

NHC Webinar Series on Clinical Outcome Assessments

Patient-Reported Outcomes and Patient-Centered Outcomes: Is There a Difference?

NOVEMBER 7, 2018



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NATIONAL HEALTH COUNCIL

ID Immune Deficiency Foundation

Arthritis Foundation™
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awareness • advocacy • action

Women Heart
The National Coalition for Women with Heart Disease

Tourette Association of America

Sjögren's Syndrome Foundation

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Spina Bifida Association of America

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NATIONAL HEMOPHILIA FOUNDATION
for all bleeding and clotting disorders

prevent cancer

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nfed LUNG CANCER ALLIANCE

National Blood Clot Alliance
Stop The Clot®

NORD
National Organization for Rare Disorders

NATIONAL PSORIASIS FOUNDATION®

Parent Project Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE

Outline

- Why are we having this webinar?
- Introduction to Clinical Outcome Assessment (COA)
- Patient centered versus patient reported
- Examples
- Q&A
- Wrap up & announcement of next webinar



Why are we having this webinar?

FDA's Patient-Focused Drug Development Initiative

Guidance 1: Collecting Comprehensive and Representative Input ▼

Guidance 2: Methods to Identify What is Important to Patients ▼

Guidance 3: Selecting, Developing or Modifying Fit-for-Purpose Clinical Outcomes Assessments ▼

Guidance 4: Title forthcoming ▼

FDA's Standard Core Clinical Outcome Assessments and Endpoints

“FDA is seeking information submissions from the public. Information is being solicited to inform planned work to promote development of publicly available standard core sets of Clinical Outcome Assessment (COA) measures for specific disease indications.”



Why are we having this webinar?

- Patient-centered outcomes research
- Interest in PROs and patient-centeredness across the healthcare ecosystem
 - Clinical practice guideline development
 - Health care delivery
 - Research
 - Payment models
 - Quality measurement

Why are we having this webinar?

Ensure NHC membership is prepared to engage in conversations about Clinical Outcome Assessment

Clinical Outcome Assessment

- First in a Webinar series
- In-person meeting Q2 2019



Clinical Outcome Assessments (COAs)

- FDA definition: “measures a patient’s symptoms, overall mental state, or the effects of a disease or condition on how the patient functions”
- COAs tell you something about how a patient feels or functions



Clinical Outcome Assessment Tools

Patient-
Reported
Outcome
Measures
(PROs)

*Report that comes from the **patient** about the status of a patient's health condition without amendment or interpretation of the patient's report by a clinician or anyone else*

Clinician-
Reported
Outcome
Measures
(ClinROs)

*Report that comes from a **trained health-care professional** after observation of a patient's health condition*

Observer-
Reported
Outcome
Measures
(ObsROs)

*Observation by **someone other than the patient or a health professional**. This may be a parent, spouse, or other non-clinical caregiver who is in a position to regularly observe and report on a specific aspect of the patient's health*

Performance
Outcome
Measures
(PerfOs)

*Based on a task(s) performed by a patient according to instructions that is **administered by some one trained such as a health care professional***



Patient Role

- Which concepts are important and should be measured?
- How should items be worded?
- Are the questions understandable?
- Do different patients interpret the questions in the same way?
- Is the recall period appropriate?
- Does the response scale make sense?
- What is the best data collection mode?



Patient Centered Versus Patient Reported



What is Patient Centered?

Any process, program or decision focused on patients in which patients play an active role as meaningfully engaged participants and the central focus is on optimizing use of patient-provided information.

- Doing things **WITH** patients, not **FOR** or **TO** patients
- Relies on meaningful engagement

What is Patient Reported?

A report that comes directly from the patient about the status of a patient's health condition without interpretation of the patient's response by a clinician or anyone else.

- *Self-report or by interview*
- *Symptoms or other unobservable concepts known only to the patient (e.g., pain severity or nausea)*
- *Patient perspective on functioning or activities that may also be observable by others*



Patient-Reported Outcomes and Patient-Centered Outcomes: Is There a Difference?

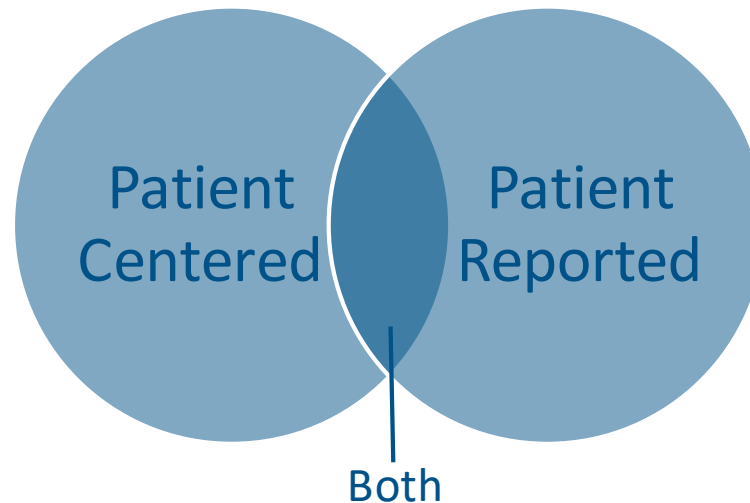


Patient-Reported Outcomes and Patient-Centered Outcomes: Is There a Difference?

YES!



Patient-Centered Versus Patient-Reported Outcomes



Patient-centered outcomes

- Outcomes patients report as important to them

Patient-reported outcomes

- Outcomes reported only by the patient on how they feel or function

Patient Centricity in Patient-Reported Outcome Measures

FDA's PRO Guidance (2009) repeatedly states that patient input is critical for developing valid PRO instruments:

- *“Without adequate documentation of patient input, a PRO instrument’s content validity is likely to be questioned.”*
- *“If symptoms known to be common to the population to be studied in the clinical trial are missing from a measure meant to capture important symptoms in that population, we will question the instrument’s content validity.”*



Doesn't that mean all PROs should be patient centered?

- Theoretically – Yes!
- But, older measures – many still in use today – may not have been developed based on understanding what is most important to patients
- Some older measures can be updated to include and emphasize concepts important to patients
- Use caution and do not assume a PRO tool is patient centered until you understand how it was developed or adapted to focus on what is important to patients

Keep in Mind

Not all patient-REPORTED outcomes
are patient CENTERED.

Not all patient-CENTERED outcomes
are patient REPORTED.

PRO measures can and should be both!



Examples



Example #1: Chronic Fatigue Syndrome

Voice-of-the-Patient Meeting held in 2013. Patients expressed they would like to have a biomarker for their disease. It would convince their families, friends, and colleagues that they really have an illness and remove the stigma associated with their disease.

- a) The biomarker is a patient-centered outcome
- b) The biomarker is a patient-reported outcome
- c) The biomarker is both a patient-centered and patient-reported outcome
- d) The biomarker is neither a patient-centered nor patient-reported outcome



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Example #2: Hemophilia A, Hemophilia B, von Willebrand Disease and Other Heritable Bleeding Disorders

Voice-of-the-Patient Meeting held in 2014. Patients expressed that joint pain is one of the most significant symptoms associated with their disease.

- a) Joint pain is a patient-centered outcome
- b) Joint pain is a patient-reported outcome
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Example #3: Non-24-hour sleep-wake disorder

Researchers developed a sleep diary to document and quantify sleep and waking behaviors. The items were selected by combining several older instruments. The researchers involved patients as study subjects to test out the diary.

- a) The diary is a patient-centered outcome measure.
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Questions?



NHC Series on COAs



Please join us
December 7 (2pm ET/ 11am PT)
for the next webinar in this series!

**FDA's Roadmap to Patient-Focused
Outcome Measurement in
Clinical Trials**

What aspects of
Clinical Outcome Assessment would
you like to see covered?

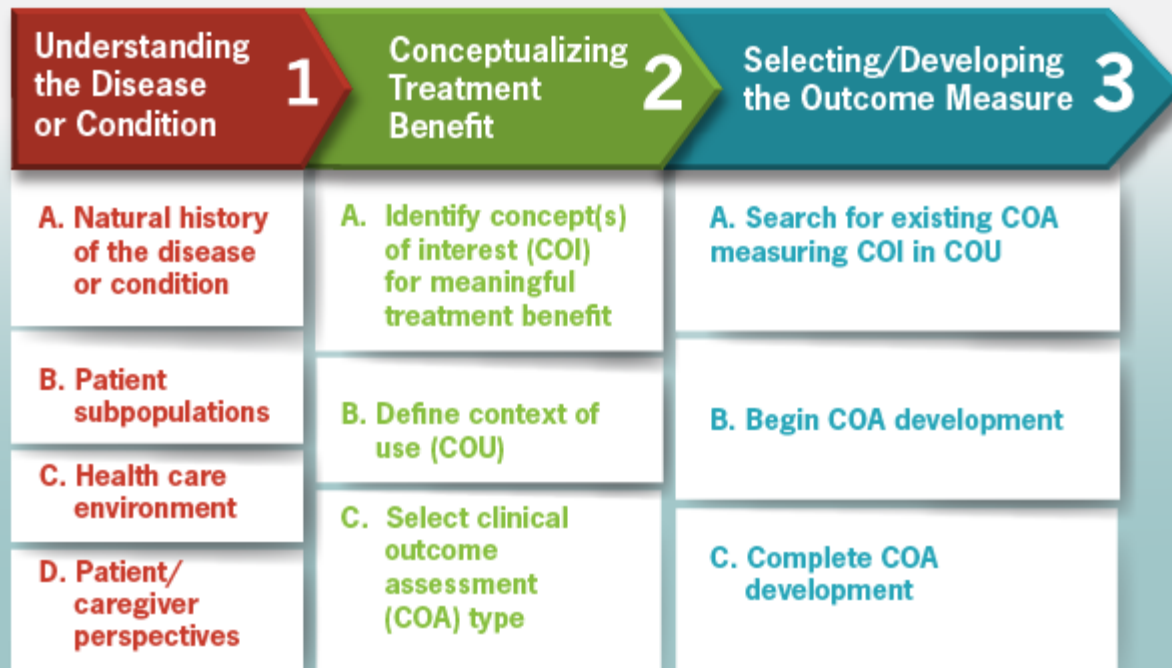
Please submit suggestions:

<http://www.nationalhealthcouncil.org/webinar-follow-patient-reported-outcomes-and-patient-centered-outcomes-there-difference>



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Roadmap to
PATIENT-FOCUSED OUTCOME MEASUREMENT
 in Clinical Trials



Qualification of **CLINICAL OUTCOME ASSESSMENTS (COAs)**

