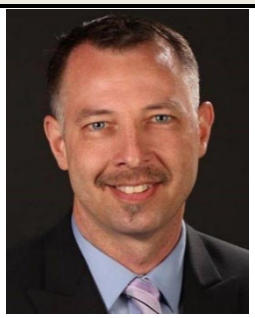
# 7th Annual Clinical Outcome Assessment in Cancer Clinical Trials Workshop

***Keeping an "open" mind: Patient-reported Outcomes in open-label trials***

**June 29, 2022 10:00 am – 3:00 PM ET (Virtual)**

# Biographies

## Workshop Welcome and Opening Remarks



**Paul G. Kluetz, MD**
**Deputy Director, Oncology Center of Excellence, FDA**

Dr. Paul Kluetz is a medical oncologist and the Deputy Director of the Oncology Center of Excellence at the U.S. FDA. In addition to assisting in the strategic and operational oversight of the OCE, he has a broad interest in trial design and endpoint selection to expedite drug development and define clinical benefit in oncology trials. Some of his initiatives include creation of the OCE's patient-focused drug development program and expansion and direction of OCE's efforts to advance real-world evidence, decentralized trial design and digital health technology. He is also active in regulatory review of Oncology products and oversees important oncology drug labeling initiatives. Dr. Kluetz remains clinically active, caring for patients and supervising medical residents at the Georgetown University Hospital.

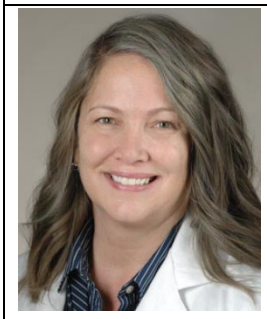
## Session 1: What is the issue?
## Exploring the realities of open-label trials in oncology and use of PROs

**Erica Horodniceanu, MPH (Moderator)**
**Health Scientist, Oncology Center of Excellence, FDA**

Erica Horodniceanu, MPH, is a health scientist in the Oncology Center of Excellence (OCE) Patient-Focused Drug Development (PFDD) program at the FDA. Her work focuses on advancing the incorporation of the patient experience into the drug development process through engagement, research, and regulatory initiatives. Her interests include patient-reported outcomes (PROs), qualitative research methods, and rare and pediatric cancers. Erica holds a Bachelor of Science degree in Health Science Education, with a

concentration in Health Promotion from the University of Florida and a Master's in Public Health degree in Public Health Practice and Policy from the University of Maryland.

## Panelists:



**Terri Armstrong, PhD, ANP-BC, FAAN, FAANP**
**Senior Investigator & Deputy Branch Chief, Neuro-Oncology Branch**
**Associate Director for Patient-Centered Outcomes, Center for Cancer Research, National Cancer Institute**

Terri S. Armstrong is a Senior Investigator and Deputy Branch Chief in the Neuro-Oncology Branch (NOB), and Associate Director for Patient-Centered Outcomes, Center for Cancer Research (CCR), National Cancer Institute (NCI), National Institutes of Health (NIH). Her program of research focuses on clinical outcomes assessment in therapeutic trials and clinical care, exploring the role of alterations in circadian biology in tumor growth and treatment toxicity and developing prediction modeling and biologically based interventions for symptom management. She also co-leads the Moonshot funded NCI-CONNECT program for rare CNS tumors. She has been an advanced practice provider for over 30 years, Quality of Life chair on multiple institutional and multi-site clinical trials and the PI of several research studies with over 4.5 million in grant support. She was inducted as a Fellow in the American Academy of Nurse Practitioners in 2009 and as a Fellow in the American Academy of Nursing in 2013.

**Martha Donoghue, MD**
**Associate Director for Pediatric and Rare Cancer Drug Development (Acting), OCE, FDA**
**Deputy Division Director, Division of Oncology 2, CDER, FDA**

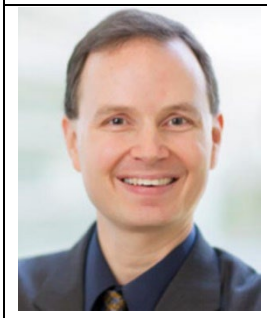
Martha Donoghue, MD, is the Deputy Director of the Division of Oncology 2 in the Office of Oncologic Diseases at the U.S. Food and Drug Administration (FDA). Dr. Donoghue provides regulatory oversight, engages in clinical review activities, and advises stakeholders regarding strategies for clinical development of drugs and therapeutic biologics for the diagnosis, prevention, and treatment of cancer. Areas of special interest include development of treatments for rare cancers and the use of innovative designs in clinical trials to optimize drug development. Prior to joining FDA in 2009, Dr. Donoghue completed a fellowship in Pediatric Hematology and Oncology at the Children's National Medical Center after working for several years as a general pediatrician in private practice. She received her medical degree from Emory University and completed a residency in general pediatrics at the Georgetown University Medical Center.

# Biographies

**Wenora Johnson**
**Patient/Research Advocate**

Wenora is a three-time Cancer survivor (Colorectal, Endometrial and Basal Cell Carcinoma), Volunteer Research/Patient Advocate and Navy Veteran. As a volunteer with various organizations, she shares her understanding of policy; research; genetic testing; hereditary cancer; patient engagement and clinical trials with patients and the healthcare community.

Being a Lynch Syndrome patient, Wenora advocates for genetic testing and awareness. She serves on various panels and review boards to provide extensive feedback on her role as a patient and research advocate with organizations such as CAP (College of American Pathologist); Clinical Trials Curator for Fight CRC; FORCE (Facing Our Risk of Cancer Empowered) research Advocate, Peer Navigator and Board Member; a Consumer Review for the DoD Peer Reviewed Cancer Research Program; a PCORI Ambassador and Clinical Trials Panel Member; IRB for local community hospital; NRG Oncology Patient Advocate Committee Member and the AACR Scientist-Survivor Program – presenting a poster on financial toxicities and disparities among minority patients and a National Quality Forum (NQF) Cancer Standing Committee Member. She has written various patient advocate blogs and participated as a guest speaker/panelist. Wenora works in administration in the greater Chicagoland area and enjoys reading and traveling with her family.



**Bryce Reeve, PhD**
**Professor of Population Health Sciences**
**Professor of Pediatric**
**Director, Center for Health Measurement**
**Duke University**

Dr. Bryce Reeve is a Professor of Population Health Sciences and Professor of Pediatrics at Duke University School of Medicine. He also serves as Director of the Center for Health Measurement since 2017. Trained in psychometric methods, Dr. Reeve's work focuses on assessing the impact of disease and treatments on the lives of patients and their caregivers. This includes the development of clinical outcome assessments using both qualitative and quantitative methods, and the integration of patient-centered data in research and healthcare delivery settings to inform decision-making. From 2000 to 2010, Dr. Reeve served as Program Director for the U.S. National Cancer Institute and oversaw a portfolio of health-related quality of life research in cancer patients. From 2010 to 2017, he served as Professor of Health Policy and

Management at the University of North Carolina. From 2011-2013, Dr. Reeve served as President of the International Society for Quality of Life Research (ISOQOL). In 2015, he received the John Ware and Alvin Tarlov Career Achievement Prize in Patient-Reported Outcomes Measures. In 2017, 2018, 2019 and 2021, he was ranked in the top 1% most-cited in his respective field over the past 11-year period.

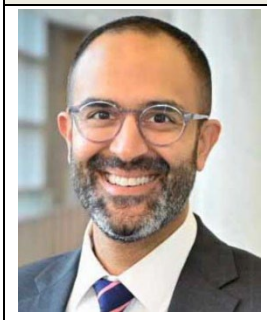
**Gita Thanarajasingam, MD**
**Assistant Professor of Medicine, Division of Hematology**
**Mayo Clinic**

Dr. Gita Thanarajasingam is an Assistant Professor of Medicine and consultant in the Division of Hematology at Mayo Clinic in Rochester, Minnesota. She is a graduate of Yale University and Mayo Clinic Alix School of Medicine and completed her internal medicine residency at the Brigham and Women's Hospital at Harvard Medical School. After Hematology/Oncology Fellowship and Advanced Lymphoma Fellowship at Mayo Clinic, she joined the faculty of the Mayo Lymphoma disease-oriented group. Her clinical practice as an oncologist is focused on Hodgkin and non-Hodgkin lymphoma, and she performs health outcomes research in lymphoma and other cancers.

As a clinical investigator, her work focuses on improving the evaluation of adverse events (AEs) of treatment and measuring their impact on treatment tolerability cancer patients. She developed the Toxicity over Time (ToxT), a longitudinal patient-focused approach to AE evaluation. She is active in the implementation of patient-reported outcomes (PRO) to better understand treatment toxicity and tolerability. She serves of as vice co-chair of the Alliance for Clinical Trials in Oncology Health Outcomes Committee and is the recipient of K and U01 grants from the U.S. National Institutes of Health. She has been funded by the Lymphoma Research Foundation in support of her work. She leads the ongoing international multi-stakeholder Lancet Haematology Commission, "Beyond maximum grade: modernizing the assessment and reporting of adverse events in hematological malignancies." She is an international advisory board member of the Lancet Haematology and an ad-hoc member of the U.S. Food and Drug Administration (FDA) Oncology Drug Advisory Committee (ODAC) with expertise in toxicity assessment. She is also currently the lead principal investigator of a multi-site prospective trial evaluating physical functioning in cancer patients with clinician-reports, PRO and wearable device data. Her research program overall endeavors to improve the accuracy and patient-centeredness of AE evaluation and better understand cancer treatment tolerability from the patient's perspective.

# Biographies

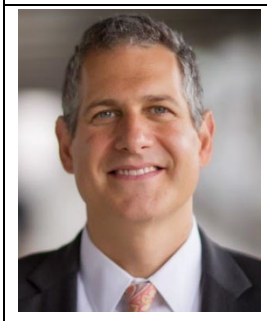
## Session 2: What have we learned?
## Analysis and interpretation of PRO data from open-label trials



**Vishal Bhatnagar, MD (Moderator)**
**Associate Director for Patient Outcomes**
**Oncology Center of Excellence, FDA**

Dr. Vishal Bhatnagar is a medical oncologist/hematologist and the Associate Director for Patient Outcomes in the OCE. His interests include patient reported outcomes, patient preference and incorporation of patient experience in oncology trials. His work focuses on the operational management of the OCE's Patient-Focused Drug Development program. Additionally, Dr. Bhatnagar has a strong clinical interest in multiple myeloma and has previously served as an Office of Hematology and Oncology multiple myeloma scientific liaison. Dr. Bhatnagar received his BA in Political Science and his medical degree at the George Washington University. He completed his internal medicine residency and hematology/oncology fellowship at the University of Maryland.

## Panelists:



**Ethan Basch, MD, MSc**
**Chief of Oncology, University of North Carolina**
**Physician-in-Chief, North Carolina Cancer Hospital**

Dr. Ethan Basch is Physician-in-Chief of the North Carolina Cancer Hospital and Chief of Oncology at the University of North Carolina, where he is Distinguished Professor in Medical Oncology and Professor of Health Policy & Management. His research group established that up to half of patients' symptomatic adverse events go undetected in clinical trials, and that patient-reported outcome questionnaires substantially improve detection. His team determined that integrating web-based patient-reported symptoms into oncology clinical practice improves clinical outcomes, including survival, and reduces health service utilization. He led a group under contract to the National Cancer Institute to create and test a system for collecting patient-reported AEs during cancer trials called the 'PRO-CTCAE.'

Dr. Basch is involved in efforts to bring PROs into comparative effectiveness research, routine care, and quality improvement. He is a member of the Board of Directors of ASCO, an Associate Editor at JAMA, and a prior member of the Board of Scientific Advisors for the National Cancer Institute and the Methodology Committee of the Patient-Centered Outcomes Research Institute. Dr. Basch received his MD from Harvard Medical School, a

Master's degree in Epidemiology from the Harvard School of Public Health, completed his Internal Medicine residency at Massachusetts General Hospital, and fellowships in Oncology and Health Services Research at Memorial Sloan Kettering Cancer Center. He has published over 300 manuscripts and is deeply committed to advancing integration of patient perspectives in oncology clinical research and care.

**Selena Daniels, PharmD, PhD**
**Clinical Outcome Assessment Team Leader**
**Office of Drug Evaluation Sciences, CDER, FDA**

Dr. Selena Daniels serves as a Team Leader in the Division of Clinical Outcome Assessment at the FDA. She leads a team of expert analysts who provide consultation and advice on clinical outcome assessment (COA) endpoint development and validation, including considerations for clinical trial design, conduct, analysis, interpretation, and reporting for regulatory determinations of medical product benefit.

Prior to joining the FDA in 2015, Dr. Daniels worked in the Health Economic and Outcomes Research (HEOR) group at Allergan, Inc for almost five years, where she developed and executed HEOR strategies, as well as developed and implemented innovative COA strategies and endpoints for clinical trials.

Dr. Daniels received her doctor of philosophy degree in Education at Nova Southeastern University and pharmacy degree at Loma Linda University.

**Mallorie Fiero, PhD**
**Lead Mathematical Statistician**
**Office of Biostatistics, CDER, FDA**

Dr. Mallorie Fiero has been at the FDA since 2016 and is a statistical team leader supporting the Division of Oncology 1 (DO1) in the Office of Oncologic Diseases (OOD) covering breast, gynecologic and genitourinary cancers. She received her BS in Statistics from UCLA and a PhD in Biostatistics from the University of Arizona. Before her current role, she was a statistical reviewer in DO2 and DO3 covering gastrointestinal, thoracic head and neck, neuro-oncology, and other rare cancers. Mallorie's research interests include estimands, missing data, and statistical analysis of patient-reported outcomes in cancer trials.

# Biographies

**Jessica Roydhouse, PhD**
**Select Foundation Senior Research Fellow, Health Services Research**
**University of Tasmania, AU**

Dr. Jessica Roydhouse is a Select Foundation Senior Research Fellow in Health Services Research at the Menzies Institute for Medical Research, University of Tasmania, Australia. As part of her role at Menzies she is also Director of the Tasmanian Cancer Registry, the state's population-based cancer registry, and Academic Lead for the Prostate Cancer Outcomes Registry – Tasmania, a clinical quality registry that collects treatment and PRO data. Her research focuses on patient-reported outcomes (PROs) in clinical trials and observational studies in cancer. She is interested in methodological issues relating to PROs, including open-label trials, missing data and causal inference.

Prior to her role at the Menzies, Dr Roydhouse was an ORISE Fellow in the Oncology office at the FDA, where she led research on PROs in open-label trials, time to patient-reported event endpoints and patient-reported side effect bother. She is the Chair of the Standards and Best Practices Committee of the International Society of Quality of Life Research (ISOQOL) and Chair-Elect of the Patient-Centered Special Interest Group for ISPOR, the professional society for health economics and outcomes research. Dr Roydhouse also serves on committees for Australian collaborative cancer trials groups, including the Primary Care Collaborative Cancer Clinical Trials Group (Scientific Committee) and the Australasian Gastro-Intestinal Cancer Trials Group (Lower GI Working Party).

**Patty Spears**
**Scientific Research Manager & Patient Advocate**
**University of North Carolina Lineberger Comprehensive Cancer Center**

Ms. Spears is an over 20-year breast cancer survivor and cancer research patient advocate. She was diagnosed with locally advanced breast cancer at the age of 40 and after neoadjuvant chemotherapy, surgery and radiation therapy, she participated in a HER2 vaccine clinical trial. Ms. Spears has concentrated her advocacy on clinical trials and serves as an advocate on the Translational Breast Cancer Research Consortium (TBCRC). She has also been an advocate for the National Clinical Trials Network (NCTN) since 2008 and is currently an advocate for the Alliance for Clinical Trials in Oncology as Associate Group Chair for Advocacy, Chair of the Alliance Patient Advocate Committee and a member of the Breast Committee. She is also a member of several NCI Committees, including the Clinical Trials and Translational

Research Advisory Committee (CTAC), Investigational Drug Steering Committee (IDSC), and Co-Chair of the Patient Advocate Steering Committee (PASC).

Ms. Spears is currently working as a scientific research manager and patient advocate at the University of North Carolina at Chapel Hill, Lineberger Comprehensive Cancer Center where she leads the UNC Lineberger Patient Advocates for Research Council (PARC) and the UNC Breast SPORE Advocates. At UNC she focuses on communicating research to the public and facilitates the engagement of patient advocates with Lineberger researchers. She also has an interest in patient reported outcome measures (PROMs) in drug development, clinical trials and clinical care.

## Session 3: Where do we go from here?
## Efforts to advance PRO to inform tolerability regardless of blinding status

**Moderators:**
**Vishal Bhatnagar, MD**
**Erica Horodniceanu, MPH**
**Paul Kluetz, MD**
**FDA, OCE**

See bios above

### Panelists:

**Yelak Biru, MS**
**Patient Advocate**
**International Myeloma Foundation**

Diagnosed at the young age of 25 with stage III multiple myeloma, Yelak is a patient turned multiple myeloma research advocate and successfully integrated multiple myeloma into his life for over a quarter of a century.

Yelak is a member of the International Myeloma Foundation board of directors, Eastern Cooperative Oncology Group's patient advocate and myeloma committees, National Cancer Institute's Myeloma Steering Committee, the NCI Council of Research Advocates, and is active on Twitter under the handle @NorthTxMsg. Yelak regularly collaborates with National Comprehensive Cancer Network, the US Food and Drug Administration, and American Association for Respiratory Care on various access disparity initiatives and publications. His areas of advocacy interest include patient education, clinical trial design,

quality of life improvements, drug accessibility, minority engagement, access disparities, and global capacity building of patient organizations.

Yelak has a master's degree in computer science. After a 25 year career in fortune 50 companies as a technology leader, Yelak was appointed president and CEO of the International Myeloma Foundation in November of 2021.

**Melanie Calvert, PhD**
**Professor of Outcomes Methodology, NIHR Senior Investigator**
**University of Birmingham, UK**

Professor Melanie Calvert, PhD, is Professor of Outcomes Methodology at the University of Birmingham UK. She is Director of Birmingham Health Partners Centre for Regulatory Science and Innovation and Director of the Centre for Patient Reported Outcomes Research which aims to optimize the use of patient reported outcomes (PROs) in clinical trials and routine care, to improve service delivery, enhance patient care and outcomes and ensure that the patient perspective is at the heart of health research and healthcare decision-making. She is the cross-cutting theme lead for PROs research within National Institute for Health Research (NIHR) infrastructure including the Biomedical Research Centre Birmingham, Surgical Reconstruction and Microbiology Research Centre and Applied Research Collaboration West Midlands. She is a member of the National Research Ethics Advisory Panel and is a NIHR Senior Investigator. She is currently co-leading the NIHR/UKRI funded Therapies for Long COVID Study.

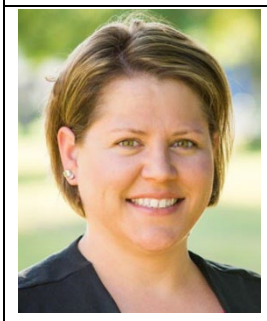
Professor Calvert has >200 peer reviewed publications in journals including the NEJM, BMJ, JAMA and the Lancet. With international collaborators she led the development of international PRO guidance including the SPIRIT-PRO extension, CONSORT-PRO extension , PRO Ethics Guidelines and is a member of the SISAQOL initiative. Recent work includes publications on the use of PROs in early phase trials and real-world evidence generation.

Her highly cited work has informed clinical guidelines, NICE and EMA guidance. Professor Calvert sits on a number of international committees leading national and international strategy for PROs research/implementation including the PROTEUS Consortium which promotes tools and resources to optimize the use of PROs in clinical trials to ensure that patients, clinicians, and other decision-makers can make the best decisions about treatment options.

# Biographies

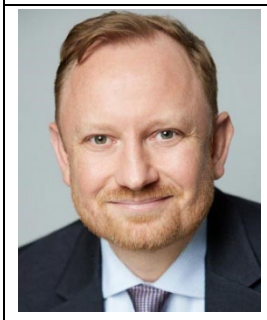
**R. Angelo de Claro, MD**
**Director, Division of Hematologic Malignancies 1**
**Office of Oncologic Diseases, CDER, FDA**

Dr. Angelo de Claro is the Division Director for Division of Hematologic Malignancies I with Office of Oncologic Diseases. He provides leadership and scientific direction to staff engaged in the review and evaluation of applications for investigational new drugs and drug approvals. Dr. de Claro is also the Associate Director (Acting) for Global Clinical Sciences with US FDA Oncology Center of Excellence (OCE). In this role, he leads OCE efforts to advance cancer drug development and regulatory science across the globe, including direction of Project Orbis, a global collaborative review program started in 2019. He is board certified in Internal Medicine, Hematology, and Medical Oncology. He completed his Hematology-Oncology fellowship at University of Washington and Internal Medicine residency at Baylor College of Medicine. He has been with FDA since 2010.



**Amylou Dueck, PhD**
**Associate Professor of Biostatistics**
**Mayo Clinic Amylou Dueck, PhD**

Dr. Amylou Dueck is an Associate Professor of Biostatistics and Vice Chair of the Department of Quantitative Health Sciences at Mayo Clinic in Arizona. She is an expert in the statistical analysis of patient-reported outcomes (PROs) in cancer clinical trials and has contributed to the development of PRO measures, including the PRO-CTCAE and MPN-SAF. Dr. Dueck is the Co-chair of the Health Outcomes Committee of the Alliance for Clinical Trials in Oncology. In this role, Dr. Dueck partners with clinical investigators to integrate, monitor, analyze, and report PROs in a wide variety of cancer clinical trials.



**Devin Peipert, MD**
**Assistant Professor, Medical Social Sciences**
**Northwestern University**

Dr. Devin Peipert is an Assistant Professor in the Department of Medical Social Sciences at Northwestern University Feinberg School of Medicine and a member of the Robert H. Lurie Comprehensive Cancer Center of Northwestern University. In this role, he acts as an investigator and psychometrician focusing on the application of patient reported outcomes (PROs) in patient focused drug development and in clinical monitoring to optimize patient management. He has a research program aiming to quantify and manage

drug intolerability in cancer. This work includes a focus on overall summary measures of treatment tolerability (specifically, FACT item GP5, 'I am bothered by side effects of treatment'), as well as a focus on tools to capture physical function and key disease symptoms in advanced cancer. This research is carried out though multiple fruitful collaborations, including as a member of the EVOLV team in the National Cancer Institute's Tolerability Consortium and through a project with the US FDA.

Devin is among the lead developers of the PROMIS® Medication Adherence Scale (PMAS). He is also a key investigator on projects that integrate PROs in clinical care to capture symptom and side effect burden to inform shared treatment decision-making. In his psychometric research areas, Devin works extensively on establishing evidence to qualify PROs as clinical outcome assessments (COAs) to implement in drug trials, largely focusing on measures from the PROMIS® and Functional Assessment of Cancer Therapy (FACT) systems. He also has a strong methodological focus on evaluating and establishing new methods to determine individual patient change on PRO measures.