Welcome To Today's Webinar

Thanks for joining us!
We'll get started in a few minutes

Today's Topic:

Final Guidance, Principles for Selecting, Developing, Modifying, and Adapting Patient-Reported Outcome Instruments for Use in Medical Device Evaluation

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Final Guidance: Principles for Selecting, Developing, Modifying, and Adapting Patient-Reported Outcome Instruments for Use in Medical Device Evaluation

Fraser Bocell, MEd, PhD
Psychometrician, Patient Science and Engagement
Office of Strategic Partnerships and Technology Innovation
Center for Devices and Radiological Health

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Final Guidance: Principles for Selecting, Developing, Modifying, and Adapting Patient-Reported Outcome Instruments for Use in Medical Device Evaluation

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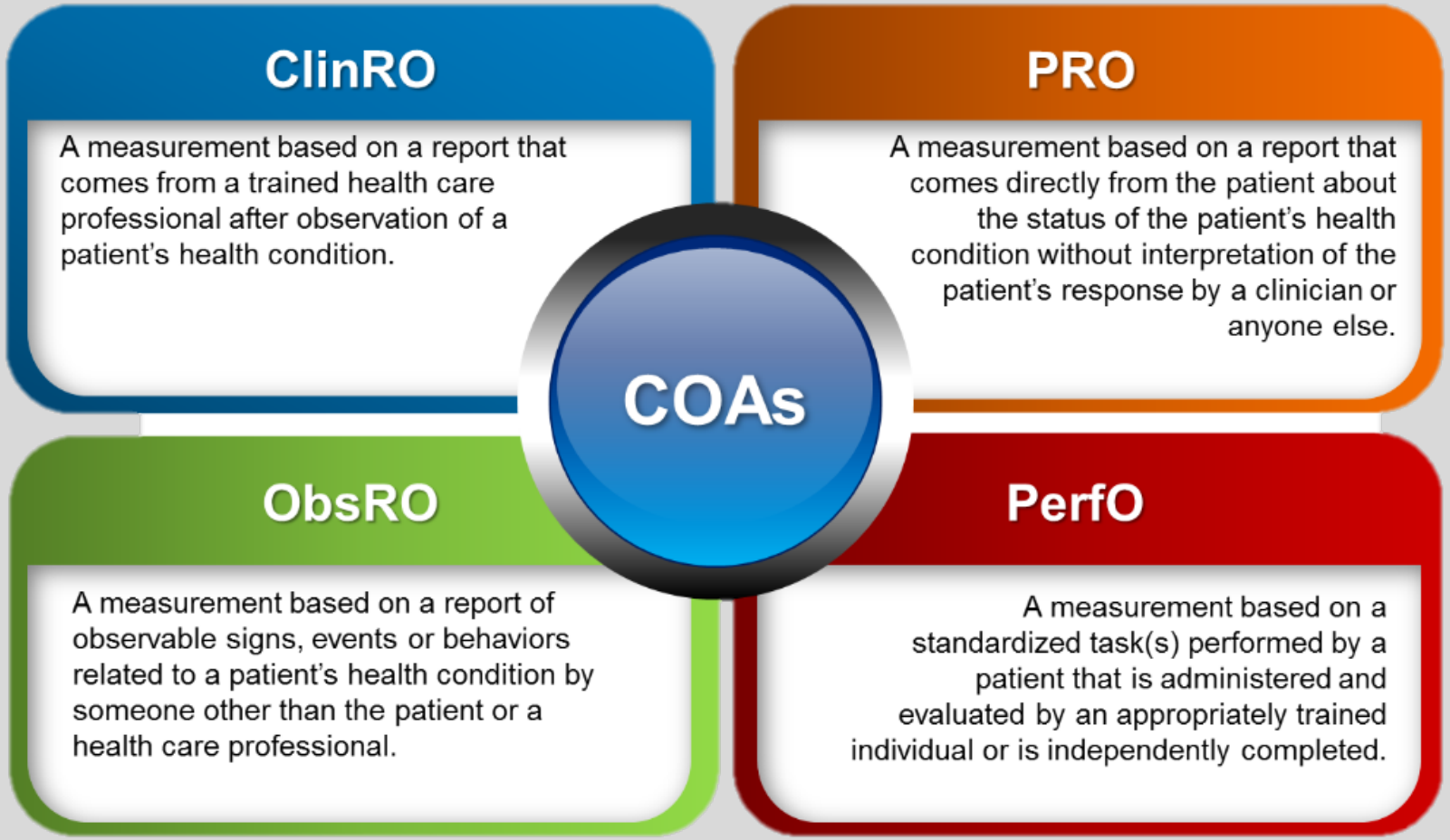
Learning Objectives

- Summarize FDA's current patient-reported outcome (PRO) guidances, as well as the Clinical Outcome Assessment efforts underway
- Describe contents of CDRH final guidance on PRO instruments including:
  - Key principles
  - Steps for ensuring instruments are fit for purpose
  - Best practices for selecting, developing, modifying and adapting PRO instruments
- List available PRO-related resources

Background on Patient-Reported Outcome Guidance Efforts

Types of Clinical Outcome Assessments (COAs)

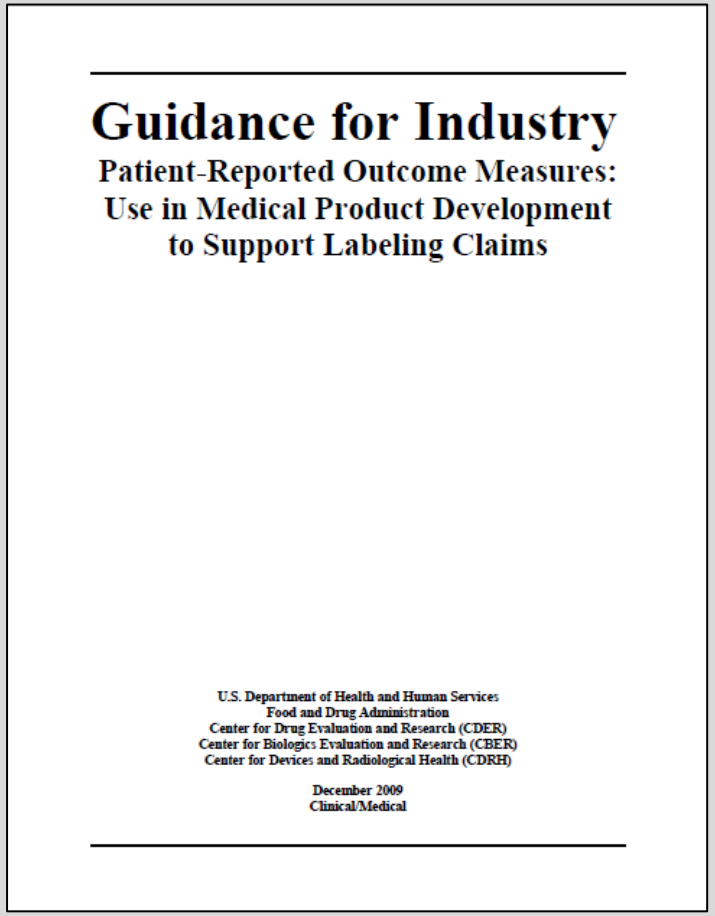


ClinRO = Clinician-Reported Outcome
ObsRO = Observer-Reported Outcome

PRO = Patient-Reported Outcome
PerfO = Performance Outcome

Biomarkers, Endpoints, and Other Tools (BEST) Resource: www.ncbi.nlm.nih.gov/books/NBK338448/

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



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

- Focused on use of PRO instruments to support efficacy claims made in medical product labeling
- Often cited in development and review of PRO instruments in literature
- Other potential uses of PRO instruments are not directly addressed

Patient-Focused Drug Development (PFDD) Guidance Series

Guidance 1: Collecting Comprehensive and Representative Input	▪ Workshop held December 18, 2017 ▪ Issued Final Guidance June 18, 2020
Guidance 2: Methods to Identify What is Important to Patients	▪ Workshop held October 15-16, 2018 ▪ Issued Draft Guidance December 2019
Guidance 3: Selecting, Developing or Modifying Fit-for-Purpose Clinical Outcome Assessments	▪ Workshop held October 15-16, 2018 ▪ Draft in progress
Guidance 4: Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision Making	▪ Workshop held December 6, 2019 ▪ Draft in progress

www.fda.gov/drugs/development-approval-process-drugs/fda-patient-focused-drug-development-guidance-series-enhancing-incorporation-patients-voice-medical

Contents of the Final Guidance: Principles for Selecting, Developing, Modifying, and Adapting Patient-Reported Outcome Instruments for Use in Medical Device Evaluation

Objectives of Guidance

1. Describe principles that may be considered when using PRO instruments in the evaluation of medical devices
2. Provide recommendations about importance of ensuring PRO instruments are fit-for-purpose
3. Outline best practices to help ensure relevant, reliable, and sufficiently robust PRO instruments are developed, modified, adapted using the least burdensome approach

Scope of Guidance

- Applies to PRO instruments used in medical device evaluation across total product life cycle
- Intended to supplement the aforementioned resources
- Communicates what the FDA believes are some best practices
- Does not detail methods and steps of developing, modifying or adapting a PRO instrument

Key Principles

1. Establish and define the concept of interest (COI) that the PRO instrument intends to capture
2. Clearly identify the role of the PRO (e.g., primary, secondary, ancillary, effectiveness, safety) in clinical study protocol and statistical analysis plan
3. Provide evidence showing that the PRO instrument reliably assesses COI
4. Effectively and appropriately communicate PRO-related results in labeling to inform healthcare provider and patient decision making

Ensuring PRO Instruments are Fit-For-Purpose

Fit-for-purpose: A conclusion that the level of validation associated with a biomarker or COA is sufficient to support its context of use.

Factors to consider:

1. Is the concept (and change) being measured by the PRO instrument meaningful to patients?

2. What role (e.g., primary, secondary, ancillary, effectiveness, safety) will the PRO instrument serve in the clinical study protocol and statistical analysis plan?

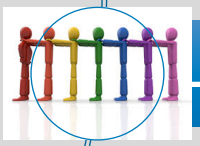
3. Does the evidence support the PRO instrument's use in measuring the concept of interest as specified in the clinical study protocol and statistical analysis plan?

Best Practices for Least Burdensome Selection, Development, Modification, and Adaptation of Patient-Reported Outcome Instruments

Measure concepts important to patients

Ensure PRO instruments are understandable to patients

Be clear about the role of PRO instrument in the clinical study protocol and statistical analysis plan



Leverage existing PRO instrument and validity evidence

Consider alternative platforms and parallel development for generating validity evidence for PRO instruments

Collaborate with others in the pre-competitive space

CDRH PRO Guidance: Best Practices

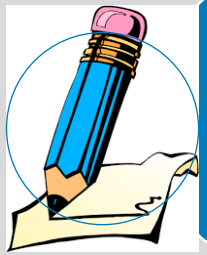
Measure concepts important to patients

- Helps integrate factors important to patients in FDA benefit-risk determinations
- Outcomes important to patients appropriately included in labeling help inform patient and healthcare provider conversations

Ensure patient-reported outcome instruments are understandable to patients

- Plain language helps ensure patients can provide informed responses
- Engage with patients to help generate evidence

CDRH PRO Guidance: Best Practices



Be clear about role of PRO instrument in clinical study protocol and statistical analysis plan

- Strength of evidence needed to support the instrument depends on its role in the clinical study and whether it is fit-for-purpose

Leverage existing PRO instruments and validity evidence

- Peer-reviewed literature is a starting point for identifying validity evidence
- Existing measures may be modified or adapted, and new validity evidence may be needed, depending on the modification(s) made

CDRH PRO Guidance: Best Practices

Consider alternative platforms and parallel development for generating validity evidence for PRO instruments

- Early feasibility, phased clinical studies, pivotal clinical studies, post-market clinical studies, and/or real-world data platforms can be used to generate validity evidence

Collaborate with others in the pre-competitive space

- Consider partnering with relevant stakeholders such as patient organizations, health professional organizations, and research institutions

Patient-Related Outcomes Resources

FDA Guidances

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

www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-reported-outcome-measures-use-medical-product-development-support-labeling-claims

- **Principles for Selecting, Developing, Modifying, and Adapting Patient-Reported Outcome Instruments for Use in Medical Device Evaluation**

www.fda.gov/regulatory-information/search-fda-guidance-documents/principles-selecting-developing-modifying-and-adapting-patient-reported-outcome-instruments-use

FDA Website Resources

- **Clinical Outcome Assessments in Medical Device Decision Making**
  www.fda.gov/about-fda/cdrh-patient-science-and-engagement-program/clinical-outcome-assessments-coas-medical-device-decision-making

- **FDA Patient Focused Drug Development Guidance Series**
  www.fda.gov/drugs/development-approval-process-drugs/fda-patient-focused-drug-development-guidance-series-enhancing-incorporation-patients-voice-medical

Contact Us

Patient-Reported Outcome or other Clinical Outcome Assessment Questions:

CDRH-PRO@fda.hhs.gov

Summary

- New guidance complements current 2009 PRO guidance, as well as the PFDD guidances under development
- Key principles highlight considerations important to evaluation of use of a PRO instrument in a clinical study
- These best practices may help ensure relevant, reliable, and sufficiently robust PRO instruments are developed, modified, or adapted using a least burdensome approach
- Use of PRO instruments is voluntary if other methods of measuring the concept of interest exist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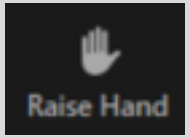
Let's Take Your Questions

- **To Ask a Question:**
  1. Please "Raise Your Hand" 

  2. Moderator will Announce Your Name to Invite You to Ask Your Question
  3. Unmute yourself when called

- **When Asking a Question:**
  - Ask 1 question only
  - Keep question short
  - No questions about individual submissions

- **After Question is Answered:**
  - Please lower hand
  - If you have more questions - raise your hand again

Thanks for Joining Today!

- **Presentation and Transcript will be available at CDRH Learn:**
  - www.fda.gov/Training/CDRHLearn
    Patient Engagement
- **Additional questions about today's presentation**
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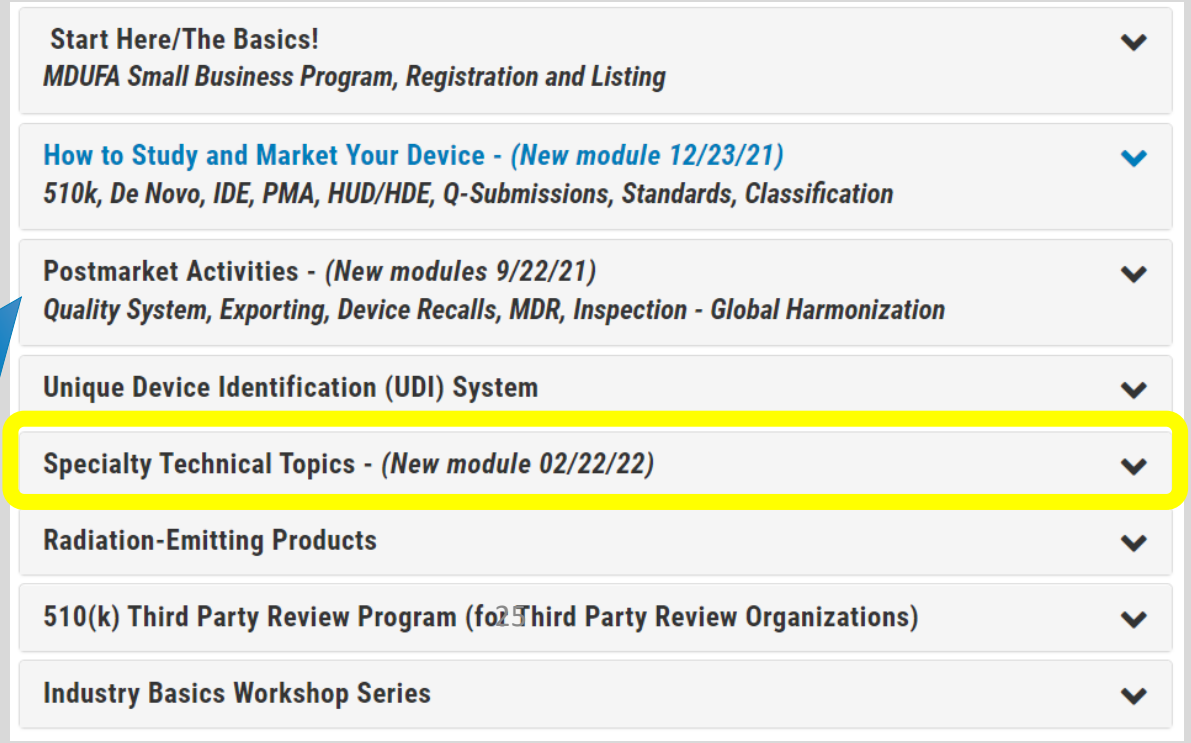


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