Tenth Annual Patient-Reported Outcome Consortium Workshop

April 24 – 25, 2019

Sheraton Silver Spring Hotel
8777 Georgia Avenue
Silver Spring, MD 20910

On April 24-25, 2019 the *Tenth Annual Patient-Reported Outcome Consortium Workshop* was held in Silver Spring, Maryland. The overall Workshop objectives were to:

- Provide an update on FDA's Clinical Outcome Assessment (COA) Qualification Program and other initiatives;
- Describe new and emerging approaches to the assessment of clinical outcomes in rare diseases;
- Discuss efforts to qualify a performance outcome assessment tool for early Alzheimer's disease;
- Explore the construction of clinical endpoints using activity monitor data in chronic heart failure trials;
- Discuss how sponsors and eCOA providers can work together to optimize COA data collection in trials; and
- Describe ways to use patient input to estimate meaningful within-patient change at the scale score level.

The following Workshop Agenda provides an overview of the day-and-a-half-long meeting as well as links to the slide sets and posters presented.

Request Session Recordings

Agenda – Day 1

7:30–8:30 am	**Registration and Continental Breakfast – Cypress Ballroom**
	Day 1 Morning Moderator: *Michelle Campbell, PhD* – Senior Clinical Analyst for Stakeholder Engagement and Clinical Outcomes, Division of Neurology Products (DNP), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)
8:30–8:50 am	**Welcome and Patient-Reported Outcome Consortium Update** *Stephen Joel Coons, PhD* – Executive Director, Patient-Reported Outcome (PRO) Consortium, Program Officer, Clinical Outcome Assessment Program, Critical Path Institute (C-Path)

8:50–10:20 am	**Session 1: Update from FDA** **Moderator:** *Michelle Campbell, PhD* – Senior Clinical Analyst for Stakeholder Engagement and Clinical Outcomes, DNP, OND, CDER, FDA **Presenters:***Peter Stein, MD* – Director, OND, CDER, FDA*Elektra Papadopoulos, MD, MPH* – Associate Director, Clinical Outcome Assessments (COA) Staff, OND, CDER, FDA *Michelle Campbell, PhD* – Senior Clinical Analyst for Stakeholder Engagement and Clinical Outcomes, DNP, OND, CDER, FDA **Q & A**
10:20–10:45 am	**Break – 25 min**
10:45 am–12:15 pm	**Session 2: Leveraging Information that Can Inform the Evaluation of Clinical Benefit in Rare Diseases** **Moderator:** *Michelle Campbell, PhD* – Senior Clinical Analyst for Stakeholder Engagement and Clinical Outcomes, DNP, OND, CDER, FDA **Presenters:***Lucas Kempf, MD* – Associate Director, Rare Diseases Program (acting), OND, CDER, FDA*Dylan Trundell, MSc* – Senior Outcomes Research Scientist, Patient-Centered Outcomes Research, Roche *Mindy Leffler, MEd* – President, Casimir **Panelists:** *Billy Dunn, MD* – Director, Division of Neurology Products**,** OND, CDER, FDA *Lili Garrard, PhD* – Senior Statistical Reviewer, Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences (OTS), CDER, FDA *Montserrat Vera-Llonch, MD, MPH, MSc* – Senior Director, Global Outcomes Research and Epidemiology, Takeda **Q & A**
12:15–1:15 pm	**Lunch – Magnolia Ballroom, Elm I and Elm II**
	Day 1 Afternoon Moderator: *Linda M. Nelsen, MHS* – Senior Director and Head, Patient Centered Outcomes, Value Evidence and Outcomes, GlaxoSmithKline and Industry Co-Director, PRO Consortium

1:15–2:45 pm	**Session 3: Cognition Working Group Case Study** **Moderator:** *Katy Benjamin, PhD* – Director, HEOR, Patient Centered Outcomes, AbbVie Inc. **Presenters:***Richard Keefe, PhD* – CEO, VeraSci, Inc., Professor of Psychiatry, Psychology and Neurosciences, Duke University Medical Center*Philip D. Harvey, PhD* – Leonard M. Miller Professor of Psychiatry, University of Miami Miller School of Medicine *Katy Benjamin, PhD* – Director, HEOR, Patient Centered Outcomes, AbbVie Inc. **Panelist:** *Billy Dunn, MD* – Director, Division of Neurology Products**,** OND, CDER, FDA **Q & A**
2:45–3:10 pm	**Break – 25 min**
3:10–4:40 pm	**Session 4: Endpoint Construction from Activity Monitor Data: Chronic Heart Failure** **Moderator:** *Maria Mattera, MPH* – Assistant Director, PRO Consortium, C-Path **Presenters:***Chad Gwaltney, PhD* – President, Gwaltney Consulting*Maria Mattera, MPH* – Assistant Director, PRO Consortium, C-Path *Jeremiah (Jay) Trudeau, PhD* – Director, Patient-Reported Outcomes, Janssen Global Services *Wayne Amchin, RAC, MIA, MPA* – Senior Consumer Safety Officer, Division of Cardiovascular and Renal Products, OND, CDER, FDA *Bill Byrom, PhD* – Vice President of Product Strategy and Innovation, CRF Bracket **Panelist:** *Ebony Dashiell-Aje, PhD* – Reviewer, COA Staff, OND, CDER, FDA **Q & A**
4:40–5:00 pm	**Day 1 Closing Remarks** – *Linda Nelsen* and *Sonya Eremenco* **Adjourn**
5:00–5:30 pm	**Open**

5:30–7:00 pm	**Reception and Poster Session – Magnolia Ballroom** Asthma Working Group Chronic Heart Failure Working Group Cognition Working Group Depression Working Group Functional Dyspepsia Working Group Electronic Patient-Reported Outcome Consortium Irritable Bowel Syndrome Working Group Multiple Sclerosis Working Group Pediatric Asthma Working Group Rheumatoid Arthritis Working Group

Agenda – Day 2

7:30–8:30 am	**Registration and Continental Breakfast – Cypress Ballroom**
	Day 2 Moderator: *Sonya Eremenco, MA* – Associate Director, PRO Consortium and Acting Director, Electronic Patient-Reported Outcome (ePRO) Consortium, C-Path
8:30–10:00 am	**Session 5: eCOA – Continuing to Get Better Together** **Moderator:** *Sonya Eremenco, MA* – Associate Director, PRO Consortium and Acting Director, ePRO Consortium, C-Path **Presenters:***Katherine Zarzar* – Senior Manager, Patient-Centered Outcomes Research**,** Genentech, A Member of the Roche Group *Paul O'Donohoe, MSc* – Scientific Lead, eCOA and Mobile Health**,** Medidata Solutions and Vice Director, ePRO Consortium *Alexandra (Alex) Barsdorf, PhD* – Director, Clinical Outcome Assessments, Clinical Outcomes Solutions *Megan Turner* – Scientist, COA Implementation, Value Evidence and Outcomes, GlaxoSmithKline *Patricia (Trish) Shepherd Delong, MS* – Manager, Patient-Reported Outcomes, Global Commercial Strategy Organization (GCSO), Janssen *Andres Escallon, DM* – Director, eCOA Clinical Data Management, ERT **Panelist:** *David Reasner, PhD* – Vice President, Data Science and Head, Study Endpoints, Ironwood Pharmaceuticals **Q & A**
10:00–10:25 am	**Break – 25 min**

10:25–11:55 am	**Session 6: Using Patient Input to Estimate Clinically Meaningful Within-Patient Change at the Scale Score Level** **Moderator:** *Lori McLeod*, PhD – Vice President, Patient-Centered Outcomes Assessment, RTI Health Solutions **Presenters:***Jean Paty PhD* – Vice President and Head of Patient Centered Endpoints, IQVIA*Kate Sully, PhD* – Senior Research Manager, Patient-Centered Outcomes, Adelphi Values *Kelly McCarrier, PhD, MPH* – Director and Qualitative Research Lead, Pharmerit International *Cheryl D. Coon, PhD* – Principal, Outcometrix **Panelists:** *Scott Komo, DrPH* – Team Leader, Division of Biometrics III, Office of Biostatistics, OTS, CDER, FDA *Michelle Campbell, PhD* – Senior Clinical Analyst for Stakeholder Engagement and Clinical Outcomes, DNP, OND, CDER, FDA **Q & A**
11:55 am–12:15 pm	**Closing Remarks** – *Stephen Coons* **Adjourn**