WELCOME!

SECOND ANNUAL WORKSHOP ON CLINICAL OUTCOME ASSESSMENTS IN CANCER CLINICAL TRIALS

April 25, 2017 ■ Bethesda, MD

Co-sponsored by

FDA U.S. FOOD & DRUG ADMINISTRATION

CRITICAL PATH INSTITUTE
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Workshop Packet Contents

- Welcome Letter
- Workshop Agenda
- Presenters and Panelists Biographical Sketches
- Pre-Registrant List
- ***Workshop Feedback Form***

Active Participation During the Q&A Portion of Each Session is Encouraged

Before you speak, please step to a microphone or let us bring a microphone to you

The workshop is being audio recorded

Please turn off cell phones or set to vibrate

Wireless Network Connection

- Select @Hyatt_Meeting
- Open browser to Hyatt Bethesda Log-In Screen
- Select “I agree”
- Login Information
  - Meeting Name: Critical Path
  - Passcode: COA2017

Acknowledgments

We will end the workshop later today with more specific expressions of gratitude, but to begin the day we want to sincerely thank the dedicated FDA and C-Path staff members who, along with the distinguished panelists, worked so hard to bring this year's workshop from concept to reality.

And, thank you all for being here today. We look forward to a very interactive, productive, and exceptionally worthwhile workshop.

Overall Workshop Goal

This annual workshop was initiated last year to advance our understanding of the complex regulatory, healthcare policy, and scientific issues surrounding the use of patient-reported (PRO) measures in cancer clinical trials

In this year's workshop, we have assembled a broad array of individuals (e.g., patients, clinicians, regulators, measurement scientists) to discuss the use of PRO measures in the assessment of safety and tolerability. We will explore the utility of information derived from new and existing PRO measures, and identify potential ways to improve alignment and strategic use of patient-reported adverse event data to support oncology drug development and better inform treatment decisions.

Workshop Topics

- Session 1: Exploring the Concepts of Safety and Tolerability – Incorporating the Patient Voice
- Session 2: *Assessment* of Safety and Tolerability – Emerging Patient-Reported Methods
- Session 3: *Analysis and Display* of PRO-Based Tolerability Data – Metrics and Paths Forward
- Session 4: From Individual Symptoms to Overall Side Effect Burden

FDA Oncology:
Patient Focused Drug Development

Recent Legislative Efforts to Promote Patient Focused Drug Development

- 2012 FDA Safety and Innovation Act
  - Patient Focused Drug Development (PFDD)
- 2016 Vice President Biden's Cancer Moonshot
  - Blue Ribbon Panel prioritizes incorporating the patient into improved symptom assessment and management for cancer therapies
- 2016 21st Century Cures Act
  - Set up disease-specific cross-center institutes

Oncology Center of Excellence

- FDA Inter-center Institute as Part of 21st Century Cures Act
- Integrated approach to clinical evaluation of cancer products
- Leverages combined skills of regulatory scientists and reviewers from the 3 key centers who review cancer products

Center for Drug Evaluation and Research (CDER)
- Drugs and Antibodies.
- Office of Hematology and Oncology Products

Center for Biologics Evaluation and Research (CBER)
- Cellular and Gene Therapies, Vaccines.

Center for Devices and Radiologic Health (CDRH)
- Devices, In Vitro Diagnostics, Diagnostic and Therapeutic Radiologics.

OCE Patient Focused Drug Development (PFDD) Program Mission Statement:

Cancer therapies are developed to treat patients, not their disease. Cancer patients experience disease symptoms and symptomatic treatment side effects that can impact their ability to function and other aspects of their health related quality of life. The Oncology Center of Excellence PFDD program fosters collaboration between FDA Centers and external stakeholders involved in patient outcomes research in cancer populations. The OCE PFDD program focuses on three key areas: Actively engaging with patients and advocacy groups, fostering research into measurement of the patient experience, and generating science-based recommendations for regulatory policy. The overarching goal is to identify rigorous methods to assess the patient experience that will complement existing survival and tumor information to better inform a cancer therapy's effect on the patient.

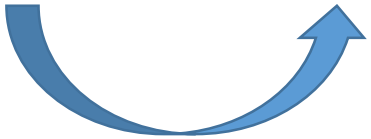
https://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/OCE/ucm544143.htm

Oncology Center of Excellence (OCE)
Patient Focused Drug Development (PFDD) Program

Oncology PFDD Education and Outreach
- Symposia/Workshops
- Reviewer Education
- Patient Engagement

Oncology PFDD Science
- Analysis and Presentation Methods
- Real-World COA Data
- Preference Data
- Mobile Devices / Wearables

Oncology PFDD Regulatory Policy
- Consistency of advice
- SOPs, Guidances
- Review Practices



CDRH

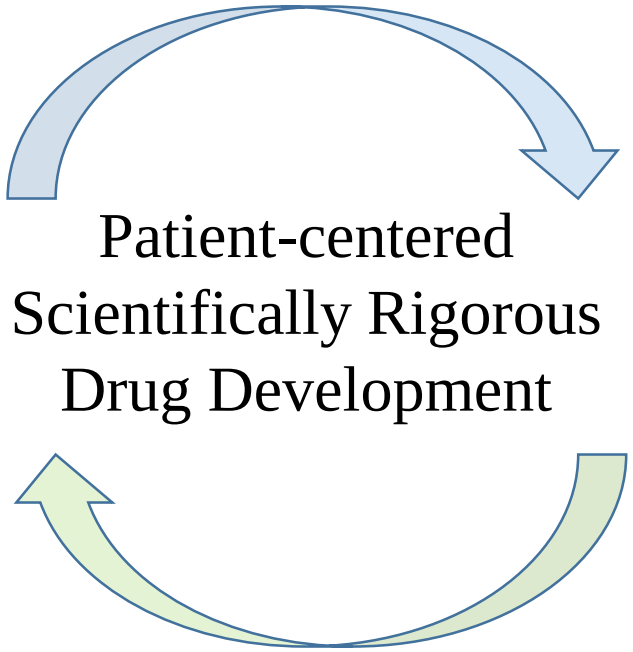
CBER

CDER
- COA Staff
- Office of Biostatistics
- Office of Prescription Drug Promotion
- Office of Strategic Programs
- Office of Medical Policy

Patient Engagement is a Dialogue

Patients

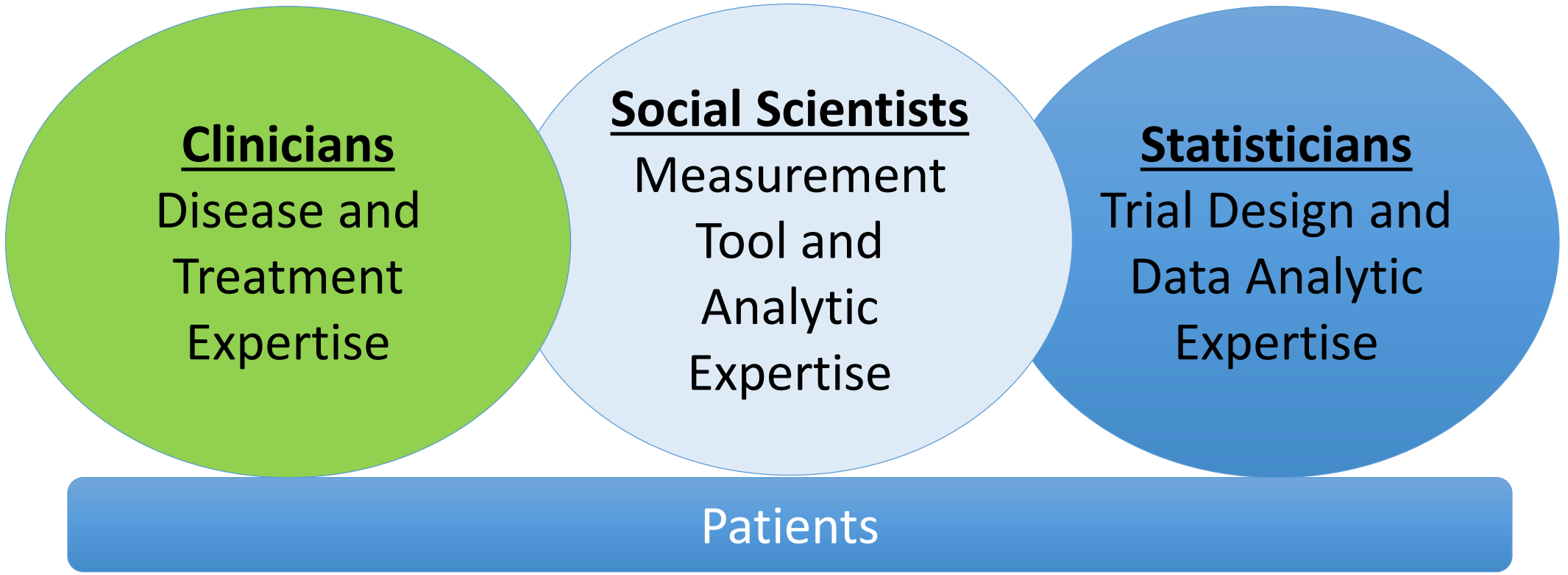
- Experts in how they experience their disease
- Identify what matters most to patients
- Identify areas to make clinical trials more patient-friendly



Clinicians/ Trialists/ Health Policy Leaders

- Experts in clinical trial design and conduct
- Medical expertise
- Assess feasibility of trial modifications and outcome measures

Advancing COA Science- Everyone to the Table



Goal: Translating what is important to patients into rigorous clinical trial data

FDA Oncology: Patient Focused Drug Development

- Great momentum to advance the science of PRO measurement, analysis and presentation

- Oncology Center of Excellence has prioritized patient-focused drug development as one of its initial programs

- **We will continue to seek international collaboration to advance measurement of the patient experience**

So Let's Get Started- Session 1

Clinical Trial Measures are Intended to Address a Specific Objective

One Important Objective is Safety and Tolerability- what are these terms and how do they differ from one another?

Session 1- Moderator: **Bindu Kanapuru, Clinical Reviewer, U.S. FDA**