

Strategic Plan and Blueprint for Addressing Thalassemia in the United States

Committee

Jewel Mullen

Chair

Jewel Mullen is the founding Associate Dean for health equity at the University of Texas at Austin Dell Medical School, as well as an Associate Professor in the Departments of Population Health and Internal Medicine. An internist, epidemiologist, and public health expert, Dr. Mullen formerly served as the principal deputy assistant secretary for health in the U.S. Department of Health and Human Services and commissioner of the Connecticut Department of Public Health. She has held faculty appointments at the New York University, University of Virginia, Yale University and Tufts University schools of medicine. Board-certified in internal medicine, Dr. Mullen received her bachelor's degree and Master of Public Health from Yale University, where she also completed a post-doctoral fellowship in psychosocial epidemiology. She graduated from the Mount Sinai School of Medicine, where she was elected to Alpha Omega Alpha national medical honor society, and completed her residency at the Hospital of the University of Pennsylvania. She also holds a Master of Public Administration from Harvard University's John F. Kennedy School of Government. Dr. Mullen has served on numerous National Academies' study committees and currently chairs the Committee on Newborn Screening: Current Landscape and Future Directions.

Mei Baker

Member

Mei Baker is a Professor in the Division of Genetics and Metabolism in the Department of Pediatrics at the University of Wisconsin School of Medicine and Public Health. She is also the Director of the Newborn Screening Program at the Wisconsin State Laboratory of Hygiene. Dr. Baker practiced medicine before being trained in both biochemical and molecular genetics, obtaining a clinical biochemical genetics certification from the American Board of Medical Genetics and Genomics in 2009. She has 20 years of experience in routine newborn screening (NBS) with specific interest in, and a successful track record of, applying emerging technologies to implement new screening tests for disorders and to improve ongoing screening tests. Dr. Baker served as a member of the Department of Health and Human Services' Advisory Committee on Heritable Disorders in Newborns and Children from 2016 to 2021. Dr. Baker received her MD from Anhui Medical University. Currently, she is a member of the National Academies' consensus study committee on Newborn Screening: Current Landscape and Future Directions. Dr. Baker's work has led to one patent involving DNA extraction from dried blood samples and one patent application for a newborn screening assay, with one patent currently licensed by QuantaBio. She has also received research funding from Ultragenyx from 2022 to 2024 to develop an assay for Angelman syndrome screening that could be integrated into current state public health newborn screening programs.

Anirban Basu

Member

Anirban Basu is a Professor of Health Economics and the Stergachis Family Endowed Director of The CHOICE Institute at the University of Washington, Seattle, with joint appointments in the Departments of Health Systems & Population Health and Economics. He is a Research Associate at the US National Bureau of Economic Research and an elected Fellow at the American Statistical Association. His research focuses on understanding the economic value of health care, generating causal evidence, and in mitigating discrimination with machine learning and artificial intelligence algorithms. From 2018-2024, Dr. Basu co-led the NHLBI's Cure Sickle Cell Consortium on Economic Impact and Analysis. He has served on the 2nd Panel on Cost-effectiveness Analysis in Health and Medicine and currently serves on the Editorial Advisory Board of Value in Health Journal. In 2024, he served as a member of the Advisory Council on Financing and Access to Sickle Cell Disease Treatment and Other High-Cost Drugs & Treatment for the Office of the Governor of Illinois. Dr. Basu is a recipient of many academic honors, including the 2018 Mid-Career Excellence Award from the Health Policy Statistics Section of the American Statistical Association, the 2007 and 2016 Research Excellence Award for Methodological Excellence, and the 2009 Bernie O'Brien New Investigator Award from the International Society for Pharmacoeconomics and Outcomes Research. He received his MS in Biostatistics from UNC-Chapel Hill and a PhD in Public Policy (Health Economics) from The University of Chicago. Dr. Basu is also the founder of Salutis Consulting, LLC, which provided services for Novartis in 2020 and Merck in 2023.

Michael A. Bender

Member

Michael A. Bender (known as Bender) is a pediatric hematologist and basic scientist with a lifelong commitment to all aspects of hemoglobinopathies. He is currently Professor Emeritus at the University of Washington and remains active in special projects. He previously ran the Odessa Brown / Seattle Children's Hospital comprehensive hemoglobinopathy program and a basic science laboratory at the Fred Hutchinson Cancer Center. Clinically, Dr. Bender has a long-standing commitment to red blood cell disorders with emphasis on patient and provider education, immigrant populations, community outreach and optimizing access to respectful health care. A current focus has been on newborn screening with emphasis on increasing health equity, access to receiving a definitive diagnosis and improving public health. His research has focused on gene therapy, novel agents to treat hemoglobinopathies, and the regulation of beta-globin chromatin structure and gene expression. He received the American Society of Hematology's Champion for Advocacy award, was on the NIH Sickle Cell Disease Advisory Committee, is on the CDC / Association of Public Health Laboratories (APHL) Hemoglobinopathy Work Group and is involved in Western States Collaboratives to improve hemoglobinopathy care. Dr. Bender did a combined MD-PhD focused on gene therapy for hemoglobinopathies at the University of Washington, followed by pediatrics and hematology/oncology.

Alexander Boucher

Member

Alexander Boucher is an Associate Professor of hematology at the University of Minnesota Medical School. He is Program Director of the pediatric hematology/oncology fellowship and Associate Program Director for the adult hematology, oncology, and transplantation fellowship, where he leads the classical hematology track. He has joint appointments within the Departments of Pediatrics and Medicine and leads the University's Sickle Cell Program across the lifespan. His academic and clinical efforts are focused on optimizing care for individuals with red blood cell disorders with particular emphasis on transitions from pediatric to adult care for people with hemoglobinopathies as well as identifying and mitigating sources of bias and health inequities within healthcare systems. Dr. Boucher has mentored several trainees across multiple specialties and has had national leadership roles within educational committees at the University and within the American Society of Hematology, where he is a longstanding member. He is a member of the Minnesota Newborn Screening Advisory Committee (2024-2027) and the ASH Sickle Cell Disease (SCD) Task Force (2025-2026), which is working to develop SCD initiatives for the next 5 years. Dr. Boucher has received awards as a clinical educator within both departments and as a health equity champion within pediatrics. He received his MD from the University of Tennessee Health Science Center, completed a combined medicine-pediatrics residency at the University of Minnesota, and later a pediatric hematology-oncology fellowship at Cincinnati Children's Hospital Medical Center. Dr. Boucher was a site principal investigator for clinical trials on hemopexin for Sickle Cell Disease in 2019-2025 with CSL Behring and on Adzynma for chronic thrombotic thrombocytopenic purpura (cTTP) in 2023-2024 with Takeda Pharmaceutical Company.

Sean P. David

Member

Sean P. David is the Endeavor Health Site Leader for the Institute for Translational Medicine (CTSA), Program Director for Translational Science for the Health Outcomes Research Collaborative, and Vice Chair for Research for the Department of Family Medicine at Endeavor Health and the University of Chicago. He is a clinical and translational physician scientist with interests in alcohol and nicotine genomic discovery science, pharmacogenomics, precision medicine, tobacco cessation, implementation science, community-engaged research, and health equity. His work is presently using AI and implementation science frameworks to evaluate genomic and pharmacogenomic screening in primary care and population health. He is the recipient of the American Board of Family Medicine Puffer Fellowship with the National Academy of Medicine. Dr. David received his MD at the University of Washington, completed his residency in family medicine at Dartmouth-Hitchcock Medical Center, received his master's (SM) in public health from the Harvard School of Public Health, and his doctorate (DPhil) in pharmacology from the University of Oxford. He also took part in the Harvard Business School Program for Leadership Development and the Design Thinking Bootcamp at Stanford University's d.school. Dr. David previously served on the National Academies' Consensus Study Committee on the Evidence Base for Genetic Testing.

Lee A. Fleisher

Member

Lee A. Fleisher (NAM) is Professor Emeritus of Anesthesiology and Critical Care at the University of Pennsylvania Perelman School of Medicine and continues to practice clinically. He is also the Founding Principal and CEO at Rubrum Advising, LLC. He serves as a Senior Advisor of the Bipartisan Policy Center and FasterCures of the Milken Institute, and Visiting Fellow of the Duke-Margolis Center. He is a member of the Health Information Technology Advisory Committee (HITAC) of the Office of the National Coordinator for Health Information Technology (ONC). From July 2020-July 2023, he was the Chief Medical Officer and Director of the Center for Clinical Standards and Quality for the Centers for Medicare and Medicaid Services. From 2004 through July 2020, he was the Robert D. Dripps Professor and Chair of Anesthesiology and Critical Care and Professor of Medicine at the University of Pennsylvania. In 2007, he was elected as a member of the National Academy of Medicine (NAM). Dr. Fleisher received his MD from the State University of New York at Stony Brook and an ML from the University of Pennsylvania Law School.

Ellen Fung

Member

Ellen Fung is an Adjunct Professor in the Division of Hematology, Department of Pediatrics within the School of Medicine at the University of California, San Francisco (UCSF). She is also the Director of the Bone Density Clinic at UCSF Benioff Children's Hospital Oakland and trains medical staff in assessment and interpretation of bone health examinations. She has a long-standing interest on the impact of nutrition and physical activity on bone health in children and adults, particularly those with hemoglobin disorders including thalassemia and sickle cell disease. She has published over 100 research articles and book chapters and is co-author of the first textbook related to the assessment of pediatric bone health, *Bone Health Assessment in Pediatrics: Guidelines for Clinical Practice* as well as first text on *Nutritional Guidelines for Patients with Thalassemia*, published in 2023 by the Thalassemia International Federation. In 2024, Dr. Fung was presented with the Career Achievement Award by the UCSF Benioff Children's Hospital Oakland. She holds a doctorate in nutritional science from the University of California, Berkeley, is a Registered Dietitian with the Academy of Nutrition and Dietetics and is a Certified Clinical Densitometrist with the International Society for Clinical Densitometry.

Cindy Gillespie

Member

Cindy Gillespie served as Secretary of the Arkansas Department of Human Services from March 2016 to October 2022. As secretary, she led the state's health, social services, and children and family programs, including Medicaid, behavioral health, aging and special populations, child welfare, juvenile justice, as well as childcare and early childhood education. She also oversaw the state's psychiatric hospital, nursing home, and five human development centers for the developmentally disabled. For the past twenty years, Ms. Gillespie has focused on health policy and innovations in coverage and insurance models. Prior to her appointment as Secretary, she was a principal in the Washington office of the multinational law firm Dentons US, LLP where she led the firm's Health Policy and Health Insurance Exchange teams. Before joining the firm, Ms. Gillespie served as a senior advisor to Governor Mitt Romney of Massachusetts overseeing executive branch initiatives and helping develop the state's health reforms. She has served on boards overseeing state government employee health plans, a health insurance marketplace, and the National Academies' Board on Health Care Services. Ms. Gillespie is a graduate of Trevecca Nazarene University and has an MA in Organizational Communications from Auburn University. She recently served as a member of the National Academies' committee on strategies to improve access to behavioral health services in Medicaid, Medicare and Marketplace plans. She currently serves as a strategic advisor to BioInsite on state child welfare and long-term care guardianship programs and to Gentreo on health care and insurance programs.

Aaron Goldenberg

Member

Aaron Goldenberg is a Professor and Vice-Chair in the Department of Bioethics at Case Western Reserve University (CWRU). He is also Director of the CWRU Bioethics Center for Health and Genomics (CHANGE). He has a background in bioethics, health behavior/health education, public health ethics, and public health genetics, and has expertise in qualitative and quantitative research design and analysis. His work has focused on the ethical, legal, and social issues (ELSI) associated with research and clinical care for rare diseases and the integration of new genomic technologies into research, clinical and public health settings. A major interest is on increasing community voices in the considerations of the ethical and social implications of expanded genetic and genomic screening in newborns and children – especially addressing how genetic information may change care decisions and impact the diagnostic and intervention odysseys of families. Dr. Goldenberg is currently the co-chair for the Ethical, Legal, Social & Policy Implications (ELSPI) sub-committee of the Association of Public Health Laboratories (APHL) and sits on APHL's steering committee for their NewSTEPS newborn screening resource center. He received his MA and PhD in bioethics from Case Western Reserve University and his MPH from the University of Michigan. He has previously served as a member of the National Academies' Planning Committee for a Workshop on Exploring the Current Landscape of Consumer Genomics and Workshop on Next-Generation Newborn Screening - The Promise and Perils of DNA Sequencing of Newborns at Birth.

Mathew Kiang

Member

Mathew Kiang is an assistant professor in the Department of Epidemiology and Population Health at Stanford University School of Medicine. He is a computational social scientist and social epidemiologist whose research focuses on improving health in small or hard to reach populations using a range of quantitative methods such as Bayesian modeling, microsimulation, and demographic models. Dr. Kiang received his doctorate in quantitative methods and social epidemiology from the Harvard TH Chan School of Public Health in the Department of Social and Behavioral Sciences and his MPH from New York University. Before becoming an assistant professor, Dr. Kiang received his postdoctoral training at Stanford University, a fellowship in the Data Science for Social Good program at the University of Chicago, and a visiting fellowship at the Harvard FXB Center for Health and Human Rights. He recently served on the National Academies' consensus study committee on Evaluating the Effects of Opioids and Benzodiazepines on All-Cause Mortality in Veterans.

Radhika Sawh

Member

Radhika Sawh is a board-certified genetic counselor based in New York State, currently working as part of the team at the Long Island University Genetic Counseling Graduate Program as the Clinical Supervisor for their Simulation Lab. She has enjoyed a diverse career, which included clinical genetic counseling at a high-volume prenatal center at a community hospital in Queens, NY, educational and advocacy driven efforts as the National Director of Patient Services for the Cooley's Anemia Foundation, nearly ten years on the faculty of the Joan H. Marks Graduate Program in Human Genetics at Sarah Lawrence College, and work on the federally funded NYMAC Region Genetics Network grant which focused on expanding access to genetic services in underserved communities. Radhika was diagnosed at birth with transfusion dependent thalassemia major and has woven her personal experience and professional interest in thalassemia throughout her career and research, including invited lectures, presentations and research on thalassemia and other blood disorders. She has spoken several times at the annual Cooley's Anemia Foundation Patient-Family conference and participated in the recent revisions of the American College of Medical Genetics and Genomics (ACMG) Hemoglobinopathies ACT Sheets. She earned her MS in Human Genetics from Sarah Lawrence College in 2001 and a dual bachelor's degree in biology and psychology from Boston College in 1997. Radhika is an active member of the Cooley's Anemia Foundation Board of Directors and Executive Committee and currently serves as the Director at Large for the Community Outreach Committee of the NYS Genetics Task Force. She has participated in Vertex's Transfusion Dependent Thalassemia Patient Advisory Board for single events in 2023 and 2024 and was a Bristol-Myers-Squibb (BMS) patient ambassador from April 14, 2023 to March 10, 2025.

Donna Charissa A. Doebler

Staff Officer

Donna Doebler is a Senior Program Officer at the National Academies of Sciences, Engineering, and Medicine (NASEM). She is part of the Health and Medicine Division's Board on Population Health and Public Health Practice. Prior to joining NASEM, Dr. Doebler was the Associate Vice President of Medicare Advantage at UPMC Health Plan in Pittsburgh, PA where she led product, strategy, and financial and clinical analytics to help manage the care of over 200,000 member lives. In addition, she worked with UPMC hospitals to develop the first community health needs assessments and implementation plans, as part of new requirements of the ACA for all non-profit hospitals. Dr. Doebler was also an Assistant Professor at the University of Pittsburgh Graduate School of Public Health, served as a biostatistician at VA Pittsburgh Health System's Center for Health Equity Research and Promotion, and worked at the Institute of Medicine and the U.S. Government Accountability Office. Dr. Doebler completed a Kellogg Health Scholars postdoctoral fellowship, has a Dr.PH. in behavioral and community health sciences, an M.S. in biostatistics—both from the University of Pittsburgh, and an M.P.H. in epidemiology from the George Washington University. Her focus is on achieving health equity and understanding the social, community, and contextual factors that impact population health, especially with the aging population.