CRITICAL PATH INSTITUTE

# 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

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| 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**<br>*Stephen Joel Coons, PhD* – Executive Director, Patient-Reported Outcome (PRO) Consortium, Critical Path Institute (C-Path) |

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

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

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

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

# Agenda – Day 2

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

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