

## **Ninth Annual Patient-Reported Outcome Consortium Workshop**

**April 25 – 26, 2018**

**Sheraton Silver Spring Hotel**

**8777 Georgia Avenue**

**Silver Spring, MD 20910**

On April 25-26, 2018 the NINTH 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 address changes associated with the 21<sup>st</sup> Century Cures Act and PDUFA VI;
- Describe the development of the three versions of the *Diary of Irritable Bowel Syndrome Symptoms (DIBSS)*;
- Discuss ways in which clinical trial sponsors and eCOA system providers can work collaboratively to optimize electronic COA data collection in trials;
- Describe results of projects aimed at advancing the science of clinical trial data collection by leveraging available and emerging technologies;
- Provide multiple stakeholders' perspectives regarding the challenges and opportunities associated with the application of existing PRO measures in drug development; and
- Discuss some emerging approaches to outcome assessment in rare diseases and pediatric populations.

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	<b>Registration and Continental Breakfast – Cypress Ballroom</b>
	<b>Day 1 Morning Moderator:</b> <i>Michelle Campbell, PhD</i> – Reviewer and Scientific Coordinator, Clinical Outcome Assessments (COA) Qualification Program, COA Staff, Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)
8:30–8:50 am	<a href="#"><u>Welcome and Patient-Reported Outcome Consortium Update</u></a> <i>Stephen Joel Coons, PhD</i> – Executive Director, Patient-Reported Outcome (PRO) Consortium, Critical Path Institute (C-Path)

8:50–10:20 am	<p><b><u>Session 1: Update from FDA Regarding the Clinical Outcome Assessment Qualification Program</u></b></p> <p><b>Moderator:</b> <i>Michelle Campbell, PhD</i> – Reviewer and Scientific Coordinator, COA Qualification Program, COA Staff, OND, CDER, FDA</p> <p><b>Presenter:</b> <i>Elektra Papadopoulos, MD, MPH</i> – Associate Director, COA Staff, OND, CDER, FDA</p> <p><b>Panelists:</b></p> <p><i>Laura Lee Johnson, PhD</i> – Acting Director, Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences, CDER, FDA</p> <p><i>Theresa Mullin, PhD</i> – Associate Director for Strategic Initiatives, CDER, FDA</p> <p><b>Q &amp; A</b></p>
10:20–10:45 am	<p><b>Break – 25 min</b></p>
10:45 am–12:15 pm	<p><b><u>Session 2: Case Study: The Diary of Irritable Bowel Syndrome Symptoms (DIBSS)</u></b></p> <p><b>Moderator:</b> <i>Jennifer Hanlon, MPH</i> – Associate Director, Study Endpoints, Ironwood Pharmaceuticals</p> <p><b>Presenters:</b> <i>Claire Ervin, MPH</i> – Senior Director, Patient-Centered Outcomes Assessment, RTI Health Solutions</p> <p><i>Lori McLeod, PhD</i> – Vice President, Patient-Centered Outcomes Assessment, RTI Health Solutions</p> <p><i>Adam Butler</i> – Senior Vice President, Strategic Development, Bracket</p> <p><i>Robyn Carson, MPH</i> – Executive Director and Head, Patient-Centered Outcomes Research, Allergan</p> <p><b>Panelists:</b></p> <p><i>Stephen Joel Coons, PhD</i> – Executive Director, PRO Consortium, C-Path</p> <p><i>Sheri Fehnel, PhD</i> – Vice President, Patient-Centered Outcomes Assessment, RTI Health Solutions</p> <p><i>Sarrit Kovacs, PhD</i> – Reviewer, COA Qualification Program, COA Staff, OND, CDER, FDA Q &amp; A</p> <p><b>Q &amp; A</b></p>
12:15–1:15 pm	<p><b>Lunch – Cedar, Walnut, Persimmon I and Persimmon II Rooms (First Floor)</b></p>

	<p><b>Day 1 Afternoon Moderator:</b> <i>Elizabeth (Nicki) Bush, MHS</i> – Director, Patient-Focused Outcomes Center of Expertise, Eli Lilly and Company and Industry Co-Director, PRO Consortium</p>
1:15–2:45 pm	<p><b><u>Session 3: eCOA: How Do We Get Better Together?</u></b></p> <p><b>Moderator:</b> <i>Jean Paty, PhD</i> – Vice President, Consulting Services, Leading Patient Centered Endpoints Activities, QuintilesIMS</p> <p><b>Presenters:</b> <i>Emily Nash Smyth, PharmD</i> – Senior Research Scientist, Global Patient Outcomes and Real World Evidence, Early Phase Oncology, Eli Lilly and Company</p> <p><i>Paul O’Donohoe, MSc</i> – Scientific Lead, eCOA and Mobile Health, Medidata Solutions</p> <p><i>Kristina Lowe, BS</i> – Vice President, Business Development, ERT</p> <p><i>Katie Zarzar</i> – Senior Manager, Patient-Centered Outcomes Research, Genentech, A Member of the Roche Group</p> <p><b>Panelists:</b></p> <p><i>Robyn Carson, MPH</i> – Executive Director and Head, Patient-Centered Outcomes Research, Allergan</p> <p><i>Katarina Halling, MSc</i> – Global Head Patient Reported Outcomes, AstraZeneca</p> <p><i>Sean Stanton</i> – Chief Executive Officer, Lifecore Solutions</p> <p><b>Q &amp; A</b></p>
2:45–3:10 pm	<p><b>Break – 25 min</b></p>

3:10–4:40 pm	<p><b><u>Session 4: Advancing the Science of Clinical Trial Data Collection</u></b></p> <ul style="list-style-type: none"> <li>• EQ-5D-5L Study Results</li> <li>• BYOD Study Results</li> <li>• IMI PROactive Project Overview</li> </ul> <p><b>Moderator:</b> <i>Sonya Eremenco, MA</i> – Associate Director, PRO Consortium, C-Path</p> <p><b>Presenters:</b></p> <p><i>Jason Lundy, PhD</i> – Principal, Outcometrix</p> <p><i>Louise Newton, MSc</i> – Senior Director, Clinical Outcome Assessments, Clinical Outcome Solutions</p> <p><i>Niklas Karlsson, PhD</i> – Patient Reported Outcomes Director Respiratory, AstraZeneca</p> <p><b>Panelists:</b></p> <p><i>Bill Byrom, PhD</i> – Vice President, Product Strategy and Innovation, CRF Health and Vice Director, ePRO Consortium</p> <p><i>Wen-Hung Chen, PhD</i> – Team Leader, COA Staff, OND, CDER, FDA</p> <p><i>David Reasner, PhD</i> – Vice President, Data Science and Head, Study Endpoints, Ironwood Pharmaceuticals</p> <p><b>Q &amp; A</b></p>
4:50–4:25 pm	<p><b><u>An Overview and Discussion with Members of the Friends of Cancer Research Working Group: Comparative Tolerability Trial Design</u></b></p> <p><i>Alicyn Campbell, MPH</i> – Global Head, Patient Centered Outcomes Research for Oncology, Genentech, A Member of the Roche Group</p> <p><i>Lee Jones, MBA</i> – Patient/Research Advocate, Fight Colorectal Cancer, SWOG, Cancer Action Coalition of VA, Cancer Policy and Advocacy Team, Clinical Trials Advisory Panel. Georgetown University Oncology Institutional Review Board</p> <p><i>Paul G. Kluetz, MD</i> – Associate Director of Patient Outcomes (Acting), Oncology Center of Excellence, FDA</p> <p><i>Mark Stewart, PhD</i> – Senior Science Policy Analyst, Friends of Cancer Research</p> <p><b>Q &amp; A</b></p>
5:25–5:30 pm	<p><b>Day 1 Closing Remarks</b></p> <p><b>Adjourn</b></p>

5:30–7:00 pm	<b>Reception and Poster Session – Cedar Room (First Floor)</b> <a href="#">Asthma Working Group</a> <a href="#">Cognition Working Group</a> <a href="#">Depression Working Group</a> <a href="#">Electronic Patient-Reported Outcome (ePRO) Consortium</a> <a href="#">Functional Dyspepsia Working Group</a> <a href="#">Irritable Bowel Syndrome (IBS) Working Group</a> <a href="#">Multiple Sclerosis Working Group</a> <a href="#">Myelofibrosis Working Group</a> <a href="#">Non-Small Cell Lung Cancer (NSCLC) Working Group</a> <a href="#">Pediatric Asthma Working Group</a> <a href="#">Rheumatoid Arthritis Working Group</a>
--------------	---

## Agenda – Day 2

7:30–8:30 am	<b>Registration and Continental Breakfast – Cypress Ballroom</b>
	<b>Day 2 Moderator:</b> <i>Maria Mattera, MPH</i> – Assistant Director, PRO Consortium, C-Path
8:30 – 10:00 am	<p><b><u>Session 5: Why Reinvent the Wheel?</u></b></p> <p><b>Moderator:</b> <i>Maria Mattera, MPH</i> – Assistant Director, PRO Consortium, C-Path</p> <p><b>Presenters:</b> <i>Elizabeth (Nicki) Bush, MHS</i> – Director, Patient-Focused Outcomes Center of Expertise, Eli Lilly and Company and Industry Co-Director, PRO Consortium</p> <p><i>Elektra Papadopoulos, MD, MPH</i> – Associate Director, COA Staff, OND, CDER, FDA</p> <p><i>Dave Cella, PhD</i> – Professor and Chair, Department of Medical Social Sciences, Feinberg School of Medicine, Northwestern University</p> <p><i>Sonya Eremenco, MA</i> – Associate Director, PRO Consortium, C-Path</p> <p><b>Panelist:</b></p> <p><i>Billy Dunn, MD</i> – Director, Division of Neurology Products, OND, CDER, FDA</p> <p><b>Q &amp; A</b></p>
10:00–10:25 am	<b>Break – 25 min</b>

10:25–11:55 am	<p><b><u>Session 6: Overcoming Challenges in Outcome Measurement in Rare Diseases and Pediatric Populations</u></b></p> <p><b>Moderator:</b> <i>Michelle Campbell, PhD</i> – Reviewer and Scientific Coordinator, COA Qualification Program, COA Staff, OND, CDER, FDA</p> <p><b>Presenters:</b> <i>Nerissa Kreher, MD, MS, MBA</i> – Chief Medical Officer, AVROBIO, Inc.</p> <p><i>Bryce Reeve, PhD</i> – Professor and Director of Center for Health Measurement, Duke University School of Medicine</p> <p><i>Ebony Dashiell-Aje, PhD</i> – Reviewer, COA Staff, OND, CDER, FDA</p> <p><b>Panelist:</b></p> <p><i>Ron Bartek, MA, BS</i> – Co-Founder/Founding President, Friedreich’s Ataxia Research Alliance (FARA)</p> <p><b>Q &amp; A</b></p>
11:55–12:15 pm	<p><b><u>Closing Remarks</u></b></p> <p><b><u>Adjourn</u></b></p>