



## **ISOQOL MEASURING WHAT MATTERS SYMPOSIUM:**

*Bridging industry regulatory needs with what matters to patients and clinicians*

25 – 26 July 2016

Washington, DC, United States







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25 July 2016

Welcome to ISOQOL's inaugural "Measuring What Matters" Symposium. I am pleased to have you join us for what promises to be an informative, comprehensive, and interactive learning experience.

ISOQOL is proud to deliver innovative educational programs that promote our mission to advance the scientific study of health-related quality of life and other patient-centered outcomes. This Symposium incorporates the knowledge and expertise of leading researchers and clinicians in the field. Over the course of the next two days, participants will review advances in the application of PROs in the regulatory approval process and best practices for engaging patient and clinician partners in the PRO development and selection process. In addition, attendees will participate in interactive case study groups that will prioritize recommendations for implementing PRO measurement strategies that triangulate the patient, clinician, and industry perspectives and set a research agenda moving forward.

As a leader in the field of quality of life and patient-centered outcomes, ISOQOL offers numerous opportunities and resources for professional development, engagement, and networking. Among these resources are its journals *Quality of Life Research* and the soon-to-be released *Journal of Patient-Reported Outcomes*; numerous educational webinars and online education initiatives; the new Introductory Education Course, and the Annual Conference. To see our full range of learning offerings and supplemental resources, please visit [www.isoqol.org](http://www.isoqol.org).

Again, many thanks for participating in the Measuring What Matters Symposium. I look forward to seeing you at future ISOQOL programs.

Sincerely,

Claire Snyder, PhD  
ISOQOL President



*Dedicated to the promotion of excellence in the science of health-related quality of life.*

International Society for Quality of Life Research (ISOQOL) established in 1993, is a non-profit society to advance the scientific study of health-related quality of life and other patient-centered outcomes to identify effective interventions, enhance the quality of health care and promote the health of populations. ISOQOL provides the premiere opportunity for those in the quality of life research field to connect and network.

Quality of life has become a prominent subject in philosophy, social science, clinical medicine, health services, and outcomes research. With over 600 members representing 43 countries, ISOQOL is an international society with activities focused on promotion of high quality research in the science of health-related quality of life (HRQOL) measurement and patient-reported outcomes (PRO).

### **Mission Statement**

*"The mission of the International Society for Quality of Life Research (ISOQOL) is to advance the scientific study of health-related quality of life and other patient-centered outcomes to identify effective interventions, enhance the quality of health care and promote the health of populations."*

### **Patient Engagement Statement**

*"ISOQOL supports the patient voice in quality of life and patient-reported outcomes research. Patient engagement initiatives are intended to align ISOQOL's priorities and infrastructure with efforts to establish best practices in patient-engaged quality of life research and create educational efforts and model programs that promote best practices and fulfill ISOQOL's mission."*

### **Programs and Projects**

#### **Education Programs**

- Webinar Events and Recordings
- 23<sup>rd</sup> Annual Conference
- "Measuring What Matters" Symposium
- Introductory Education Course
- Mentor/Mentee Program

#### **Publications**

- *Quality of Life Research Journal*
- *Journal of Patient Reported Outcomes* (Coming Soon)
- *ISOQOL Dictionary of Quality of Life and Health Outcomes Measurement* (2015)
- International Society for Quality of Life Research commentary on the draft European Medicines Agency reflection paper on the use of patient-reported outcome (PRO) measures in oncology studies (2015)
- ISOQOL Recommends Minimum Standards for Patient-Reported Outcome Measures Used in Patient-Centered Outcomes and Comparative Effectiveness Research (2013)
- Patient-Reported Outcomes in Randomized Clinical Trials (2012/2013)
- Using Patient-Reported Outcome Measures to Improve Clinical Practice (2012)
- User's Guide to Implementing Patient-Reported Outcomes Assessment in Clinical Practice (2011)
- *Quality of Life: The Assessment, Analysis and Interpretation of Patient-reported Advancing Health Outcomes Research Methods and Clinical Applications Outcomes*
- *Assessing Quality of Life in Clinical Trials*, 2nd Edition
- *Measuring and Valuing Health Benefits for Economic Evaluation*



# **MEASURING WHAT MATTERS SYMPOSIUM:**

***Bridging industry regulatory needs with what matters to patients and clinicians***

Thank you to the planning committee that contributed to the creation of this Symposium:

Steve Blum, MBA MA - Co-Chair

Sarah Ahmed, PhD - Co-Chair

Susan Bartlett, PhD - Board Liaison

## **Committee Members:**

Deborah M. Miller, PhD LISW-S

Ana María Rodríguez, PhD MSc BS PT

John T. Farrar, MD MSCE PhD

Bellinda King-Kallimanis, PhD

Emuella Flood

Helen Kitchen, MSc

Wen-Hung Chen, PhD

I-Chan Huang, PhD



# **MEASURING WHAT MATTERS SYMPOSIUM:**

*Bridging industry regulatory needs with what matters to patients and clinicians*

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Additional articles, recordings, and resources can be found at [www.isoqol.org/about-isoqol/what-is-health-related-quality-of-life-research](http://www.isoqol.org/about-isoqol/what-is-health-related-quality-of-life-research)



# MEASURING WHAT MATTERS SYMPOSIUM

## Program Agenda

### MONDAY, 25 JULY

<u>TIME</u>	<u>SESSION</u>	<u>MEETING ROOM</u>
10:00 AM – 5:00 PM	Registration Open	16 <sup>th</sup> Floor Foyer
1:00 PM – 1:20 PM	Welcome & Introductions	Galaxy Ballroom
1:20 PM – 2:35 PM	<b>Session I</b>  <b><i>Patient/Caregiver And Clinicians Engagement In Planning A PRO Measurement Strategy</i></b>  Speakers:  <i>A Roadmap to Patient-Focused Outcome Measurement in Drug Development - <b>Elektra Papadopoulos, MD, MPH, US Food and Drug Administration (FDA)</b></i>  <i>US Perspectives on Patient Engagement - <b>Suzanne Schrandt, JD, Patient-Centered Outcomes Research Institute (PCORI)</b></i>  <i>Voice of Experience: Patient Perspective on Engagement - <b>Amye Leong, MBA - Healthy Motivation, Patient Research Partner</b></i>	Galaxy Ballroom
2:35 PM – 2:55 PM	Break	
2:55 PM – 4:00 PM	<b>Session II</b>  <b><i>Incorporating The Disease Model Into Your PRO Measurement Strategy</i></b>  Speakers:  <i>Conceptual Disease Model/Historical Perspective - <b>James Witter, MD PhD FACR, National Institute of Health (NIH)</b></i>  <i>Apply Disease Model/Insights to Develop TPP and Measurement Strategy - <b>Diana Rofail, PhD CPsychol, Roche Products Limited</b></i>  <i>Why Reinvent the Wheel? - <b>David Cella, PhD, Northwestern University</b></i>	Galaxy Ballroom



# MEASURING WHAT MATTERS SYMPOSIUM

## Program Agenda

<b>4:00 PM – 4:45 PM</b>	<b>Case Study Breakout Session</b>	
	<i>Multiple Sclerosis</i>	<b>Galaxy Ballroom</b>
	<i>Pediatrics</i>	<b>Stars 1</b>
	<i>Cancer</i>	<b>Stars 2</b>
<b>4:45 PM – 5:00 PM</b>	<b>Case Study Summary</b>	<b>Galaxy Ballroom</b>
<b>5:00 PM</b>	<b>Adjourn for the day</b>	
<b>5:30 PM – 6:30 PM</b>	<b>Evening Reception</b>	<b>Concourse Room</b>

## TUESDAY, 26 JULY

<u>TIME</u>	<u>SESSION</u>	<u>MEETING ROOM</u>
<b>7:00 AM – 3:00 PM</b>	<b>Registration Open</b>	<b>16<sup>th</sup> Floor Foyer</b>
<b>8:00 AM – 8:30 AM</b>	<b>Continental Breakfast</b>	<b>Galaxy Ballroom</b>
<b>8:30 AM – 8:45 AM</b>	<b>Review of Day 1 &amp; Kick-Off for Day 2</b>	<b>Galaxy Ballroom</b>
<b>8:45 AM – 10:20 AM</b>	<b>Session III</b>	<b>Galaxy Ballroom</b>
	<i>Assessing PRO-based Endpoints that Reflect Patient Perspectives and Meet Regulatory Expectations</i>	
	Speakers:	
	<i>Engaging with FDA to Develop PROs for Clinical Trials- Michelle Campbell, PhD, US Food and Drug Administration (FDA)</i>	
	<i>EMA Review and Qualification Process - Lena Ring, PhD, Medical Products Agency (MPA) and Uppsala University</i>	
	<i>A Consortium Approach to Advancing the Assessment of PRO-based Clinical Trial Endpoints - Stephen Joel Coons, PhD, Patient-Reported Outcome Consortium, Critical Path Institute</i>	
	<i>Role of Patients in Advisory Panels - Phil Posner, PhD, Patient Research Partner</i>	



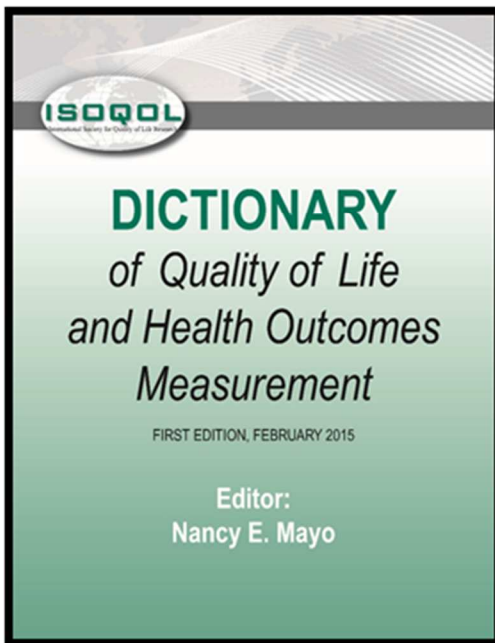
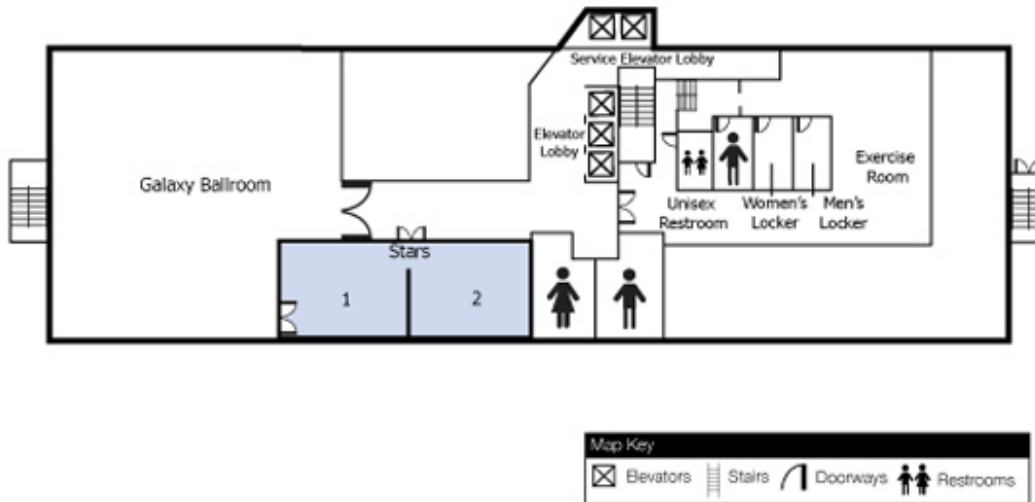


# MEASURING WHAT MATTERS SYMPOSIUM

## Program Agenda

10:20 AM – 10:35 AM	Break	
10:35 AM – 11:45 AM	<b>Session IV</b>  <b><i>Implementing PRO Measurement Strategies in Clinical Development Programs</i></b>  Speakers:  <i>Implementation challenges – Industry Perspective -</i> <b><i>Diana Rofail, PhD CPsychol - Roche Products Limited</i></b>  <i>Scoring/Psychometrics -</i> <b><i>Laura Lee Johnson, PhD - Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences, Center for Drug Evaluation and Research U.S. Food and Drug Administration (FDA)</i></b>  <i>Interpretation of Results – Clinician Perspective -</i> <b><i>John T. Farrar, MD PhD - University of Pennsylvania</i></b>  <i>Interpreting and Disseminating Results to Patients -</i> <b><i>Amye Leong, MBA - Healthy Motivation, Patient Research Partner</i></b>	Galaxy Ballroom
11:45 AM – 12:15 PM	Break (Boxed Lunch Available for Pick-Up)	
12:15 PM – 1:45 PM	<b>Case Study Breakout Session</b>  <i>Multiple Sclerosis</i>  <i>Pediatrics</i>  <i>Cancer</i>	Galaxy Ballroom  Stars 1  Stars 2
1:45 PM – 2:15 PM	<b>Case Study Synthesizing Panel Output</b>  <i>Multiple Sclerosis</i>  <i>Pediatrics</i>  <i>Cancer</i>	Galaxy Ballroom  Stars 1  Stars 2
2:15 PM – 2:45 PM	Case Study Read-Out Panel	Galaxy Ballroom
2:45 PM – 3:00 PM	Closing Remarks	Galaxy Ballroom

## Sheraton Pentagon City Hotel 16th Floor/Floor Plan



### ISOQOL Dictionary Of Quality Of Life And Health Outcomes Measurement

Over 600 definitions of terms related to quality of life and health outcomes measurement, study design, and analysis are covered, along with over 350 references. Look up a term, or read the Dictionary from A to Z to see the vast array of terms and concepts that apply to this important field.

Many thanks to Editor Nancy E. Mayo, PhD, for her dedication and hard work on this publication.

Purchase your e-version or print version now at [www.isoqol.org](http://www.isoqol.org). All profits go to support ISOQOL's activities to advance the scientific study of quality of life and other patient-centered outcomes.

## **General Information**

### **Title and Theme**

*Measuring What Matters Symposium: Bridging industry regulatory needs with what matters to patients and clinicians*

There is growing consensus among patients, clinicians, payers, regulators, and product developers that incorporating the patient voice is necessary in product development and testing to support product-labeling claims. PROs provide the most direct method for gauging individuals' symptoms, functional status, participation, health status, and quality of life. PROs play a particularly important role in the approval process for chronic conditions where the goal of treatment is not curative but rather to alleviate symptoms, and improve functional ability and quality of life. While the application of PRO instruments in the regulatory process has developed over the past 6 years with the creation of the Food & Drug Administration (FDA) PRO Guidance and the European Medicines Agency (EMA) paper on use of PROs in oncology studies, there remain methodological and technical challenges associated with the development of a PRO measurement strategy that is based on scientifically rigorous and patient-centered approaches.

This workshop will review advances in the application of PROs in the regulatory approval process, and best practices for engaging patient and clinician partners in the PRO development and selection process. The workshop will also include an interactive component that will prioritize recommendations for implementing PRO measurement strategies that triangulates the patient, clinician, and industry perspectives, and set a research agenda moving forward

### **Hotel Information**

Sheraton Pentagon City Hotel

900 South Orme Street

Arlington, Virginia, USA 22204

Phone: +1(703) 521-1900

Website: <http://www.starwoodhotels.com/sheraton/property/overview/index.html?propertyID=829>

Check in: 3:00 PM

Check out: 12:00 PM

### **Session Rooms**

All sessions at the Measuring What Matters Symposium will be held on the 16th Floor at the Sheraton Pentagon City Hotel.

### **Registration Desk**

ISOQOL accepts MasterCard, Visa, American Express, and Discover credit cards.

Payment by check is accepted so long as the check is in US dollars and drawn on a US bank account. Cash will not be accepted at the Registration Desk.

### **Registration Desk Hours**

Monday, 25 July 10:00 am – 5:00 pm

Tuesday, 27 July 7:00 am – 3:00 pm

## **General Information**

### **Cancellation Policy**

ISOQOL reserves the right to cancel any event due to lack of enrollment or other factors. In the event of a cancellation, registered participants will be notified by e-mail and will have the option to exchange their ticket for an available alternative, or to receive a complete refund.

### **Evaluation**

Please take time to complete the Measuring What Matters evaluation that will be distributed electronically immediately following the conclusion of the symposium. Your input and comments are essential in planning future educational events.

### **Session Recording**

Session content is copyright-protected by ISOQOL. Recording of any session without the consent of ISOQOL is prohibited. Any recording done with consent of ISOQOL is for personal use only and cannot be reproduced or distributed.

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## **Committee Members**



**Sara Ahmed, PhD**

*Co-Chair*

Dr. Sara Ahmed is an Associate Professor in the Faculty of Medicine, McGill University, and an FRQS research scholar. Originally trained as a Physical Therapist, she is a health services and clinical and health informatics researcher. Supported by federal, provincial, and private industry funds, her research includes studies that 1) address the challenges of using patient reported outcomes (PRO) (e.g. health-related quality of life) in chronic disease management programs, and the use of advanced psychometric approaches for improving the precision and efficiency of outcome evaluations, 2) develop and evaluate the impact of chronic disease computer-enabled self-management interventions integrated into electronic personal health records, and 3) knowledge exchange and transfer related to best practices for chronic disease management. She works with clients who have respiratory conditions, musculoskeletal conditions, and neurological conditions. She co-leads the ISOQOL Canada PRO Special Interest Group and the Canada PRO Network that aims to facilitate the harmonized use of state-of-the-art PROs in Canadian research, clinical practice, and population health monitoring.



**Steven Blum, MBA MA**

*Co-Chair*

Steven Blum is a health economist and outcomes researcher with over 24 years of experience in the pharmaceutical industry. He currently serves as Director, Patient Reported Outcomes at GlaxoSmithKline, where he provides strategic and technical support for the development and implementation of patient focused outcomes across various GSK clinical development programs. Since 2013, Steve has served as a member of the PCORI Advisory Panel on Patient Engagement. He received a BBA from University of Massachusetts, a MBA from Fordham University, and a MA in Economics from The New School for Social Research.

## **Committee Members**



**Susan Bartlett, PhD**

*Board Liaison*

Susan Bartlett, PhD is Associate Professor of Medicine in the Division of Clinical Epidemiology at McGill University and Adjunct Associate Professor of Medicine at Johns Hopkins School of Medicine. Dr. Bartlett was Co-chair of the 2014 ISOQOL annual conference in Berlin.



**Deborah M. Miller, PhD LISW-S**

*Committee Member*

Deborah M. Miller, PhD, LISW, is member of the Professional Staff at the Mellen Center for Multiple Sclerosis Treatment and Research of Cleveland Clinic and is an Associate Professor of Medicine at the Cleveland Clinic Lerner College of Medicine of Case Western Reserve University. Her research interests focus on health services research and the use patient reported measures in assessing outcomes of care. A clinical social worker, her practice interests focus on marital and family adjustment to the consequences of neurological diseases.



**John T. Farrar, MD MSCE PhD**

*Committee Member*

John T. Farrar, MD, MSCE, PhD, is an Associate Professor of Epidemiology, Neurology, and Anesthesia, at the University of Pennsylvania, Perelman School of Medicine. His research includes randomized trials of various pain therapies, new methodologies for understanding how patients report their pain, and functional brain imaging in people with pain, with funding from NIH, FDA, private foundations, and industry sources. He has served on advisory boards for the FDA, the NAS committee on Missing Data in Clinical Trials, and IOM committee on Relieving Pain in America. At the University of Pennsylvania, he serves as the co-Director of the Master of Science in Clinical Epidemiology, Co-Director of the Biostatistics and Epidemiology Consulting Center, and still sees patients, predominately in a palliative care setting.

## **Committee Members**



**Bellinda King-Kallimanis, PhD**

*Committee Member*

Bellinda King-Kallimanis received her PhD in psychometrics from the University of Amsterdam, the Netherlands. Since then, she worked as a postdoctoral research fellow on the Longitudinal Study on Ageing at Trinity College Dublin, Ireland investigating the measurement of the frailty syndrome, and currently works at Pharmerit International developing fit for purpose patient reported outcomes (PRO) from conducting the qualitative interviews of patients through to psychometric validation. In 2015, Bellinda was the recipient of the 2015 International Society for Quality of Life Research Emerging Leader Award.



**Emuella Flood**

*Committee Member*

Emuella Flood is Senior Principal of Clinical Outcomes Assessments (COA) at ICON and leads the US East Coast COA team. She began her career in COA research and consulting nearly 20 years ago and has developed numerous COA instruments for both adult and pediatric populations and across a wide range of therapeutic areas.



**Helen Kitchen, MSc**

*Committee Member*

Helen is a Senior Consultant in Clinical Outcomes Assessment at DRG Abacus. Helen has worked in outcomes research for over 8 years and has developed and validated multiple COAs including PROs and ClinROs for clinical trials and clinical practice. Helen has experience across a range of therapy areas and specializes in qualitative and mixed methods research including innovative patient-centered research methods. Helen holds an MSc in Health Psychology from Sheffield Hallam University and a BA(Hons) in Psychology from the University of Manchester.

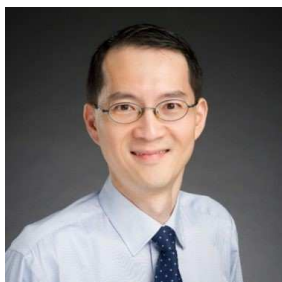
## **Committee Members**



**Wen-Hung Chen, PhD**

*Committee Member*

Wen-Hung Chen is currently a reviewer in the Clinical Outcome Assessment Staff in the Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), FDA. Dr. Chen's responsibility includes supporting the review divisions in issues related to the development and interpretation of study endpoints, and supporting the drug development tool qualification program. He was involved with the development of the pain item bank for the NIH led patient reported-outcome measurement information system (PROMIS) project. He was also involved in the development of the Exacerbations of Chronic Pulmonary Disease Tool (EXACT) developed under the EXACT-PRO Initiative. Dr. Chen earned his Master's degree and Doctorate in quantitative psychology, with a minor in Biostatistics, from University of North Carolina at Chapel Hill.



**I-Chan Huang, PhD**

*Committee Member*

Dr. I-Chan Huang is an Associate Member/Associate Professor in the Department of Epidemiology and Cancer Control at St. Jude Children's Research Hospital, Memphis, TN. Dr. Huang's main research interests include developing PRO methodologies and tools for policy application and clinical decision-making, as well as designing interventions to improve PROs based on bio-psycho-social mechanisms. He has led and recently completed a NIAMS-funded PROMIS Pediatric Asthma Study. Currently, Dr. Huang leads a NCI-funded study to examine the impact of symptom changes over a 25-year period on the occurrence of chronic health conditions in childhood cancer survivors. He is also leading a NIAMS-funded PEPR study to evaluate the clinical validity of the PROMIS Pediatric measures for childhood cancer survivors. Dr. Huang is serving as an Associate Editor for Quality of Life Research.



## **Symposium Speakers**

### **Michelle Campbell, PhD**

US Food and Drug Administration (FDA)  
Washington DC, United States



Michelle Campbell is a reviewer on the Clinical Outcome Assessments (COA) Staff and Scientific Coordinator of the COA Qualification Program in the Office of New Drugs (OND) in the Center for Drug

Evaluation and Research (CDER). COA Staff advises OND review divisions and other FDA centers by providing consultation and advice on clinical outcome assessment development, validation, and interpretation of clinical benefit endpoints in clinical trials to support drug development, labeling, and promotion. Additionally, the COA Staff leads and manages CDER's Clinical Outcome Assessment qualification program and engages with internal and external stakeholders to advance good scientific clinical outcome measurement standards and policy development.

### **David Cella, PhD**

Northwestern University  
Department of Medical Social Sciences  
Institute for Public Health and Medicine (IPHAM)  
Center for Patient-Centered Outcomes  
Evanston, IL, United States



Dr. Cella is currently Professor and Chair of the Department of Medical Social Sciences at Northwestern University Feinberg School of Medicine. He also directs the Center for Patient Centered Outcomes Research at Northwestern's Institute for Public Health and Medicine.

His research interests and accomplishments are focused in the areas of basic measurement research, descriptive studies of quality of life in chronic illness, psychosocial and behavioral health intervention, and the analysis and interpretation of patient-reported outcomes data in clinical trials. Prior to his work on PROMIS, Dr. Cella developed and validated a wide array of questionnaires, known collectively as the Functional Assessment of Chronic Illness

Therapy Measurement System (FACIT), that are in wide use internationally.

Over the past 25 years, beginning with a FIRST Award in 1989, Dr. Cella has had continuous NIH funding as PI on projects related to measurement and interventions to improve the quality of life of people affected by chronic illnesses such as cancer, arthritis, heart disease and neurologic disorders. Since its inception in 2004, Dr. Cella has chaired the Steering Committee of the NIH Common Fund project known as PROMIS: The Patient Reported Outcomes Measurement Information System. He also served as principal investigator of its Statistical Center. Currently, he is the PI of The National Person-Centered Assessment Resource (PCAR) grant which combines PROMIS, Toolbox, Neuro-QoL and ASCQ-Me into one research resource. Dr. Cella also has parallel funded projects, Neuro-QoL and PROsetta Stone which, while scientific accomplishments in their own right, extend the reach and potential of PROMIS to be truly transformative and unifying, enabling the patient's voice to be heard more loudly and clearly than ever before.

### **Stephen Joel Coons, PhD**

Patient-Reported Outcome Consortium  
Critical Path Institute  
Tuscon, Arizona, United States



Stephen Joel Coons is Executive Director of Critical Path Institute's (C-Path) Patient-Reported Outcome (PRO) Consortium. He joined C-Path in 2009 after a 23-year career in academia. Stephen earned a BS degree at the University of Connecticut and

MS, MEd, and PhD degrees at the University of Arizona. His post-doctoral training in health outcomes research was completed at the University of California, San Diego. Stephen is a fellow in the American Association of Pharmaceutical Scientists and a professor emeritus at the University of Arizona. For over two decades, the primary focus of his research has been the assessment of patient-reported outcomes.

## **Symposium Speakers**

### **John T. Farrar, MD, PhD**

Associate Professor of Epidemiology, Neurology  
and Anesthesiology  
University of Pennsylvania  
Philadelphia, PA, United States



John T. Farrar, MD, MSCE, PhD, is an Associate Professor of Epidemiology, Neurology, and Anesthesia, at the University of Pennsylvania, Perelman School of Medicine. His research includes randomized trials of various pain therapies, new methodologies for understanding how patients report their pain, and functional brain imaging in people with pain, with funding from NIH, FDA, private foundations, and industry sources. He has served on advisory boards for the FDA, the NAS committee on Missing Data in Clinical Trials, and IOM committee on Relieving Pain in America. At the University of Pennsylvania, he serves as the co-Director of the Master of Science in Clinical Epidemiology, Co-Director of the Biostatistics and Epidemiology Consulting Center, and still sees patients, predominately in a palliative care setting.

### **Laura Lee Johnson, PhD**

Associate Director  
Division of Biometrics III  
Office of Translational Sciences  
Center for Drug Evaluation and Research  
U.S. Food and Drug Administration (FDA)  
Washington, DC, United States



Dr. Laura Lee Johnson is the liaison between the U.S. Food and Drug Administration's Center for Drug Evaluation and Research's Office of Biostatistics and the Clinical Outcome Assessment Team in the Office of New Drugs providing guidance on design, logistics, implementation, and analysis of research studies ranging from person reported outcome measure qualification to safety and randomized studies of all sizes. Dr. Johnson also serves on the FDA-NIH Interagency Clinical Outcome Assessments Working Group and co-directs the NIH Principles and Practice of Clinical Research course. Prior to working at the FDA she spent over a decade at the NIH working on and overseeing clinical research and research support programs. She has been involved with numerous projects developing, validating, and using clinical outcome assessments in both patient care and research. Dr. Johnson received her Ph.D. in Biostatistics from the University of Washington.



## **Symposium Speakers**

### **Amye Leong, MBA**

Healthy Motivation  
Patient Research Partner  
Santa Barbara, California, United States



Amye Leong is an internationally recognized patient advocate leader, health communications and policy strategist, speaker, author and educator. She is President/CEO of Healthy Motivation, a health education and advocacy consulting firm in

Santa Barbara, California. Clients include governments, industry and non-government organizations in 18 countries who seek expertise in patient-centered care and communications, patient engagement in research, advocacy and patient advisor and education programs, as well as strategic planning, group facilitation, marketing to patients, conflict resolution, and building networks and collaborators. Diagnosed with rheumatoid arthritis at age 18 and later with Sjogren's syndrome and osteoporosis, she became wheelchair-bound within 6 years. She spent every day for 5 years in a wheelchair and over 290 days hospitalized, and was a recipient of Social Security Disability. After 16 surgeries and 12 joint replacements, she developed America's largest network of 40-plus young adult arthritis education/advocacy programs, voluntarily halted Disability payments and started Healthy Motivation to become a respected medical and motivational speaker, advocate, strategic advisor and facilitator. She is a recognized key opinion leader in musculoskeletal care and communications.

She has advised the US Food and Drug Administration, Critical Path, National Institutes of Health (NIH), Patient-Centered Outcomes Research Institute (PCORI), and the Agency for Health Research and Quality (AHRQ) on the topics and processes of integrating the voices of patients and stakeholders into research development. She served on the NIH Director's Council of Public Representatives and on the Editorial Board of the International Journal on Self-Help and Self Care. She also served as the primary patient advocate on the task force developing treatment guidelines for rheumatoid arthritis for the American College of Rheumatology.

### **Elektra Papadopoulos, MD, MPH**

US Food and Drug Administration (FDA)  
Washington DC, United States



Elektra Papadopoulos, MD, MPH, serves as the Acting Associate Director of the Clinical Outcome Assessments (COA) Staff in the Office of New Drugs in the Center for Drug Evaluation and Research (CDER). The COA Staff provides consultation

to CDER's Review Divisions as well as other FDA Centers on the development, validation, and interpretation of clinical outcome assessments, including patient-reported outcome instruments, in medical product development. The COA Staff also manages CDER's Clinical Outcome Assessment Drug Development Tool Qualification Program and participates in other FDA initiatives to promote patient-focused outcome measurement.

### **Phil Posner, PhD**

Patient Research Partner  
Arlington, VA, United States



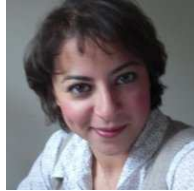
Philip Posner, PhD, is a patient with multiple sclerosis and atrial fibrillation. He has been involved in research and teaching at various academic medical centers for over 35 years, and has experience reviewing research proposals for various funding agencies. He has served as a patient representative for the FDA, DOD (CDMRP), and PCORI, and volunteers with the National Capital MS Society.

He is currently a patient participant in the CaRe-Align project on complex medical diseases. He has served as Vice Chair of WMATA's Accessibility Advisory Committee, is a member of the Virginia GRC, and has participated in Richmond and Capitol Hill advocacy for various patient and research groups. He has been elected to the MS Society Advocacy Hall of Fame and awarded the Richard W. Hedding Award for his efforts to improve paratransit.

## Symposium Speakers

### **Diana Rofail, PhD CPsychol**

Roche Products Ltd  
Global Head of Patient Centered Outcomes Research  
Neuroscience Product Development  
Welwyn Garden City, United Kingdom



At Roche, Dr. Rofail is the Global Head of Patient Centered Outcomes Research for Neuroscience. She leads a team of clinical outcome assessment experts to help maximize the value of multiple agents by defining treatment benefit and developing holistic measurement strategies during early clinical development through Phase IV. Her work supports PRO labeling and promotional claims, as well as key messages to facilitate rapid market access for pharmaceutical products.

### **Lena Ring, PhD**

Medical Products Agency (MPA)  
Uppsala University



Lena Ring, PhD is a researcher at the Medical Products Agency (MPA) in Sweden, in addition she is Adjunct Professor in Quality of Life Research in Health Care at Uppsala University. Her research focus is on Patient

Reported Outcomes (PRO) studies e.g. incorporation the patient perspectives into outcomes assessments in relation to the evaluation of treatment and care of patients in clinical oncology practice. At the MPA her focus is on including patients'/consumers perspectives and experiences in ongoing projects as well as increasing their engagement in the agencies processes as well.

## **ISOQOL 23<sup>rd</sup> ANNUAL CONFERENCE**

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with the challenges in quality of life  
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## **Symposium Speakers**

### **Suzanne Schrandt, JD**

Deputy Director, Patient Engagement  
Patient-Centered Outcomes Research Institute  
(PCORI)  
Washington, DC, United States



Suzanne Schrandt, JD, is the Deputy Director of Patient Engagement at the Patient-Centered Outcomes Research Institute (PCORI). She is responsible for supporting the Director of Patient

Engagement in creating networks and engaging patients across the nation to provide broad-based input on the development and execution of PCORI's research. Schrandt has been involved in patient education and advocacy since being diagnosed with a form of rheumatoid arthritis as a teenager.

For more than 15 years, she has advocated on behalf of children and adults with arthritis and has been engaged in numerous patient and provider education initiatives aimed at increasing early diagnosis and appropriate, patient-centered management of chronic disease. Before coming to PCORI, Schrandt served as the health reform strategy team leader for the Kansas Health Institute, where she educated the state's policymakers, providers, and consumers on the implications of the Affordable Care Act. While there, Schrandt also led the Kansas Legislative Health Academy, an intensive educational experience for select Kansas legislators.

Schrandt also previously served as the Coordinator of Public Health and Public Policy for the Arthritis Foundation in Kansas City and as a Research Associate for a Human Genome Research Institute Ethical, Legal, and Social Issues project. She is a member of the Kansas Bar and the American Health Lawyers Association.

### **James Witter, MD PhD FACR**

Chief Science Officer for PROMIS® and PEPR  
Medical Officer/Rheumatic Diseases Clinical  
Program  
National Institute of Arthritis and Musculoskeletal  
and Skin Disease (NIAMS)  
National Institutes of Health



James P. Witter is the Program Director of the Rheumatic Diseases Clinical Program at the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS) at the National Institutes of Health (NIH). He has been the Chief Science Officer (CSO) of the

Patient-Reported Outcomes Measurement Information System (PROMIS®), a 10-year initiative funded by the NIH Roadmap/Common Fund for Medical Research. He currently also serves as the CSO for the Validation of Pediatric Patient-Reported Outcomes in Chronic Diseases (PEPR) consortium. He received his Ph.D. in Medical Microbiology/Immunology with a minor in Bacteriology from the University of Wisconsin Medical School, and his medical degree from the Medical College of Wisconsin. He is a Harvard trained, board-certified rheumatologist who has been a member of several American College of Rheumatology (ACR) committees.

Before joining NIH, Dr. Witter was a Medical Officer at the Food and Drug Administration (FDA), and a Staff Physician in Rheumatology at the National Naval Medical Center. Dr. Witter has received multiple awards and honors in his career, including the NIH Director's Award, and is frequent presenter at professional rheumatology meetings.

Dr. Witter manages a research portfolio that supports patient-relevant clinical research and the design, development and execution of clinical trials and registries in rheumatic diseases in adults and children, such as rheumatoid arthritis, lupus, scleroderma, ankylosing spondylitis, psoriatic arthritis, gout, inflammatory myopathies, and vasculitis.

## Measuring What Matters Symposium: Case Study Template

### **Case Study Focus Areas:**

- **Pediatrics**
- **Multiple Sclerosis**
- **Cancer**

<b>Case Study Description</b>
Provide a case description from the patient's perspective, including sufficient insight about the day-to-day life of a patient with this condition and what happens in a typical clinical encounter [i.e. Provide a description of the manifestation of the disease and common impact of the condition and treatment on symptoms, activity limitations, and health-related quality of life].
<b>Case Study Breakout Session Day 1 Overview</b>
<ol style="list-style-type: none"><li>1. Develop a patient/caregiver engagement strategy (which organization, sub-populations, engagement plan - e.g. focus groups, interviews, social media etc.) and describe the pros and cons of the different options. Also consider engagement of all stakeholders.</li><li>2. Develop a measurement strategy guided by the patient perspective, disease model, and conceptualization of outcomes.<ul style="list-style-type: none"><li>• What are the important domains to measure?</li><li>• Think about existing or new measures required.</li><li>• What are potential barriers and solutions to developing and submitting a measurement strategy for approval (e.g. perceived benefit of longer term outcomes).</li></ul></li></ol>
<b>Case Study Breakout Session Day 2 Overview</b>
<ol style="list-style-type: none"><li>1. Develop an end point model for a clinical trial.<ul style="list-style-type: none"><li>• Identify the measurement concepts.</li><li>• Identify primary and secondary end points (scientific, patient, clinical) [provide examples and definitions of possible end points – e.g. time to event, responder].</li><li>• What is the hierarchy between end points?</li><li>• What will be the frequency of data collection for each concept?</li><li>• Will data collection be electronic or paper-based?</li><li>• Develop a mock label claim.</li></ul></li></ol>

# Roadmap to **PATIENT-FOCUSED OUTCOME MEASUREMENT** in Clinical Trials

## Understanding the Disease or Condition

# 1

### A. Natural history of the disease or condition

- Onset/Duration/Resolution
- Diagnosis
- Pathophysiology
- Range of manifestations

### B. Patient subpopulations

- By severity
- By onset
- By comorbidities
- By phenotype

### C. Health care environment

- Treatment alternatives
- Clinical care standards
- Health care system perspective

### D. Patient/caregiver perspectives

- Definition of treatment benefit
- Benefit-risk tradeoffs
- Impact of disease

## Conceptualizing Treatment Benefit

# 2

### A. Identify concept(s) of interest (COI) for meaningful treatment benefit, i.e., How a patient:

- Survives
- Feels (e.g., symptoms)
- Functions

### B. Define context of use (COU) for clinical trial:

- Disease/Condition entry criteria
- Clinical trial design
- Endpoint positioning

### C. Select clinical outcome assessment (COA) type:

- Patient-Reported Outcome (PRO)
- Observer-Reported Outcome (ObsRO)
- Clinician-Reported Outcome (ClinRO)
- Performance Outcome (motor, sensory, cognition)

## Selecting/Developing the Outcome Measure

# 3

### A. Search for existing COA measuring COI in COU:

- Measure exists
- Measure exists but needs to be modified
- No measure exists
- Measure under development

### B. Begin COA development

- Document content validity (qualitative or mixed methods research)
- Evaluate cross-sectional measurement properties (reliability and construct validity)
- Create user manual
- Consider submitting to FDA for COA qualification for use in exploratory studies

### C. Complete COA development:

- Document longitudinal measurement properties (construct validity, ability to detect change)
- Document guidelines for interpretation of treatment benefit and relationship to claim
- Update user manual
- Submit to FDA for COA qualification as effectiveness endpoint to support claims





## **Engagement Rubric for Applicants**

Updated: June 6, 2016

Published: February 4, 2014

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To cite this document, please use: PCORI Engagement Rubric. PCORI (Patient-Centered Outcomes Research Institute) website. <http://www.pcori.org/sites/default/files/Engagement-Rubric.pdf>.  
Published February 4, 2014. Updated October 12, 2015. Accessed [fill in date]".





## PCORI Engagement Principles

As you use the rubric and fill out your Engagement Plan, demonstrate how you espouse the six PCORI Engagement Principles in your work. They are:

- **Reciprocal Relationships:** This principle is demonstrated when the roles and decision-making authority of all research partners, including the patient and other stakeholder partners, are defined collaboratively and clearly stated.
- **Co-Learning:** This principle is demonstrated when the goal is not to turn patients or other stakeholder partners into researchers, but to help them understand the research process; likewise, the research team will learn about patient-centeredness and patient/other stakeholder engagement, and will incorporate patient and other stakeholder partners into the research process.
- **Partnerships:** This principle is demonstrated when time and contributions of patient and other stakeholder partners are valued and demonstrated in fair financial compensation, as well as in reasonable and thoughtful requests for time commitment by patient and other stakeholder partners. When projects include priority populations, the research team is committed to diversity across all project activities and demonstrates cultural competency, including disability accommodations, when appropriate.
- **Transparency, Honesty, and Trust:** These principles are demonstrated when major decisions are made inclusively and information is shared readily with all research partners. Patients, other stakeholders, and researchers are committed to open and honest communication with one another.

## Definitions

- “Patient partners” is intended to include patients (those with lived experience), family members, caregivers, and the organizations that are representative of the population of interest in a particular study.
- It is important that patient partners are not confused with patient subjects; patient partners are members of the research team and involved in the planning, conduct, and dissemination of the research, whereas patient subjects are those individuals actually enrolled into the study as participants.
- “Stakeholder partners” may include members of constituencies based on professional, rather than personal, experience. For example, these constituencies can include: clinicians, purchasers, payers, industry, hospitals and health systems, policy makers, and training institutions. Some individuals may fit into several categories.

## Key Considerations for Planning, Conducting, and Disseminating Engaged Research:

- For the proposed intervention, think through the project from original concept to implementation, and identify the various stakeholders and patients who would need to be included in order for the project to be as successful as possible.



## Guidance for Applicants Completing a PCORI Funding Announcement (PFA) Engagement Plan

The Engagement Rubric is divided into three sections; planning, conduct, and dissemination. Each section includes descriptions of the types of activities likely to take place within each phase of research and examples of engagement from PCORI-funded projects. Each numbered section below corresponds to a numbered section in the engagement plan that accompanies each PFA.

1. **PLANNING THE STUDY:** Describe how patient and stakeholder partners will participate in study planning and design. (As you fill out Section 1 of your Engagement Plan, refer to the information below.)

Potential activities include:

- Developing the research question and relevant outcomes to be studied, to ensure that the project and its results will be useful and important to patient and stakeholder communities.
- Defining the characteristics of study participants, to minimize the risk that certain patients will be included or excluded due to criteria that are not relevant.
- Designing the study to minimize disruption to patient and stakeholder study participants, thereby promote retention of study participants.

Real-World Examples:

- Mental health study: Patient partners and community members helped craft the study name and materials to reduce the potential for stigma and to reframe the goal of the study as a movement toward emotional well-being rather than away from a mental health challenge. The anticipated benefit of this input is improved recruitment of study participants and greater acceptance of the study by the community in which it is occurring.
- Large pragmatic study comparing surgery to antibiotics: Over 800 patients were surveyed about their preferences for these treatment options and that input was used to shape the proposal. In this same study, significant clinician input changed the study inclusion criteria, study logistics, and criteria for "failure" for one of the arms.
- Diabetes study: Clinicians who reviewed the initial study design indicated that clinical practice is quite variable and suggested that a three-arm approach would be more appropriate for the study. The study design was revised accordingly and those changes aim to make the study more reflective of real clinical settings.
- Study on use of prescription drug for stroke patients: stroke survivors serving as patient partners on the study identified "home-time" or the number of days when a patient is living at home, not hospitalized or in another institution as an important new outcome. This input from patients was vital in directing the study toward an outcome that they truly cared about.
- Chronic pain study: The initial survey tool was lengthy and to be administered over the phone. Patient partners, feeling that a lengthy phone survey would create a barrier for chronic pain patients, shortened and redesigned the tool to be self-reported and -paced, facilitating greater ease of participation.



3. **DISSEMINATING THE STUDY RESULTS:** Describe how patient and stakeholder partners will be involved in plans to disseminate study findings and to ensure that findings are communicated in understandable, usable ways. (As you fill out Section 3 of your Engagement Plan, refer to the information below.)

Potential activities include:

- Identifying partner organizations for dissemination, to ensure meaningful and direct connections with end-users.
- Planning dissemination efforts, shaping study design and protocol from the very beginning to be focused on the final product.
- Participating in dissemination efforts, such as authoring manuscripts and presenting study findings, to offer the patient and stakeholder perspective and to reach new and different audiences
- Identifying opportunities to present or share information about the study, even as it is in progress, to move away from traditional models of dissemination and think more creatively about how to get information into the hands of those who need it.

Real-World Examples:

- Trauma study: The research team will convene a policy summit with relevant professional societies during the third year of the study to focus on identifying ways to speed the implementation of findings into practice.
- Care planning study: A national investing and financial planning firm is expanding their educational services to seniors, families, and their own financial planners. This firm will link to the study's website from its national website, and will disseminate the tool through their newsletters, financial planning conferences, and directly to high-impact planners.
- Large pragmatic study comparing surgery to antibiotics: Seven payers, three policymakers, and four large employers provided letters of support for the study and have agreed to disseminate findings to their networks when results are available. This involvement will ensure broad-based and diverse dissemination.
- Neurology study: The research team presented at a neurology patient advocacy conference to inform the community that this research was ongoing and to stay tuned for future results.
- Large pragmatic study about chronic pain: Physical therapists partnering in the study will design a Continuing Education (CE) program that will be delivered as part of the intervention. Patient partners will contribute by providing feedback on what kind of therapy and communication techniques will be more or less likely to be effective during an acute pain episode. Though also used during the course of the study, this CE can be an important dissemination tool upon study conclusion.

## **References**

PCORI Engagement Rubric. PCORI (patient-Centered Outcomes Research Institute) website. <http://www.pcori.org/sites/default/files/Engagement-Rubric.pdf>. Published February 4, 2014. Updated October 12, 2015. Accessed July 11, 2016.

FDA Roadmap to Patient-Focused Outcome Measurement in Clinical Trials FDA (US Food and Drug Administration website. <http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugDevelopmentToolsQualificationProgram/ucm284077.htm>. Page updated April 1, 2016. Accessed July 11, 2016.









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