Product Development in Hemophilia Workshop
Speakers-Panelists Biographical Information
December 6, 2018

Speakers and *Panelists*
By order of appearance

Welcome
Opening Remarks

Peter Marks, MD, PhD, Director, Center for Biologics Evaluation and Research (CBER), Acting Associate Director of Patient Outcomes, Oncology Center of Excellence (OCE), FDA
Dr. Peter Marks is the Director of the Center for Biologics Evaluation and Research, FDA. Dr. Marks 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.

Albert Deisseroth, MD, PhD, FDA, Center for Drug Evaluation and Research (CDER)
Dr. Al Deisseroth is best known for the development of new directions in the treatment of leukemias and solid tumors using molecular targeting and genetic therapy. He has also been active in the development of new targeting and genetic modification techniques for the treatment of solid tumors. Following several years of training at the University of Rochester, Harvard University, the NIH and the Dana Farber Cancer Center, Dr. Deisseroth held several academic positions at the NCI as Head of the Experiment Hematology Section, Ensign Professor of Medicine and Chief of Medical Oncology at Yale University School of Medicine, President and CEO of the Sidney Kimmel Cancer Center in San Diego, the Anderson Professor of Cancer Treatment and Research and Chairman of the Department of Hematology at the UT MD Anderson Cancer Center, and Professor of Medicine at UCSF and Chief of the Medical Oncology/Hematology Division at the San Francisco VAMC. He now works at the US FDA as Associate Director of the Division of Hematology Products of the Office of Hematology and Oncology Drug Products.

Jay Lozier, MD, PhD, FDA, CBER
Dr. Jay Lozier is a Medical Officer at the FDA's Clinical Hematology Branch, Office of Tissues and Advanced Therapies, Center for Biologics Evaluation and Research (CBER), FDA. He trained as a Coagulation Fellow and Hematology Fellow at UNC-Chapel Hill. He was supported by the National Hemophilia Foundation on a Judith Graham Pool Fellowship award, and worked in Kathy High's laboratory on the molecular biology of factor IX and hemophilia B. He was a researcher at the National Human Genome Research Institute Gene Therapy Branch from 1995-1999, where he studied factor VIII and factor IX gene transfer and identified a factor VIII gene inversion in the Chapel Hill hemophilia A dog colony like the common intron22 inversion in humans with hemophilia A. He reviewed various hemophilia-related products in the FDA/CBER Office of Blood Research and Review from 2001-2006 and studied the immune response to factor VIII and factor IX proteins in animals. He was the director of the Hematology Consult Service at the NIH Clinical Center and oversaw clinical hemostasis testing in the Department of Laboratory Medicine from 2006-2017. He currently reviews hemophilia gene therapy, CAR T-cell, and related protocols in the FDA/CBER Office of Tissue and Advanced Therapies.

Laurel Menapace, MD, FDA, CDER
Dr. Laurel Menapace is a Medical Officer in the Division of Hematology Products (DHP), Center for Drug Evaluation and Research (CDER) at the FDA and holds an academic appointment at the National Heart, Lung, and Blood Institute (NHLBI) as attending staff in the Sickle Cell Branch. She received her medical degree from the University of Rochester School of Medicine in Rochester, New York and completed Internal Medicine residency at University of Rochester/Strong Memorial Hospital. She continued post-graduate training by pursing a Hematology Oncology fellowship at the Cleveland Clinic, Taussig Cancer Institute in Cleveland, Ohio. During her fellowship, Dr. Menapace focused on the treatment of benign hematologic conditions and researched cancer associated thrombosis (CAT) and novel thromboprophylaxis regimens under the tutelage of Dr. Alok Khorana. She joined the FDA in 2017 and reviews benign hematology drug development. She has particular interest in novel drug development for patients with sickle cell disease (SCD), hemophilia, and thalassemia.

Session 1: Overview of Product Development in Hemophilia
Moderator: Lori Ehrlich (CDER)

Lori Ehrlich, MD, PhD, FDA, CDER
Dr. Lori Ehrlich is a Medical Officer at the FDA's Division of Hematology Products in the Office of Hematology and Oncology Products, Center for Drug Evaluation and Research (CDER). Lori completed her residency and fellowship training as a pediatric hematologist-oncologist at the Children's Hospital of Philadelphia. She received her medical degree and doctorate from the University of Pittsburgh School of Medicine. She holds a clinical appointment in benign hematology at Children's National in Washington, D.C. She joined the FDA in 2014 and reviews benign and malignant hematology drug development with a focus on pediatric drug development.

Margaret Ragni, MD, MPH, University of Pittsburgh Medical Center
Dr. Margaret Ragni is a Professor of Medicine and Clinical Translational Science in the Department of Medicine, Division of Hematology/Oncology, at the University of Pittsburgh, and Medical Director of the Hemophilia Center of Western Pennsylvania in Pittsburgh, PA. She received her MD degree from the University of Pittsburgh School of Medicine and MPH degree from the Graduate School of Public Health, University of Pittsburgh, where she completed medical residency, Hematology/Oncology fellowship, and Coagulation fellowship. Her career has been focused on clinical translational research and novel therapies for congenital disorders of hemostasis. Her studies were among the first multi-center NIH-funded investigator-initiated studies in hemophilia and von Willebrand Disease. She has chaired clinical trials, prospective cohort studies, observational and case-control studies, and investigator-initiated new drug trials in hemophilia. She has authored over 250 peer-reviewed publications and serves as Associate Editor for *Blood Advances*. She is a clinical and research mentor to medical students, residents, fellows, and young faculty, and is the recipient of the Kenneth Brinkhous Research Award (NHF), Kenneth E. Schuit Educator Award (Pitt), G. David Roodman Mentoring Award (UPCI), Murray Thelin Research Award (NHF), the Max Wintrobe Endowed Lectureship (Utah), and the Leadership in Research Award (NHF). She has served on the NHLBI Hemostasis Thrombosis Study Section, the FDA Blood Products Advisory Committee, the NHF Medical & Scientific Advisory Committee, and Co-Chair of Working Group-1 of the recent NHLBI State of the Science Workshop on Inhibitor Eradication.

Session 2: Clinical Endpoints
Moderator: Najat Bouchkouj (CBER)

Najat Bouchkouj, MD, FDA, CBER
Dr. Najat Bouchkouj is a Medical Officer at the FDA's Clinical Hematology Branch, Office of Tissues and Advanced Therapies (OTAT), CBER, FDA. She holds an academic appointment as Assistant Professor at the School of Medicine at Georgetown University and a clinical appointment in oncology at Children's National Health System, D.C. Dr. Bouchkouj received her medical degree from University of Damascus, Faculty of Medicine. She completed her residency in Pediatrics at State University of New York, Downstate Medical Center, and her fellowship at Children's National Medical Center in Pediatric Hematology and Oncology and was a guest researcher at the National Cancer Institute. Prior to Joining FDA in 2016, Dr. Bouchkouj was an attending physician at Medstar Georgetown University Hospital. She currently reviews gene therapy, CAR T cell, and other benign and malignant hematology products in OTAT, and she also serves as OTAT's patient voice representative.

Robert Montgomery, MD, Medical College of Wisconsin, Children's Hospital of Wisconsin
Dr. Robert R. (Bob) Montgomery is a physician scientist who has studied von Willebrand factor (VWF) and its relationship with Factor VIII (FVIII). He is a Senior Investigator at the Blood Research Institute at the Blood Center of Wisconsin and Professor of Pediatric Hematology at the Medical College of Wisconsin and Children's Hospital of Wisconsin in Milwaukee. He has NHLBI funding for studies on the molecular and clinical biology of VWF and has studied the relationships between VWF and FVIII in plasma, cellular synthesis, and cell trafficking. In mice his group demonstrated using tissue-specific knockouts that plasma FVIII could be abrogated by eliminating FVIII synthesis in endothelial cells. He helped develop the hemophilia gene therapy approach using ectopic platelet expression of FVIII by lentiviral FVIII transduction of hematopoietic stem cells under the control of the platelet-specific integrin αIIb promoter. In mice, rats, and dogs, this gene therapy approach is efficacious even in the face of high titer plasma FVIII inhibitors. The FVIII produced is present only in platelet storage granules, absent in plasma, but delivered to the site of vascular injury where the activated platelet releases FVIII thereby promoting "normal" hemostasis.

Marilyn Manco-Johnson, MD, University of Colorado, Children's Hospital of Colorado
Dr. Marilyn Manco-Johnson is the Director, Hemophilia & Thrombosis Center, University of Colorado, Children's Hospital Colorado in Denver, CO. A researcher in pediatric hemostasis and thrombosis with a passion for patient care, Dr. Manco-Johnson is well known for her pioneering work in the Joint Outcomes Study which led to a dramatic change in clinical practices in the United States toward adoption of primary prophylaxis therapy in children to prevent long-term joint damage. She contributes significantly to pediatric thrombosis, including thrombolytic therapies, global assays of hemostasis, thrombotic outcomes and rare pediatric thrombotic disorders.

Poornima Sharma, MD, FDA, CBER
Dr. Poornima Sharma is a Medical Officer and a hematologist oncologist at the FDA' s Clinical Hematology Branch, Office of Tissues and Advanced Therapies, Center for Biologics Evaluation and Research (CBER). Dr. Sharma received her MBBS at Lady Hardinge Medical College, University of Delhi, India. She completed her residency in Internal Medicine at NYU Downtown hospital, New York. Subsequently, Dr. Sharma completed her fellowship in Hematology and Oncology at University of Cincinnati, Ohio. Prior to Joining the FDA, Dr. Sharma worked as consultant physician at Tate Cancer Center, UM Baltimore-Washington Medical Center and Clinical assistant professor, University of Maryland. She continues to hold her clinical and academic appointments.

Session 3: Overview of Patient-Reported Outcomes (PROs)
Moderator: Laurel Menapace (CDER)

Elektra Papadopoulos, MD, MPH, FDA, CDER
Dr. Elektra Papadopoulos serves as the Associate Director of the Clinical Outcome Assessments Staff in the Office of New Drugs in the Center for Drug Evaluation and Research (CDER). The staff provides consultation to CDER's Review Divisions as well as other FDA Centers on clinical outcome assessments (COAs) regarding their development, validation, interpretation and overall suitability to support labeling claims and manages the COA drug development qualification program.

Christine Kempton, MD, MSc, Emory School of Medicine
Dr. Christine Kempton is an Associate Professor in the Department of Hematology and Medical Oncology at Emory University School of Medicine where she is the Director of the Hemophilia of Georgia Center for Bleeding and Clotting Disorders of Emory. She also serves as the Regional Medical Director for the Southeastern Region of the Hemophilia Treatment Center Network. Her clinic and research focus is on hemophilia and its complications.

Christopher Templin, Hemophilia Patient Advocate
Christopher M. Templin has been active in hemophilia education, advocacy and outreach since the age of 4, when the only form of treatment for his Hemophilia B was whole blood and fresh frozen plasma, RICE and medically ordered bed rest. Over the years, available therapies for hemophilia improved and the demand for advocacy increased due to costs of treatment and when patients in the community were exposed to HIV and HCV via plasma-derived clotting factors and blood products. Mr. Templin has been a guest speaker and presenter at various regional, national and international conferences within the bleeding disorders community. He is more motivated than ever to make sure female patients with bleeding disorders get proper medical care from hemophilia treatment centers, since the birth of his daughter, Allyson, 8 years ago, who was diagnosed with mild Hemophilia B. He is determined to ensure the insurance industry follows all local, state and federal laws and continues to provide the lifesaving and life sustaining medications needed by the members of the bleeding disorder community.

Speakers and *Panelists*

Shelby Smoak, Hemophilia Patient Advocate
Shelby Smoak is a writer and musician. His book Bleeder: A Memoir (Michigan State University Press) received praise from sources as diverse as The Minneapolis Star Tribune, Library Journal, and Glamour, and has won several awards, including "Best of the Best" by the American Library Association. Shelby has been playing and touring with his music since the 1990s. He has released several albums and been involved in numerous projects. More recently, he formed indie-rock band Bleeder which released their debut album in June 2017. Awarded a Pen/American grant for writers living with HIV, Smoak holds a Ph.D. in Literature and an M.A. in English. He lives, writes, and plays music in the Shenandoah Mountains of Virginia.

George Stone, Hemophilia Patient Advocate
George M. Stone, Jr. is an independent hemophilia patient. Mr. Stone is 65 years old and has severe Hemophilia A. He is a retired federal government employee who worked for Department of the Interior Agencies. He has served as an advocate in the Hemophilia community in various capacities. He is considered to be a long-term survivor of HIV and HCV (cured) acquired from plasma-derived clotting factor products in the 1980s. Mr. Stone recently completed Genentech's HAVEN 3 study for HEMLIBRA® where he had bleed-free results over 18 months. He has experienced the full panoply of severe hemophilia problems and treatments, including five joint replacements. He holds a Master of Public Administration degree from the George Washington University and a Bachelor of Politics degree from Fairfield University. He resides in Shenandoah Valley, Virginia with his wife, Cyndi.

Miriam Goldstein, Hemophilia Patient Advocate
Miriam Goldstein lives in Arlington, Virginia, and is the mother of two adult sons with hemophilia. Miriam is an Associate Director for Policy at Hemophilia Federation of America (HFA), where her work includes monitoring and analyzing federal legislation and regulations impacting patient access to care; insurance, Medicaid, and Medicare issues; blood safety; and more. Before joining HFA, Miriam worked as a lawyer with the federal government and volunteered for many years with her local hemophilia association and the Committee of Ten Thousand. Miriam graduated from Yale University (B.A.) and University of Pennsylvania (J.D.). While Miriam is a staff member of HFA, she is participating in this workshop in her personal capacity as a member of the bleeding disorders community and as such, is speaking on her own behalf. Her comments today express her own opinions and do not necessarily reflect HFA's positions or views.

Christine Guelcher, RN-BC, MS, PPCNP-BC, Children's National Health System
Chris Guelcher has been a hematology/oncology clinical nurse for more than 25 years. After graduating from Georgetown University with a Bachelor of Science in Nursing she worked at Children's Memorial Hospital in Chicago, Illinois and later at Mott Children's Hospital in Ann Arbor, Michigan. Since coming to Children's National in 1992, she has worked on the inpatient unit and in the outpatient clinic as a Hematology/Oncology nurse. After completing her Master's and becoming certified as a Pediatric Nurse Practitioner, she has been the Hemophilia Nurse Coordinator at CNMC since 1997. Chris was promoted to Lead Advanced Practice Provider within the Center for Cancer and Blood Disorders at Children's National in 2017.

Virginia Kwitkowski, MS, ACNP-BC, FDA, CDER
Virginia Kwitkowski is the Associate Director for Labeling in the Division of Hematology Products at CDER/FDA. In this role, she advises review team members and division leadership on methods for developing clear, meaningful, and scientifically accurate prescription drug labeling that conforms to regulations, guidance, and policies. She is also a Patient Reported Outcomes lead for the Division of Hematology Products. Ms. Kwitkowski completed her Master of Science degree at the University of Maryland Graduate Program with a certification as an Acute Care Nurse Practitioner-Oncology.

Bellinda King-Kallimanis, PhD, FDA, CDER
Dr. Bellinda King-Kallimanis is a psychometrician working in the Office of Hematology and Oncology Products. She provides support to the three oncology divisions with respect to clinical outcome assessments (COAs) as well as works on advancing science with respect to understanding how current COA strategies in cancer clinical trials can be improved. Bellinda has been working in the field of COAs for the past 10 years across both academia and industry.

Session 4: Factor Activity as a Surrogate Endpoint
Moderator: Mikhail Ovanesov (CBER)

Mikhail Ovanesov, PhD, FDA, CBER
Dr. Ovanesov is a Research Biologist and Principal Investigator in the Office of Tissues and Advanced Therapies at CBER, FDA. He graduated from the Moscow Institute of Physics and Technology and received his PhD degree in Biology from the Russian Academy of Medical Sciences. Prior to joining the FDA, Dr. Ovanesov completed postdoctoral fellowships with the American Red Cross in Rockville, MD, Johns Hopkins University Medical School in Baltimore, MD, and Janelia Farm Research Campus of the Howard Hughes Medical Institutes in Ashburn, VA. Dr. Ovanesov participates in the review of plasma-derived and recombinant plasma protein concentrates, transfusion products including frozen and dried plasma and platelets, and gene therapies for hemophilia. His research laboratory investigates the mechanisms of action of procoagulant therapeutics and participates in international collaborative studies on international standards for plasma protein activity.

Elaine Gray, PhD, The National Institute for Biological Standards and Control
Dr. Elaine Gray is a Principal Scientist with over 35 years of experience in control and standardization of biological, specializing in hemostasis and thrombosis. She worked at the National Institute for Biological Standards and Control (NIBSC) as a pre-graduate student and completed her PhD at NIBSC. Her current main responsibilities are batch control, standardization and research and development in hemostasis and related areas. Dr. Gray serves on many national and international committees both as Chair and as a member. She has extensive expertise in the standardization of blood products, being responsible for the production of World Health Organization (WHO) and British standards for coagulation factors II, IX, X, XI; plasma coagulation inhibitors such as Antithrombin and protein C and antithrombotics such as unfractionated and low molecular weight heparin. She is one of NIBSC Genetic Reference Materials team members producing reference materials for in vitro diagnosis of genetic disorders.

Steven W. Pipe, MD, University of Michigan School of Medicine
Dr. Steven Pipe is a Professor and the Laurence A. Boxer Research Professor of Pediatrics and Communicable Diseases, as well as Professor of Pathology at the University of Michigan, Ann Arbor, Michigan, USA. He is the medical director of the Pediatric Hemophilia and Coagulation Disorders Program and medical director of the Special Coagulation Laboratory at the University of Michigan. His

clinical interests include bleeding and thrombotic disorders and congenital vascular anomalies. Dr. Pipe also directs a basic research lab investigating coagulation factor VIII and the molecular mechanisms of hemophilia A. Dr. Pipe completed his undergraduate medical training at the University of Toronto, Ontario, Canada, and his pediatric training at McMaster University in Hamilton, Ontario. His pediatric hematology and oncology training was completed at the University of Michigan. He was a fellow of the Pediatric Scientist Development Program. Dr. Pipe is certified by the American Board of Pediatrics in pediatric hematology/oncology. He was awarded a Young Investigator Award from both the American Society of Pediatric Hematology and Oncology and the International Society on Thrombosis and Haemostasis, and a Faculty Scholar Award from the American Society of Hematology. He was the 2015 recipient of the Leadership in Research Award from the National Hemophilia Foundation. He has 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 the Chair of the Medical and Scientific Advisory Committee to the National Hemophilia Foundation.

Kenneth D. Friedman, MD, Medical College of Wisconsin

Dr. Kenneth Friedman is trained in Internal Medicine, adult hematology and transfusion medicine. He serves as medical director of the Hemostasis Reference Laboratory at BloodCenter of Wisconsin, recently renamed a part of Versiti, and is professor of medicine at the Medical College of Wisconsin. The Hemostasis Reference Laboratory that he directs supports a local hemophilia treatment center and has participated in field studies evaluating several of the newly licensed hemophilia treatment products. The lab also provides central lab services for the Zimmerman Program evaluating the clinical and molecular biology of von Willebrand disease. Dr. Friedman's patient care responsibilities include participation in the care of patients with hemophilia at two treatment centers in Wisconsin, and in providing consultation in benign hematology at the hospitals associated with the Medical College of Wisconsin. His research interests are related to laboratory evaluation of patients with bleeding and thrombotic disorders, focused mainly upon von Willebrand disease and thrombotic microangiopathies.

Johannes Dodt, PhD, Paul-Ehrlich-Institut, Langen, Germany

Dr. Johannes Dodt is Head of Section, Blood Coagulation Products II in the Department of Hematology and Transfusion Medicine of the Paul-Ehrlich-Institut, the Federal Institute for Vaccines and Biomedicines in Germany. He received a MS and PhD in Chemistry from the Ludwig-Maximilians-University in Munich. He continued as a research group leader in the Biochemistry Faculty of the Technical University Darmstadt, where he received his teaching license for biochemistry. His research focused on structure function relationship of proteinases and proteinase inhibitors. After working as a senior fellow at Georgetown University School of Medicine in Washington DC and at the University College London Medical School in London, he joined the Paul-Ehrlich-Institut in 1995. Amongst others, the section Blood Coagulation Products II is responsible for the evaluation of the quality aspects of coagulation products. Johannes Dodt is the current chairmen of the Expert Group 6B (blood products) of the European Pharmacopoeia.

Richard Marlar, PhD, University of New Mexico

Dr. Marlar is Director of the Special Coagulation Laboratory and Routine Coagulation Laboratories (16 hospital labs) at TriCore Reference Laboratories in Albuquerque, New Mexico. He is also Professor of Pathology at the University of New Mexico Health Sciences Center. He has been directing Special Coagulation and Routine Coagulation Laboratories for over 37 years, including establishing 3 new Special Coagulation Laboratories at three different institutions. He has served as Chairholder of the CLSI Expert Committee on Hematology, Coagulation and Immunoassays. He is chairman of the international

consortium of national EQA organizations (EQATH, External Quality Assurance in Thrombosis and Hemostasis). He is currently the Editor-in-Chief of the international journal Blood Coagulation and Fibrinolysis. He serves as director for Proficiency Testing and Quality Assurance for the North American Special Coagulation Laboratory Association. He just completed 4 years as chairman of the Plasma Coagulation Inhibitors Subcommittee of the International Society of Thrombosis and Hemostasis. His research focuses on laboratory coagulation method development and validation procedures and writing guidelines for clinical laboratory testing. He also conducts research on the genetics of venous thrombosis and thrombophilia with special interest in protein C deficiencies. He is the author of over 180 articles, book chapters and monographs. He received his PhD from Wayne State University in Detroit, Michigan in Biochemistry and Physiology; and completed a Fellowship at the Scripps Clinic and Research Foundation in Blood Coagulation.

Session 5: Clinical Trial Design
Moderator: Jay Lozier (CBER)

Amy Shapiro, MD, Indiana Hemophilia and Thrombosis Center

Dr. Amy Shapiro is the Medical Director and CEO of the Indiana Hemophilia and Thrombosis Center in Indianapolis and Adjunct Professor of Pediatrics at Michigan State University in East Lansing. 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.

An author or co-author of more than 300 journal articles, abstracts, and textbook chapters, Dr. Shapiro is clinically focused on improving treatment for people with rare bleeding disorders. She has served on the National Hemophilia Foundation's Medical and Scientific Advisory Council and as well as several boards for the National Institutes of Health in Data Safety Monitoring and Clinical Trial Review. 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. Dr. Shapiro has been honored as the National Hemophilia Foundation Physician of the Year Award and most recently received their Leadership in Research Award. Among other accomplishments, she has also received the Distinguished Hoosier Award in Indiana.

Stacey Huppert, PhD, Cincinnati Children's Hospital Medical Center

Dr. Stacey S. Huppert, is an Associate Professor of Gastroenterology, Hepatology and Nutrition at Cincinnati Children's Hospital Medical Center and the University of Cincinnati College of Medicine. Dr. Huppert's research program explores how intercellular signaling pathways are integrated to coordinate cell fate decisions and allocate cell types during both organogenesis and regeneration. One aspect of her research program is specifically focused on hepatic cell plasticity, commitment, and the therapeutic potential of differentiated hepatocytes to augment or regenerate truncated or damaged intrahepatic bile duct systems. Dr. Huppert's work has established a three-dimensional blueprint for the formation of intrahepatic bile duct architecture. A second area of focus is the molecular regulation of hepatocyte differentiation via transcriptional networks and epigenetic landscape. The etiology of pediatric liver toxicity and ability of liver to heal itself in the face of pediatric acute liver failure are unknown in many cases. Specified hepatocytes undergo a process of postnatal differentiation where they adopt the physiological functions and morphology associated with the adult liver. It is unclear how transcription factors, expressed in both embryonic and adult liver, coordinate the gene expression changes that are necessary for organ formation and mature hepatocyte cell function. Notch signaling is a third focus of Dr. Huppert and one pathway that holds promise as a therapeutic target for treatment of many cancers

and congenital diseases. Her work, demonstrating that proper cell fate decisions stem from Notch signaling imparting feedback regulation of ligand and receptor asymmetric expression, continues to be a central concept in canonical Notch signaling. Dr. Huppert combines cellular and biochemical screens with advanced methods to quantify three-dimensional changes of in vivo vascular and epithelial structures enabling the search for targets that fine-tune regulation of Notch pathway activity.

Mark Sands, PhD, Washington University School of Medicine
Dr. Mark Sands received his Bachelor of Science from Rochester Institute of Technology, Rochester NY in Nuclear Medicine and in 1990 he received his PhD in Molecular Pharmacology at State University of New York at Stony Brook. While at SUNY – Stony Brook he studied the interactions of transcription factor IIIA with the 5S rRNA gene and the gene product. He then performed a post-doctoral fellowship at The Jackson Laboratory in Bar Harbor, ME where he characterized the murine model Mucopolysaccharidosis Type VII. He then spent one year at the University of Pennsylvania, School of Veterinary Medicine developing gene transfer vectors for lysosomal storage diseases. In 1994 he joined the faculty of Washington University School of Medicine as an independent investigator. The goals of his laboratory are to better understand the underlying pathophysiology and develop effective therapies for lysosomal storage diseases, in particular, mucopolysaccharidoses, Krabbe disease and infantile Batten disease.

Theo Heller, MD, National Institute of Health
Dr. Theo Heller, M.D. is Chief of the Translational Hepatology Section, Liver Diseases Branch, NIDDK at the National Institutes of Health. He received an M.D. degree from the University of the Witwatersrand, Johannesburg, South Africa and then underwent residency training in Internal Medicine At Georgetown University Hospital. He was a postdoctoral fellow in the laboratory of Dr. Robert Purcell at the NIH after residency and then did his Gastroenterology fellowship at the University of Maryland. His Hepatology Fellowship was in the Liver Diseases Branch at the NIH where he has since stayed on as faculty. Dr. Heller's scientific interests focus on clinical work in humans and include factors causing the progression of liver disease, rare liver diseases, the gut-liver axis, and the microbiome. He has published over 140 scientific papers. He has received multiple awards for mentoring and teaching including the Hugh H. Hussey Award from Georgetown University Hospital in 1993, certificates of recognition for education of postbaccalaureate trainees in 2008 and 2010, the NIDDK Nancy Nossal Mentorship Award in 2008 and 2012, the NIDDK "You Make A Difference" Award in 2010, and the faculty teacher of the year award, Department of Medicine, Division of Gastroenterology, University of Maryland School of Medicine in 2014. In addition, he has been honored with an NIH Plain Language Award in 2010, NIDDK Early Career Investigator Award by the sponsors of the Congressional Breakfast to honor the 60th anniversary of NIDDK in 2010, Fellow of the American Association for the Study of Liver Disease in 2017, and a NIAID Merit Award in 2017. He has received multiple bench to bedside awards from the Clinical Center in 2011, 2012, 2015 (as principal investigator), and 2016. He currently serves as an editorial board member for *Gastroenterology*, and *Hepatology*.

Bindu George, MD, FDA, CBER
Dr. George is the Chief of the Clinical Hematology Branch (CHB) at the Office of Tissues and Advanced Therapies (OTAT) at CBER. She received her MBBS at Stanley Medical College, Tamil Nadu Dr. M.G.R. Medical University India. She completed her residency in Internal Medicine at Harbor Hospital Center, Baltimore, Maryland. Subsequently, she completed her fellowship in Hematology and Oncology at University of Maryland, Baltimore. Dr. George joined the FDA in 2007 as a medical officer, served as team leader since 2009, and subsequently as branch chief since 2015. She is responsible for supervisory oversight of clinical review of cell and gene therapy products for malignant and non-malignant hematological diseases.

Session 6: Closing Session

Ann Farrell, MD, FDA, CDER
Dr. Ann Farrell is a hematologist, supervisory medical officer, and Division Director for the Division of Hematology Products, Office of Hematology and Oncology Drug Products, Center for Drug Evaluation and Research, FDA. Her division reviews hematology therapies for benign disorders and malignancies. Prior to her FDA career, Dr. Farrell was an Assistant Professor of Medicine in the Division of Hematology/Oncology at the University of Massachusetts in Worcester, Massachusetts. She received her Bachelor of Arts from the University of Chicago and her medical degree from Rush Medical College in Chicago. She completed her internship and residency at a Yale program, the Hospital of St. Raphael in New Haven, CT. She completed her hematology/oncology fellowship at the University of Massachusetts.