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

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FDA Disclaimer

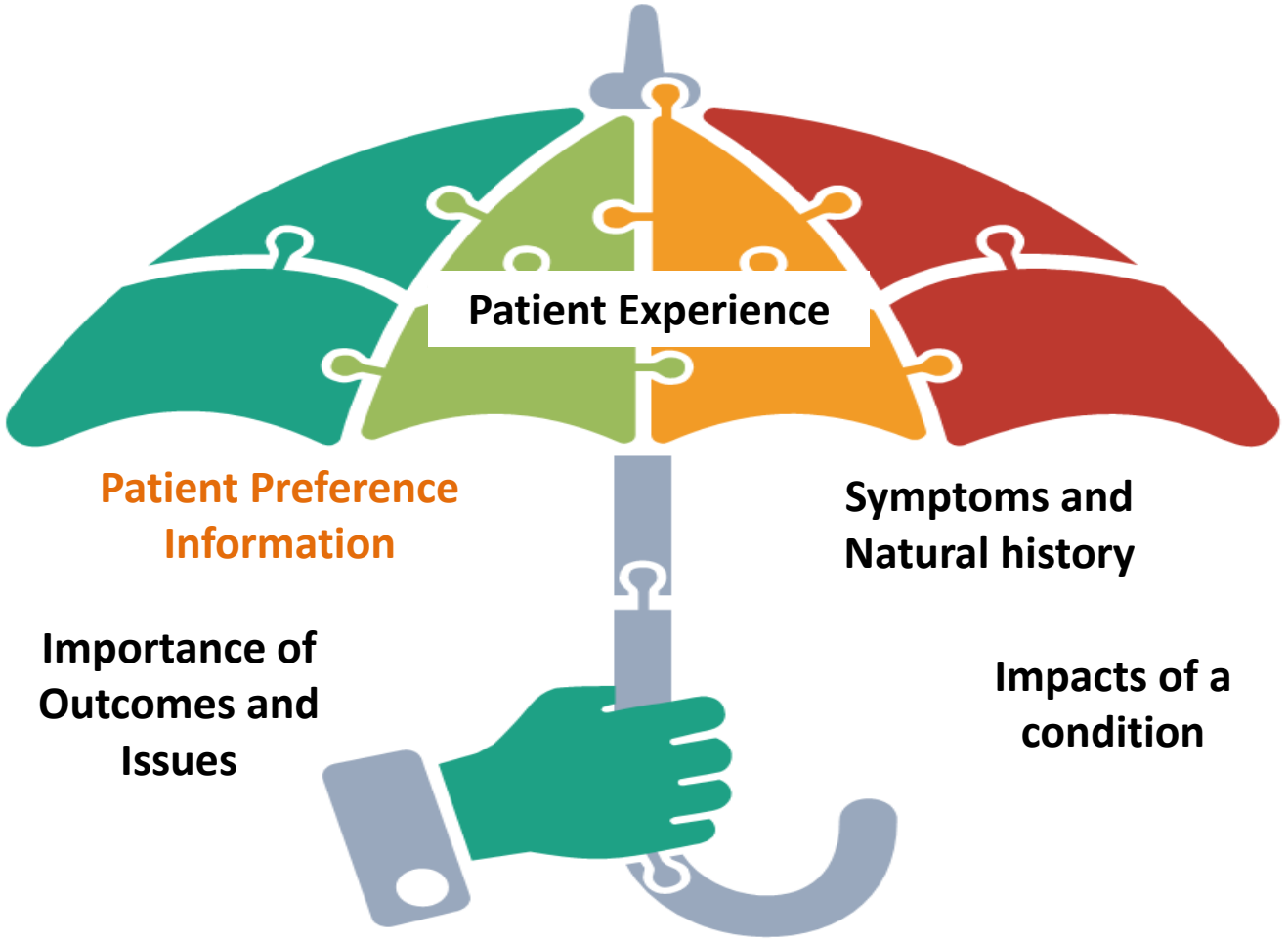
- The views expressed in this presentation are those of the speaker, and do not necessarily represent an official FDA position.

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

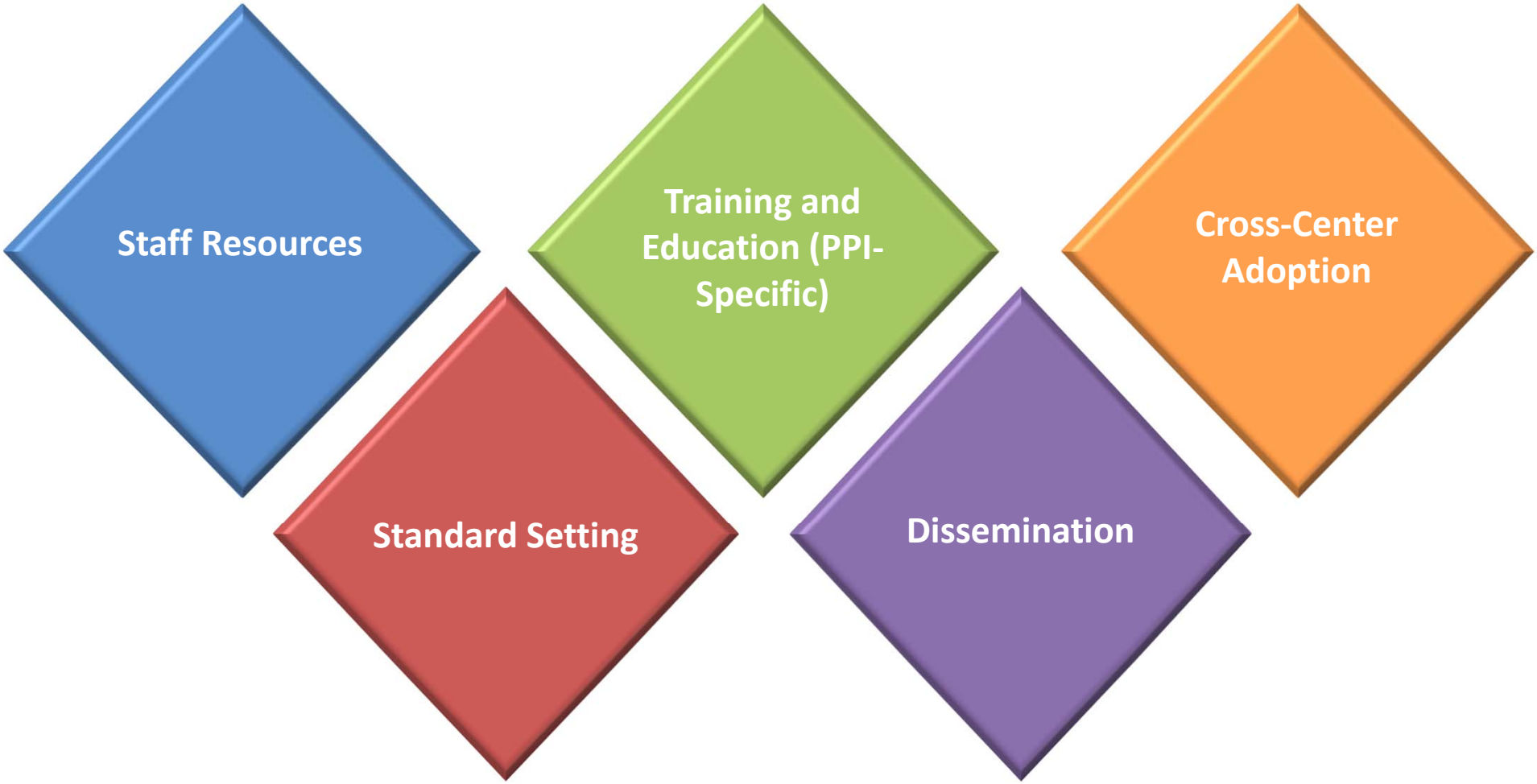
Context: Usefulness of PPI Studies within the Regulatory Context

Patient Preference Information: Measuring the Patient Experience

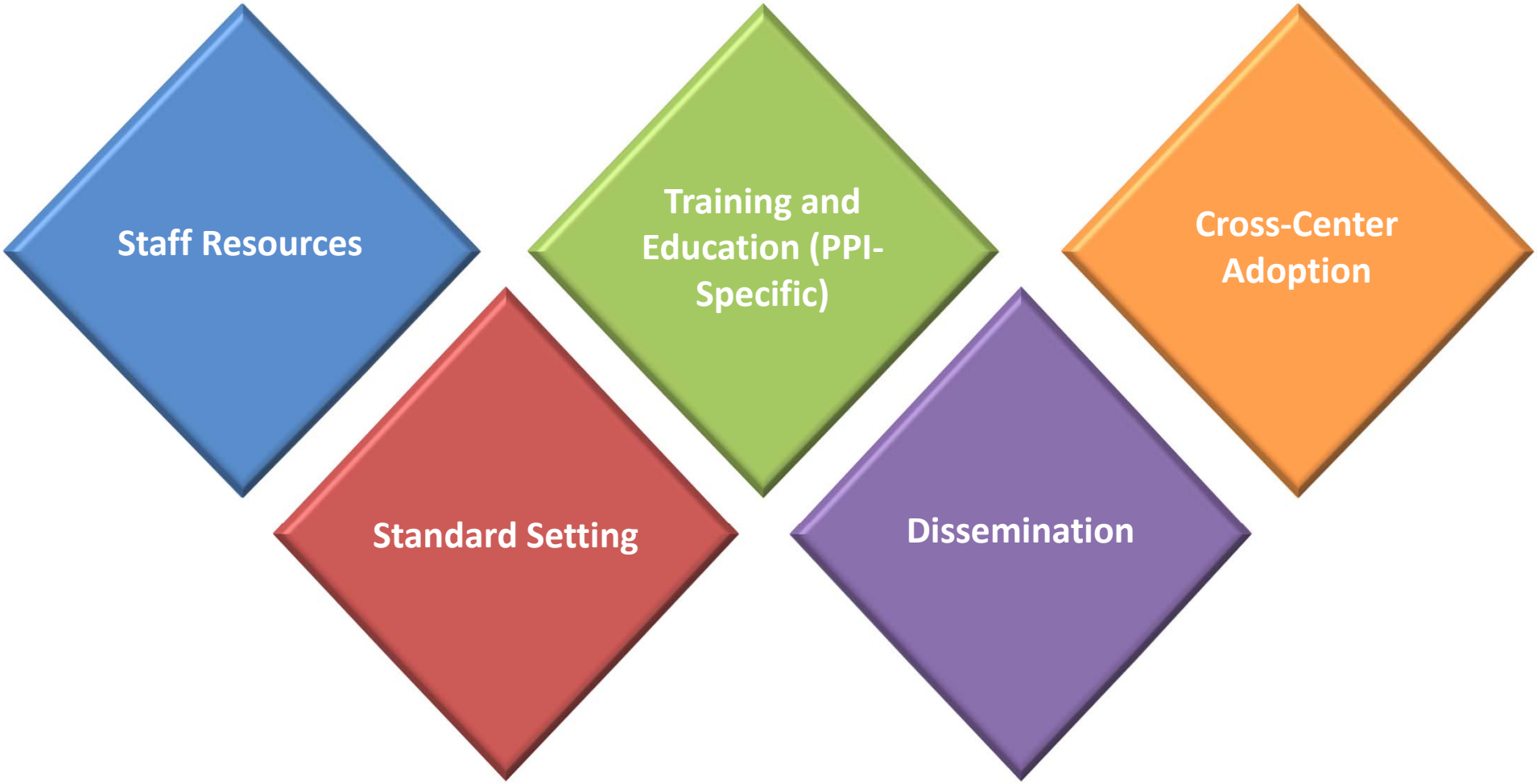


Building Capacity for Adoption and Implementation : Key Considerations

Key Considerations for Capacity Building within the FDA



Challenges and Barriers for Capacity Building, PPI Adoption and Implementation

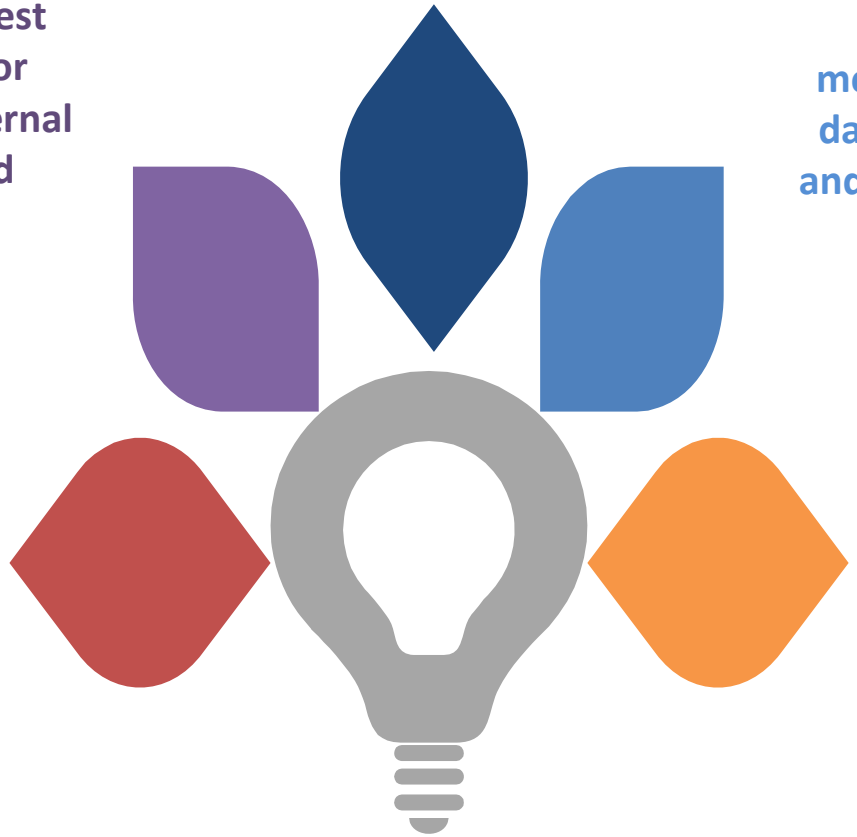


Key Considerations Related to Challenges

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?

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



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

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

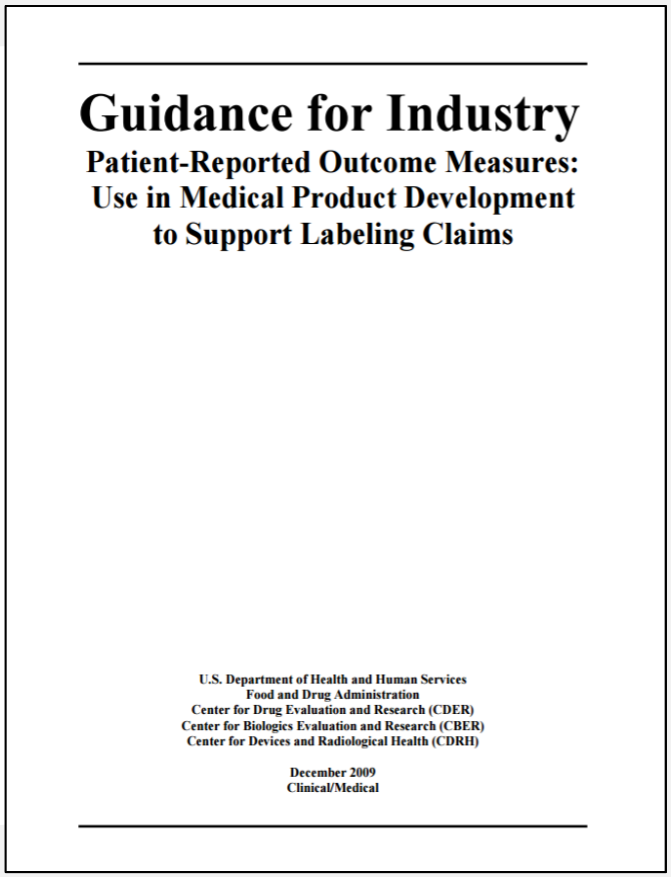
Infrastructure

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)



Guidance for Industry

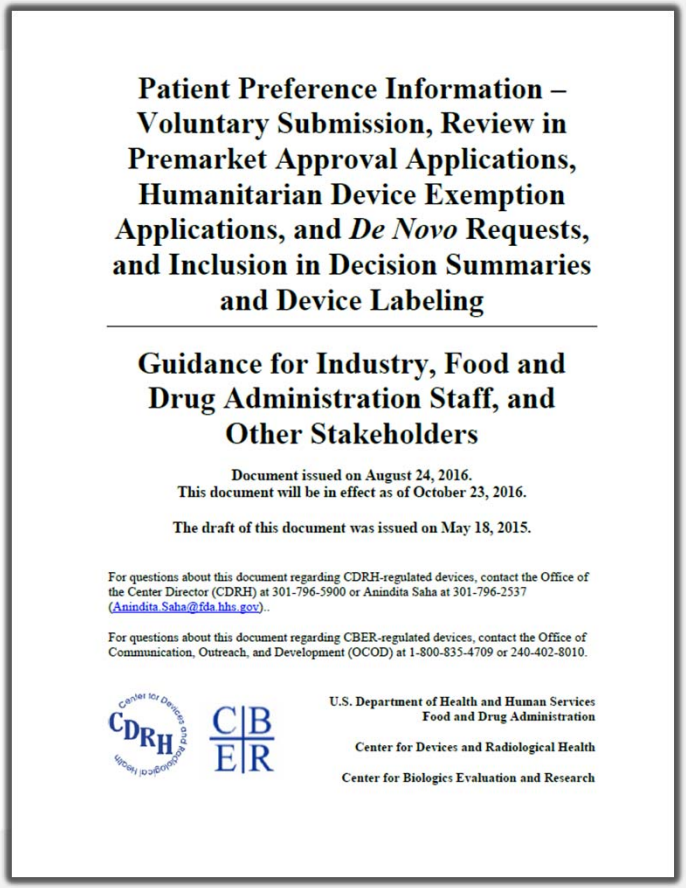
Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Center for Devices and Radiological Health (CDRH)

December 2009
Clinical/Medical

- 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)



Patient Preference Information – Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and *De Novo* Requests, and Inclusion in Decision Summaries and Device Labeling

Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

Document issued on August 24, 2016.
This document will be in effect as of October 23, 2016.

The draft of this document was issued on May 18, 2015.

For questions about this document regarding CDRH-regulated devices, contact the Office of the Center Director (CDRH) at 301-796-5900 or Anindita Saha at 301-796-2537 (Anindita.Saha@fda.hhs.gov)..

For questions about this document regarding CBER-regulated devices, contact the Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration

Center for Devices and Radiological Health

Center for Biologics Evaluation and Research

- 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
- PRO Guidance: http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM193282.pdf
- PPI Guidance: https://www.fda.gov/downloads/medicaldevices/deviceregulationandguidance/guidancedocuments/ucm446680.pdf
- DDT Qualification Guidance: http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM230597.pdf
- COA DDT Qualification Website: http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugDevelopmentToolsQualificationProgram/ucm284077.htm
- Medical Device Development Tool Qualification Guidance: https://www.fda.gov/downloads/medicaldevices/deviceregulationandguidance/guidancedocuments/ucm374432.pdf
- Critical Path Innovation Meeting Website & Guidance: http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugInnovation/ucm395888.htm
- CDER COA Compendium: https://www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/DevelopmentResources/UCM481225.pdf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