Patient Centered Outcomes Research and EHRs

Kevin Larsen MD
Medical Director, Meaningful Use
Office of the National Coordinator of Health IT

November 19, 2013
Meaningful Use

Data capturing and sharing

Advanced clinical processes

Improved outcomes

Stage 1

Stage 2

Stage 3
“I am the expert about me.”
Patient Reported Outcomes

• MU 2 Measures
  – Functional status pre and post hip surgery
  – Functional status pre and post knee surgery
  – Functional status with heart failure over time
  – Functional status with rheumatoid arthritis
  – Depression remission

• Measures in development
  – Change in functional status (delta)
  – Shared care plan goal attainment
  – ADHD outcome
Only those who provide care can improve care
Small Data is our Short Term Focus.
Dr. Joe Kimura
Patient Centered Outcome Measures

- NQF project
- 3 characteristics
  - Meaningful to consumers, built with consumers
  - Care bundles (measures patients through their experience, rather than a single environment or program)
  - Patient Reported Outcomes
Quality Measurement Alignment

Current EHR Reporting

MU, PQRS, IQR, ACO, VBP, HRSA, CDC

Unified Measures
REPORT ONCE- using standards

Eligible Providers

Eligible Hospitals

CA Hospitals

CQMs@direct.mihin.org
Complex Adaptive System
Structured Data Capture Goals

SDC will focus on solving a specific interoperability challenge through the development of **four** new standards that will enable EHRs to capture and store structured data:

1. Standard for the CDEs that will be used to fill the specified templates
2. Standard for the structure or design of the template (container)
3. Standard for how EHRs interact with the template
4. Standard to auto-populate template
Structured Data Capture Standards Overlay

3. EHR Interaction Standard:
Find, display, cache, store/transmit

1. CDE Standard
- Selects form/template

2. Structure Standard
- Converts, populates & displays form

3. EHR Interaction Standard:
Find, display, cache, store/transmit

4. Pre-populate Standard
- Storages/data

5. Displays form

6. Caches data

7. Extracts, transforms, & loads data by form/template

8. Provider/End User Actor Key

9. EHR System Actor Key

CDE Library
- Clinical Research CDEs
- AHRQ CDEs [Common Formats]
- Other domain CDEs

Form Library Standard
- Patient Safety Forms
- Other domain Forms

Structured Captured Data
- EHR System
- End User
Structured Data Capture Conceptual Workflow

1. Selects form/template
2. Finds form/template
3. Converts, populates & displays form
4. Inputs data
5. Caches data
6. Stores/transmits data
7. Extract, Transform, & Load Data by form/template

Actor Key:
- Provider/End User
- EHR System

CDE Library
- Clinical Research CDEs
- AHRQ CDEs [Common Formats]
- Other domain CDEs

Form Library
- Patient Safety Forms
- Other domain Forms

EHR System
- Specified Form/Template
- Displayed Form
- Structured Captured Data
- External Data Repository
Questions?

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For more information about ONC visit: healthIT.gov

Office of the National Coordinator for Health Information Technology
Patient Reported Outcome Measures in CMS Programs

Kate Goodrich, MD MHS
Director, Quality Measurement and Health Assessment Group
Centers for Medicare and Medicaid Services
The strategy is to concurrently pursue three aims

<table>
<thead>
<tr>
<th>Aim</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Better Care</strong></td>
<td>Improve overall quality by making health care more patient-centered, reliable, accessible and safe.</td>
</tr>
<tr>
<td><strong>Healthy People / Healthy Communities</strong></td>
<td>Improve population health by supporting proven interventions to address behavioral, social and environmental determinants of health, in addition to delivering higher-quality care.</td>
</tr>
<tr>
<td><strong>Affordable Care</strong></td>
<td>Reduce the cost of quality health care for individuals, families, employers and government.</td>
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</table>
CMS framework for measurement maps to the six National Quality Strategy priorities

- Measures should be patient-centered and outcome-oriented whenever possible
- Measure concepts in each of the six domains that are common across providers and settings can form a core set of measures

Clinical quality of care
- Care type (preventive, acute, post-acute, chronic)
- Conditions
- Subpopulations

Person- and Caregiver-centered experience and outcomes
- Patient experience
- Caregiver experience
- Preference- and goal-oriented care

Safety
- All-cause harm
- HACs
- HAIs
- Unnecessary care
- Medication safety

Care coordination
- Patient and family activation
- Infrastructure and processes for care coordination
- Impact of care coordination

Efficiency and cost reduction
- Cost
- Efficiency
- Appropriateness

Population/ community health
- Health Behaviors
- Access
- Physical and Social environment
- Health Status
CMS’ Vision for Quality Measurement

- Align measures with the National Quality Strategy and Six Measure Domains/Priorities
- Implement measures that fill critical gaps within the 6 domains, particularly patient experience and Patient Reported Outcomes
- Align measures across CMS programs whenever possible
- Parsimonious sets of measures; core sets of measures
- Removal of measures that are no longer appropriate (e.g., topped out)
- Align measures with external stakeholders, including private payers and boards and specialty societies
- Major aim of measurement is improvement over time
Landscape of Quality Measurement

- Historically a siloed approach to quality measurement
  - Different measures within each quality program
  - Different reporting criteria for each quality program
- No clear measure development strategy
- Typically Disease Specific measures
- Confusing and Burdensome to stakeholders
- Burdensome to CMS with stovepipe solutions to quality measurement
The Future of Quality Measurement for Improvement and Accountability

- Meaningful quality measures increasingly need to transition away from setting-specific, narrow snapshots
- Reorient and align measures around patient-centered outcomes that span across settings
- Measures based on patient-centered episodes of care
- Capture measurement at 3 main levels (i.e., individual clinician, group/facility, population/community)
- Why do we measure?
  - Improvement

Source: Conway PH, Mostashari F, Clancy C. The Future of Quality Measurement for Improvement and Accountability. JAMA 2013 June 5; Vol 309, No. 21 2215 - 2216
Percentage of Medicare Beneficiaries with Multiple Chronic Conditions

Multiple Conditions is the Norm

- 3/4 persons ≥65 years have multiple conditions
- 1/4 adults < 65 who receive health care have multiple conditions
- 65+ y.o. with ≥ 2 conditions →~ 80% Medicare costs
- All adults: Majority of health care used by those with ≥ 2 conditions

Anderson G (RWJF.org)

- 60% take 5-9 medications
- 20% take 10+ medications

Tinetti, M. CMS Grand Rounds, October 2012
Most important outcome among older adults with multiple conditions when faced with tradeoff

- Varied in their outcome priority
  - Maintain function: 42%
  - Relief of pain or other symptoms: 32%
  - Keep alive: 27%

Policy changes that support patient-centered care with MCC

- Replace disease-focused quality metrics with...
- Patient-centered metrics (e.g. ascertain goals, shared decision-making, function, symptoms, appropriate prescribing for health outcome goal)

Tinetti, M. CMS Grand Rounds, October 2012
Current activities that foster appropriate care for MCC

- Payment and delivery system innovations that foster integration
- **Patient-Reported outcomes (PROs) measure use and development**
- EHRs: care plans and patient-centered outcomes shared across providers for decision making
- A few available quality metrics (e.g. shared decision making)

Tinetti, M. CMS Grand Rounds, October 2012
Patient Experience of Care Measures

• HCAHPs used for Hospital VBP – weighted at 30% of total score starting in FY 2015
• CG-CAHPS used in the PQRS, ACO and Physician VM programs for groups of 25 or more
  – CMS is exploring expansion of this measure for all clinicians
  – Specialty specific CAHPS? (e.g. S-CAHPS)
• CAHPS measures are in use or in development for every setting of care
  – Post Acute Care (LTCH, IRF, Home Health)
  – In-Center Dialysis
• First caregiver experience measure implemented in the Hospice quality reporting program
CMS Activities on Patient Reported Outcome Measures

- In 2012, CMS funded the NQF to develop guidance on development of PROMs.
- CMS currently uses a number of PROMs in our clinician reporting programs (e.g. depression, functional status).
- CMS and HHS working to identify existing PROMs that can be rapidly incorporated into our quality reporting programs, including the ACO program and CMMI models.
- CMS and ONC are currently developing PROMs for the hospital and outpatient setting:
  - Disease-specific functional status
  - General functional status
- CMS now includes patients in all measure development work, in order to understand the outcomes that are most important to patients and families.
Measures Using Patient-Reported Outcomes
Phyllis Torda
November 2013
Today

Working on performance measures for assessment of functional status for the following

- Hip and knee replacement
- Congestive heart failure
- Asthma, rheumatoid arthritis, pain

• For use in Meaningful Use and other CMS eligible professional programs
## Complex Chronic Conditions: Heart Failure - Generic Tools

<table>
<thead>
<tr>
<th>PROM</th>
<th># of items</th>
<th>Dartmouth-Hitchcock</th>
<th>University of Penn</th>
<th>Cleveland Clinic</th>
<th>Oxford Report¥</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>PROMIS (global)</td>
<td>10</td>
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<td>√</td>
<td></td>
<td></td>
<td>2</td>
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<tr>
<td>EQ-5D*</td>
<td>5</td>
<td></td>
<td>√</td>
<td>√</td>
<td></td>
<td>2</td>
</tr>
<tr>
<td>SIP</td>
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<td>SF-12*</td>
<td>12</td>
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<tr>
<td>VR-12</td>
<td>12</td>
<td>√</td>
<td></td>
<td></td>
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<td>1</td>
</tr>
</tbody>
</table>

*Proprietary tools
¥Patient-Reported Outcome Measurement Group, Oxford: A Structured Review of Patient-Reported Outcomes Measures (PROMs) for Heart Failure
# Complex Chronic Conditions: Heart Failure - Generic vs. Condition-Specific Tools

<table>
<thead>
<tr>
<th>PROM</th>
<th>Dartmouth-Hitchcock</th>
<th>University of Penn</th>
<th>Cleveland Clinic</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>Generic PROM only</td>
<td></td>
<td>√*</td>
<td></td>
<td>1</td>
</tr>
<tr>
<td>Condition-specific PROM only</td>
<td></td>
<td></td>
<td></td>
<td>0</td>
</tr>
<tr>
<td>Generic and condition-specific PROM</td>
<td>√</td>
<td></td>
<td>√*</td>
<td>2</td>
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</table>

*University of Pennsylvania and Cleveland Clinic are also using the PHQ-9, and Cleveland Clinic is using the GAD-7 for anxiety.
Key Themes: Use of PROMs

- Experts provided feedback that standardized functional status assessments are of interest, but generally are not used in clinical practice
  - When they are used, they are not used systematically
  - These instruments are generally calibrated for individual patient assessment
  - We will need consider risk adjustment to achieve equitable population-level evaluation for outcome measures

- Expert support for pairing process measure with goal setting tied to functional status assessments

- Expert recommendation to specify several assessment tools because using a single assessment tool may affect face validity

- Issues may be different for assessment of procedures than for use with chronic conditions

- Expert discomfort with outcomes at aggregate level
Building to Outcomes: PROM Performance Measures

- Improvement across patients
- Goal attainment
- Goal setting
- Assessment using standardized PROMs
Measuring Goal-Setting and Goal-Attainment

**STEP 1**
Complete FSA, enter score
- Which FSAs?
- How and where is FSA completed?
- Global score?
- Subscale?
- Item?

**STEP 2**
Discuss and determine goal
- Is it possible to relate a qualitative discussion to a FSA score?
- Should we measure one goal or more than one?
- If more than one, should we ask for “importance” and “difficulty”?

**STEP 3**
Record FSA goal
- Should we capture the interventions related to achieving the goal?
- If so, how do we do this with structured data?

**STEP 4**
Retake FSA, record score
- What is the appropriate time interval?
- Should it vary by condition?
- If so, how do we do this with structured data?

**STEP 5**
Determine if goal met
- May need “yes/no” as well as score
Next Steps

- Explore how to construct outcome measures
- Decide on standardized tools
- Explore licensing options
- Field test
- Final specifications final specs for potential inclusion in Meaningful Use Stage 3
Patient Reported Outcomes:
Examples of Measures in MN

- Depression Remission
- Asthma Control
- Orthopedic Functional Status

Collette Pitzen, BSN CPHQ
MN Community Measurement
Case Study # 1 Depression

- Condition specific PROM performance measure
- PROM = PHQ-9
  - Tool in public domain
  - 9 question tool, easy to administer & score
  - Valid for diagnostic and assessment over time
- Widely implemented in MN
  - Publically reported since 2009
  - Primary Care and Behavioral Practices
  - 80,000+ patients annually via direct data submission
- Implemented in EMR systems → pop-up, templates-values stored in discrete fields (not dependent on LOINC)
- NQF Endorsed/ e-Measure/ MU 2

<table>
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<tr>
<th>Score</th>
<th>Depression Severity</th>
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<td>0 to 4</td>
<td>None/ minimal</td>
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<tr>
<td>5 to 9</td>
<td>Mild</td>
</tr>
<tr>
<td>10 to 14</td>
<td>Moderate</td>
</tr>
<tr>
<td>15 to 19</td>
<td>Moderately Severe</td>
</tr>
<tr>
<td>20 to 27</td>
<td>Severe</td>
</tr>
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</table>
Depression Remission at Six Months

- Patients with diagnosed major depression or dysthymia AND elevated PHQ-9 > 9
- Prospective/longitudinal, based on index visit

**PHQ-9 < 5 (remission) at six months +/- 30 days**
Adults 18 + w major depression or dysthymia & PHQ-9 > 9

- Not assessed = not in remission

<table>
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<th>Date</th>
<th>2/1/2012</th>
<th>3/15/2012</th>
<th>4/10/2012</th>
<th>6/20/2012</th>
<th>7/15/2012</th>
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<td>Index Visit</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Diag</td>
<td>296.23</td>
<td>Major depression, severe</td>
<td>PHQ-9 = 18</td>
<td>PHQ-9 = 12</td>
<td>PHQ-9 = 8</td>
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<tr>
<td></td>
<td></td>
<td>PHQ-9 = 21</td>
<td></td>
<td></td>
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<tr>
<td></td>
<td>7/15/2012</td>
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<td></td>
<td>PHQ-9 = 3</td>
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<tr>
<td></td>
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<td>Remission</td>
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<td></td>
<td>8/1/2012</td>
<td>Six Month Marker</td>
<td></td>
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<tr>
<td></td>
<td>8/31/2012</td>
<td>plus 30 days</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

MN Community Measurement
### Depression: Remission (Feeling Better)

Depression is more than feeling sad or "blue". Depression can interfere with daily life. Most people who seek treatment can improve to where they feel better and have few symptoms of depression or none at all. This is called being in remission.

The Depression Remission measure reports on how well clinics help patients with depression reach remission and improve to where they say they have few symptoms of depression or none at all.

<table>
<thead>
<tr>
<th>Clinic Name</th>
<th>City</th>
<th>ZIP</th>
<th>Remission Rate</th>
<th>View Profile</th>
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<tbody>
<tr>
<td>Essentia Health - Aurora Clinic</td>
<td>Aurora</td>
<td>55705</td>
<td>29%</td>
<td>view profile</td>
</tr>
<tr>
<td>Aspen Medical Group - Hopkins</td>
<td>Hopkins</td>
<td>55343</td>
<td>28%</td>
<td>view profile</td>
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<tr>
<td>Essentia Health - Deer River Clinic</td>
<td>Deer River</td>
<td>55705</td>
<td>25%</td>
<td>view profile</td>
</tr>
<tr>
<td>Allina Health - Prescott</td>
<td>Prescott</td>
<td>54021</td>
<td>24%</td>
<td>view profile</td>
</tr>
<tr>
<td>Mayo Clinic - Northwest</td>
<td>Rochester</td>
<td>55901</td>
<td>24%</td>
<td>view profile</td>
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<tr>
<td>Sawtooth Mountain Clinic, Inc</td>
<td>Grand Marais</td>
<td>55604</td>
<td>22%</td>
<td>view profile</td>
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<tr>
<td>Entira Family Clinics - North St. Paul (formerly Family Health Services Minnesota - North St. Paul Clinic)</td>
<td>North St. Paul</td>
<td>55109</td>
<td>21%</td>
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<tr>
<td>Allina Health - Brooklyn Park</td>
<td>Brooklyn Park</td>
<td>55443</td>
<td>20%</td>
<td>view profile</td>
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<tr>
<td>Hennepin County Medical Center (HCMC) Clinics - St. Anthony Village Clinic</td>
<td>St. Anthony</td>
<td>55418</td>
<td>20%</td>
<td>view profile</td>
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</tbody>
</table>
Illustrated Example of the Lifecycle of a Measure

Depression Remission at Six Months

Courtesy of the National Quality Forum
Depression Remission/ Response and Follow-up Rates at Six Months

Depression Remission and Response at Six Months

Small incremental improvement ... but lost to follow-up is at 72%
Challenges with Patient Follow-up

Of patients assessed:

- 25% in remission
- 25% major to severe depression symptoms
Case Study # 2 Asthma

• Condition specific PROM performance measure

• PROM = Three Tools (choice) to indicate if asthma is in control
  ▪ ACT/ C-ACT Asthma Control Test [Score = 20 or >]
  ▪ ACQ Asthma Control Questionnaire [Score = 0.75 or <]
  ▪ ATAQ Asthma Therapy Assessment Questionnaire [Score = 0]

• Less complicated than depression measure

• Most recent assessment in the measurement period in control?

• 76% of population with completed test (↑ from 55%)
  ▪ 99% of practices using ACT or C-ACT
Optimal Asthma Care - Control Component

Most recent asthma control test with score in control
Patients age 5 to 50 with a diagnosis of asthma

• Not assessed = not in control
• Rush to implement → low rates first year as groups implementing tools
Case Study # 3

Total Knee and Lumbar Spine Surgery

- Condition specific PROM performance measure administered pre-operatively and post-operatively to patients
- Currently in pilot
- Yes, specialists can collect and report data
- Implementation into work flow is key
  - Groups rated tool administration to the patients more difficult that getting the info into or out of EMR
- Issues with proprietary tools
Orthopedic/ Neurosurgery Measures

- Measuring the average or percent change between pre and post op scores
- For each patient → measure change
- Rates by practice or practice/ location
- Not assessed = not in measure
- Anticipate at least 70% one year capture rate
- Assessing clinical variables for risk adjustment
  - Obesity/ BMI
  - Tobacco Status
  - Pre-operative functional status score

<table>
<thead>
<tr>
<th>Population</th>
<th>Pre-op</th>
<th>Three Month</th>
<th>One Year</th>
<th>Functional Status</th>
<th>Quality of Life</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total Knee Replacement</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
<td>Oxford Knee</td>
<td>EQ5D-5L</td>
</tr>
<tr>
<td>Lumbar Disc/Laminotomy</td>
<td>✓</td>
<td>✓</td>
<td></td>
<td>Oswestry (ODI) Pain Scale</td>
<td>EQ5D-5L</td>
</tr>
<tr>
<td>Lumbar Fusion</td>
<td>✓</td>
<td></td>
<td>✓</td>
<td>Oswestry (ODI) Pain Scale</td>
<td>EQ5D-5L</td>
</tr>
</tbody>
</table>
Oxford Knee Score

3. During the past 4 weeks...
   Have you had any trouble getting in and out of a car or using public transportation because of your knee? (whichever you would tend to use)
   - No trouble at all
   - Very little trouble
   - Moderate trouble
   - Extreme difficulty
   - Impossible to do
   □ 4 □ 3 □ 2 □ 1 □ 0

4. During the past 4 weeks...
   For how long have you been able to walk before pain from your knee becomes severe? (with or without a cane)
   - No pain/more than 30 minutes
   - 16 to 30 minutes
   - 5 to 15 minutes
   - Around the house only
   - Not at all/severe pain when walking
   □ 4 □ 3 □ 2 □ 1 □ 0

5. During the past 4 weeks...
   After a meal (sitting at a table), how painful has it been for you to stand up from a chair because of your knee?
   - Not at all painful
   - Slightly painful
   - Moderately painful
   - Very painful
   - Unbearable
   □ 4 □ 3 □ 2 □ 1 □ 0
Oswestry Disability Index

• A low back pain specific functional status tool; gold standard used in the field over 20 years. Tool in public domain.

• Expressed as % disability
  ▪ 20 to 40 moderate disability
  ▪ 40 to 60 severe disability
  ▪ 60 to 80 crippled
  ▪ 80 to 100 bedbound or exaggerating

10 Questions related to low back function

- Pain intensity
- Personal care
- Lifting
- Walking
- Sitting
- Standing
- Sleeping
- Sex life (if applicable)
- Social life
- Travelling

Valid Tool = at least 8 of 10 questions answered
Valid Version = 2.1a
Learning Via Pilot

• If a tools are “newer” to the practice
  • Time to implement & build into work flow
  • Follow-up post-op
  • Unfamiliarity / skipping questions

• Public domain tools preferable
  • “Permission to Use” → barriers for electronic admin

• Frequently desired measure point is lengthy
  • One year (nine to fifteen months post-op) post fusion
  • Initial discectomy 3 month follow-up good

• Balance between desire and burden
  • Development work group function, quality of life and pain
  • Needed to narrow variables for RA (started with > 20)
PROs in EHR: Regulatory Considerations for Use in Clinical Trials

Ann Marie Trentacost, M.D. Medical Lead
OND/CDER/SEALD
November 19, 2013
Outline

- Instrument Selection: Measuring the Right Thing in the Right Way
- Special Considerations for Electronic Data Collection
Evolution of EHR

EHR

Medical Record Keeping

Quality Assessment

Public Reporting

Research

Comparative Effectiveness

Adverse Event Reporting

Efficacy or Safety Claims
Guidance for Industry
Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims

- PRO: A measurement based on a report that comes directly from the patient about the status of a patient’s health condition without amendment or interpretation of the patient’s response by a clinician or anyone else.

- Defines how the Agency interprets “well-defined and reliable” for PRO measures intended to provide evidence of treatment benefit that support labeling claims.
PRO Instrument

A means to capture data (i.e., a questionnaire) plus all the information and documentation that supports its use

- Clearly defined methods and instructions for administration or responding
- Standard format for data collection
- Well-documented methods for scoring, analysis, and interpretation of results
PRO Instrument Selection
“Fit for Purpose”

Step 1: Define disease population

Step 2: Define other aspects of context of use

Step 3: Define the concept of interest that will define treatment benefit

Step 4: Select or develop well-defined and reliable PRO Measures (including data collection method)

PRO Instrument
Defining Context of Use

Each of the following variables can impact the adequacy of a PRO to support a claim:

- **Disease definition including, if appropriate**
  - Disease subtype
  - Disease severity
  - History of previous treatment

- **Patient subpopulations**
  - Patient demographics
  - Reporting ability
  - Culture and language

- **Clinical trial design and objectives**
  - Endpoint positioning
  - Endpoint definitions
  - Analysis plan
  - Methods for interpretation of study results
  - Targeted labeling claim

- **Clinical practice and study setting**
  - Inpatient vs. outpatient
  - Geographic location
  - Clinical practice variation
Considerations for Mode of Administration Selection

- Who is the target population?
- Where will the assessment be completed? (e.g., patient home)
- What is the timeframe for reporting (immediate or some recall)?
- Characteristics of the items and response options?
- Infrastructure of collection of data electronically (e.g., internet connectivity variation)
- Patient burden and length of instrument or batteries of instruments
- Multiple languages needed?
## Considerations for Selecting an Appropriate Mode of Administration

<table>
<thead>
<tr>
<th>Consideration</th>
<th>Paper</th>
<th>Electronic Hand Held Device</th>
<th>Web or browser-based</th>
<th>IVRS</th>
</tr>
</thead>
<tbody>
<tr>
<td>Technology Availability and Acceptance</td>
<td>Paper and Pens available and acceptable to all</td>
<td>Device provided by Sponsor; studies show acceptance across broad spectrum</td>
<td>Must have computer or web-enabled device; studies show acceptance across broad spectrum</td>
<td>Must have phone (or Sponsor provides one); studies show acceptance across broad spectrum</td>
</tr>
<tr>
<td>Graphics supported?</td>
<td>Yes</td>
<td>Yes</td>
<td>Yes</td>
<td>No</td>
</tr>
<tr>
<td>Large number of questions and/or responses supported?</td>
<td>Yes</td>
<td>Yes</td>
<td>Yes</td>
<td>No</td>
</tr>
<tr>
<td>Alarm option to minimize missing data?</td>
<td>No</td>
<td>Yes (multiple alarms possible)</td>
<td>No (although possibility for email reminders)</td>
<td>Yes (incoming phone call)</td>
</tr>
<tr>
<td>Logical branching / adaptive questions possible?</td>
<td>No (branching possible, but with patient confusion and burden)</td>
<td>Yes</td>
<td>Yes</td>
<td>Yes</td>
</tr>
<tr>
<td>Literacy required?</td>
<td>Yes</td>
<td>Yes</td>
<td>Yes</td>
<td>No (but cognitive load may be higher for auditory vs. visual items)</td>
</tr>
<tr>
<td>Out of range data avoided?</td>
<td>No</td>
<td>Yes</td>
<td>Yes</td>
<td>Yes</td>
</tr>
<tr>
<td>Transcription errors avoided?</td>
<td>No</td>
<td>Yes</td>
<td>Yes</td>
<td>Yes</td>
</tr>
<tr>
<td>Time stamp available?</td>
<td>No</td>
<td>Yes</td>
<td>Yes</td>
<td>Yes</td>
</tr>
</tbody>
</table>
Thinking about the last 24 hours...
Please rate how difficult it was to stand upright without falling while your eyes were closed.

0 - 1 - 2 - 3 - 4 - 5 - 6 - 7 - 8 - 9 - 10
Not at all difficult  Extremely difficult
Existing instruments that switch from paper to electronic data capture are evaluated as a modified instrument

- At a minimum documentation of cognitive debriefing should demonstrate that content validity is not altered between the 2 instruments
Data Collection Method Review

- Data collection method, procedures and protocols associated with instrument administration mode
  - Instructions to interviewers, self-administration, or supervising self-administration.

- Data quality control procedures specific to the data collection method or instrument administration mode
  - Case report forms or screen shots of electronic PRO instruments.

- Comparability of data obtained when using multiple data collection methods or administration modes within a single clinical trial
When Reviewing Electronic PROs

- The content of electronic instruments is evaluated the same as in any other PRO instrument
- Documentation of development and validation needed for review of evidence to support labeling claims
  - PRO Guidance defines the principles of good measurement science for developing PROs
- Additional documentation may be important to review with electronic assessments
  - Program specifications and rationale for design features (e.g., forced responses, branches, prompts)
  - Usability testing
  - Training materials
  - Documentation related to migration from paper to electronic
- Electronic data capture does not overcome problems with content validity
  - Capturing the wrong data really well is not useful!
Includes all information in original records, certified copies of original records of clinical findings, observations, or other activities used for reconstructing and evaluating the investigation.

FDA and sponsors have access to source data to ensure adequate protection of rights, welfare, and safety of human subjects.

Must be attributable, legible, contemporaneous, original, and accurate.
Electronic PRO Instruments

Sponsors:

- Must ensure that regulatory requirements for record keeping, transmission, maintenance, storage, and access are met
- Provide investigators with all information necessary to conduct the trials in accordance with the investigational plan and permitting FDA to access, copy, and verify records and reports relating to the investigation (i.e., source data verification)
Computerized System Safeguards

- Internal Security Safeguards
  - Limited Access: limited to authorized individuals only
  - Audit Trails: Use of computer generated stamped audit trails
  - Date/Time Stamp

- External Security Safeguards
  - Procedures and controls to prevent the altering, browsing, querying, or reporting of data via external software applications
FDA Guidances

What is the Potential for Using PROs in Performance Measurement?

PCORI PRO Infrastructure Workshop
Atlanta
November 19, 2013

Karen Beckman Pace, PhD, MSN
Senior Director, Performance Measures
kpace@qualityforum.org
Potential for PROs to Improve Care

- Facilitate person-centered care
- Improve patient-provider communication and decision-making
- Identify patient needs in a timely manner
- Assist clinical providers in care management
- Assist patients with self-care management and monitoring
- Outcomes such as function and symptom relief are reasons for seeking and delivering care
- Therefore, PROs should be considered for performance measurement
NQF Endorses Performance Measures

- NQF is a voluntary consensus standards setting organization
- Endorses performance measures for use in BOTH improvement and accountability applications (public reporting and payment)
- Endorses PRO-based performance measures, not individual-level PRO instruments, tools, or scales
- Does not develop measures -- evaluates against standard criteria
  - Importance to measure and report
  - Scientific acceptability of measure properties
  - Feasibility
  - Usability and use
  - Related and competing measures
Guiding Principles
- Psychometric Soundness
- Person-Centered
- Meaningful
- Amenable to Change
- Implementable

PRO domains included
- health-related quality of life/functional status
- symptom and symptom burden
- experience with care (incl. engagement, shared decision-making)
- Health-related behaviors
### Distinctions among PRO, PROM, and PRO-PM

<table>
<thead>
<tr>
<th>PRO (patient-reported outcome)</th>
<th>Definition</th>
<th>Example: Patients With Clinical Depression</th>
</tr>
</thead>
<tbody>
<tr>
<td>The concept of any report of the status of a patient’s health condition that comes directly from the patient, without interpretation of the patient’s response by a clinician or anyone else.</td>
<td>Symptom: depression</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>PROM (instrument, tool, single-item measure)</th>
<th>Definition</th>
<th>Example: Patients With Clinical Depression</th>
</tr>
</thead>
<tbody>
<tr>
<td>Instrument, scale, or single-item measure used to assess the PRO concept as perceived by the patient, obtained by directly asking the patient to self-report (e.g., PHQ-9).</td>
<td>PHQ-9®, a standardized tool to assess depression</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>PRO-PM (PRO-based performance measure)</th>
<th>Definition</th>
<th>Example: Patients With Clinical Depression</th>
</tr>
</thead>
<tbody>
<tr>
<td>A performance measure that is based on PROM data aggregated for an accountable healthcare entity (e.g., percentage of patients in an accountable care organization whose depression score as measured by the PHQ-9 improved).</td>
<td>Percentage of patients with diagnosis of major depression or dysthymia and initial PHQ-9 score &gt;9 with a follow-up PHQ-9 score &lt;5 at 6 months (NQF #0711)</td>
<td></td>
</tr>
</tbody>
</table>
1. **Identify the quality performance issue or problem**  
   • Include input from all stakeholders including consumers and patients

2. **Identify outcomes that are meaningful to the target population and are amenable to change**  
   • Ask persons who are receiving the care and services  
   • Identify evidence that the outcome responds to intervention

3. **Determine whether patient-/person-reported information (PRO) is the best way to assess the outcome of interest**  
   • If a PRO is appropriate, proceed to step 4
4. Identify existing PROMs for measuring the outcome (PRO) in the target population of interest
   • Many PROMs (instrument/scale/single-item) were developed and tested primarily for research

5. Select a PROM suitable for use in performance measurement
   • Identify reliability, validity, responsiveness, feasibility in the target population

6. Use the PROM in the real world with the intended target population and setting to:
   • Assess status or response to intervention, provide feedback for self-management, plan and manage care or services, share decision-making
   • Test feasibility of use and collect PROM data to develop and test an outcome performance measure
Pathway from PRO to NQF-endorsed PRO-PM

PRO-PM

7. Specify the outcome performance measure (PRO-PM)
   • Aggregate PROM data such as average change; percentage improved or meeting a benchmark

8. Test the PRO-PM for reliability, validity, and threats to validity
   • Analysis of threats to validity, e.g., measure exclusions; missing data or poor response rate; case mix differences and risk adjustment; discrimination of performance; equivalence of results if multiple PROMs specified
9. Submit the PRO-PM to NQF for consideration of NQF endorsement
   • Detailed specifications and required information and data to demonstrate meeting NQF endorsement criteria

10. Evaluate the PRO-PM against the NQF endorsement criteria
    • Importance to Measure and Report (including evidence of value to patient/person and amenable to change)
    • Scientific Acceptability of Measure Properties (reliability and validity of PROM and PRO-PM; threats to validity)
    • Feasibility
    • Usability and Use
    • Comparison to Related and Competing Measures to harmonize across existing measures or select the best measure
11. Use the endorsed PRO-PM for accountability and improvement
   • Refine measure as needed

12. Evaluate whether the PRO-PM continues to meet NQF criteria to maintain endorsement
   • Submit updated information to demonstrate meeting all criteria including updated evidence, performance, and testing; feedback on use, improvement, and unintended adverse consequences

Feedback to step 1
Sample of Methodological Questions for Discussion

- What should be considered in choosing an approach to aggregate PROM data for an outcome performance measure (e.g., average/median amount of change; percentage of patients who improve/reach benchmark/have meaningful change)?

- What are the implications of various aggregation approaches on:
  - reliability of the PRO-PM score
  - validity of conclusions about quality?

- Are there any unique considerations for risk adjustment of a PRO-PM (as compared to other quality outcome performance measures)?
Next steps

- PRO-PMs are ripe for the “measure incubator” concept to fill important measure gaps
  - Select a candidate PRO (e.g., functional status) and take down the pathway
- NQF upcoming projects
  - Measure Gaps – Person-centered care and outcomes
  - CDP endorsement of performance measures – Person and family-centered care
  - Patient engagement “Action Team”
Resources from NQF Project

- Project Report - **Patient-Reported Outcomes (PROs) in Performance Measurement**
- Commissioned papers
  - *Methodological Issues in the Selection, Administration and Use of Patient-Reported Outcomes in Performance Measurement in Health Care Settings*
    David Cella, Ph.D., Elizabeth A. Hahn, M.A., Sally E. Jensen, Ph.D., Zeeshan Butt, Ph.D., Cindy J. Nowinski, M.D., Ph.D., Nan Rothrock, Ph.D.
  - *Patient-Reported Outcomes in Performance Measurement Commissioned Paper on PRO-Based Performance Measures for Healthcare Accountable Entities*
    Anne Deutsch, RN, PhD, CRRN; Laura Smith, PhD; Barbara Gage, PhD; Cynthia Kelleher, MPH, MBA; Danielle Garfinkel, BA
- Available on NQF website: [PROs in Performance Measurement](#)