Capacity Building and Sustainability in Patient Preference Research: A Regulatory Perspective

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FDA Disclaimer

• The views expressed in this presentation are those of the speaker, and do not necessarily represent an official FDA position.
Objectives

To Discuss:

– Key considerations for building capacity within FDA and externally to facilitate high-quality patient preference information (PPI) study design, conduct and review.

– Existing infrastructure within the FDA that can be leveraged for PPI study adoption and implementation

– Next steps for moving toward wider adoption and implementation of PPI studies within the regulatory context
Context: Usefulness of PPI Studies within the Regulatory Context
Patient Preference Information: Measuring the Patient Experience

- Patient Preference Information
- Symptoms and Natural history
- Importance of Outcomes and Issues
- Impacts of a condition
Building Capacity for Adoption and Implementation: Key Considerations
Key Considerations for Capacity Building within the FDA

- Staff Resources
- Training and Education (PPI-Specific)
- Standard Setting
- Dissemination
- Cross-Center Adoption
Challenges and Barriers for Capacity Building, PPI Adoption and Implementation

- Staff Resources
- Training and Education (PPI-Specific)
- Standard Setting
- Cross-Center Adoption
- Dissemination
Key Considerations Related to Challenges

What elements are necessary to deem a PPI Study suitable for regulatory review?

What are the best mechanisms for internal and external education and outreach?

How do we increase awareness regarding the usefulness of PPI studies in the pre-and post-market environment?

What are PPI methodological and data considerations and how can these be better adopted externally?

What do regulatory reviewers need to know to be prepared to review a study?
Surmounting Challenges: Existing Mechanisms to Help Facilitate PPI Study Adoption and Implementation
Infrastructure
Foundations for Successful Adoption and Implementation

• Patient-Focused Medical Product Development Initiatives
  – Specific to each Center (CDER, CDRH, CBER) and implemented to provide a formal mechanism for FDA to develop a more systematic way of gathering patient perspective. Designed to help provide greater patient input on benefit-risk to inform FDA analyses both during and outside of medical product review.

• Clinical Outcome Assessment Staff
  – Established in 2002 within the Office of New Drugs to serve as a cross-divisional resource on patient-reported outcomes (PROs) intended to support labeling claims.
  – The COA Staff capacity and responsibilities have since expanded to better meet needs. Likewise, to remain scientifically relevant, expertise has expanded beyond PROs to other COAs (ClinROs, ObsROs, PerFOs) and other patient experience data (e.g., patient preference information).
Guidances
FDA PRO Guidance (2009)

- Defines **good measurement principles** to consider for “well-defined and reliable” (21 CFR 314.126) PRO measures intended to provide evidence of clinical benefit

- All clinical outcome assessments can benefit from the good measurement principles described within the guidance

- Provides **optimal approach** to PRO development; **flexibility** and judgment needed to meet practical demands
Patient Preference Information Guidance (2016)

- Provides guidance on **best practices and good principles** to consider for PPI that may be used by FDA in PMAs, HDE applications and *de novo* requests within CDRH and CBER

- The guidance also provides several hypothetical examples that illustrate how PPI may inform regulatory decision-making
Communication Pathways
Pathways for FDA Clinical Outcome Assessment Review & Advice: PPI Study Application

1. Medical Product Development Pathway
   - **Within** a medical product development program
   - Sponsor submissions to FDA
   - Potential to result in *labeling* claims

2. Qualification Pathway
   - **Outside** of a medical product development program
   - Development of novel or existing COAs for use in multiple medical product development programs addressing unmet measurement needs
   - Potential to result in *qualification* of COA

3. Informal Pathways for General Advice
   - **Outside** of an individual medical product development program
   - Potential for *general advice* on specific methodology or technology in its early stages of development

COA = Clinical Outcome Assessment
Summary

• Next steps in moving toward wider PPI study adoption and implementation within the regulatory context:
  – Build internal staff capacity – utilize and build upon existing infrastructure
  – Encourage external stakeholder capacity building
  – Increase expertise through internal and external education and outreach
  – Further establish and disseminate standards and best practice documents to help guide PPI study design and implementation and PPI data standards
  – Encourage and engage in early planning and discussion between FDA and external stakeholders to ensure that PPI study designs and data are suitable for use within the regulatory context
  – Continue to evolve – learning from experiences and embracing growth
Helpful links

- FDA COA Staff Website: http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ucm349031.htm#Endpoints