

# State of the Science Summit

September 12-15, 2021

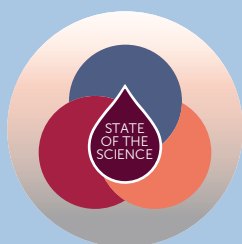


**NATIONAL HEMOPHILIA FOUNDATION**  
*for all bleeding disorders*

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**Research creates discovery,  
discovery creates change  
and change creates impact  
for people with inherited  
bleeding disorders.”**

-Leonard Valentino, MD, NHF President & CEO



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# Welcome

Dear colleagues and friends,

Working together, we have made tremendous progress in the diagnosis, treatment and lifelong management of inherited bleeding disorders. Yet we can all agree that there is much more to be done.

With improved technologies and greater attention to the community, I see tremendous opportunities to foster dramatic changes that can advance care in a more holistic and equitable way and can redefine what it means to live with an inherited bleeding disorder.

But to make this happen, we need to listen, understand and coordinate our efforts around the areas that matter the most for individuals with these disorders. This warrants broad community alignment on defined research priorities driven by the voices of the community – the individuals, families and all of the allied health professionals and partners who are invested in this effort.

This is why the National Hemophilia Foundation (NHF), as the largest national organization dedicated to improving lives for people with inherited bleeding disorders, is championing this community-driven effort to define and prioritize patient-centered research with quantifiable outcomes. Read our announcement for our roadmap to bring this to life.

As part of this effort, we are convening the State of the Science Research Summit, a virtual community dialogue, on September 12-15, 2021. The Summit will bring together voices from across our community to address some of the most pressing issues faced by people with these disorders, and to agree on specific and actionable activities that should be guided via a National Research Blueprint, to be published early next year.

We are looking forward to several days of active discussion, featuring the experiences of patients, providers and allied professionals who support the community. We will address critical topics, including underserved areas such as ultra-rare disorders and care for women and girls with these disorders. Sessions will feature plenary presentations and panel discussions from multi-disciplinary experts, with opportunities for live patient and caregiver participation throughout. I encourage you to review the interactive program here for more information.

NHF is serving as the catalyst for this important journey, but this is much bigger than us. This is about all of us coming together to change lives for the better. Research creates discovery, discovery creates change and change creates impact for people with inherited blood disorders, both today and for generations to come.

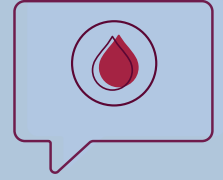
I look forward to welcoming you, virtually, to the State of the Science Research Summit!

Sincerely,

*Leonard A. Valentino, MD*

Leonard A. Valentino, MD  
NHF President and CEO

Why I'm joining the summit



“

Because research takes all of us - we need to know how to improve the lives of everyone with an inherited bleeding disorder.”

## Conference Organizer

NHF Conference & Travel Services Department

### **Logistics Lead**

Karina Lopez  
Operations Specialist

## Program Content Development

### **Program Managers**

Michelle Witkop, DNP  
Head of Research  
Maria Santaella, MSN, RN-BC, OPHON

# Conference Code of Conduct

## CONFERENCE BEHAVIOR

All participants, including attendees, exhibitors, vendors, NHF staff, chapters, volunteers and other stakeholders at NHF meetings, events and conferences both in-person and virtually will conduct themselves in a professional manner that is welcoming to all participants and free from any form of discrimination, harassment or retaliation.

Participants will treat each other with respect and consideration to create a collegial, respectful, inclusive and professional environment at NHF conferences both in-person and virtually. Creating a supportive environment to enable scientific discourse at NHF meetings is the responsibility of all participants.

We expect participants to follow NHF's conference behavior guidelines onsite (if applicable) and online, within the virtual platform and social media networks.

## UNACCEPTABLE CONFERENCE BEHAVIOR

Participants are expected to avoid any inappropriate actions or statements based on individual characteristics such as age, race, ethnicity, sexual orientation, gender identity, gender expression, marital status, nationality, political affiliation, disability status, educational background or any other individual characteristics. Disruptive or harassing behavior of any kind will not be tolerated.

Harassing conduct includes but is not limited to: unlawful harassment, derogatory comments, epithets, slurs or negative stereotyping; threatening, intimidating or hostile acts; denigrating jokes; and written, electronic or graphic material that denigrates or shows hostility or aversion toward an individual or group, including during online networking, via text, chat functions, virtual question-and-answer sessions or otherwise on the NHF virtual platform and onsite (when applicable).

## CONSEQUENCES OF UNACCEPTABLE BEHAVIOR

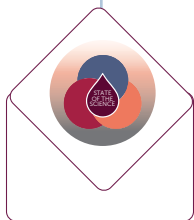
Upon receiving a report of unacceptable behavior, NHF's priority will be the safety and security of participants. Sanctions for violating this Code of Conduct may range from verbal warning, to being denied access to remainder of the meeting, event or

conference, to notification of appropriate authorities. Retaliation for complaints of inappropriate conduct will not be tolerated.

The following Agreements and Disclaimers pertain to meetings, conferences and events organized by the National Hemophilia Foundation, to the meeting/conference/event speakers, presenters, sponsors, exhibitors and volunteers (collectively referred to herein as the "NHF") and the person intending to attend and participate in the conference.

The information presented at NHF conferences is solely intended for consumers, chapters and healthcare professionals. You understand and agree that you are being provided the information for educational and informational purposes only and that information will be provided to educate you on the latest scientific, clinical, therapeutic and educational advancements. Although NHF strives to present only current and accurate information at its conferences, you agree not to solely rely on this information or consider it to be any form of professional advice without additional independent review of its accuracy, completeness, efficacy, and timeliness. Any decisions you make based on the information provided is voluntary and should only be undertaken after an independent review of its accuracy, completeness, efficacy and timeliness and/or only after consultation with a healthcare professional. Information provided during the conference or otherwise offered by NHF, is intended to provide you with updates on scientific, clinical, therapeutic and educational advancements and is not meant to replace or in any way modify the advice of a healthcare professional. Any decisions as to how you use this information is at your sole discretion and NHF will not be liable for any liability that may arise from such use.

Given the stated purpose of the information presented at NHF meetings, events and conferences, you agree that NHF is not responsible in any manner or to any extent for the consequences of any action(s) taken by any individual(s) as a result of using the information presented by NHF during the conference. Furthermore, NHF is not responsible for any information or content, or any actions that are based on such information or content, that is provided during the conference.



## Registration

To access the Summit, you will need the e-mail address used to complete registration as well as your registration number. This number can be found in your confirmation e-mail, as well as the final e-mail containing login instructions.

## Social Media



Use the hashtag

**#NHFSOS**

to connect with NHF and other attendees on Twitter and Instagram.

We will also be live tweeting sessions and sharing photos. Follow us on Twitter and Instagram at: **NHF\_hemophilia**

## Time Zone

**All times listed are ET.**

Sessions will be available on demand 24 hours after the live session takes place.

Please note, the “**JOIN**” session button will be active three minutes prior to the session listed time.

**Join**

## VIRTUAL HELP DESK

If you have any Summit or registration-related questions, please reach out to our Conference Help Desk by contacting [NHFConferences@hemophilia.org](mailto:NHFConferences@hemophilia.org)

**VIRTUAL HELP DESK SCHEDULE** *All times listed are in Eastern Time*

Sunday, September 12	10:00 A.M.	6:45 P.M.
Monday, September 13	10:00 A.M.	6:45 P.M.
Tuesday, September 14	10:00 A.M.	6:45 P.M.
Wednesday, September 15	10:00 A.M.	2:15 P.M.

## State of the Science Co-Chairs

### Leonard A. Valentino, MD



Leonard A. Valentino, MD, has been the President and Chief Executive Officer of the National Hemophilia Foundation since February 2020. He is a board-certified pediatric hematologist oncologist and practiced at Rush University Medical Center as a Professor of Pediatrics, Internal Medicine, Immunology-Microbiology and Biochemistry and the Director of the Rush University Hemophilia and Thrombophilia Center in Chicago for over two decades before retiring from academic medicine in 2013. Dr. Valentino then worked in the biopharmaceutical industry at Baxter Healthcare, Baxalta, Shire and most recently at Spark Therapeutics as the Vice President and strategy lead for hematology. He has treated many patients with hemophilia, von Willebrand Disease, platelet disorders, rare bleeding disorders and those with hereditary thrombotic disorders. He has published over 160 peer-reviewed manuscripts and participated in phase 1, 2 and 3 clinical research trials. He led a research laboratory investigating the molecular basis of joint disease in hemophilia patients.

### Michael Recht, MD, PhD



Michael Recht, MD, PhD, specializes in caring for children, adolescents and young adults with bleeding and clotting disorders. He has been part of more than 100 clinical research trials testing new treatments for children and adults with bleeding and clotting disorders. He also has been a leader in research into gene therapy for hemophilia. Dr. Recht is the Chief Science Officer for the American Thrombosis and Hemostasis Network, a nonprofit organization dedicated to improving the lives of people affected by bleeding and clotting disorders. Dr. Recht was the director of the Hemophilia Center at Oregon Health & Science University from 2007 to 2020. He also was director of the Mountain States Hemophilia Network, a collaboration of hemophilia treatment centers in the western United States.

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## State of the Science Steering Committee

### Leonard A. Valentino, MD



Leonard A. Valentino, MD, has been the President and Chief Executive Officer of the National Hemophilia Foundation since February 2020. He is a board-certified pediatric hematologist oncologist and practiced at Rush University Medical Center as a Professor of Pediatrics, Internal Medicine, Immunology-Microbiology and Biochemistry and the Director of the Rush University Hemophilia and Thrombophilia Center in Chicago for over two decades before retiring from academic medicine in 2013. Dr. Valentino then worked in the biopharmaceutical industry at Baxter Healthcare, Baxalta, Shire and most recently at Spark Therapeutics as the Vice President and strategy lead for hematology. He has treated many patients with hemophilia, von Willebrand Disease, platelet disorders, rare bleeding disorders and those with hereditary thrombotic disorders. He has published over 160 peer-reviewed manuscripts and participated in phase 1, 2 and 3 clinical research trials. He led a research laboratory investigating the molecular basis of joint disease in hemophilia patients.



### **Michael Recht, MD, PhD**



Michael Recht, MD, PhD, specializes in caring for children, adolescents and young adults with bleeding and clotting disorders. He has been part of more than 100 clinical research trials testing new treatments for children and adults with bleeding and clotting disorders. He also has been a leader in research into gene therapy for hemophilia. Dr. Recht is the Chief Science Officer for the American Thrombosis and Hemostasis Network, a nonprofit organization dedicated to improving the lives of people affected by bleeding and clotting disorders. Dr. Recht was the director of the Hemophilia Center at Oregon Health & Science University from 2007 to 2020. He also was director of the Mountain States Hemophilia Network, a collaboration of hemophilia treatment centers in the western United States.

### **Kevin Mills, PhD**



Kevin Mills, PhD, obtained his PhD in Biology from the Massachusetts Institute of Technology and completed a postdoctoral fellowship at Harvard Medical School. In 2005, Dr. Mills joined the faculty of The Jackson Laboratory in Bar Harbor (Maine) and led an NIH-funded laboratory investigating the biology of DNA repair in hematology and oncology. In 2012, he co-founded Cyteir Therapeutics, a publicly traded biotechnology company developing and commercializing new therapies targeting DNA repair pathways. As co-founder and Chief Scientific Officer for Cyteir, Dr. Mills raised \$80 million in pre-IPO financing and led the team that developed a first-in-class RAD51 inhibitor and brought it into clinical trials in oncology.

### **Shannon Carpenter, MD, MS**



Shannon Carpenter, MD, MS, focuses her research on bleeding and clotting disorders. She has published multiple collaborative manuscripts regarding the risk for and prevention of venous thromboembolism in hospitalized pediatric patients. She currently leads a national trial investigating the development of inhibitors in untreated children with hemophilia. Additionally, she is involved in studies of women and girls with rare bleeding disorders. Dr. Carpenter also collaborates with Child Abuse Pediatricians regarding the work-up for bleeding disorders in children suspected of being abused.

### **Esmeralda Vázquez**



Esmeralda Vázquez is a member of the inherited bleeding disorder community. She was diagnosed with Glanzmann's Thrombasthenia, an ultra-rare bleeding disorder, at age 1. She is a member of the NHF SOS Steering Committee and a member of the Diversity, Equity and Inclusion Working Group. Esmeralda has developed an interest in healthcare inequalities, improving healthcare access and working with underserved populations in urban areas.

### Jill Johnsen, MD



Jill Johnsen, MD, is a physician scientist studying benign (classical) hematology. She is an Associate Member at the Bloodworks Research Institute, an Assistant Professor of Medicine at the University of Washington and sees patients in the Washington Center for Bleeding Disorders (WACBD). Dr. Johnsen studies the genetics and biology of clotting factors and blood groups (blood types), with emphasis on coagulation factor VIII (FVIII), factor IX (FIX) and von Willebrand factor (VWF), and clinically relevant blood group genes, particularly the ABO and Rh systems. Her research uses new sequencing approaches to identify simple and complex DNA variation and performs computational and functional studies of the consequences of DNA changes to better understand causes of disease and to improve interpretation of genetic data. A major research focus is the molecular basis of how inherited bleeding disorders uniquely impact females, particularly in hemophilia and von Willebrand Disease.

### Barbara Konkle, MD



Barbara Konkle, MD, has built a career in clinical and laboratory-based research in disorders of hemostasis, including hemophilia and von Willebrand Disease and in mentoring trainees and junior faculty in these areas. She has a longstanding interest in the genetic diagnosis of bleeding disorders and recently led the MyLifeOurFuture project, which genotyped over 11,000 patients and potential genetic carriers of hemophilia in the U.S. She has researched outcomes in hemophilia and VWD, with special interest in the impact of these disorders on women, and has led a number of clinical trials, including in gene therapy for hemophilia. She has received NIH, CDC and industry support for her studies and has published widely in these areas. Dr. Konkle is a member of the Board of Directors of the World Federation of Haemophilia and is a founding Board member of the Foundation for Women and Girls with Blood Disorders.

### Sarah O'Brien, MD, MSc



Sarah O'Brien, MD, MSc, is a pediatric hematologist in the Nationwide Children's Hospital Hemostasis and Thrombosis Center, an investigator in the Center for Health Equity and Outcomes Research at the Abigail Wexner Nationwide Children's Research Institute and an Associate Professor of Pediatrics at The Ohio State University College of Medicine. She serves as the Director of Experimental Therapeutics for the Division of Pediatric Hematology/Oncology/BMT and leads a multi-disciplinary hematology and adolescent gynecology clinic at Nationwide Children's Hospital. Her clinical and research interests include pediatric thrombosis and thromboprophylaxis, the evaluation and diagnosis of mild bleeding disorders and the intersections between hematology and women's health.

### Steven Pipe, MD



Steven Pipe, MD, is a Professor and the Laurence A. Boxer Research Professor of Pediatrics and Professor of Pathology at the University of Michigan. He is the medical director of the Pediatric Hemophilia and Coagulation Disorders Program and the Special Coagulation Laboratory. His clinical interests include bleeding and thrombotic disorders and congenital vascular anomalies. Dr. Pipe directs a research lab investigating coagulation factor VIII and the molecular mechanisms of hemophilia A. He has been actively involved in clinical trials with novel therapeutics for hemophilia, including gene therapy. In 2015, he received the Leadership in Research Award from the National Hemophilia Foundation. He served on the Board of Directors for the Hemostasis and Thrombosis Research Society, as Chair of the Board of Directors for the American Thrombosis and Hemostasis Network and currently serves as Chair of NHF's Medical and Scientific Advisory Council.

## **Donna DiMichele, MD**



Donna DiMichele, MD, served as Deputy Director of the Division of Blood Diseases and Resources at NHLBI from 2010 to 2019. Previously, she was Professor of Pediatrics and Public Health at the Weill Cornell Medical College of Cornell University, Director of the New York Center for Hemophilia and Coagulation Disorders and Attending Physician at the New York Presbyterian Hospital. Since early 2020, she has embarked on a new venture as the principal consultant with Donna DiMichele Consulting, LLC, with the goal of partnering with the Hematology Community at large and the Bleeding Disorders Community in particular, to strategize a future informed by critical research and supported by a dedicated workforce. She is thrilled to engage again with the Inherited Bleeding Disorders Community through her work with the National Hemophilia Foundation on the organization of the NHF State of the Science Research Summit 2021.

## **Michelle Witkop, DNP, FNP-BC**



As the Head of Research at the National Hemophilia Foundation, Michelle Witkop, DNP, FNP-BC, leads the NHF's research initiatives. She is a recipient of the Nurse of the Year Award from the American Nurses Credentialing Center for Transformational Leadership and an ATHN/HRSA Demonstration Project of National Significance Grantee with the project, "Evaluating a Nurse Practitioner Medically Led Hemophilia Treatment Center in Comparison to a Physician Medically Led Hemophilia Treatment Center." With extensive experience and research in pain management, she has been the principal or co-investigator for multiple hemophilia pain research studies, including the National Pain Study and the IMPACT Quality of Life Studies, and has published and lectured extensively.

## **Maria E. Santaella, PhD candidate, RN-BC, MSN, CPHON**



Maria Santaella is a board-certified nurse with more than 18 years of experience in inherited bleeding disorders (IBDs). She works at the Research Department of the National Hemophilia Foundation (NHF), focusing primarily on the implementation of Community Voices in Research (CVR). A community-powered registry, CVR gathers the experiences of persons living with IBDs and their non-affected relatives longitudinally. It provides their unique and important perspectives and complements and enhances the value of other research efforts. Its ultimate goal is to empower participants to be more active members of their healthcare team, identify research questions important to the community and improve quality of life. Previously, she was the bleeding disorders nurse coordinator for the University of Miami Hemophilia Treatment Center.

## **State of the Science Advisory Committee**

### **Craig Hooper, PhD**



Craig Hooper, PhD, received his BA from Wake Forest University and his PhD in Pathology from The Ohio State University. After completing a post-doctoral fellowship at the Mayo Clinic, he joined the Centers for Disease Control and Prevention in the Division of Host Factors. He has remained with what is now known as the Division of Blood Disorders for the past 33 years. His activities have revolved around translational research in oncology, vascular biology and hemostasis, with emphasis on the molecular and inflammatory aspects of thrombosis, hemophilia and red cell disorders. He has long been interested in the health disparities that underlie venous thromboembolism and genes that may modify the hemophilia phenotype. He has authored or co-authored over 190 peer review publications and currently serves as the Director, Division of Blood Disorders.

### **Keith Hoots, MD**



W. Keith Hoots, MD, is director of NHLBI's Division of Blood Diseases and Resources. Dr. Hoots received his AB in English and chemistry and his MD from the University of North Carolina at Chapel Hill, NC. While a senior at UNC, he worked in the hemostasis laboratory of Kenneth Brinkhous, MD. He then completed his pediatric internship and residency at Children's Medical Center, Parkland Memorial Hospital, in Dallas, TX. He returned to UNC for his fellowship in pediatric hematology oncology and worked in the laboratory of Harold Roberts, MD. Dr. Hoots then joined the faculty at MD Anderson Cancer Center.

His work includes the creation of longitudinal follow-up of hemophilia cohorts with HIV and hepatitis, gene therapy trials for hemophilia A and B, clinical trials of new clotting concentrates for hemophilia A and B and the impact of care and clotting factor product on hemophilia patient outcomes.

### **Kathryn McLaughlin, MPH**



Kathryn McLaughlin, MPH, has been the Director and Project Officer for the National Hemophilia Program, consisting of the Regional Hemophilia Networks and the National Hemophilia Program Coordinating Center, since 2011. These programs are funded out of the Division of Services for Children with Special Health Needs (DSCSHN), within the Maternal and Child Health Bureau (MCHB) at the Health Resources and Services Administration (HRSA). Ms. McLaughlin received her MPH in Health Policy & Management from the University of Massachusetts at Amherst. Previously, she was a senior research associate at America's Health Insurance Plans (AHIP), specializing in coverage, quality improvement and performance measurement and evidence-based medicine.

### **Peter Marks, MD, PhD**



Peter Marks, MD, PhD, received his graduate degree in cell and molecular biology and his medical degree at New York University and completed Internal Medicine residency and Hematology/Medical Oncology training at Brigham and Women's Hospital in Boston. He has worked in academic settings teaching and caring for patients and in industry on drug development. He joined the FDA in 2012 as Deputy Center Director for CBER and became Center Director in January 2016.



## Working Group 1: Research Priorities for Hemophilia A & B

### “Bobby” Duc Q. Tran, MD, MSc



“Bobby” Duc Q. Tran, MD, MSc, is an assistant professor in the Department of Hematology and Medical Oncology at the Emory University School of Medicine in Atlanta. He is an adult hematologist at Hemophilia of Georgia Center for Bleeding & Clotting Disorders at Emory. Dr. Tran received his medical degree from the University of South Carolina in Columbia. He completed a residency in internal medicine at the University of South Florida in Tampa, where he also served as Chief Resident. He then completed a fellowship in hematology at Emory University. Dr. Tran is an active clinical researcher in the fields of hemophilia and bleeding disorders. He is particularly interested in patient-reported outcomes research in people with hemophilia.

### Annette von Drygalski, MD, PharmD, RMSK



Annette von Drygalski, MD, PharmD, RMSK, is a Professor of Clinical Medicine and Director of the Hemophilia and Thrombosis Treatment Center at the University of California, San Diego. Dr. von Drygalski is dedicated to the care of patients with hemophilia and bleeding disorders, including addressing the needs of aging patients with hemophilia, such as new therapies, cardiovascular disease and hypertension as well as the progression of hemophilic joint disease. Dr. von Drygalski investigates gene therapy and other novel molecules and elucidates effects of new treatments on the pathobiology of joint health, both at the bench and in translational studies. She has pioneered musculoskeletal ultrasound for rapid joint bleed detection and joint evaluation in hemophilia. Dr. von Drygalski developed a focus in international outreach to facilitate training for hematologists from developing countries and establish care for patients with hemophilia and bleeding disorders in Mozambique, as well as address mortality from post-partum bleeding.

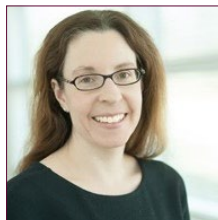
## Working Group 2: Research Priorities for von Willebrand Disease, Platelet Dysfunction & Other Mucocutaneous IBDs

### Robert Sidonio, MD, MSc



Robert Sidonio, MD, MSc, graduated from the University of Alabama-Birmingham medical school, completed his pediatric residency at the University of Louisville and completed his fellowship at the University of Pittsburgh, where he also obtained his master's degree in Clinical Investigation. Dr. Sidonio has been the Associate Director of Hemostasis and Thrombosis at Emory University since 2014. Dr. Sidonio's clinical and research interest is in investigating the bleeding phenotype and genotype of women with hemophilia carriage and low VWF. He also is the co-creator of the Atlanta Protocol, which combines emicizumab and FVIII for ITI. He is co-PI of the HOG VWD project focused on characterization of children with low VWF in Georgia. He also is the PI for the emicizumab PUP and Nuwiq ITI trial, MOTIVATE study, ATHN 9 study (severe VWD).

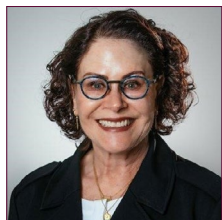
### Veronica Flood, MD



Veronica Flood, MD, is a pediatric hematologist and researcher at the Medical College of Wisconsin. She is a member of the Comprehensive Center for Bleeding Disorders in Milwaukee, where she sees patients with von Willebrand Disease and other bleeding and clotting disorders, as well as children admitted to the hematology service. Dr. Flood also served as the clinical chair of the VWD Management Guideline Committee through ASH/ISTH/NHF/WFH. Dr. Flood's research interests include the diagnosis of VWD and the interaction of von Willebrand Factor (VWF) with platelets and vascular collagens. Through the Zimmerman Program for the Molecular and Clinical Biology of VWD, she has worked on assays of VWF function and the genetics of VWF. She also has a grant from the National Heart, Lung, and Blood Institute to look at VWF interactions with type IV collagen and the impact of these interactions on hemostasis.

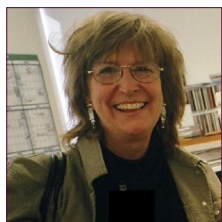
## Working Group 3: Research Priorities for Ultra-Rare IBDs

### Amy Shapiro, MD



Amy Shapiro, MD, is Medical Director and CEO of the Indiana Hemophilia and Thrombosis Center in Indianapolis and Adjunct Senior Investigator, Clinical Track at the Blood Research Institute in Milwaukee, WI. After receiving her medical training at New York University School of Medicine in New York City, Dr. Shapiro completed her pediatric internship, residency and fellowship in pediatric hematology/oncology at the University of Colorado Health Sciences Center in Denver. She has served on the National Hemophilia Foundation's Medical and Scientific Advisory Council as well as several boards for the National Institutes of Health in Data Safety Monitoring and Clinical Trial Review. Dr. Shapiro currently serves on the FDA Blood Products Advisory Committee. As one of the founders of the American Thrombosis and Hemostasis Network (ATHN), she has served as Co-Chairman of the Board of Directors and remains active on various ATHN committees.

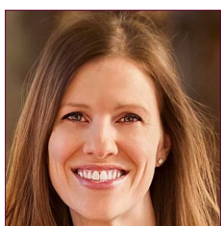
### Diane Nugent, MD



Diane Nugent, MD, is Chief of Hematology at Children's Health Orange County/UC Irvine, Center for Inherited Blood Disorders; is Board certified in pediatrics and pediatric Hematology/ Oncology and is an internationally recognized expert clinician and researcher specializing in all aspects of bleeding and clotting disorders. She is principal investigator of numerous studies advancing diagnostics and treatments, authored over 150 publications in peer-reviewed journals and leads several federal government grants to transform regional healthcare delivery and outcomes, including the HRSA-funded Western States Regional Hemophilia Network and the Pacific Sickle Cell TDP and CDC Prevention of Bleeding Disorder Complications in Region IX. Dr. Nugent serves on the Medical and Scientific Advisory Council of the National Hemophilia Foundation. To increase access to care and diagnosis for patients of all ages, she founded the safety net nonprofit Center for Inherited Blood Disorders (CIBD-ca.org) and the Hematology Advanced Diagnostic Laboratory, which is CAP/CLIA certified nationally.

## Working Group 4: Research Priorities for the Health of Women & Girls & Persons with the Potential for Menstruation

### Angela Weyand, MD



Angela Weyand, MD, is a native of Kansas City, KS and graduate of Northwestern University in Evanston, IL. She attended medical school at the University of Michigan and completed her pediatrics residency at the University of Washington/ Seattle Children's Hospital. She then returned to the University of Michigan for a pediatric hematology and oncology fellowship and subsequently stayed on as faculty. Clinically, she is interested in von Willebrand Disease and young women and girls with bleeding or clotting disorders. She is co-director of a combined hematology/ gynecology program and an active member of the Foundation for Women and Girls with Blood Disorders. Her research interests are in women and girls with bleeding disorders, hormone-provoked thrombosis and von Willebrand Disease. She was a member of the management panel for the latest ASH/ISTH/NFH/WFH VWD guidelines and is national Co-PI on an observational study of severe VWD.

### **Maureen K. Baldwin, MD, MPH**



Maureen K. Baldwin, MD, MPH, is an Associate Professor of Obstetrics & Gynecology at Oregon Health & Science University, where she received her medical degree and master's in Public Health. Dr. Baldwin completed her Ob/Gyn residency and Fellowship in Family Planning at OHSU, and has a Practice Focus Designation in Pediatric and Adolescent Gynecology. She is Clinical Director of the Early Pregnancy Assessment Clinic and Co-Director of the interdisciplinary Hematology and Gynecology Adolescent "Spots, Dots and Clots" clinic, which takes care of teens with heavy menstrual bleeding. She serves as a Medical Advisor for the National Hemophilia Foundation and the Foundation for Women & Girls with Bleeding Disorders. Her research has included abortion safety, diagnosis of early pregnancy loss, early postpartum IUD outcomes, system approaches to long-acting contraceptive uptake and teaching with simulation.

## **Working Group 5: Diversity, Equity & Inclusion, Health Services Research & Implementation Science**

### **Judith Baker, DrPH, MHSA**



Judith Baker, DrPH, MHSA, is a national leader in rare blood disorders in regional healthcare systems. She researches organizational influences on quality, cost, outcomes and equity. At the Center for Inherited Blood Disorders in Orange, CA, Dr. Baker co-directs federal grants. She is Regional Administrator for the Western States Hemophilia Network's 13 centers serving California, Hawaii, Nevada and the US Pacific; and Public Health Director for HRSA's 13-State Pacific Sickle Cell Regional Collaborative plus the new Networking California for Sickle Cell Care \$15 million initiative. Dr. Baker co-led the development of the first hemophilia centers in Guam, Hawaii and Nevada, and an Adult Sickle Cell Clinic in Los Angeles. She co-chairs the US HTC Network's National Patient Satisfaction Survey, serves on FDA's Blood Products Advisory Committee and received a National Hemophilia Foundation Meritorious Service Award. Dr. Baker obtained her Doctor of Public Health degree from UCLA.

### **Tyler Buckner, MD, MSc**



Tyler Buckner, MD, MSc, provides care to children and adults with bleeding and clotting disorders. Dr. Buckner received his medical degree and completed Internal Medicine and Pediatrics residency and a combined fellowship in Hematology (adult) and Pediatric Hematology/Oncology at the University of North Carolina-Chapel Hill. He also completed a master's degree in clinical research at the UNC School of Public Health and a post-doctoral fellowship at the UNC Cecil G Sheps Center for Health Services Research. In 2015, Dr. Buckner joined the Hematology faculty at the University of Colorado. His research seeks to identify clinical management strategies that lead to improved patient outcomes for patients with bleeding and clotting disorders.

### **Vanessa Byams, DrPH, MPH**



Vanessa Byams, DrPH, MPH, joined the Centers for Disease Control and Prevention in 2001 and has worked in inherited bleeding disorders for her entire tenure. As team lead, she provides scientific and programmatic leadership for surveillance and health promotion activities to improve the health of people with inherited bleeding disorders. Dr. Byams is privileged to serve the bleeding disorders community alongside many dedicated and talented colleagues inside and outside of CDC. She earned her undergraduate degree from Emory University and her master's degree from Boston University School of Public Health. She completed her doctoral training in public health leadership at the University of Illinois at Chicago.

## Working Group 6: Facilitating Priority Research in the IBDs Community

### Margaret Ragni, MD, MPH



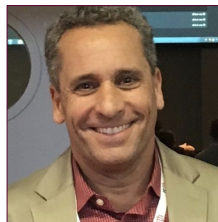
Margaret Ragni, MD, MPH, has been involved in clinical translational studies relevant to congenital hemostatic and thrombotic disorders. She has served as the chair of clinical trials, prospective epidemiologic, observational, case-control studies, cost-effectiveness analyses and investigator-initiated new drug trials in hemophilia and VWD. Her studies were among the first multi-center NIH-funded investigator-initiated studies in hemophilia malignancy (NCI), hemophilia inhibitor formation (NHLBI), hemophilia HIV/HCV infection (NHLBI), hemophilia AIDS therapy (NIAID) and hemophilia adult prophylaxis (NHLBI). She is the Medical Director of the Hemophilia Center of Western PA and is a member of the National Hemophilia Foundation Medical & Scientific Advisory Committee (MASAC). She also is a member of the ASH Women in Hematology Committee and serves as an Associate Editor of the ASH online publication Blood Advances. She mentors students, residents and fellows in patient care and clinical translational research, including T32, T35, K23, and K12 recipients, which she considers critical to the future of benign hematology.

### Jordan Shavit, MD, PhD



Jordan Shavit, MD, PhD, received his MD and PhD from Northwestern University after completing his undergraduate education at the University of Michigan. He returned to Ann Arbor to complete a residency in pediatrics and a fellowship in pediatric hematology/oncology. Dr. Shavit is a Professor of Pediatrics and the Henry and Mala Dorfman Family Professor of Pediatric Hematology/Oncology at the University of Michigan. His research interests are in “clinically directed basic science” using zebrafish as a model and his work is supported by grants from the National Institutes of Health and the National Hemophilia Foundation. He is a member of the NHF’s Medical and Scientific Advisory Council (MASAC), the American Society of Hematology, International Society on Thrombosis and Haemostasis, American Society for Clinical Investigation and the American Pediatric Society. He also is on the board of directors for the Hemostasis and Thrombosis Research Society.

### Guy Young, MD



Guy Young, MD, received his medical degree from the State University of New York at Stony Brook School of Medicine. He completed a residency in pediatrics at Schneider Children’s Hospital at Long Island Jewish Medical Center, Albert Einstein College of Medicine in New York, and a fellowship in pediatric hematology/oncology at Children’s National Medical Center, George Washington University School of Medicine in Washington, D.C. Dr. Young is the Director of the Hemostasis and Thrombosis Center at Children’s Hospital Los Angeles and a Professor of Pediatrics at the University of Southern California’s Keck School of Medicine. His research activities encompass clinical trials in hemophilia, the use of novel anticoagulants in children and the development of global hemostasis assays to monitor the effects of medications used to treat bleeding in hemophilia. He was awarded the National Hemophilia Foundation Physician of the Year Award in 2013.



## State of the Science Subject Matter Experts

**Mark Skinner, JD**



Mark Skinner, JD, is President/CEO of the Institute for Policy Advancement Ltd, specializing in patient-centered outcomes research. He is an Assistant Professor in the Department of Health Research Methods, Evidence and Impact at McMaster University and the principal investigator for the Patient Reported Outcomes Burdens and Experiences (PROBE) global research study. He has previously led both the World Federation of Hemophilia and National Hemophilia Foundation, where he currently serves on the Medical and Scientific Advisory Council. He holds degrees in Public and Business Administration from Kansas State University and a JD from Washburn University School of Law.

**Amar Haidar**



Amar Haidar lives with Severe Factor XIII deficiency, as do three of her family members. She is a student studying Graphic Design, actively participates in different art activities throughout the city and is active within her hemophilia chapter, Hemophilia Foundation of Michigan. She was part of a study for a new treatment for FXIII for 10 years. With new treatment opportunities that recently became available, her quality of life continues to improve.

**Shatara Askew**



Shatara Askew was diagnosed at 15 with factor 7 deficiency. She is interested in learning more about NHF and the different type of factors and is looking to meet more people who are living with bleeding disorders.

**Nikole Scappe**



Nikole Scappe is an Education Specialist at the National Hemophilia Foundation (NHF) and works on Inhibitor Series, Education for Empowerment Programming and rare bleeding disorders educational content development. She has been actively involved in the bleeding disorders community since 2003 and has von Willebrand disease. In addition, Nikole was an intern for NHF and the Western Pennsylvania Chapter and served on NHF's Board of Directors as an NYLI representative, NHF's VWD working group and the Annual Meeting Planning Committee. She currently serves on the World Federation of Hemophilia's Youth Committee. Nikole earned her bachelor's degree from La Roche University in Pittsburgh, Pa., where she double majored in marketing and management.

### Shellye Horowitz, MA



Shellye Horowitz, MA, is a licensed school counselor and school administrator with over 25 years of experience in the field of education. Shellye has strong ties to the bleeding disorders community, with six traceable generations of hemophilia A in her family. Like many women, Shellye struggled to receive a hemophilia diagnosis, growing up in a time when women were believed to be “just carriers.”

After attending multiple hemophilia conference events and advocacy training sessions, Shellye synthesized information and developed an understanding of how to present information about bleeding issues in a compelling way to medical providers. Shellye works to empower women and has spoken with local hemophilia chapters and at national conferences, sharing crucial information on partnering with providers for bleeding disorders diagnosis and treatment. Shellye writes a column for Hemophilia News Today addressing issues regarding women and bleeding disorders called “The Forgotten Factor.”

### Esmeralda Vázquez



Esmeralda Vázquez is a member of the inherited bleeding disorder community. She was diagnosed with Glanzmann’s Thrombasthenia, an ultra-rare bleeding disorder, at age 1. She is a member of the NHF SOS Steering Committee and a member of the Diversity, Equity and Inclusion Working Group. Esmeralda has developed an interest in healthcare inequalities, improving healthcare access and working with underserved populations in urban areas.

### Ziva Mann



Ziva Mann is a perennially curious systems geek, with a deep respect for the capacity of people, communities and organizations to change their world. She is the Director of Assessment and Development at Ascent Leadership Networks, where she uses data and motivation to assess and develop leaders and organizations. Previously, she worked in a range of roles, including patient partnership for redesigning processes, policies and structures for team-based, person-centered care at Cambridge Health Alliance; faculty member for the Institute of Healthcare Improvement, the Gold Foundation, and core team for grants from the Robert Wood Johnson Foundation and PCORI. Her work for RWJF and IHI has supported the development of generative, collaborative leaders, working for equity, health and wellbeing in their communities. A member of NHF’s board of directors, Ziva lives with and loves someone with a bleeding disorder. She lives in Massachusetts with her husband and sons.

## Additional Featured Summit Speakers

### Suchitra Acharya, MD, MBBS



Suchitra Acharya, MD, MBBS, is a trained Pediatric Hematologist/Oncologist with a committed interest and passion for diagnosing and treating bleeding and clotting disorders in children and young adults. Dr. Acharya established the first North American ultraRare Bleeding Disorder (NARBD) registry as a fellow in training, which provided an estimate of the distribution of these disorders. More recently as part of the American Thrombosis and Hemostasis Network (ATHN) ultraRare Bleeding Disorders (RBDs) subcommittee, she analyzed data on ultraRBDs collected as part of the ATHN- CDC project, which shed light on the need for accurate data collection. She serves on the Steering committee for ATHN-10, offering genetic testing for ultraRBDs using NexGen sequencing. Dr. Acharya also is a member of the Working Group and Co-Chair of WG 3 for ultra-rare bleeding disorders for the NHF's State of the Science Research Summit.

### Charles Bailey, MD, PhD



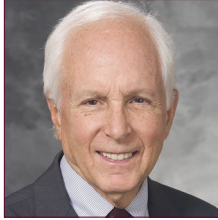
Charles Bailey, MD, PhD, is a pediatric hematologist/oncologist and a clinical informatician at the Children's Hospital of Philadelphia. His informatics work focuses on creating a pediatric learning health system --a health care partnership designed not to dispense expertise to patients, but rather to constantly learn, using today's experience to deliver better health care tomorrow. He is the informatics lead for PEDSnet, a collaboration among children's hospitals designed to accelerate research through collaboration and data standardization, and directs the network's Data Coordinating Center. His research examines the reuse of data collected during clinical care to perform more effective clinical research and to better measure the quality of health care. He also is working to develop better ways to determine how well clinical data fits for a particular study, and how best to communicate that to people who rely on the results of the study.

### Melissa Creary, PhD, MPH



Melissa Creary, PhD, MPH, is Senior Director for the Office of Public Health Initiatives at the American Thrombosis and Hemostasis Network (ATHN) and Assistant Professor in the Department of Health Management and Policy in the School of Public Health at the University of Michigan. She received her PhD and MPH at Emory University. Over a nine-year career at the Centers for Disease Control and Prevention in the Division of Blood Disorders, she helped create and co-lead the first national program and data collection system for sickle cell disease at the agency. Her area of study includes ELSI, race and racism, identity politics, health equity and health policy. Her most recent project analyzes how equity-based scientific and public health policies are incongruent to the very justice they are trying to produce. She has been published in the Journal of Law, Medicine, & Ethics, Social Science and Medicine, Genetics in Medicine and American Journal of Bioethics.

### **Norman Fost, MD, MPH**



Norman Fost, MD, MPH, is Professor Emeritus of Pediatrics and Bioethics at the University of Wisconsin-Madison and past-Director of the Bioethics Program, which he founded in 1973. He was Chair of the UW Hospital Ethics Committee and Health Sciences IRB for 31 years; director of the Pediatric Residency Training Program for 21 years; founder and leader of the Child Protection Program for 33 years; and Vice Chair of the Department of Pediatrics and the Department of Medical History and Bioethics for 10 years. He served on President Clinton's Health Care Task Force, and was an elected member of the Princeton University Board of Trustees. He has received three lifetime achievement awards for his work in bioethics. Dr. Fost is a graduate of Princeton (AB), Yale (MD) and Harvard (MPH). He completed residency training in Pediatrics at Johns Hopkins, including two years as Chief Resident.

### **Wendy Kohrt, PhD**



Wendy Kohrt, PhD, is a Distinguished Professor of Medicine in the Division of Geriatric Medicine and the Nancy Anschutz Chair in Women's Health Research at the University of Colorado Anschutz Medical Campus. She is the Director of Research for Geriatric Medicine, Acting Director of the Eastern Colorado VA Geriatric Research, Education and Clinical Center (GRECC), and Associate Director of the Center for Women's Health Research. She has received continuous funding from the NIH as a principal investigator since 1990 and has more than 260 research publications. Dr. Kohrt is the Director of the Colorado Specialized Center of Research Excellence (SCORE) on Sex Differences and Women's Health. She also serves as Chair of the Steering Committee for the NIH Common Fund Molecular Transducers of Physical Activity Consortium (MoTrPAC).

### **Keri Norris, PhD, JM, MPH, MCHES**



Keri Norris, PhD, JM, MPH, MCHES, is a public health professional with extensive training and expertise in health equity, health promotion and disease prevention at the local, state and federal levels. She has worked for premiere public health and higher-education institutions including the CDC, The Fulton DeKalb Hospital Authority, Spelman College, Baylor University and Morehouse School of Medicine. She is a graduate of Agnes Scott College, Morehouse School of Medicine, the University of South Carolina and Emory University Law School. Dr. Norris serves on the boards of several nonprofit organizations and is a member of Junior League DeKalb and Alpha Kappa Alpha Sorority Inc. Dr. Norris also is a two-time author and a TEDx Speaker on Health Equity. In her spare time, she enjoys binge watching a good series, reading and spending time with family. She has a son and a grandson.

### **James O'Donnell, MD**



James O'Donnell, MD, received his medical degree from Trinity College Dublin, and completed hematology training in the Hammersmith and Royal Free Hospitals in London. He is a Fellow of both the Royal College of Physicians of Ireland and the Royal College of Pathologists (UK). Dr. O'Donnell is Professor of Vascular Biology in the Royal College of Surgeons in Ireland and Director of the Irish Centre for Vascular Biology. The Haemostasis Research laboratory led by Dr. O'Donnell has focused on basic research related to different aspects of vascular biology in relation to clinical bleeding and thrombosis. He has published more than 160 publications in high-impact peer-reviewed journals.

# Day One: September 12

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11:00 am - 11:03 AM ET

## WELCOME

**Dr. Leonard A. Valentino**

NHF's CEO and Summit Co-Chair Dr. Leonard A. Valentino welcomes everyone to the State of the Science Research Summit, setting the stage for what to expect in the next 3½ days and specifically, in this morning's session.

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11:03 AM - 12:20 PM ET

## SUBJECT MATTER EXPERT: THE PATIENT'S PERSPECTIVE

**Mark Skinner, JD**

NHF recognizes the unique and important expertise patients have about their disorders. For that reason, we refer to them as subject matter experts (SMEs). This short presentation will provide the SME summit perspective with regard to hemophilia A and B.

## PLENARY: SIX SHIBBOLETHS ABOUT ETHICAL ISSUES IN GENE THERAPY FOR HEMOPHILIA

**Norman Fost, MD, MPH**

This session will address common misconceptions about ethical issues in research on bleeding disorders, including the distinction between legally and morally valid consent; equal access to novel therapies; the use of democratic principles in developing policy; efficiency vs fairness in allocating scarce resources; and genetic manipulation of embryos.

## WORKING GROUP CHAIR SUMMARY

**"Bobby" Duc Q. Tran, MD, MSc**

**Annette von Drygalski, MD, PharmD, RMSK**

Chairs for Working Group 1: Research Priorities for Hemophilia A and B discuss their approach and questions identified.

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**BREAK** 12:20 PM - 1:00 PM ET

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1:00 PM - 2:30 PM ET

## WORKING GROUP PANEL DISCUSSION

Moderator: Jill Johnsen, MD

Norman Fost, MD, MPH

Mark Skinner, JD

“Bobby” Duc Q. Tran, MD, MSc

Annette von Drygalski, MD, PharmD, RMSK

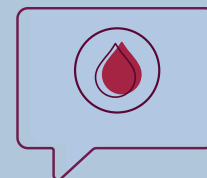
Live discussion/panel responds to questions and comments from the community generated by the session’s plenary speaker and Working Group 1: Research Priorities for Hemophilia A and B.

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**BREAK** 2:30 PM - 3:00 PM

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Our dialogue together is what  
matters, because...



“  
Patients and their families  
should be involved in every step  
of research. Without us there  
cannot be any research.”

**3:00 PM - 3:50 PM ET**

## **WELCOME**

**Leonard A. Valentino, MD**

NHF's CEO and meeting's co-chair, Dr. Leonard Valentino, welcomes everyone and explains what to expect in today's afternoon session.

## **SUBJECT MATTER EXPERT: THE PATIENT'S PERSPECTIVE**

**Shatara Askew**

**Amar Haidar**

NHF recognizes the unique and important expertise patients have about their disorders. For that reason, we refer to them as subject matter experts (SMEs). This short presentation will provide the SME summit perspective with regard to ultra-rare bleeding disorders.

## **PLENARY: THE EVOLVING PROCESS TO MOVE RARE DISEASE THERAPEUTICS THROUGH THE REGULATORY PROCESS**

**Peter Marks, MD, PhD**

Arriving at a complete understanding of ultra-rare inherited bleeding disorders has been challenging due to the difficulties of studying small and widely dispersed affected populations. These same challenges have hampered the development of targeted therapeutics for ultra-rare disorders through the standard regulatory processes. Dr. Marks will discuss innovative ideas being discussed at the FDA for overcoming these regulatory challenges and how a robust research agenda for ultra-rare bleeding disorders could contribute to the faster realization of a more robust therapeutic landscape.

## **WORKING GROUP CHAIR SUMMARY**

**Suchitra Acharya, MD, MBBS**

**Diane Nugent, MD**

**Amy Shapiro, MD**

Chairs for Working Group 3: Research Priorities for Ultra-Rare Inherited Bleeding Disorders discuss their approach and questions identified.

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**BREAK 3:50 PM - 4:45 PM ET**

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4:45 PM - 6:15 PM ET

## WORKING GROUP PANEL DISCUSSION

Moderator: Michael Recht, MD, PhD

Suchitra Acharya, MD, MBBS

Shatara Askew

Amar Haidar

Peter Marks, MD, PhD

Diane Nugent, MD

Amy Shapiro, MD

Live discussion/panel responds to questions and comments from the community generated by the session's plenary speaker and Working Group 3: Research Priorities for Ultra-Rare Inherited Bleeding Disorders.

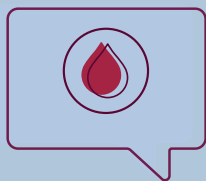
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6:15 PM - 6:30 PM ET

## SUMMARY OF THE DAY

Leonard A. Valentino, MD

NHF's CEO and Summit Co-Chair, Dr. Leonard A. Valentino, welcomes everyone and explains what to expect in today's afternoon session.



“

I was so happy to be involved in the Working Group. I learned so much and I felt listened to.”



# Day Two: September 13

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11:00 AM - 11:03 AM ET

## WELCOME

**Michael Recht, MD, PhD**

ATHN's Chief Science Officer and Summit Co-Chair Dr. Michael Recht welcomes everyone and explains what to expect in today's morning session.

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11:03 AM - 12:20 PM ET

## SUBJECT MATTER EXPERT: THE PATIENT'S PERSPECTIVE

**Nikole Scappe**

NHF recognizes the unique and important expertise patients have about their disorders. For that reason, we refer to them as subject matter experts (SMEs). This short presentation will provide the SME summit perspective with regard to von Willebrand Disease, platelet dysfunction and other mucocutaneous inherited bleeding disorders.

## PLENARY: UNDERSTANDING PHENOTYPIC VARIABILITY – IMPLICATIONS FOR DIAGNOSIS AND PERSONALIZED TREATMENT IN VON WILLEBRAND DISEASE

**James O'Donnell, MD**

People with von Willebrand Disease vary significantly in their bleeding manifestations (phenotype), which cannot always be predicted on the basis of standard diagnostic testing. Dr. O'Donnell has been studying the biological mechanisms that are responsible for causing decreased von Willebrand Factor levels to search for a better understanding of this condition and its symptoms. He will share his observations and highlight the research still needed to achieve the goals of better diagnostic accuracy and prediction of bleeding.

## WORKING GROUP CHAIR SUMMARY

**Veronica Flood, MD**

**Robert Sidonio, MD, MSc**

Chairs for Working Group 2: Research Priorities for von Willebrand Disease, Platelet Dysfunction and Other Mucocutaneous Inherited Bleeding Disorders discuss their approach and questions identified.

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**BREAK 12:20-1:00 PM**

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1:00 PM - 2:30 PM ET

## WORKING GROUP PANEL DISCUSSION

**Moderator:** Barbara Konkle, MD

Veronica Flood, MD

James O'Donnell, MD

Nicole Scappe

Robert Sidonio, MD, MSc

Live discussion/panel responds to questions and comments from the community generated by the session's plenary speaker and Working Group 2: Research Priorities for von Willebrand Disease, Platelet Dysfunction and Other Mucocutaneous Inherited Bleeding Disorders.

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## BREAK 2:30 PM - 3:00 PM ET

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3:00 PM - 4:30 PM ET

## WELCOME

**Michael Recht, MD, PhD**

ATHN's Chief Science Officer and Summit Co-Chair Dr. Michael Recht welcomes everyone and explains what to expect in today's afternoon session.

## SUBJECT MATTER EXPERT: THE PATIENT'S PERSPECTIVE

**Shellye Horowitz, MA**

NHF recognizes the unique and important expertise patients have about their disorders. For that reason, we refer to them as subject matter experts (SMEs). This short presentation will provide the SME summit perspective with regard to the health of women, girls and those with the potential for menstruation.

## PLENARY: BIOENERGETIC AND METABOLIC ACTIONS OF ESTROGENS

**Wendy Kohrt, PhD**

Through the discussion of metabolic consequences of the loss of gonadal function, Dr. Kohrt will provide the Bleeding Disorders Community with actionable insights on how the exploration of sex and gender biology might be applied to the study of inheritable bleeding disorders, including the sex and gender differences in other symptoms and response to treatment across the lifespan.

## WORKING GROUP CHAIR SUMMARY

Maureen K. Baldwin, MD, MPH

Angela Weyand, MD

Chairs for Working Group 4: Research Priorities for the Health of Women, Girls and Persons with the Potential to Menstruate discuss their approach and questions identified.

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## BREAK 4:30 PM - 4:45 PM ET

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4:45 PM - 6:15 PM ET

## WORKING GROUP PANEL DISCUSSION

Moderator: Sarah O'Brien, MD, MSc

Maureen K. Baldwin, MD, MPH

Shellye Horowitz, MA

Wendy Kohrt, PhD

Angela Weyand, MD

Live discussion/panel responds to questions and comments from the community generated by the session's plenary speaker and Working Group 4: Research Priorities for the Health of Women, Girls and Persons with the Potential to Menstruate.

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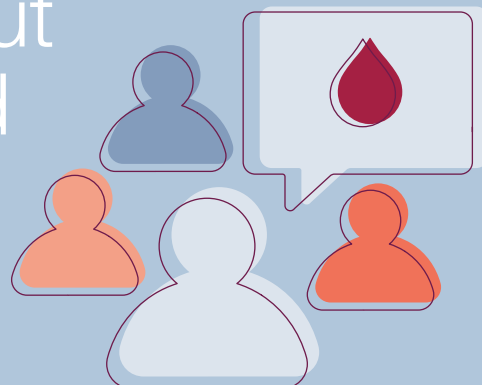
6:15 PM - 6:30 PM ET

## SUMMARY OF THE DAY

Michael Recht, MD, PhD

“

It's exciting to be involved in discussions about research that could benefit my life.”



# Day Three: September 14

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11:00 AM - 11:03 AM ET

## WELCOME

Kevin Mills, PhD

NHF's Chief Scientific Officer, Dr. Kevin Mills, welcomes everyone and explains what to expect in today's morning session.

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11:03 AM - 12:30 PM ET

## SUBJECT MATTER EXPERT: THE PATIENT'S PERSPECTIVE

Esmeralda Vázquez

NHF recognizes the unique and important expertise patients have about their disorders. For that reason, we refer to them as subject matter experts (SMEs). This short presentation will provide the SME summit perspective with regard to diversity, equity and inclusion, health services research and implementation science.

## PLENARY: THE INTERSECTION OF RESEARCH, JUSTICE AND PUBLIC HEALTH

Keri Norris, PhD, JM, MPH, MCHES

This session aims to educate attendees on how research, social justice and public health are interrelated – and how their intersection lends itself to equitable access, care, treatment and outcomes for those in the inherited bleeding/blood disorders community.

## WORKING GROUP CHAIR SUMMARY

Judith Baker, DrPH, MHSA

Tyler Buckner, MD, MSc

Vanessa Byams, DrPH, MPH

Chairs for Working Group 5: Diversity, Equity & Inclusion, Health Services Research, & Implementation Science discuss their approach and questions identified.

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**BREAK** 12:30 PM - 1:00 PM ET

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1:00 PM - 2:30 PM ET

## WORKING GROUP PANEL DISCUSSION

**Moderator:** Melissa Creary, PhD, MPH

Judith Baker, DrPH, MHSA

Tyler Buckner, MD, MSc

Vanessa Byams, DrPH, MPH

Keri Norris, PhD, JM, MPH, MCHES

Esmeralda Vazques

Live discussion with the community about ideas generated by the session's plenary speaker and Working Group 5: Diversity, Equity & Inclusion, Health Services Research, & Implementation Science.

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## BREAK 2:30 PM -3:00 PM ET

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3:00 PM - 4:33 PM ET

## WELCOME

**Kevin Mills, PhD**

NHF's Chief Scientific Officer, Dr. Kevin Mills, welcomes everyone and explains what to expect in today's afternoon session.

## SUBJECT MATTER EXPERT: THE PATIENT'S PERSPECTIVE

**Ziva Mann**

NHF recognizes the unique and important expertise patients have about their disorders. For that reason, we refer to them as subject matter experts (SMEs). This short presentation will provide the SME summit perspective with regard to facilitating priority research in the inherited bleeding disorder community (resource/funding, workforce and infrastructure).

## PLENARY: LEARNING HEALTH SYSTEMS - BRINGING CLINICAL CARE AND RESEARCH TOGETHER THROUGH INFORMATICS

**Charles Bailey, MD, PhD**

The integration of clinical care and research through the use of electronic health records is critical to advancing the standard of care in areas of hematology. This work requires a partnership among the disciplines of epidemiology, health services research and clinical informatics to understand the behavior of EHR data and how to effectively draw valid conclusions from it. Dr. Bailey will discuss the research in pediatric cancer and other rare diseases that has been made possible through the integration of pediatric clinical care and informatics, and how these research tools could advance research in inherited bleeding disorders.

## WORKING GROUP CHAIR SUMMARY

Margaret Ragni, MD, MPH

Jordan Shavit, MD, PhD

Guy Young, MD

Chairs for Working Group 6: Research Priorities for Facilitating Priority Research in the Inherited Bleeding Disorder Community (Resource/Funding, Workforce and Infrastructure) discuss their approach and questions identified.

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## BREAK 4:33 PM - 4:45 PM ET

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4:45 PM - 6:15 PM ET

## WORKING GROUP PANEL DISCUSSION

Moderator: Shannon Carpenter, MD, MS

Charles Bailey, MD, PhD

Ziva Manna

Margaret Ragni, MD, MPH

Jordan Shavit, MD, PhD

Guy Young, MD

Live discussion with the community about ideas generated by the session's plenary speaker and Working Group 6: Research Priorities for Facilitating Priority Research in the Inherited Bleeding Disorder Community (Resource/Funding, Workforce and Infrastructure).

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6:15 PM - 6:30 PM ET

## SUMMARY OF THE DAY

Kevin Mills, PhD

# Day Four: September 15

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11:00 AM - 11:15 AM ET

## WELCOME

Leonard A. Valentino, MD

NHF's CEO and Summit Co-Chair, Dr. Leonard A. Valentino, welcomes everyone to the last session of the State of the Science Research Summit and explains what to expect today.

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11:15 PM - 12:00 PM ET

## WORKSHOP SUMMARY AND NEXT STEPS

Kevin Mills, PhD

Michael Recht, MD, PhD

Leonard A. Valentino, MD

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12:00 PM - 1:45 PM ET

## GENERAL PANEL DISCUSSION

Moderator: Donna DiMichele, MD

Suchitra Acharya, MD, MBBS

Judith Baker, DrPH, MHSA

Maureen K. Baldwin, MD, MPH

Tyler Buckner, MD, MSc

Vanessa Byams, DrPH, MPH

Shannon Carpenter, MD, MS

Veronica Flood, MD

Craig Hooper, PhD

Keith Hoots, MD

Jill Johnsen, MD

Barbara Konkle, MD

Peter Marks, MD, PhD

Kathryn McLaughlin, MPH

Kevin Mills, PhD

Diane Nugent, MD

Sarah O'Brien, MD, MSc

Steven Pipe, MD

Margaret Ragni, MD, MPH

Michael Recht, MD, PhD

Amy Shapiro, MD

Jordan Shavit, MD, PhD

Robert Sidonio, MD, MSc

"Bobby" Duc Q. Tran, MD, MSc

Leonard A. Valentino, MD

Esmeralda Vázquez

Annette von Drygalski, MD, PharmD, RMSK

Angela Weyand, MD

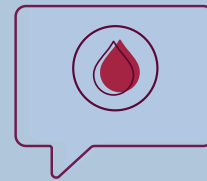
Michelle Witkop, DNP, FNP-BC

Guy Young, MD

1:45 PM - 2:00 PM ET

## ADJOURN

Leonard A. Valentino, MD



“

There are so many opportunities for research to understand more about the experience of living with a bleeding disorder, and to make lives better until we can find a cure.”



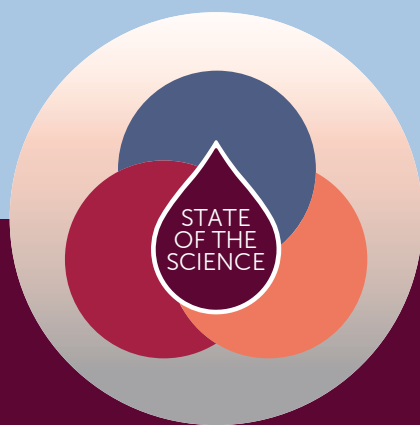
# State of the Science Summit Social Media Handles

Follow along on social media using hashtag #NHFSOS and be sure to connect with your favorite speakers on social media. Don't forget to tag NHF on your favorite platform using @nhf\_hemophilia!

SPEAKER	LINKEDIN	TWITTER
Charles Bailey	<a href="https://www.linkedin.com/in/charles-bailey-66a65216/">https://www.linkedin.com/in/charles-bailey-66a65216/</a>	
Judith Baker	<a href="https://www.linkedin.com/in/judith-r-baker-71186311/">https://www.linkedin.com/in/judith-r-baker-71186311/</a>	
Tyler Buckner		@TBuckTN
Vanessa Byams	<a href="https://www.linkedin.com/in/judith-r-baker-71186311/">https://www.linkedin.com/in/judith-r-baker-71186311/</a>	
Melissa Creary	<a href="https://www.linkedin.com/in/shadowblossom/">https://www.linkedin.com/in/shadowblossom/</a>	@MelissaSCreary
Veronica Flood	<a href="https://www.linkedin.com/in/veronica-flood-01b51283/">https://www.linkedin.com/in/veronica-flood-01b51283/</a>	
Ziva Mann	<a href="https://www.linkedin.com/in/ziva-mann-6239834">https://www.linkedin.com/in/ziva-mann-6239834</a>	@MannZiva
Kevin Mills	<a href="https://www.linkedin.com/in/kevin-mills-20783914/">https://www.linkedin.com/in/kevin-mills-20783914/</a>	
Diane Nugent	<a href="https://www.linkedin.com/in/diane-nugent-b0a7a623/">https://www.linkedin.com/in/diane-nugent-b0a7a623/</a>	
James O'Donnell	<a href="https://www.linkedin.com/in/james-o-donnell-13aaa81a/">https://www.linkedin.com/in/james-o-donnell-13aaa81a/</a>	
Margaret Ragni	<a href="https://www.linkedin.com/in/margaret-ragni-54b1b697/">https://www.linkedin.com/in/margaret-ragni-54b1b697/</a>	@ragnmv
Michael Recht	<a href="https://www.linkedin.com/in/michael-recht-md-phd-mba-268590121/">https://www.linkedin.com/in/michael-recht-md-phd-mba-268590121/</a>	
Amy Shapiro	<a href="https://www.linkedin.com/in/amy-shapiro-7613a9121/">https://www.linkedin.com/in/amy-shapiro-7613a9121/</a>	
Jordan Shavit		@clot1
Robert Sidonio	<a href="https://www.linkedin.com/in/robert-sidonio-md-0556a185/">https://www.linkedin.com/in/robert-sidonio-md-0556a185/</a>	@nashgreenie
Leonard Valentino	<a href="https://www.linkedin.com/in/leonardvalentinomd/">https://www.linkedin.com/in/leonardvalentinomd/</a>	@LenValentino1
Annette von Drygalski	<a href="https://www.linkedin.com/in/annette-von-drygalski-8888499b/">https://www.linkedin.com/in/annette-von-drygalski-8888499b/</a>	
Angela Weyand	<a href="https://www.linkedin.com/in/angela-weyand-229577173/">https://www.linkedin.com/in/angela-weyand-229577173/</a>	@acweyand
Guy Young	<a href="https://www.linkedin.com/in/guy-young-b7438962/">https://www.linkedin.com/in/guy-young-b7438962/</a>	@GuyYoungMD

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**March 7-12 – Washington, D.C.**



# **Bleeding Disorders Conference 2022**

**Aug 25-27 – Houston, Texas**

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