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.

Agenda – Day 1

7:30–8:30 am	**Registration and Continental Breakfast – Cypress Ballroom**	
	Day 1 Morning Moderator: *Michelle Campbell, PhD* – 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	**Welcome and Patient-Reported Outcome Consortium Update** *Stephen Joel Coons, PhD* – Executive Director, Patient-Reported Outcome (PRO) Consortium, Critical Path Institute (C-Path)	
8:50–10:20 am	**Session 1: Update from FDA Regarding the Clinical Outcome Assessment Qualification Program** **Moderator:** *Michelle Campbell, PhD* – Reviewer and Scientific Coordinator, COA Qualification Program, COA Staff, OND, CDER, FDA **Presenter:** *Elektra Papadopoulos, MD, MPH* – Associate Director, COA Staff, OND, CDER, FDA **Panelists:** *Laura Lee Johnson, PhD* – Acting Director, Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences, CDER, FDA *Theresa Mullin, PhD* – Associate Director for Strategic Initiatives, CDER, FDA **Q & A**	
10:20–10:45 am	**Break – 25 min**	

10:45 am–12:15 pm	**Session 2: Case Study: The Diary of Irritable Bowel Syndrome Symptoms (DIBSS)** **Moderator:** *Jennifer Hanlon, MPH* – Associate Director, Study Endpoints, Ironwood Pharmaceuticals **Presenters:** *Claire Ervin, MPH* – Senior Director, Patient-Centered Outcomes Assessment, RTI Health Solutions *Lori McLeod, PhD* – Vice President, Patient-Centered Outcomes Assessment, RTI Health Solutions *Adam Butler* – Senior Vice President, Strategic Development, Bracket *Robyn Carson, MPH* – Executive Director and Head, Patient-Centered Outcomes Research, Allergan **Panelists:** *Stephen Joel Coons, PhD* – Executive Director, PRO Consortium, C-Path *Sheri Fehnel, PhD* – Vice President, Patient-Centered Outcomes Assessment, RTI Health Solutions *Sarrit Kovacs, PhD* – 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: *Elizabeth (Nicki) Bush, MHS* – Director, Patient-Focused Outcomes Center of Expertise, Eli Lilly and Company and Industry Co-Director, PRO Consortium	

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

3:10–4:40 pm	**Session 4: Advancing the Science of Clinical Trial Data Collection** • EQ-5D-5L Study Results • BYOD Study Results • IMI PROactive Project Overview **Moderator:** *Sonya Eremenco, MA* – Associate Director, PRO Consortium, C-Path **Presenters:** *Jason Lundy, PhD* – Principal, Outcometrix *Louise Newton, MSc* – Senior Director, Clinical Outcome Assessments, Clinical Outcome Solutions *Niklas Karlsson, PhD* – Patient Reported Outcomes Director Respiratory, AstraZeneca **Panelists:** *Bill Byrom, PhD* – Vice President, Product Strategy and Innovation, CRF Health and Vice Director, ePRO Consortium *Wen-Hung Chen, PhD* – Team Leader, COA Staff, OND, CDER, FDA *David Reasner, PhD* – Vice President, Data Science and Head, Study Endpoints, Ironwood Pharmaceuticals **Q & A**	

4:50–4:25 pm	**An Overview and Discussion with Members of the Friends of Cancer Research Working Group: Comparative Tolerability Trial Design** *Alicyn Campbell, MPH* – Global Head, Patient Centered Outcomes Research for Oncology, Genentech, A Member of the Roche Group *Lee Jones, MBA* – 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 *Paul G. Kluetz, MD* – Associate Director of Patient Outcomes (Acting), Oncology Center of Excellence, FDA *Mark Stewart, PhD* – 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	

Agenda – Day 2

7:30–8:30 am	**Registration and Continental Breakfast – Cypress Ballroom**	
	Day 2 Moderator: *Maria Mattera, MPH* – Assistant Director, PRO Consortium, C-Path	

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

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