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)

AGENDA	
10:00 AM – 10:15 AM	**Workshop welcome and opening remarks**
Paul Kluetz – Medical Oncologist, FDA	
10:15 AM – 11:30 AM	**Session 1: What is the issue?** **Exploring the realities of open-label trials in oncology and use of PROs**

Moderator: Erica Horodniceanu – Health Scientist, FDA

Panelists:

- Terri Armstrong – Outcomes Researcher, NCI
- Martha Donoghue – Pediatric Hematologist/Oncologist, FDA
- Wenora Johnson – Patient Advocate
- Bryce Reeve – Professor, Duke University School of Medicine
- Gita Thanarajasingam – Lymphoma Hematologist, Mayo Clinic

Key Questions:

1. *Why are open-label trial designs currently used in oncology drug development? Are they more prevalent in certain treatment settings?*
2. *Why is it important to include PROs in open-label trials and how have PROs been incorporated in this trial setting?*
3. *How might the impact of open-label bias differ depending on the PRO objective (e.g., tolerability, disease symptoms)?*
4. *Are there unique considerations for PRO core outcome measurement in open-label trials?*

11:30 AM – 11:45 AM	**Break**
11:45 AM – 1:00 PM	**Session 2: What have we learned?** **Analysis and interpretation of PRO data from open-label cancer trials**

Moderator: Vishal Bhatnagar - Medical Oncologist, FDA

Panelists:

- Ethan Basch – Chief of Oncology, University of North Carolina
- Selena Daniels – Social Science Analyst, FDA
- Mallorie Fiero – Statistician, FDA
- Jessica Roydhouse – Researcher, University of Tasmania
- Patty Spears – Patient Advocate, University of North Carolina

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Key Questions:

1. *Is it possible to use available data to determine if open-label bias has influenced PRO results (e.g., examine use of concomitant supportive medications)?*
2. *What are the differences between descriptive and comparative PRO data analyses in open-label oncology trials and how should each be interpreted?*
3. *What analytic challenges exist when a comparative treatment effect is being proposed in an open-label trial of an oncology drug?*
4. *What are ways to mitigate the potential of open-label bias in oncology trials (e.g., avoidance of asymmetric missingness)?*
5. *How can analysis of PROs from open-label trials inform improvements in routine clinical care?*

1:00 PM – 1:15 PM	**Break**
1:15 PM – 2:45 PM	**Session 3: Where do we go from here?** **Efforts to advance PRO to inform tolerability regardless of blinding status in oncology**

Moderators:

Vishal Bhatnagar - Medical Oncologist, FDA
Erica Horodniceanu – Health Scientist, FDA
Paul Kluetz – Medical Oncologist, FDA

Panelists:

- Yelak Biru – Patient Advocate, International Myeloma Foundation
- Melanie Calvert – Professor, University of Birmingham
- Angelo de Claro – Hematologist/Oncologist, FDA
- Amylou Dueck– Biostatistician, Mayo Clinic
- Devin Peipert – Assistant Professor, Northwestern University

Key Questions:

1. *What are some specific examples of PRO data in open-label trials and what are best practices to communicate these data?*
2. *Are there differences in how descriptive PRO data (similar to safety/tolerability) should be communicated versus comparative treatment effects from open-label trials?*
3. *How should PROs be used to characterize tolerability in oncology trials moving forward?*
4. *How can PRO data to inform tolerability be communicated outside of the drug label (e.g., Project Patient Voice)?*

2:45 PM – 3:00 PM	**Workshop conclusion and adjourn**

Vishal Bhatnagar, Erica Horodniceanu, Paul Kluetz