9th Annual Clinical Outcome Assessment in Cancer Clinical Trials Workshop

Modernizing Tolerability Assessment in Cancer Clinical Trials

June 25, 2024 11:00 am – 2:30 pm ET (Virtual)

Biographies

Welcome and opening remarks

Vishal Bhatnagar, MD
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 was previously a clinical reviewer in the Division of Hematology Products. 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.

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 holds an acting role overseeing the solid tumor and toxicology Divisions within the Office of Oncologic Diseases. Paul has a broad interest in trial design and endpoint selection as well as evidence modernization 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.

Biographies

Session 1: Revisiting Core Item Sets in Oncology Trials – Where are we and where do we want to go?

Terri Armstrong, PhD, ANP-BC, FAAN, FAANP (Moderator)
Senior Investigator, 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.

Panelists:

Yelak Biru
Patient Advocate
President & CEO, International Myeloma Foundation

Yelak Biru's journey is one of resilience, strategy, and unwavering determination. Diagnosed with myeloma at age 25, 28 years ago, he transformed his personal battle into a relentless pursuit of a cure. As a patient advocate, Yelak championed better resources and support for those facing similar challenges, leveraging his voice to bridge the gap between patients and research.

With a background in computer science, Yelak recognized the power of data to drive research initiatives and personalized care, ensuring treatments can be tailored to individual needs.

Drawing on his corporate experience at Fortune 50 companies, Yelak brings strategic acumen and leadership skills to his role as CEO and President of the International Myeloma Foundation (IMF). Under his guidance, the IMF has experienced remarkable growth, expanding research funding, forging global partnerships, and advocating for equitable access to care.

Biographies

Yelak's unique ability to understand and articulate both patient and research perspectives makes him a bridge builder, connecting patients' emotional urgency with researchers' scientific rigor. His leadership embodies the unwavering spirit of the myeloma community, driving collective progress toward a future free from this disease.

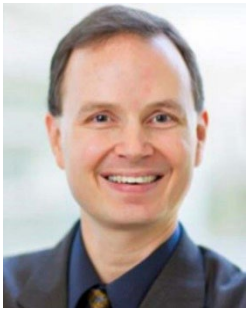
Erica Horodniceanu, MPH
Senior Health Scientist, Patient Focused Drug Development
Oncology Center of Excellence, FDA

Erica Horodniceanu, MPH, is a health scientist in the Oncology Center of Excellence (OCE) Patient-Focused Drug Development program at the FDA. Over the past 20 years, Erica has provided healthcare research, health education, communications, and project management services to industry and the federal government. She has previously held positions within consulting firms focused on outcomes research and has been working in the field of clinical outcome assessments (COAs) and patient-reported outcomes (PROs) for the past 10 years. 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.

Tito Mendoza, PhD
Senior Scientist, Office of Patient-Centered Outcomes Research
Center for Cancer Research, National Cancer Institute

Dr. Mendoza received his Ph.D. in Quantitative Methods and his M.S. in Statistics from the University of Wisconsin-Madison. He also obtained an MEd in Research and Measurement from the University of Toledo. After earning his doctorate degree, he joined the faculty of the University of Texas MD Anderson Cancer Center and left as a professor after having served for 25 years. He is currently a senior scientist with the Office of Patient-Centered Outcomes Research in the Center for Cancer Research at the US National Cancer Institute. He is a psychometrician who was instrumental in the development and validation of several multi-symptom and single symptom assessment tools. He was also involved in several aspects of the development and validation of the patient-reported outcomes version of the Common Terminology Criteria Adverse Event (PRO-CTCAE) funded by the National Cancer Institute. His collaborative interests are in the design, analysis and interpretation of studies incorporating patient-reported outcomes in early phase cancer clinical trials.

Biographies



Bryce Reeve, PhD
Professor of Population Health Sciences
Professor of Pediatrics
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. 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, 2021, 2022, and 2023, 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 Associate 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 completion of the Hematology/Oncology Fellowship Program and an Advanced Lymphoma Fellowship at Mayo Clinic, she joined the faculty of the Mayo Clinic Rochester Lymphoma Disease-Oriented Group. Her clinical practice as a hematologist 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 in 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 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. 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.

Lynne I. Wagner, PhD
Professor & Clinical Health Psychologist, Department of Health Policy & Management
University of North Carolina Gillings School of Global Publish Health

Dr. Lynne Wagner is a clinical health psychologist and a Professor in the Department of Health Policy and Management in the UNC Gillings School of Global Public Health at the University of North Carolina at Chapel Hill. Dr. Wagner is a Member of the Cancer Prevention and Control Program at the UNC Lineberger Comprehensive Cancer Center, where she is currently leading the Patient-Reported Outcomes Center of Excellence.

Dr. Wagner is Multiple-PI of the ECOG-ACRIN NCI Community Oncology Research Program (NCORP) Research Base and is the Co-Director of the ECOG-ACRIN Cancer Control and Outcomes Program. In this role, Dr. Wagner is responsible for leading a program of cancer prevention, survivorship, quality of life, and cancer care delivery research within the National Clinical Trials Network (NCTN). She has contributed to the successful conduction of numerous cancer survivorship trials and the collection of patient-centered outcomes on over 35 NCTN and NCORP trials. Dr. Wagner's research expertise includes patient-reported outcomes clinical trials research, cancer treatment tolerability, psychosocial oncology, eHealth interventions in cancer survivorship, and cancer symptom management. Dr. Wagner has expertise in integrating patient-reported outcomes into clinical care, with the goal of systematically bringing the patient's voice to the clinical encounter. She has served as the Principal Investigator or Co-Investigator on numerous extramurally-funded projects and has authored or co-authored over 150 peer-reviewed publications.

Biographies

Session 2: Revisiting Core Item Sets in Oncology Clinical Trials – How do we get there?

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

See above entry

Panelists:

Ethan Basch, MD, MSC
Distinguished Professor, Chief of Oncology & Physician-in-Chief
Cancer Hospital at the University of North Carolina

Ethan Basch, MD, MSC is Distinguished Professor and Chief of Oncology, and Physician-in-Chief of the Cancer Hospital at the University of North Carolina. His research focuses on patient-reported outcomes for assessing tolerability in clinical trials, and for remote symptom monitoring during cancer clinical care. His team led development of the PRO-CTCAE for the National Cancer Institute, and established that PRO symptom monitoring improves quality of life, hospitalizations, and survival. He is committed to bringing the patient voice into oncology research and care delivery.

Cheryl Coon, PhD
Vice President, Clinical Outcome Assessment Program
Critical Path Institute (C-Path)

Cheryl D. Coon, PhD, is Vice President of the Clinical Outcome Assessment (COA) Program at Critical Path Institute (C-Path). Dr. Coon is a psychometrician with two decades of experience developing and evaluating COAs for use in constructing patient-centered endpoints. She spent much of her career as a consultant, advising sponsors on the use, analysis, and interpretation of COAs in clinical trials. She joined C-Path in 2023, where she helps to bring sponsors, regulators, and patients together in a pre-competitive space to address critical endpoint issues that can be tackled more quickly and thoroughly in a collaborative environment. Dr. Coon is a recognized thought-leader in the field of COAs and seeks to generate evidence that allows the benefits of new therapies to be clearly communicated to patients and clinicians for informing treatment decisions. Dr. Coon received her PhD in Quantitative Psychology from the University of North Carolina at Chapel Hill, during which

time her research focused on item response theory and included early work on the Patient-Reported Outcomes Measurement Information System.

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

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.

Megan Fitter, PhD
Clinical Outcome Assessment Reviewer
Office of Drug Evaluation Science, CDER, FDA

Dr. Megan Fitter is a Clinical Outcome Assessment (COA) reviewer in the Division of Clinical Outcome Assessment in the Office of Drug Evaluation Science in the Center for Drug Evaluation and Research at the Food and Drug Administration (FDA).

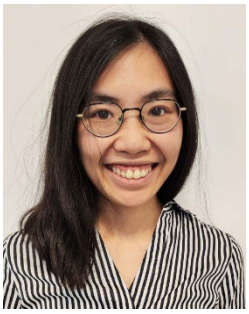
As a COA reviewer, Dr. Fitter provides guidance on the development, validation, and selection of COAs that accurately capture meaningful aspects of the patient experience to support reliable and well-defined clinical trial endpoints.

Dr. Fitter earned a Doctor of Philosophy degree in Developmental Psychology and a certificate in Measurement, Statistics, and Evaluation at the University of Maryland College Park.

Jan Geissler
Patient Advocate
Founder & CEO, Patvocates

Jan Geissler is the founder and CEO of Patvocates, a consultancy and think tank in patient advocacy and patient involvement. He was the Director of the European Patients Academy (EUPATI) from 2012-2017. He has been a leukemia patient since 2001. Jan co-founded the

patient organizations LeukaNET in 2002, the European Cancer Patient Coalition in 2003, the CML Advocates Network in 2007, the Leukemia Patient Advocates Foundation in 2011, the Workgroup of European Cancer Patient Advocacy Networks (WECAN) in 2015 and the Acute Leukemia Advocates Network in 2016. Jan represents patients on a number of advisory boards and committees, e.g. the European Cancer Organization, the European Hematology Association, EuroBloodNET, the International CML Foundation, the German National Decade Against Cancer and the Ethics Committee of the Bavarian Chamber of Physicians. Jan is a work package leader of the EU-funded HARMONY Alliance on Big Data for Better Outcomes in Hematology as well as SafePolyMed. In 2016, he was awarded with the Order of Merit of the Federal Republic of Germany for his work as a patient advocate.



Madeline Pe, PhD
Head of Quality of Life Department
European Organisation for Research and Treatment of Cancer

Madeline Pe, PhD is the Head of the Quality-of-Life Department at the European Organisation for Research and Treatment of Cancer (EORTC). In this role, she oversees the various quality of life (QOL) research activities, ensuring that it is in line with the mission of the EORTC. She leads a team that provide advice and methodological guidance for the inclusion of QOL endpoints in EORTC cancer trials. She ensures that appropriate support is provided to the EORTC Quality of Life Group network for the development of the QOL measures and implementation of their research activities. Dr. Pe's expertise is on statistical and methodological research for participant/patient-reported outcomes and is the scientific lead for the "Setting International Standards in Analysing Patient-Reported Outcomes and Quality of Life Endpoints Data" (SISAQOL) Consortium, which aims to develop consensus recommendations for the design, analysis and interpretation of patient-reported outcomes and quality of life endpoints in cancer clinical trials. She is a member of the International Advisory Board for the Lancet Oncology and collaborates with different international stakeholder groups to promote the inclusion of QoL into their cancer research programs.

Ashley Wilder Smith, PhD, MPH
Chief, Outcomes Research Branch,
National Cancer Institute

Ashley Wilder Smith, PhD, MPH, is Chief of the Outcomes Research Branch (ORB) at the National Cancer Institute (NCI). ORB supports investigations to understand and improve health outcomes and quality care for cancer patients, survivors, caregivers, and families with a cross-cutting emphasis on health equity. Dr. Smith carries out the mission of ORB by facilitating research designed to integrate the perspectives of cancer patients/survivors across the lifecourse, their caregivers and family members, and to integrate those

perspectives into healthcare settings with a goal to optimize patient engagement, health and well-being. In addition to overseeing the entire portfolio of ORB grants, contracts and research activities, Dr. Smith collaborates on efforts across the NIH and the Department of Health and Human Services to study and improve the measurement and evaluation of patient-reported care quality and health outcomes in observational studies, clinical trials, and for use in clinical care. She is currently the NCI Chief Science Officer of IMPACT: **I**mproving the **M**anagement of sym**P**toms during **A**nd following **C**ancer **T**reatment, a Research Consortium designed to accelerate the use of systematic cancer symptom management systems integrated into electronic health record systems to collect patient-reported data and support clinical responses consistent with evidence-based guidelines. She also served as the National Institutes of Health (NIH) Chief Science Officer of a trans-NIH initiative to make four person-centered health outcome assessment systems available: PROMIS®, the NIH Toolbox®, Neuro-QOL, and ASCQ-Me™. These tools were transitioned to independence from NIH funding and are now offered through an integrated platform for automated use in one publicly available resource, HealthMeasures. Dr. Smith earned her MS and PhD degrees in Health Psychology from the University of Pittsburgh. She completed an NCI Cancer Prevention Fellowship, which included earning an MPH in Epidemiology, also from the University of Pittsburgh. Dr. Smith joined the ORB in 2006 and became Branch Chief in 2014.