

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 21st 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	Registration and Continental Breakfast – Cypress Ballroom
	Day 1 Morning Moderator: <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	<u>Welcome and Patient-Reported Outcome Consortium Update</u> <i>Stephen Joel Coons, PhD</i> – Executive Director, Patient-Reported Outcome (PRO) Consortium, Critical Path Institute (C-Path)

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

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

3:10–4:40 pm	<u>Session 4: Advancing the Science of Clinical Trial Data Collection</u> • EQ-5D-5L Study Results • BYOD Study Results • IMI PROactive Project Overview Moderator: <i>Sonya Eremenco, MA</i> – Associate Director, PRO Consortium, C-Path Presenters: <i>Jason Lundy, PhD</i> – Principal, Outcometrix <i>Louise Newton, MSc</i> – Senior Director, Clinical Outcome Assessments, Clinical Outcome Solutions <i>Niklas Karlsson, PhD</i> – Patient Reported Outcomes Director Respiratory, AstraZeneca Panelists: <i>Bill Byrom, PhD</i> – Vice President, Product Strategy and Innovation, CRF Health and Vice Director, ePRO Consortium <i>Wen-Hung Chen, PhD</i> – Team Leader, COA Staff, OND, CDER, FDA <i>David Reasner, PhD</i> – Vice President, Data Science and Head, Study Endpoints, Ironwood Pharmaceuticals Q & A
4:50–4:25 pm	<u>An Overview and Discussion with Members of the Friends of Cancer Research Working Group: Comparative Tolerability Trial Design</u> <i>Alicyn Campbell, MPH</i> – Global Head, Patient Centered Outcomes Research for Oncology, Genentech, A Member of the Roche Group <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 <i>Paul G. Kluetz, MD</i> – Associate Director of Patient Outcomes (Acting), Oncology Center of Excellence, FDA <i>Mark Stewart, PhD</i> – Senior Science Policy Analyst, Friends of Cancer Research Q & A
5:25–5:30 pm	Day 1 Closing Remarks Adjourn

5:30–7:00 pm	Reception and Poster Session – Cedar Room (First Floor) Asthma Working Group Cognition Working Group Depression Working Group Electronic Patient-Reported Outcome (ePRO) Consortium Functional Dyspepsia Working Group Irritable Bowel Syndrome (IBS) Working Group Multiple Sclerosis Working Group Myelofibrosis Working Group Non-Small Cell Lung Cancer (NSCLC) Working Group Pediatric Asthma Working Group Rheumatoid Arthritis Working Group
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Agenda – Day 2

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

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