTOD), one component of the CW Bill Young Cell Transplantation Program. As such he has responsibility for all aspects of collection and use of data to fulfill CIBMTR's contractual obligations. Dr. Rizzo has more than 20 years' experience collecting, managing and analyzing HCT data, and has been integrally involved in CIBMTR initiatives studying late effects and quality of life, regimen intensity and toxicity, and health economics and access disparities. He has participated in numerous quality of care initiatives within and beyond the HCT community. He has international recognition and plays an important part of CIBMTR's collaborative international presence. Dr. Rizzo has been a key contributor to both screening and practice guideline efforts for HCT survivors from the CIBMTR.

**Sara Rosenbaum, J.D.** is the Harold and Jane Hirsh Professor of Health Law and Policy and Founding Chair of the Department of Health Policy at the Milken Institute School of Public Health, George Washington University. She also holds professorships in the Trachtenberg School of Public Policy and Public Administration and the Schools of Law and Medicine and Health Sciences. A graduate of Wesleyan University and Boston University Law School, Professor Rosenbaum has devoted her career to issues of health justice for populations who are medically underserved as a result of race, poverty, disability, or cultural exclusion. An honored teacher and scholar, a highly popular speaker, and a widely-read writer on many aspects of health law and policy, Professor Rosenbaum has emphasized public engagement as a core element of her professional life, providing public service to six Presidential Administrations and nineteen Congresses. She is best known for her work on national health reform, Medicaid and private insurance, Medicaid managed care, health care access for medically underserved communities and populations, and civil rights and health care. Professor Rosenbaum's current research

focuses on the transformation of Medicaid and its effects on poor populations and communities. Her research also focuses on national health reform, Medicaid managed care, and the nation's community health centers, the largest primary health care system for medically underserved rural and urban populations. She is best known for her research into the impact of laws affecting health care access and coverage, as well as the potential effects of major shifts in laws affecting low income and medically underserved populations.

**Karen L. Syrjala, Ph.D.** is a Professor and Director of Biobehavioral Sciences, and Co-Director of the Survivorship Program at the Fred Hutchinson Cancer Research Center. At the University Of Washington School Of Medicine, she is a Professor in the Department of Psychiatry and Behavioral Sciences. She is a licensed clinical psychologist who established the Fred Hutch Psychiatry/Psychology Service and co-founded the Fred Hutch Survivorship Program. Among other national responsibilities, she has been on invited national panels to develop cancer-related guidelines, including for hematopoietic cell transplantation (HCT) and general cancer survivorship, pain, anxiety/depression, and other symptom management. Her research is focused on examining long term outcomes and interventions to improve the lives of cancer survivors, with a focus on HCT. In prospective longitudinal studies, she has examined musculoskeletal, sexual, emotional and physical function trajectories, neurocognitive recovery, healthcare adherence and barriers to healthcare access in HCT survivors. Recent multicenter randomized controlled trials are looking at digital strategies for individualizing programs to meet HCT and other cancer survivor health needs, with a focus on cardiometabolic and subsequent malignancy healthcare adherence and management of emotional needs. She also leads mouse to human studies targeting the mechanisms for late complications in cancer survivors. Among journal editorial board invited memberships, she has been Associate Editor for the Journal of Cancer Survivorship. She mentors numerous junior faculty and fellows in cancer research across disciplines, and provides clinical services to oncology patients.

**Alexis A. Thompson, M.D., M.P.H.** is the Hematology Section Head at Ann and Robert H. Lurie Children's Hospital of Chicago in Chicago, Illinois. She holds the A. Watson and Sarah Armour Endowed Chair for Blood Diseases and Cancer at Lurie Children's. She is also a Professor of Pediatrics at the Northwestern University Feinberg School of Medicine. Dr. Thompson is also the Associate Director for Equity and Minority Health at the Robert H. Lurie Cancer Center and Northwestern University Feinberg School of Medicine. She has served on regional and national on advisory committees for governmental agencies as well as non-profit organizations focused on improving healthcare access, increasing workforce diversity and reducing health disparities. Currently Dr. Thompson is President of the American Society of Hematology. She is an



investigator on multi-center trials and as well as her own institutional clinical studies in thalassemia, sickle cell disease, and hemophilia. She has been a leader in multicenter collaborations, such as the NHLBI-funded Thalassemia Clinical Research Network and the Sickle Cell Disease Implementation Consortium. Her clinical interests include hemoglobinopathies (thalassemia and sickle cell disease), bone marrow failure syndromes and stem cell transplantation in pediatric patients.

**John Tisdale, M.D.** is Chief of the Cellular and Molecular Therapeutics Branch, National Heart, Lung, and Blood Institute (NHLBI), National Institutes of Health. Dr. Tisdale received his medical degree from the Medical University of South Carolina in Charleston after obtaining his B.A. in Chemistry from the College of Charleston. He completed an internal medicine and chief residency at Vanderbilt University Medical Center in Nashville and then trained in hematology in the Hematology Branch, NHLBI, where he served as a postdoctoral fellow. He joined the Molecular and Clinical Hematology Branch of NHLBI in 1998. In 2020 the College of Charleston recognized Dr. Tisdale as one of their Top 25 History makers in honor of the schools 250-year anniversary and was Samuel J. Heyman Service to America Medal finalist. He was recently elected to the American Society for Clinical Investigation and is a member of the American Society of Hematology. He serves as an editorial board member of the journals Stem Cells, Experimental Hematology, and Molecular Therapy Methods & Clinical Development. He is a frequent ad hoc reviewer for Blood, New England Journal of Medicine, Human Gene Therapy, and Nature Medicine Experimental Hematology Molecular Therapy to name a few. He has served on the NIDDK/NIAMS Institutional Review Board for over 15 years, is a founding member of the NIH Bone Marrow Transplant Consortium, and is an active member of the NIH Intramural Gene Therapy Task Force. Dr. Tisdale's research and clinical work center on sickle cell disease. His group focuses on developing curative strategies for sickle cell disease through transplantation of allogeneic or genetically modified autologous bone marrow stem cells. He has published over 200 first- and co-author publications.