14th Annual Patient-Reported Outcome Consortium Workshop

April 19 – 20, 2023

DoubleTree by Hilton Silver Spring DC North
8777 Georgia Avenue
Silver Spring, MD 20910

On April 19–20, 2023, the *14th Annual Patient-Reported Outcome Consortium Workshop* was held in Silver Spring, MD.

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

Agenda – Day 1

7:30–8:30 am	**Registration and Breakfast – Cypress Ballroom**	
	Day 1 Morning Moderator: *Michelle Campbell, PhD* – Associate Director, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience (ON), 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** **Overview:** Provide a high-level summary of the recent accomplishments and ongoing activities within the Patient-Reported Outcome Consortium **Presenter:** *Sonya Eremenco, MA* – Executive Director, Patient-Reported Outcome (PRO) Consortium, Critical Path Institute (C-Path)	

8:50–10:20 am	**Session 1: FDA Update** **Overview:** Provide an update on FDA's Clinical Outcome Assessment Qualification Program and other FDA initiatives **Moderator:** *Michelle Campbell, PhD* – Associate Director, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience, Office of New Drugs, Center for Drug Evaluation and Research, U.S. Food and Drug Administration **Presenters:** *Robyn Bent, RN, MS* – Director, Patient-Focused Drug Development Program, Center for Drug Evaluation and Research, U.S. Food and Drug Administration *Selena Daniels, PharmD, PhD* – Clinical Outcome Assessment Team Leader, Division of Clinical Outcome Assessment, Office of Drug Evaluation Sciences, Office of New Drugs, Center for Drug Evaluation and Research, U.S. Food and Drug Administration *Lili Garrad, PhD* – Lead Mathematical Statistician Team Leader, Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences, Center for Drug Evaluation and Research, U.S. Food and Drug Administration *Laura Lee Johnson, PhD* – Director, Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences, Center for Drug Evaluation and Research, U.S. Food and Drug Administration *Jessica Mavadia-Shukla, PhD* – Program Director, Medical Device Development Tools, Center for Devices and Radiological Health, U.S. Food and Drug Administration *David S. Reasner, PhD* – Division Director, Division of Clinical Outcome Assessment, Office of Drug Evaluation Sciences, Office of New Drugs, Center for Drug Evaluation and Research, U.S. Food and Drug Administration **Q & A**	
10:20–10:45 am	**Break – 25 min**	

10:45–12:15 pm	**Session 2: Patient Experience Data: Use in Regulatory Decision Making and Labeling** **Overview:** Discuss patient experience data and its use in regulatory decision making **Moderator:** *Sonya Eremenco, MA* – Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute **Presenters and Panelists:** *Robyn Bent, RN, MS* – Director, Patient-Focused Drug Development Program, Center for Drug Evaluation and Research, U.S. Food and Drug Administration *Michelle Campbell, PhD* – Associate Director, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience, Office of New Drugs, Center for Drug Evaluation and Research, U.S. Food and Drug Administration *Ellyn Kodroff, BS* – President and Founder, CURED (Campaign Urging Research for Eosinophilic Diseases) Nfp *Sarrit Kovacs, PhD* – Clinical Reviewer, Division of Gastroenterology, Office of Immunology and Inflammation, Office of New Drugs, Center for Drug Evaluation and Research, U.S. Food and Drug Administration *Marc Yale* –Advocacy and Research Coordinator, International Pemphigus Pemphigoid Foundation (IPPF) **Q & A**	
12:15–1:15 pm	**Lunch – Magnolia Ballroom, Elm I and Elm II**	
	Day 1 Afternoon Moderator: *Josephine Norquist, MS* – Executive Director, Patient-Centered Endpoints & Strategy Lead, Merck & Co., Inc. and Industry Co-Director, Patient-Reported Outcome Consortium	

1:15–2:45 pm	**Session 3: Qualitative Methods in Clinical Trials: Opportunities and Challenges** **Overview:** Discuss methodological approaches to qualitative data collection in clinical trials, and the opportunities and challenges involved **Moderator:** *Maria Mattera, MPH* – Scientific Director, Patient-Reported Outcome Consortium, Critical Path Institute **Presenters:** *Asha Hareendran, PhD* – Patient Centred Outcomes Research (PCOR) Excellence Lead, UCB Biopharma Srl *Nicola Williamson, MSc* – Associate Director, Patient-Centered Outcomes, Adelphi Values *Jane R. Wells, MSc* – Clinical Outcomes Assessment Lead, Sanofi *Calvin N. Ho, PhD* – Associate Director, Patient Centered Science, AstraZeneca *Bellinda King-Kallimanis, PhD* – Director of Patient-Focused Research, LUNGevity Foundation **Panelists:** *Naomi Knoble, PhD* – Associate Director of Rare Disease Measurement Science, Division of Clinical Outcome Assessment, Office of Drug Evaluation Sciences, Office of New Drugs, Center for Drug Evaluation Research, U.S. Food and Drug Administration *Marc Yale* – Advocacy and Research Coordinator, International Pemphigus Pemphigoid Foundation (IPPF) **Q & A**	
2:45–3:10 pm	**Break – 25 min**	

3:10–5:00 pm	**Session 4: Applying Methodologies for Estimating Meaningful Within-person Change Thresholds: Considerations and Alternative Approaches** **Overview:** Examine four examples that advance the estimation and use of meaningful within-person change thresholds **Moderator:** *Rebecca M. Speck, PhD, MPH* – Clinical Outcome Assessment Scientist, Eli Lilly and Company **Presenters:** *Elizabeth (Nicki) Bush, MHS* – Senior Director, Endpoints and Measurement Strategy, Janssen Pharmaceutical Companies of Johnson & Johnson *Devin Peipert, PhD* – Assistant Professor of Medical Social Sciences, Northwestern University *Karon Cook, PhD* – Research Professor (Retired), Feinberg School of Medicine, Northwestern University *Bill Byrom, PhD* – Vice President, Product Intelligence and Positioning, and Principal, eCOA Science, Signant Health **Panelists:** *Selena Daniels, PharmD, PhD* – Clinical Outcome Assessment Team Leader, Division of Clinical Outcome Assessment, Office of Drug Evaluation Sciences, Office of New Drugs, Center for Drug Evaluation and Research, U.S. Food and Drug Administration *Monica Morell, PhD* – Patient-Focused Statistical Support Reviewer, Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences, Center for Drug Evaluation and Research, U.S. Food and Drug Administration **Q & A**	
5:00–5:10 pm	**Day 1 Closing Remarks** *Sonya Eremenco, MA* – Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute**Adjourn**	
5:10–5:30 pm	**Open**	

5:30–7:00 pm	**Reception and Poster Session – Magnolia Ballroom**Chronic Heart Failure Working Group Cognition Working Group Depression Working Group 2.0 Functional Dyspepsia Working Group Irritable Bowel Syndrome Working Group Multiple Sclerosis Working Group Pediatric Asthma Working Group Rheumatoid Arthritis Working Group Small Cell Lung Cancer Working Group Rare Disease COA Consortium eCOA Consortium	

Agenda – Day 2

7:30–8:30 am	**Registration and Breakfast – Cypress Ballroom**	
	Day 2 Moderator:*Lindsey Murray, PhD, MPH* – Executive Director, Rare Disease Clinical Outcome Assessment Consortium, Critical Path Institute	

8:30–10:00 am	**Session 5: Strategies for Use of COAs in Rare Disease Pediatric Populations** **Overview:** Explore methodological challenges in research involving rare pediatric populations, especially in children less than 5 years of age **Moderator:** *Lindsey Murray, PhD, MPH* – Executive Director, Rare Disease Clinical Outcome Assessment Consortium, Critical Path Institute **Presenters:** *Lindsey Murray, PhD, MPH* – Executive Director, Rare Disease Clinical Outcome Assessment Consortium, Critical Path Institute *Dawn Phillips, PT, MS, PhD* – Director, Clinical Scientist, Outcomes Research, REGENXBIO Inc. *Ebony Dashiell-Aje, PhD* – Executive Director & Head, Patient Centered Outcomes Science, BioMarin Pharmaceutical, Inc. *Naomi Knoble, PhD* – Associate Director of Rare Disease Measurement Science, Division of Clinical Outcome Assessment, Office of Drug Evaluation Sciences, Office of New Drugs, Center for Drug Evaluation Research, U.S. Food and Drug Administration **Panelists:** *Teresa Buracchio, MD* – Director (Acting), Office of Neuroscience, Office of New Drugs, Center for Drug Evaluation and Research, U.S. Food and Drug Administration *Emily Freilich, MD* – Deputy Director (Acting), Division of Neurology I, Office of Neuroscience, Center for Drug Evaluation and Research, U.S. Food and Drug Administration *Cara O'Neill, MD* – Chief Science Officer, Cure Sanfilippo Foundation **Q & A**	
10:00–10:25 am	**Break – 25 min**	

10:25–11:55 am	**Session 6: eCOA: COA Program Projects and Collaborations Update** **Overview:** Explore two collaborative projects leveraging sensor-based measurement of activity and discuss the challenges involved in assessing algorithms for deriving interpretable metrics from raw sensor data **Moderator:** *Scottie Kern, BSc (Hons)* – Executive Director, Electronic Clinical Outcome Assessment Consortium, Critical Path Institute **Presenters:** *Scottie Kern, BSc (Hons)* – Executive Director, Electronic Clinical Outcome Assessment Consortium, Critical Path Institute *Josephine Norquist, MS* – Executive Director, Patient-Centered Endpoints & Strategy Lead, Merck & Co., Inc. and Industry Co-Director, Patient-Reported Outcome Consortium *Christine Guo, PhD* – Chief Scientific Officer, ActiGraph *Ronenn Roubenoff, MD, MHS* – Global Head, Translational Medicine Discovery & Profiling, Novartis Institutes for Biomedical Research *Gül Erdemli, MD, PhD* – Global Program Regulatory Director, Novartis Pharmaceuticals Corporation **Panelist:** *David S. Reasner, PhD* – Division Director, Division of Clinical Outcome Assessment, Office of Drug Evaluation Sciences, Office of New Drugs, Center for Drug Evaluation and Research, U.S. Food and Drug Administration **Q & A**	
11:55–12:15 pm	**Workshop Wrap Up** *Sonya Eremenco, MA* – Patient-Reported Outcome Consortium, Critical Path Institute *Cheryl D. Coon, PhD* – Vice President, Clinical Outcome Assessment Program, Critical Path Institute **Adjourn**	