2015 Science Writers Symposium

FDA's Patient-Focused Drug Development Initiative and the Role of Patient-Reported Outcome Assessments

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Goal of Clinical Outcome Assessment

- In clinical trials, clinical outcome assessments are measurements that are used to assess a particular patient outcome, such as:
  - How long a patient lives
  - How a patient feels or functions in their daily lives

Types of Clinical Outcome Assessments (COAs)

- Clinician-reported outcomes
- Observer-reported outcomes
- Performance outcome assessments
- **Patient-reported outcomes (PROs)**

About PROs

- ***Patient-reported outcome*** —
  A measurement based on a report that comes directly from the patient (i.e., study subject) about the status of a patient's health condition without amendment or interpretation of the patient's response by a clinician or anyone else

- PRO instruments vary in complexity:
  - Simple: e.g., a single item 0–10 numeric rating scale of pain intensity
  - Complex: e.g., health-related quality of life

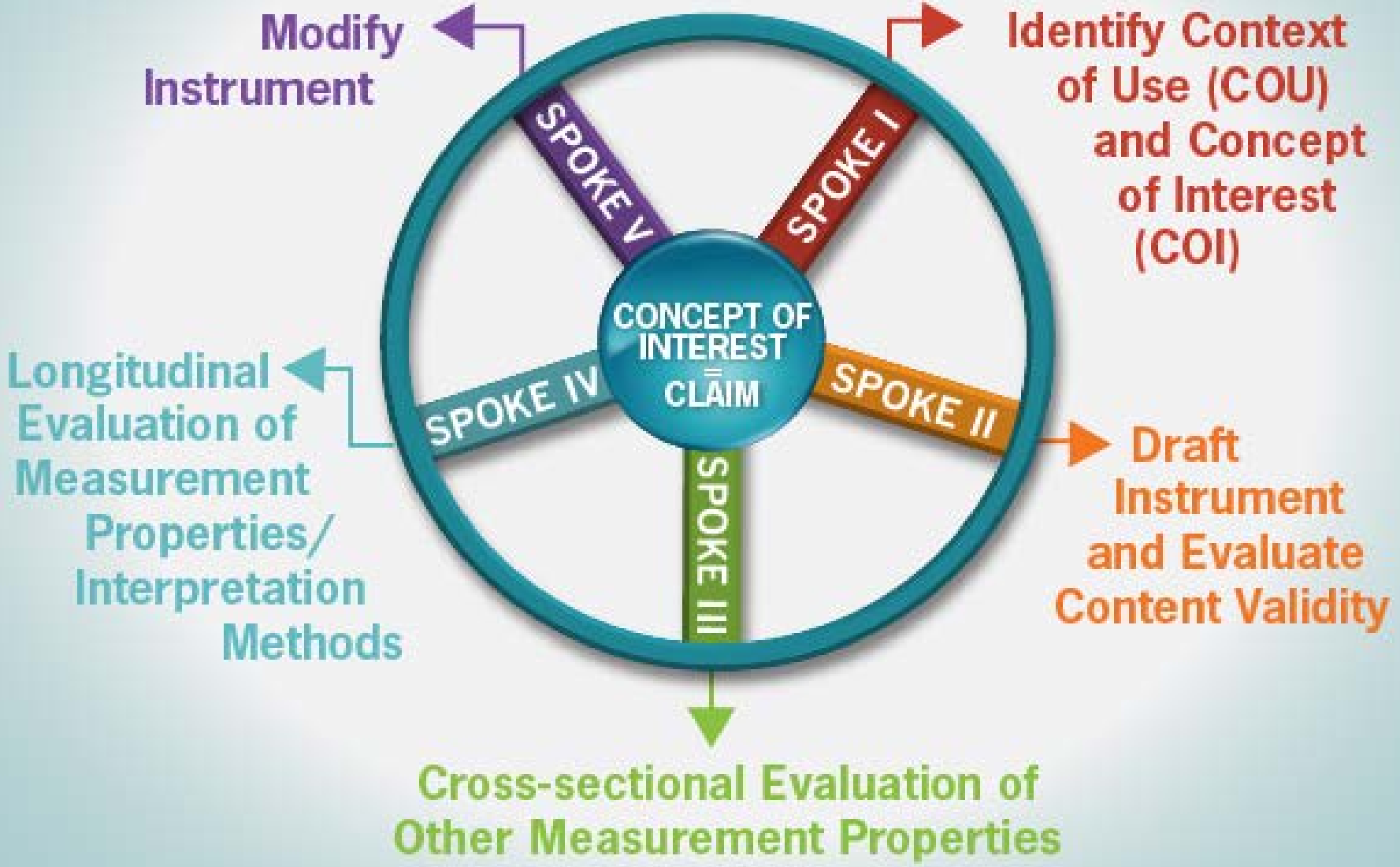
The Problem:

- Well-developed and fit-for-purpose PRO instruments do not exist for many diseases/conditions

- Development of fit-for-purpose PROs and other COAs can be time- and resource-intensive to ensure that they are fit-for-purpose (i.e., well-defined and reliable)

Qualification of

CLINICAL OUTCOME ASSESSMENTS (COAs)



Ways FDA Can Work with Stakeholders to Develop Clinical Outcome Assessments

- **The traditional way**
  - Within an individual drug development program
    - IND applications to FDA; clinical trials
    - Discuss strategy with FDA as early as possible!

- **A newer process**
  - Within the DDT qualification program; outside of an individual drug development program

Drug Development Tool Qualification

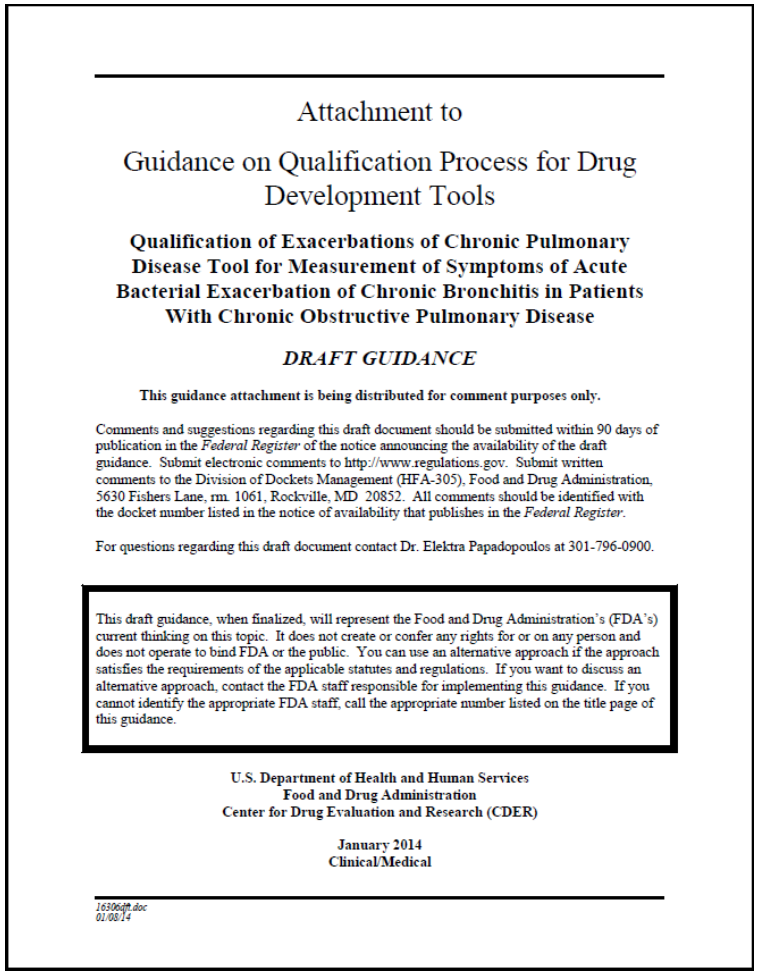
- **A process for FDA input into development of drug development tools, including PROs, intended for use in multiple drug development programs**
  - "Qualification" is a regulatory conclusion that the drug development tool measurement can be relied upon to have a stated interpretation and utility in drug development "fit-for-purpose"
- Qualified tools are made publicly available
- Qualification program for PROs and other COAs includes 36 projects in the consultation and advice stage; one PRO tool is currently qualified
- Many qualification projects are led by public-private partnerships or patient advocacy groups

Ongoing Clinical Outcome Assessment Qualification Efforts

- CDER partnering with other government agencies, consortia, patient groups, academics, researchers, and others on COA qualification projects, including:
  - Critical Path Institute PRO-Consortium (includes 7 distinct working groups: Asthma, Functional Dyspepsia, Irritable Bowel Syndrome, Non-Small Cell Lung Cancer, Rheumatoid Arthritis, Depression, Cognition)
  - FNIH Biomarkers Consortium
  - Critical Path Institute Multiple Sclerosis Outcomes Assessments Consortium (MSOAC)
  - Aging in Motion, a patient-advocacy organization
  - NIH

Example

First Clinical Outcome Assessment Qualified in January 2014



Attachment to

Guidance on Qualification Process for Drug Development Tools

Qualification of Exacerbations of Chronic Pulmonary Disease Tool for Measurement of Symptoms of Acute Bacterial Exacerbation of Chronic Bronchitis in Patients With Chronic Obstructive Pulmonary Disease

DRAFT GUIDANCE

This guidance attachment is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 90 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to http://www.regulations.gov. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document contact Dr. Elektra Papadopoulos at 301-796-0900.

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U.S. Department of Health and Human Services
Food and Drug Administration
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Exacerbations of Chronic Pulmonary Disease Tool (EXACT)

- A PRO for the measurement of symptoms of acute bacterial exacerbation of chronic bronchitis in patients with chronic obstructive pulmonary disease

Summary

- Development of fit-for-purpose PROs can be time- and resource-intensive
- The science of PRO development continues to evolve and requires a multidisciplinary approach
- The Drug Development Tool Qualification Program allows CDER to collaborate with multiple stakeholder partners in a precompetitive setting in the development of PROs and other COAs suitable for drug development

Helpful Links

- FDA's Patient-Reported Outcome (PRO) Guidance for Industry:
  - http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM071975.pdf
- DDT Clinical Outcome Assessment Qualification Program webpage:
  - http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugDevelopmentToolsQualificationProgram/ucm284077.htm
    - Includes Roadmap and Wheel and Spokes diagrams
    - Includes link to list of ongoing projects in the qualification program
- FDA's DDT Qualification Program Guidance for Industry:
  - http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm230597.pdf