Advancing Use of Patient Preference Information as Scientific Evidence in Medical Product Evaluation

December 7-8, 2017

Tommy Douglas Conference Center
Building 9
10000 New Hampshire Ave
Silver Spring, MD 20903

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DAY 1 AGENDA – Thursday, December 7

Time	Topic	Speaker
Starts at 7:30 am	**Registration**	
WELCOME SESSION: Patient Input and Regulatory Science		
8:30 am – 8:45 am	**Introduction**	**Carol Linden** FDA/ORSI
	Welcome Remarks	**RADM Denise Hinton** FDA/OCS
	Welcome from the CERSIs	**G. Caleb Alexander** Johns Hopkins University
SESSION 1: Fundamental Concepts and Regulatory Context of PPI to Support Medical Product Development and Evaluation Chair: Anindita Saha (FDA/CDRH) **Objective:** This session will provide a framework of how PPI can be used in regulatory decision making. The panel will discuss the pros and cons of doing PPI studies from the perspective of FDA, patients, industry, and academics.		
8:45 am – 10:15 am	**Introduction to Session 1**	**Anindita Saha** FDA/CDRH
	FDA Perspective on Patient Preference Information in Medical Product Evaluation	**Anindita Saha** FDA/CDRH **Million A. Tegenge** FDA/CBER
	Industry Perspective on PPI to Support Medical Product Development and Evaluation	**Bennett Levitan** Janssen R&D
	Academic Perspective on Patient Preference Research	**John F. P. Bridges** Johns Hopkins University
	Patient Perspective on Landscape	**K. Kimberly McCleary** FasterCures
	Discussion and Q&A	
10:15 am – 10:30 am	**Break**	

SESSION 2: Scientific Fundamentals of PPI Studies Chairs: Martin Ho (FDA/CDRH) & Leslie Wilson (UCSF-Stanford CERSI) **Objectives:** This session will introduce the science behind patient preference studies. The panel will discuss the intersection of scientific and regulatory needs in PPI studies and how all stakeholders can contribute to advancing the field of preference studies.		
10:30 am – 12:15 pm	**Introduction to Session 2**	**Martin Ho** FDA/CDRH
	Scientific Fundamentals of PPI Studies	**Juan Marcos Gonzalez** Duke University
	Panel Discussion and Q&A **FDA** • Martin Ho (FDA/CDRH) • Telba Irony (FDA/CBER) • Laura Lee Johnson (FDA/CDER) **CERSI and Academia** • Leslie Wilson (UCSF-Stanford CERSI) • Fadia T. Shaya (University of Maryland CERSI) • Erica S. Spatz (Yale-Mayo Clinic CERSI) • Brett Hauber (University of Washington) • Juan Marcos Gonzalez (Duke University) **Industry** • Becky Noel (Eli Lilly and Company)	
12:15 pm – 1:15 pm	**Lunch**	
SESSION 3: Discussion on In-Depth Case Studies Chairs: Telba Irony (FDA/CBER) & Michelle Campbell (FDA/CDER) **Objectives:** This session will use two hypothetical case studies to explore questions about how to initiate a patient preference study, conduct the study, and use the study results to inform decision-making. Each case study will cover various relevant scientific, regulatory, and implementation issues.		
1:15 pm – 1:20 pm	**Introduction to Session 3**	**Million Tegenge** FDA/CBER
1:20 pm – 2:45 pm	**Case Study 1: Rare Pediatric Cancer** Moderator: Michelle Campbell (FDA/CDER)	
	Introduction to Case Study 1	**Michelle Campbell** FDA/CDER
	Patient Perspective	**Nancy Goodman** Kids V Cancer
	FDA Clinical Perspective	**Gregory Reaman** FDA/OCE
	Preference Study Perspective	**Deborah A. Marshall** University of Calgary
	Panel Discussion and Q&A **FDA** • Michelle Campbell (FDA/CDER) • Gregory Reaman (FDA/OCE) • Paul G. Kluetz (FDA/OCE) **CERSI and Academia** • Leslie Wilson (UCSF-Stanford CERSI) • Deborah A. Marshall (University of Calgary) **Patient Perspective & Engagement** • Nancy Goodman (Kids V Cancer)	

	• Tamar Krishnamurti (University of Pittsburgh) **Industry** • Elisabeth (Liz) Piault-Louis (Genentech)	
2:45 pm– 3:00 pm	**Break**	
3:00 pm – 4:25 pm	**Case Study 2: Neurological Degenerative Disease** Moderator: Telba Irony (FDA/CBER)	
	Introduction to Case Study 2	**Telba Irony** FDA/CBER
	FDA Regulatory and Clinical Background	**Heather Benz** FDA/CDRH
	Patient Partnership Perspective	**Catherine Kopil** Michael J. Fox Foundation
	CERSI – Preference Study Perspective	**Ellen M. Janssen** Johns Hopkins University **Ira Shoulson** Georgetown University
	Panel Discussion **FDA** • Telba Irony (FDA/CBER) • Heather Benz (FDA/CDRH) • Kerry Jo Lee (FDA/CDER) **CERSI** • Ellen M. Janssen (Johns Hopkins University) • Ira Shoulson (Georgetown University) **Patient Engagement** • Catherine Kopil (Michael J. Fox Foundation) • Janel Hanmer (University of Pittsburgh) **Industry** • Kara L. Haas (Johnson & Johnson)	
4:25 pm – 4:45 pm	**Introduction to Patient Group**	**Kathryn M. O'Callaghan** FDA/CDRH
	Patient Group Experience in Patient Preference Studies	**Andrea Ferris** LUNGevity Foundation
4:45 pm – 5:00 pm	**Day 1 Closing Remarks**	**Kathryn M. O'Callaghan** FDA/CDRH

DAY 2 AGENDA – Friday, December 8

Time	Topic	Speaker
Starts at 7:30 am	**Registration**	
8:30 am – 8:45 am	**Welcome and Patient Preference Remarks**	**Theresa M. Mullin** FDA/CDER
SESSION 4: CDRH Preference Sensitive Areas Discussion: Diseases and Conditions Where Patient Preference Studies Could Be Useful Chair: Heather Benz (FDA/CDRH)		

Objectives: This session will address the following questions: 1) What makes a topic preference sensitive? 2) What conditions might decision-making be enhanced by data from a patient preference study? 3) When does a patient preference study add value?		
8:45 am – 10:00 am	**Introduction to Session 4**	**Heather Benz** FDA/CDRH
	FDA Perspective	**Vishal Bhatnagar** FDA/CDER **Million A. Tegenge** FDA/CBER
	CERSI Perspective	**Liana Fraenkel** Yale University
	Consortium Perspective	**Stephanie Christopher** Medical Device Innovation Consortium
	Patient Partnership Perspective	**Melissa West** Kidney Health Initiative **Frank Hurst** FDA/CDRH
	Q&A	
10:00 am – 10:15 am	**Break**	
SESSION 5: Capacity Building and Sustainability Chairs: Michelle Tarver (FDA/CDRH) & Fadia Shaya (University of Maryland) **Objective:** This session will identify the needs for building capacity in patient preference research for all stakeholders and outline the best practices from other multidisciplinary programs to develop and sustain expertise and programs to expand the field of patient preference research.		
10:15 am – 12:30 pm	**Introduction to Session 5**	**Michelle Tarver** FDA/CDRH
	Patient Perspective	**Cynthia Grossman** FasterCures
	FDA Perspective	**Ebony Dashiell-Aje** FDA/CDER
	Industry Perspective	**Matt Reaney** Sanofi
	Professional Society Perspective	**Shelby D. Reed** ISPOR
	Academic Perspective	**C. Daniel Mullins** University of Maryland
	Panel Discussion and Q&A • Michelle Tarver (FDA/CDRH) • Cynthia Grossman (FasterCures) • Ebony Dashiell-Aje (FDA/CDER) • Matt Reaney (Sanofi) • Shelby D. Reed (ISPOR) • C. Daniel Mullins (University of Maryland) • Joseph S. Ross (Yale University) • R. Scott Braithwaite (New York University)	
12:30 pm – 12:45 pm	**The CERSI Success Story: How the CERSI Collaborations Have Helped Advance the Field of Patient Preference**	**Frank F. Weichold** FDA/ORSI
12:45 pm – 1:00 pm	**Summary of the Day and Wrap-Up**	**Telba Irony** FDA/CBER **Frank F. Weichold** FDA/ORSI

CASE STUDY 1 - Rare Pediatric Cancer

Moderator: Michelle Campbell, PhD, Clinical Outcomes Assessment Staff, Office of New Drugs, CDER/FDA

Speakers:
Nancy Goodman, MPP, JD, Founder and Executive Director, Kids V Cancer
Gregory Reaman, MD, Associate Director (Acting) for Pediatric Oncology, Oncology Center of Excellence, Office of the Commissioner, FDA
Deborah A. Marshall, PhD, Professor and Canada Research Chair, Health Systems and Services Research, Cumming School of Medicine, University of Calgary

Additional Panel Participants:
Paul G. Kluetz, MD, Associate Director of Patient Outcomes (Acting), Oncology Center of Excellence, FDA
Elisabeth (Liz) Piault-Louis, PharmD, Associate Director Patient Centered Outcomes Research Oncology, Genentech, a member of the Roche Group
Tamar Krishnamurti, PhD, Assistant Professor of Medicine, University of Pittsburgh
Leslie Wilson, PhD, Professor of Clinical Pharmacy, UCSF School of Pharmacy

Background:
A patient group has approached the FDA about conducting a preference study for a rare pediatric cancer. The current standard of care is chemotherapy with limited effectiveness and several side effects, including nausea. Regardless of whether a child undergoes chemotherapy, the mortality rate is high.

Given the limited choices of treatment, the patient group is looking to spur medical product development by better understanding what outcomes are most important to patients and their parents. Additionally, recruiting for clinical trials is challenging, so the patient group is also looking to see what parents and children are willing and not willing to accept in a clinical trial protocol, including design features like blood draws and how many times patients must come in to see the doctor. They would also like to see if preferences vary between the parents and the children.

Questions for the Panel:
- How do you ensure that abstract concepts such as benefits, harms, uncertainty, and tradeoffs are understandable to children?
- What patient preference elicitation approaches and methods would be feasible in this population?
- How can industry and decision-makers use the results of the patient preference study?
- How do decision-makers use preference information if there are differences between parents and children? Would the same survey tool or a different survey tool be used for the two groups?
- What are ways to incorporate the patient preference information in the design of a clinical trial?

CASE STUDY 2 - Neurological Degenerative Disease

Moderator: Telba Irony, PhD, Deputy Director, Office of Biostatistics and Epidemiology, CBER/FDA

Speakers:
Heather Benz, PhD, Staff Fellow, Office of the Center Director, CDRH/FDA
Catherine Kopil, PhD, Director, Research Partnerships, The Michael J. Fox Foundation
Ellen Janssen, PhD, Assistant Scientist, Department of Health Policy and Management, Johns Hopkins Bloomberg School of Public Health
Ira Shoulson, MD, Professor of Neurology, Pharmacology and Human Science; Director, Program for Regulatory Science & Medicine (PRSM); Principal Investigator, Georgetown University CERSI

Panel Participants:
Kara Haas, MD, MPH, FACS, RAC, Global Regulatory Affairs Policy and Intelligence, Medical Device Evidence and Outcomes, Johnson & Johnson
Kerry Jo Lee, MD, Medical Officer, Guidance and Policy Team, Immediate Office, Office of New Drugs, CDER/FDA
Janel Hanmer, MD, PhD, Assistant Professor of Medicine, University of Pittsburgh; Medical Director, UPMC Patient Reported Outcomes Center

Background:
A medical product developer has approached the FDA about conducting a patient preference study for a neurological degenerative disease. The disease includes progressive loss of motor and cognitive function over the course of several years. Individual patients experience different rates of disease progression. The company notes that there has been stagnation in medical product development for the disease, and that there is no "cure," only symptom management options. Additionally, most clinical trials for this disease focus on motor symptoms. There is a significant unmet need, as there are no current treatments for the cognitive decline that patients experience.

The medical product developer would like to conduct a patient preference study to inform benefit-risk assessment for a new medical product. The medical product is unlike any existing products in this space:

- It has shown promise in treating both motor and cognitive decline.
- The likelihood of significant adverse events associated with this medical product is higher than for existing treatments for this disease.

Questions for the Panel:

- How can a patient preference study inform the benefit-risk assessment for this medical product, which has a new benefit-risk profile in this space?
- How would you approach constructing a patient preference survey when the benefits and risks and their likelihood is not completely known?
- How would you select, specify and confirm the attributes and their levels for the study out of many possible benefits and risks?
- In light of the varying rate of progressive loss of motor and cognitive function, are there considerations associated with preference heterogeneity or clinically relevant subgroups that should be incorporated in the design of this patient preference study?
- What are approaches to incorporating patient or care partner perspectives associated with the final stages of the disease, when the loss of cognitive function prevents some patients from communicating their preferences?

SPEAKER LISTING

Speaker biographies available at www.ucsfstanfordcersi.org/pp-workshop

Welcome Session: Patient Input and Regulatory Science

Carol Linden, PhD, Director, Office of Regulatory Science and Innovation (ORSI), Office of the Chief Scientist, Office of the Commissioner, FDA

RADM Denise Hinton, Acting Chief Scientist, Office of the Chief Scientist, Office of the Commissioner, FDA

G. Caleb Alexander, MD, MS, Johns Hopkins Bloomberg School of Public Health, Center for Drug Safety and Effectiveness; Program Director, Johns Hopkins University CERSI

Session 1: Fundamental Concepts and Regulatory Context of PPI to Support Medical Product Development and Evaluation

Anindita Saha, Director, External Expertise and Partnerships, Office of the Center Director, CDRH/FDA

Million A. Tegenge, RPh, PhD, Pharmacologist, Analytics and Benefit-Risk Assessment Team, Office of Biostatistics & Epidemiology, CBER/FDA

Bennett Levitan, MD, PhD, Senior Director, Benefit-Risk / Epidemiology, Global R&D Epidemiology, Janssen Research & Development

John F P Bridges, PhD, Associate Professor, Johns Hopkins CERSI

K. Kimberly McCleary, Acting Executive Director & Managing Director, FasterCures, A Center of the Milken Institute

Session 2: Scientific Fundamentals of PPI Studies

Martin Ho, MS, Associate Director for Quantitative Innovation, Office of Surveillance and Biometrics, CDRH/FDA

Juan Marcos Gonzalez, PhD, Assistant Professor, Duke University, Department of Population Health Sciences, Duke Clinical Research Institute

Laura Lee Johnson, PhD, Director (Acting) Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences, CDER/FDA

Leslie Wilson, PhD, Professor, Health Policy and Economics, Departments of Medicine and Pharmacy, University of California San Francisco

Fadia T. Shaya, PhD, MPH, Professor and Vice-Chair for Academic Affairs PHSR; Associate Director, Center on Drugs and Public Policy; University of Maryland School of Pharmacy

Erica S. Spatz, MD, MHS, Assistant Professor, Cardiovascular Medicine, Center for Outcomes Research and Evaluation, Yale University School of Medicine

Brett Hauber, PhD, Senior Economist, RTI Health Solutions; Affiliate Associate Professor, University of Washington

Becky Noel, DrPH, MSPH, Global Benefit-Risk Leader, Global Patient Safety, Eli Lilly and Company

Session 3: Discussion on In Depth Case Studies

Million A. Tegenge, RPh, PhD, Pharmacologist, Analytics and Benefit-Risk Assessment Team, Office of Biostatistics & Epidemiology, CBER/FDA

Michelle Campbell, PhD, Reviewer and Scientific Coordinator, Clinical Outcome Assessment Staff, Office of New Drugs, CDER/FDA

Nancy Goodman, MPP, JD, Founder and Executive Director, Kids V Cancer

Gregory Reaman, MD, Associate Director (Acting) for Pediatric Oncology, Oncology Center of Excellence, Office of the Commissioner, FDA

Deborah A. Marshall, PhD, MHSA, Professor and Canada Research Chair, Health Systems and Services Research, Cumming School of Medicine, University of Calgary

Paul G. Kluetz, MD, Associate Director of Patient Outcomes (Acting), Oncology Center of Excellence, FDA

Elisabeth (Liz) Piault-Louis, PharmD, Associate Director, Patient Centered Outcomes Research Oncology, Genentech, a member of the Roche Group

Leslie Wilson, PhD, Professor, Health Policy and Economics, Departments of Medicine and Pharmacy, University of California San Francisco

Tamar Krishnamurti, PhD, Assistant Professor, University of Pittsburgh School of Medicine

Telba Irony, PhD, Deputy Director, Office of Biostatistics and Epidemiology, CBER/FDA

Heather Benz, PhD, Staff Fellow, Office of the Center Director, CDRH/FDA

Catherine Kopil, PhD, Director, Research Partnerships, The Michael J. Fox Foundation

Ellen M. Janssen, PhD, Assistant Scientist, Department of Health Policy and Management, Johns Hopkins Bloomberg School of Public Health

Ira Shoulson, MD, Professor of Neurology, Pharmacology and Human Science; Director, Program for Regulatory Science & Medicine (PRSM): Principal Investigator, Georgetown University CERSI

Kerry Jo Lee, MD, Medical Officer, Guidance and Policy Team, Immediate Office, Office of New Drugs, CDER/FDA

Janel Hanmer, MD, PhD, Assistant Professor of Medicine, University of Pittsburgh; Medical Director, UPMC Patient Reported Outcomes Center

Kara L. Haas, MD, MPH, FACS, RAC, Global Regulatory Affairs Policy and Intelligence, Medical Device Evidence and Outcomes, Johnson & Johnson

Kathryn M. O'Callaghan, CDRH Assistant Director for Strategic Programs, Office of the Center Director, CDRH/FDA

Andrea Ferris, President and CEO, LUNGevity Foundation

Day 2 Welcome

Theresa M. Mullin, PhD, Director, Office of Strategic Programs, CDER/FDA

Session 4: CDRH Preference Sensitive Areas Discussion: Diseases And Conditions Where Patient Preference Studies Could be Useful

Heather Benz, PhD, Staff Fellow, CDRH/FDA

Vishal Bhatnagar, MD, Medical Officer, Office of Hematology Oncology Products, CDER/FDA

Million A. Tegenge, RPh, PhD, Pharmacologist, Analytics and Benefit-Risk Assessment Team, Office of Biostatistics & Epidemiology, CBER/FDA

Liana Fraenkel, MD, MPH, Professor of Medicine, Yale University School of Medicine

Stephanie Christopher, MA, Program Director, Science of Patient Input, Medical Device Innovation Consortium

Melissa West, Project Director, Kidney Health Initiative

Frank Hurst, MD, Medical Officer, Renal Devices Branch, Division of Reproductive, Gastro-Renal, and Urological Devices, Office of Device Evaluation, CDRH/FDA

Session 5: Capacity Building and Sustainability

Michelle Tarver, MD, PhD**,** Ophthalmologist/Epidemiologist; Division of Ophthalmic and Ear, Nose, and Throat Devices; Office of Device Evaluation, CDRH/FDA

Cynthia Grossman, PhD, Associate Director, Science of Patient Input, *FasterCures*

Ebony Dashiell-Aje, PhD, Reviewer, Clinical Outcome Assessments Staff, Office of New Drugs, CDER/FDA

Matt Reaney, CPsychol, CSci, MSc; Global Head of Clinical Outcomes, Sanofi

Shelby D. Reed, PhD, Professor in Population Health Sciences and Medicine, Preference Evaluation Research (PrefER), Group Duke Clinical Research Institute

C. Daniel Mullins, PhD, Professor, Pharmaceutical Health Services Research Department (PHSR), University of Maryland School of Pharmacy

Joseph S. Ross, MD, MHS, Associate Professor of Medicine (General Medicine) and Public Health (Health Policy and Management), Yale University

R. Scott Braithwaite, MD, MS, FACP, Chief, Division of Comparative Effectiveness and Decision Sciences; Professor of Population Health and Medicine, Department of Population Health, New York University School of Medicine

Frank F. Weichold, MD, PhD, Director, Critical Path and Regulatory Science Initiatives, Office of Regulatory Science and Innovation, Office of the Chief Scientist, Office of the Commissioner, FDA

Telba Irony, PhD, Deputy Director, Office of Biostatistics and Epidemiology, CBER/FDA

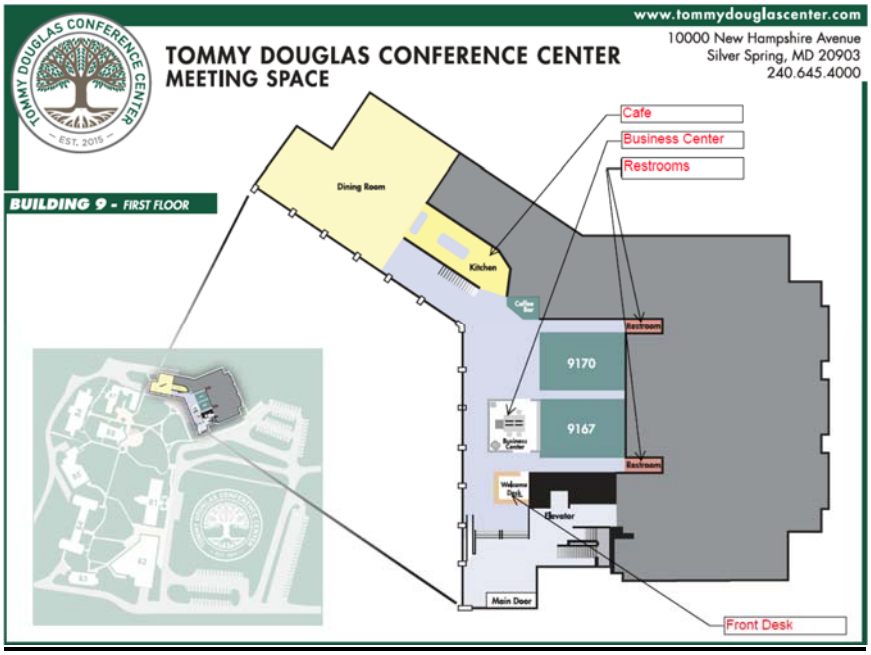
LOGISTICAL INFORMATION

Workshop Website

www.ucsfstanfordcersi.org/pp-workshop

TDCC Maps

First Floor



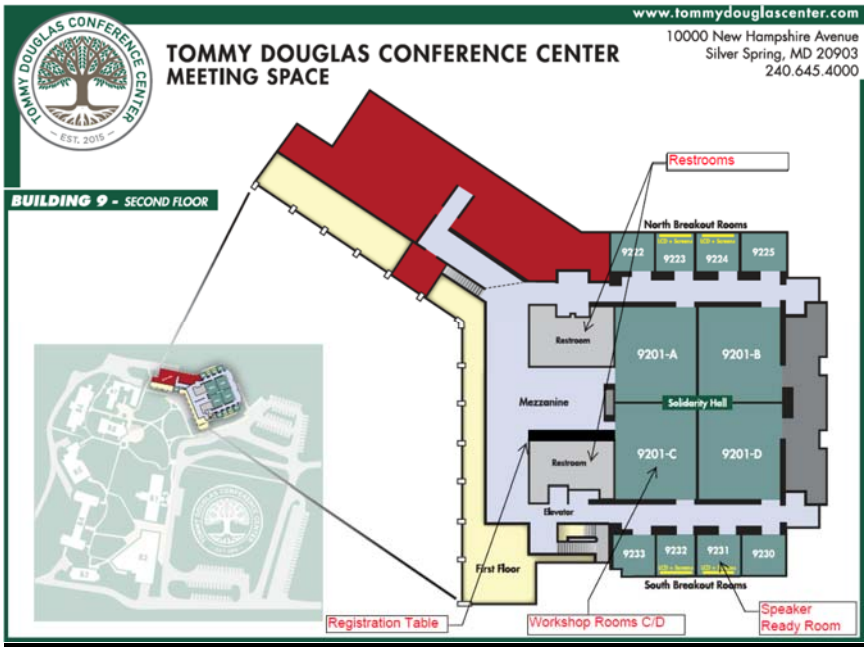
TDCC Café Hours

Thursday, December 7th
Breakfast: 7:00-9:15 am
Lunch: 11:30-1:45 pm
Dinner: 5:00-7:30 pm
The café will stay open during breaks.

Friday, December 8th
Breakfast: 7:00-9:15 am
Lunch: 11:30-2:30 pm
The café will stay open between meals.

Pre-Order Lunch or Dinner at the First Floor Front Desk – Avoid Long Lines!
You are encouraged to pre-order lunch or dinner at the TDCC first floor front desk (credit/debit card only). Meal voucher/ receipt will be provided.

Second Floor



Wifi Information

Network Name: TDCC
Password: Bufu986!

Speaker Ready Room

The Speaker Ready Room is in room 9231 on the second floor. This room is available for all scheduled speakers to organize materials.

The Speaker Ready Room will be open:
Thursday, December 7: 8:00 am to 3:00 pm
Friday, December 8: 8:00 am to 11:00 am

ACKNOWLEDGEMENTS

Sincere appreciation is extended to all the Workshop Planning Committee members from the U.S. Food and Drug Administration and the Centers for Excellence in Regulatory Science and Innovation (CERSIs), listed below, who worked collaboratively to organize and conduct this workshop.

FDA, Center for Devices and Radiological Health (CDRH):
Kathryn O'Callaghan, Anindita Saha, Mimi Nguyen, Heather Benz, Martin Ho, Michelle Tarver, Xiting (Cindy) Yang

FDA, Center for Drug Evaluation and Research (CDER):
Sara Eggers, Pujita Vaidya, Elektra Papadopoulos, Ruth Barratt, Chekesha Clingman, Laura Lee Johnson, Ebony Dashiell-Aje, Scott Komo, Michelle Campbell, Selena Daniels, Meghana Chalasani, Vishal Bhatnagar, Paul Kluetz

Center for Biologics Evaluation and Research (CBER):
Telba Irony, Megan Moncur, Maura O'Leary, Ke Liu, Million Tegenge

FDA, Office of Regulatory Science and Innovation (ORSI)/Office of the Chief Scientist (OCS):
Carol Linden, Frank Weichold, York Tomita, Khaled Bouri, Rebekah Zinn, Audrey Thomas, Amal Manseur, Donna Blum-Kemelor

Centers for Excellence in Regulatory Science and Innovation (CERSIs):
- **UCSF-Stanford CERSI:** Leslie Wilson, Lawrence Lin
- **Johns Hopkins University CERSI:** John Bridges, Ellen Janssen, Jamie Heyward
- **Georgetown CERSI:** Ira Shoulson, Erin Wilhelm
- **University of Maryland CERSI:** Fadia Shaya, Ann Anonsen, Priyanka Gaitonde, Savyasachi Shah, Doris Titus-Glover
- **Yale-Mayo Clinic CERSI:** Liana Fraenkel, Erica Spatz, Victor Montori, Jessica Ritchie, Laura Ciaccio

To learn more about the CERSIs, see:
https://www.fda.gov/ScienceResearch/SpecialTopics/RegulatoryScience/ucm301667.htm