8th Annual Clinical Outcome Assessment in Cancer Clinical Trials Workshop

Overall Side Effect Impact: A Core Oncology Patient-Reported Outcome

June 27, 2023 11:00 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 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.

Session 1: Understanding Overall Side Effect Impact

Erica Horodniceanu, MPH (Moderator)
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.

Panelists:

Selena Daniels, PharmD, PhD
Clinical Outcome Assessment Team Leader
Center for Drug Evaluation and Research (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.

Cheryl Jernigan
Patient Research Advocate

With over 25 years as a research advocate, Cheryl L. Jernigan, CPA, F.A.C.H.E., strives to inform and empower patients to be effective partners, working with researchers and clinicians to enhance and focus research on what matters to patients.

Her "lived" cancer experience includes being a 27-year breast cancer "thriver"; and a co-survivor with her husband, who had HPV tonsil cancer and died in 2018 of metastatic prostate cancer.

Cheryl is the lead advocate for Patient & Investigator Voices Organizing Together (PIVOT), a University of Kansas Cancer Center initiative; and serves on the cancer center's NCORP scientific leadership and catchment area committees. She serves as an advocate leader for KU's Frontiers Clinical & Translational Science Award, the Kansas Institute of Precision Medicine's Center for Biomedical Research Excellence (COBRE), and the Greater Plains Collaborative, a clinical research data network.

Nationally, she was a founding member of Susan G. Komen's Advocates in Science program. She continues to serve on their steering committee, is a Komen Scholar, and is active in their BD4BC (Big Data for Breast Cancer) initiative.

In SWOG for Cancer Research (a National Cancer Institute Clinical Trials Network), she serves as the Advocate Member on their Cancer Prevention & Epidemiology Committee and is active in their Patient Advocate Committee.

She is an immediate past member of the National Cancer Institute's (NCI) Central Institutional Review Board for Adult Late Phase Clinical Trials. And currently serves on Rutgers Cancer Institute of New Jersey External Advisory Board; PCORnet's Engagement Core Leadership Committee; the Greater Plains Collaborative's Patient Advocate Committee; and the Clinical Trials Transformation Initiative's Steering Committee.

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 provides 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. She is also 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.

Biographies

Devin Peipert, PhD
Assistant Professor, Department of Medical Social Sciences
Northwestern University

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.

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 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 Hematology/Oncology Fellowship and Advanced Lymphoma Fellowship at Mayo Clinic, she joined the faculty of the Mayo Clinic Rochester Lymphoma disease-oriented group. Her

clinical practice 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.

Session 2: Analysis and Communication of Overall Side Effect Impact

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 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.

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Panelists:

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.

Mallorie Flores Fiero, PhD
Master Mathematical Statistician
Office of Biostatistics, CDER, FDA

Dr. Mallorie Fiero has been at the FDA since 2016 and is a master mathematical statistician 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.

Steven Merlin
Patient Advocate
Pancreatic Cancer Action Network

Steven Merlin became a patient advocate after surviving stage IV pancreatic cancer. Coming from a 40-year career in clinical diagnostics and clinical research, he gained an understanding of the importance in self-advocacy to be treated more aggressively with standard of care treatment and participating in a clinical trial to achieve a better outcome. Combining 11 years' experience as a survivor and research career in the fields of cancer, immunology and stem cells, Steven is very active on several social media sites answering questions from patients and caregivers regarding care, treatment, molecular profiling and clinical trials and advances in pancreatic cancer research. Additional advocacy activities include the Pancreatic Cancer Action Network (PanCan.org) as the Outreach Chair for the NJ State affiliate, and a patient advocate on the GI Cancers Committee of the Eastern

Comprehensive Oncology Group (ECOG-ACRIN) providing input from a patient perspective on clinical trials being proposed or amended, and the Hematology/Oncology Pharmacy Association (HOPA). Steven is also active in government advocacy writing to and meeting with members of Congress and their health policy legislative aids advocating for increased cancer research funding and medication pricing.

Jessica Roydhouse, PhD
Select Foundation Senior Research Fellow in Health Services Research
Director, Tasmanian Cancer Registry

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. 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.

In addition, Dr. Roydhouse is Chair-Elect of the Patient-Centered Special Interest Group for ISPOR, the professional society for health economics and outcomes research. Dr Roydhouse serves on committees and working groups in Australian cooperative cancer trials groups, including Cancer Symptom Trials (Scientific Advisory Committee) and the Australia New Zealand Gynaecological Oncology Group (Ovarian Tumour Working Group). She is also the International Society for Quality-of-Life Research (ISOQOL) representative on the Steering Committee for the Cancer Quality of Life Expert Service Team, which provides advice and support to Australian cooperative cancer trials groups about the inclusion of PROs.

Lynne I. Wagner, PhD
Professor, Department of Social Sciences and Health Policy
Wake Forest University School of Medicine, North Carolina

Dr. Lynne Wagner is a clinical health psychologist and a Professor in the Department of Social Sciences and Health Policy, Division of Public Health Sciences at the Wake Forest School of Medicine. Dr. Wagner is a Member of the Atrium Health Wake Forest Baptist Comprehensive Cancer Center, where she maintains leadership roles as the Director of Research and Clinical Integration, Cancer Prevention and Control, and Co-Director of the Qualitative and Patient-Reported Outcomes Shared Resource.

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 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.

Session 3: Future Directions for Overall Side Effect Impact

Meena Murugappan, PharmD, MPH, PhD(c) (Moderator)
Visiting Scientist, Patient-Focused Drug Development
Oncology Center of Excellence, FDA

Meena Murugappan, PharmD, MPH, is a pharmacist and pharmacoepidemiologist serving as a visiting research scientist for Patient-Focused Drug Development at the Oncology Center of Excellence (OCE) within the US Food and Drug Administration (FDA). She is also a PhD candidate in health economics and outcomes research at the University of Minnesota College of Pharmacy. Meena has led numerous projects during her time at FDA including a landscape analysis of Patient-Reported Outcomes in Pediatric Oncology trials, examination of floor/ceiling effects associated with the EORTC QLQ-C30's Physical Functioning subscale, and evaluation of frailty in Multiple Myeloma. Prior to joining FDA, she served as an associate research scientist at the University of Minnesota, conducting commercial claims data analyses. She is interested in the role of Patient Reported Outcomes and Real-World Evidence in regulatory and healthcare decision making.

Panelists:

Melanie Calvert, PhD
Professor, Outcomes Methodology, NIHR Senior Investigator
University of Birmingham, United Kingdom

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 and Care Research (NIHR) infrastructure including the Biomedical Research Centre Birmingham, Blood and Transplant Research Unit in Precision and Cellular Therapeutics 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 >250 peer reviewed publications in journals including the NEJM, Nature Medicine, 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-IMI initiative. Recent work includes publications on inclusive and equitable PRO data collection, use of PROs in AI studies and real-world evidence generation. Her highly cited work has informed clinical guidelines, NICE and EMA guidance and UK Government policy. 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.

Jan Geissler
Patient Advocate
Lancet Hematology Commission

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 organisations 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 Organisation, 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.

Biographies

Lori Minasian, MD, FACP
Deputy Director, Division of Cancer Prevention
National Cancer Institute, NIH

Dr. Lori Minasian, a medical oncologist, is the Deputy Director for the Division of Cancer Prevention at the NCI. She is a leader in the NCI clinical trials enterprise, first leading the NCI's Community Clinical Oncology Program (a community-based clinical trials program) for over 15 years, and then supporting a variety of the processes in the restructuring of the NCI's clinical trials programs. She oversees one of the four boards that make up the Central Investigational Review Board (CIRB) for the NCI's clinical trials programs. She has facilitated the development of numerous cancer prevention and symptom management clinical trials and has fostered the incorporation of patient reported outcomes in cancer clinical trials.

Currently, Dr. Minasian leads the NCI's Multi-Cancer Detection Trial Team which is developing a framework for the evaluation of MCD assays for the purpose of cancer screening. She leads the development of a new clinical trials network which will systematically evaluate a variety of different technologies for the purpose of cancer screening.

Mirat Shah, MD, MHS
Medical Oncologist and Clinical Reviewer
Division of Oncological Diseases, CDER, FDA

Dr. Shah is a medical oncologist and clinical team lead on the Breast, Gynecologic, and Supportive Oncology team within the Division of Oncology 1, Office of Oncologic Diseases at FDA.

She joined the FDA in 2019, after completing her internal medicine residency at Vanderbilt University, and her medical oncology and clinical pharmacology fellowships at Johns Hopkins, where she also served as the Chief Oncology Fellow. She is focused on improving the tolerability of cancer therapies through dosage optimization, and she has served as the clinical lead for FDA Oncology Center of Excellence's Project Optimus since 2021. She is also committed to providing education in regulatory science to internal and external stakeholders. She enjoys hiking and birding in her spare time.

Biographies

Ashley Slagle, MS, PhD
Principal & Scientific and Regulatory Advisor
Aspen Consulting, LLC

Dr. Slagle is the Principal and Founder of Aspen Consulting, LLC where she provides strategic regulatory and scientific advice to drug developers on matters related to patient focused drug development (PFDD), with a particular focus on patient-centered endpoints and clinical outcome assessments (COAs) to support medical product development and approval.

Dr. Slagle's experience includes 23 years of pharmaceutical outcomes research, policy analysis, and COA development, implementation, and analysis to evaluate treatment benefit for regulatory purposes. She participates in scientific advisory boards and advises sponsors across a broad range of therapeutic areas to ensure the patient's voice is meaningfully incorporated into drug development to inform stakeholder decision-making. Dr. Slagle previously served on the COA Staff (now Division of COA) in OND/CDER/FDA. There she provided oversight on reviews and recommendations related to the development and use of COAs and worked closely with CDER review divisions to make drug approval and labeling decisions. Dr. Slagle participated in FDA policy and guidance development and legislative activities related to PFDD, COAs, and endpoints.