Public Webinar

Patient-Focused Drug Development: Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision Making– Draft Guidance

May 4, 2023

1:00 p.m. Welcome and Overview of PFDD Methodological Guidance Series

Shannon Sparklin, Patient-Focused Drug Development (PFDD), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)

Laura Lee Johnson, Division of Biometrics III (DBIII), Office of Biostatistics (OB), Office of Translational Sciences (OTS), CDER, FDA

Constructing and Analyzing Endpoints Based on Clinical Outcome Assessments

1:05 p.m. Constructing COA-based Endpoints

Lili Garrard, DBIII, OB, OTS, CDER, FDA

1:35 p.m. Obtaining Patient Input to Inform Selection of COA-based Endpoints

Arthur Stone, Director, USC Dornsife Center for Self-Report Science, University of Southern California (USC)

1:50 p.m. Analyzing COA-based Endpoints

Yuqun Abigail Luo, Therapeutics Evaluation Branch 2 (TEB 2), Division of Biostatistics (DB), Office of Biostatistics and Pharmacovigilance (OBPV), Center for Biologics Evaluation and Research (CBER), FDA

1:55 p.m. Clinician Perspective

Hylton Joffe, Office of Cardiology, Hematology, Endocrinology and Nephrology (OCHEN), Office of New Drugs (OND), CDER, FDA

Evaluating Meaningfulness of Treatment Benefit

2:05 p.m. Introduction to Evaluating Meaningfulness of Treatment Benefit

David Reasner, Division of Clinical Outcome Assessment (DCOA), Office of New Drugs (OND), CDER, FDA

2:10 p.m. Approaches for Collecting Evidence to Support Interpretability of COA-based Endpoints

Fraser Bocell, Division of All Hazards Response, Science and Strategic Partnerships (DAHRSSP), Office of Strategic Partnerships and Technology Innovation (OST), Center for Devices and Radiological Health (CDRH), FDA

2:25 p.m. Applying Information about Meaningful Score Differences or Meaningful Score Regions to Clinical Trial Data

Monica Morell, DBIII, OB, OTS, CDER, FDA

2:45 p.m. Question & Answer

Moderator: Shannon Sparklin, PFDD, CDER, FDA

Panelists:

- David Reasner, DCOA, OND, CDER, FDA
- Laura Lee Johnson, DBIII, OB, OTS, CDER, FDA
- Yuqun Abigail Luo, TEB 2, DB, OBPV, CBER, FDA
- Fraser Bocell, DAHRSSP, OST, CDRH, FDA
- Monica Morell, DBIII, OB, OTS, CDER, FDA
- Arthur Stone, University of Southern California

3:00 p.m. Conclusion / End