PATIENT-FOCUSED DRUG DEVELOPMENT

Using Methods from PFDD Guidance 1 and Guidance 2 as Tools for Including Patient Experience Data in Clinical Trials: Who to Ask and How to Ask

June 30, 2022

11:00 a.m. **Welcome**

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

11:05 a.m. **Opening Remarks**

Theresa Mullin, Office of the Center Director (OCD), CDER, FDA

11:10 a.m. **Overview of Patient-Focused Drug Development (PFDD)**

Robyn Bent, PFDD, CDER, FDA

11:15 a.m. **Session I: Research Methods to Identify and Understand What Matters to Patients**

11:15 a.m. **Approaches to Collecting Patient Input and Selection of Data Collection Methods**
Selena Daniels, Division of Clinical Outcome Assessment (DCOA), Office of New Drugs (OND), CDER, FDA

Naomi Knoble, DCOA, OND, CDER, FDA

11:45 a.m. **Who to Collect Information From: Sampling Plans and Strategies**
Laura Lee Johnson, Division of Biometrics III (DBIII), Office of Biostatistics (OB), Office of Translational Sciences (OTS), CDER, FDA

12:05 p.m. **Session II: Ideas in Practice**

12:05 p.m. **Applications of PFDD Guidance 1 and Guidance 2 as Tools for Generating Patient Experience Data to Support Medical Product Development – Who to Ask and How to Ask**
Ebony Dashiell-Aje, BioMarin Pharmaceutical Inc.

12:15 p.m. **Progress on the Science of Patient Input - PFDD Guidance Documents 1 and 2 and IMI PREFER EMA Qualification Procedure & Recommendations**
Becky Noel, Eli Lilly and Company

12:25 p.m. **Patient Focused Drug Development Guidances 1 and 2: A Patient Advocacy Perspective**
Bellinda King-Kallimanis, LUNGevity

12:35 p.m. **Clinical Regulatory Perspective**
Erica Lyons, Division of Gastroenterology (DG), OND, CDER, FDA

12:45 p.m. **Session III: Question and Answer**
Moderator: Robyn Bent, PFDD, CDER FDA
Panelists:

- Selena Daniels, DCOA, OND, CDER, FDA
- Naomi Knoble, DCOA, OND, CDER, FDA
- Laura Lee Johnson, DBIII, OB, OTS, CDER, FDA
- Becky Noel, Eli Lilly and Company
- Bellinda King-Kallimanis, LUNGevity
- Erica Lyons, DG, OND, CDER, FDA

1:00 p.m. **End**