PATIENT-FOCUSED DRUG DEVELOPMENT

Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision-Making

December 6, 2019

9:00 a.m. **Welcome**

Meghana Chalasani, Office of the Center Director (OCD), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)

9:05 a.m. **Opening Remarks**

Theresa Mullin, OCD, CDER, FDA

9:15 a.m. **Overview of FDA's Approach to Patient-Focused Drug Development (PFDD) Guidance 4**

Scott Komo, Office of Translational Sciences (OTS), CDER, FDA

9:30 a.m. **Session I: General Considerations for Developing an Endpoint From Clinical Outcome Assessment (COA) Data**

Objective

Explore and discuss factors that need to be considered when developing COA-based endpoints.

Introduction

Martin Ho, Office of Biostatistics and Epidemiology (OBE), Center for Biologics Evaluation and Research (CBER), FDA (moderator)

Moderated Panel Discussion

- Fraser Bocell, Office of Strategic Partnerships and Technology Innovation (OST), Center for Devices and Radiological Health (CDRH), FDA
- Kendra Hileman, Vice President, Head of Clinical Research and Development, Alcon
- Hylton Joffe, Office of New Drugs (OND), CDER, FDA
- Larissa Lapteva, Office of Tissues and Advanced Therapies (OTAT), CBER, FDA
- Gianna (Gigi) McMillen, Patient Advocate and Program Administrator, Bioethics Institute at Loyola Marymount University
- Linda Nelsen, Senior Director and Head, Patient-Centered Outcomes, GlaxoSmithKline
- Kevin Weinfurt, Professor and Vice Chair for Research, Department of Population Health Sciences, Duke University School of Medicine

Audience Question and Answer

10:30 a.m. **Break**

10:45 a.m. Session II: Using the Estimand Framework to Design, Conduct, and Analyze Data From a Trial With a COA-Based Endpoint

Objective

Introduce and discuss approaches for identifying the appropriate analysis population, determining clinical trial and COA timing, and adjusting for potential confounders or intercurrent events.

Introduction

Mallorie Fiero, OTS, CDER, FDA (moderator)

Moderated Panel Discussion

- Jessica Lee, OND, CDER, FDA
- Gregory Levin, OTS, CDER, FDA
- John Scott, OBE, CBER, FDA
- Daniel Serrano, Director of Psychometrics, Pharmerit
- Kevin Weinfurt, Professor and Vice Chair for Research, Department of Population Health Sciences, Duke University School of Medicine
- Lisa Weissfeld, Senior Investigator, Statistics Collaborative

Audience Question and Answer

12:00 p.m. Lunch

1:00 p.m. Session III: Considerations When There Is Heterogeneity in Disease Symptoms and Functional Status Between Patients and Within the Same Patient Over Time

Objective

Discuss considerations for COA measurement and analysis for diseases with heterogeneous patient populations and/or variable manifestations.

Introduction

Lili Garrard, OTS, CDER, FDA (moderator)

Moderated Panel Discussion

- Lisa Kammerman, Regulatory Statistics and PRO Consultant, Kammerman Consulting, LLC
- Elektra Papadopoulos, OND, CDER, FDA
- Tejashri Purohit-Sheth, OTAT, CBER, FDA
- David Reasner, Head of Data Science & Analytics, Imbria Pharmaceuticals
- Steve Roberds, Chief Scientific Officer, Tuberous Sclerosis Alliance
- Patroula Smpokou, OND, CDER, FDA
- R.J. Wirth, President and Managing Partner, Vector Psychometric Group

Audience Question and Answer

2:00 p.m. Break

2:15 p.m. Session IV: Pulling It All Together – An Example Across Guidances

Objective

Discuss a working example – Information from this panel session will inform the development of a case study example illustrating important concepts for consideration in the collection of COA data within the clinical trial context.

Introduction

Ebony Dashiell-Aje, OND, CDER, FDA (moderator)

Moderated Panel Discussion

- Bill Byrom, Vice President of Product Strategy and Innovation, Signant Health
- Michelle Campbell, OND, CDER, FDA
- Andrea Coravos, Co-founder and Chief Executive Officer, Elektra Labs
- Matthew Diamond, OST, CDRH, FDA
- Mark Frasier, Senior Vice President, Research Programs, The Michael J. Fox Foundation for Parkinson's Research
- Abigail Luo, OBE, CBER, FDA
- Andrew Potter, OTS, CDER, FDA
- Diane Stephenson, Executive Director, Critical Path for Parkinson's Consortium, Critical Path Institute

Audience Question and Answer

3:20 p.m. Session V: Identifying Key Themes and Rounding Out the Guidance Series

Objective

Reflect on the day's discussion, specifically any themes that emerged throughout the day. Discuss key considerations that should guide FDA's completion of its methodological PFDD guidance series.

Introduction

Meghana Chalasani, OCD, CDER, FDA (moderator)

Moderated Panel Discussion

- Marc Boutin, Chief Executive Officer, National Health Council
- Stephen Joel Coons, Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute
- Katarina Halling, Global Head Patient Centered Science, AstraZeneca
- Telba Irony, OBE, CBER, FDA
- Laura Lee Johnson, OTS, CDER, FDA
- Pandu Kulkarni, Vice President, Biometrics and Advanced Analytics, Eli Lilly and Company
- Michelle Tarver, OST, CDRH, FDA

Audience Question and Answer

4:30 p.m. Open Public Comment

Mary Jo Salerno, OTS, CDER, FDA (moderator)

4:50 p.m. Closing Remarks

Laura Lee Johnson, OTS, CDER, FDA