FDA-ASCO Clinical Outcome Assessment in Cancer Clinical Trials

Fifth Annual Workshop

Date: Friday, July 17, 2020

9:00 am – 4:00 pm

Drs. Paul Kluetz (FDA), Heidi Klepin (ASCO), and Kathryn Mileham (ASCO) welcome everyone to the Fifth Clinical Outcome Assessment in Cancer Clinical Trials (COA-CCT) Workshop, co-sponsored by the U.S. Food and Drug Administration (FDA) Oncology Center of Excellence (OCE) and the American Society of Clinical Oncology (ASCO). Since the first COA-CCT meeting, our overarching goal has been to provide a forum for collaborative and productive multidisciplinary discussions to advance the understanding of the complex regulatory, health care policy, and scientific issues surrounding the use of patient-reported outcome (PRO) measures in cancer clinical trials. The specific goal of this meeting will be a detailed discussion for creating regulatory-grade patient-reported data on tolerability.

This year, the workshop will be fully virtual. To provide context for this meeting, a series of interviews will be recorded and published in the weeks prior to the meeting. The link to these interviews will be made available to registered attendees prior to July 17th.

In this workshop, we will consider a hypothetical case example of a trial in which the investigational arm is a tyrosine kinase inhibitor (TKI) and the control arm is traditional cytotoxic chemotherapy being investigated for an advanced/metastatic malignancy. Each session will focus on the specific issues associated with understanding patient-reported tolerability in the context of comparing differing therapeutic classes and to provide solutions to overcome these issues. Specifically, we will discuss unbiased item selection, assessment frequency, and a global side effect bother item. Finally, with the ultimate aim of creating data that is of high quality and informs tolerability in the regulatory context, we will discuss how this information can be included in the FDA OCEs new pilot Project Patient Voice. Project Patient Voice is web-based platform for patients and caregivers, along with their health care providers to look at patient-reported symptom data collected from cancer clinical trials.

Meeting Goals:

- To provide a forum for open discussion between academia, industry, international regulatory, and patient advocacy groups to advance measurement of the patient experience as it relates to tolerability in cancer clinical trials;
- Consider how PRO instruments, assessment frequencies, and trial designs can be used to support product labeling with regard to tolerability;
- Engage key stakeholders to identify opportunities to overcome barriers to incorporation of PRO and discuss actionable next steps;
- Discuss current FDA approaches to the analysis and review of PRO data, specifically PROs related to tolerability submitted to FDA to support cancer product applications; and
- To receive feedback on Project Patient Voice pilot

Morning Session
9:00 AM – 11:00 AM

9:00 – 9:05 - Opening Remarks/Welcome

- Paul Kluetz – FDA OCE
- Heidi Klepin – Wake Forest University Baptist Medical Center/ASCO
- Kathryn Mileham – Levine Cancer Institute, Atrium Health/ASCO

9:05 – 10:00 AM – Session 1: Identifying best methods for item selection to assess tolerability

Moderator: Vishal Bhatnagar – FDA OCE
Panelists:

- Kathryn Mileham - Levine Cancer Institute, Atrium Health
- Arlene Chung – University of North Carolina
- Mary Scroggins – Patient Advocate (International Gynecologic Cancer Society; Pinkie Hugs, LLC)
- Peter Trask - Genentech
- Maxime Sasseville – Health Canada
- Ethan Basch – University of North Carolina

Objectives:

- Explore strategies to identify relevant patient-reported symptomatic adverse events in cancer clinical trials
- Discuss how the use of PROs can complement clinician safety data
- Consider strategies to reducing patient burden for item selection
- Highlight challenges faced by industry in unbiased item selection in the context of tolerability
- Discuss how to involve patients in item selection to inform safety and tolerability of medical products

10:00 AM – 10:05 AM **Break**

10:05 AM – 11 AM – Session 2: Considerations for assessment frequency and how it relates to the measurement of tolerability

Moderator: Bellinda King-Kallimanis – FDA OCE
Panelists:

- Heidi Klepin – Wake Forest University Baptist Medical Center
- Sundeep Agarwal – FDA, CDER Clinical
- Joyce Cheng – FDA, CDER, Statistics
- Wendy Sanhai – Patient Advocate
- Diane Fairclough – University of Colorado
- Jim Shaw – Bristol-Myers Squibb

Objectives:

- Characterize challenges related to assessment frequency across treatment contexts in cancer clinical trials
- Could collection of PROs at end of treatment improve our understanding of tolerability?
- Consider how windowing rules for assessments (i.e. ± number of days around scheduled assessment) can affect the apparent tolerability
- Explore opportunities and challenges of onsite and remote PRO assessments

Break
11:00 AM – 12:00 Noon

Lunch Session
12:00 Noon – 1:00 PM

<u>12:00 Noon – 1:00 PM</u> – Session 3: An interactive panel discussion to explore how a global item capturing side effect bother complements the picture of tolerability

Moderator: Paul Kluetz – FDA OCE
Panelists:

- Laura Fernandes – FDA, CDER, statistics
- Preeti Narayan – FDA, CDER Clinical
- Janet Freeman-Daily – Patient Advocate
- Sandra Spivey – Patient Advocate
- Sandy Mitchell – National Cancer Institute, NIH
- Dave Cella – Northwestern University
- Gita Thanarajasingam - Mayo Clinic

Objectives for Q&A with panelists:

- Discuss patient perspectives on global side effect bother and relevance to understanding the tolerability of the drug from the standpoint of the patient
- Identify challenges in interpreting side effect bother in the context of other patient-reported single items
- Discuss endpoints that could be assessed using global side effect bother

Break
1:00 PM – 2:00 PM

Afternoon Session
2:00 PM – 4:00 PM

<u>2:00 PM- 2:50 PM</u> – Session 4: Project Patient Voice - Methodological Perspectives

Project Patient Voice is a pilot web-based platform for patients and caregivers, along with their health care providers to look at patient-reported symptom data collected from cancer clinical trials

Moderator: Janice Kim
Speakers/Panelists:

- Paul Kluetz – FDA OCE
- Vishal Bhatnagar – FDA OCE
- Mallorie Fiero – FDA Stats
- Katarina Halling - AstraZeneca
- Claire Snyder – Johns Hopkins University
- Lori Minasian – National Cancer Institute

Session 4
Objectives:

- Provide an overview of Project Patient Voice and its potential uses in cancer clinical care
- Discuss optimal visualization methods to disseminate complex data
- Outline challenges and benefits to participating in the Project Patient Voice pilot from the industry perspective
- Discuss design challenges and consider ways to improve usability of future versions

2:50 PM – 3:00 PM **Break**

3:00 PM – 3:50 PM – Session 5: Project Patient Voice – Patient & Health Care Provider Perspective

Moderator: Bellinda King-Kallimanis – FDA OCE
Speakers/Panelists:

- Christine Hodgdon – Patient Advocate
- Lee Jones – Patient Advocate
- Ashley Housten – Washington University in St. Louis
- Karen Smith – Sibley Memorial Hospital/Johns Hopkins University
- Adedayo Onitilo – Marshfield Clinic Health System

Objectives:

- Hear from patients, caregivers, and providers about their experience with Project Patient Voice
- Seek feedback from the community about potential improvements to Project Patient Voice and the next version
- Understand how community clinicians have utilized Project Patient Voice to inform their understanding of tolerability

3:50 – 4:00 PM - Workshop Wrap-Up

Closing comments from:

- Paul Kluetz
- Heidi Klepin
- Kathryn Mileham

4:00 PM - Adjourn