Patient Reported Outcomes (PROs): New Frontier in Surgical Outcomes Quality Improvement?

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• Nothing to disclose
Outline

• What is a PRO?
• Why are PROs important?
• Nuts and bolts of measurement
• Research examples
• Limitations
What is a PRO?

“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”

- U.S. Department of Health and Human Services
What is a PRO?

• Complete reversal of measurement approach
• Outcomes that are important to patients
  – Ex: Mobility after knee replacement
  – Ex: Sexual function after rectal cancer surgery
• Health related quality of life, physical, mental, emotional health
• Compliments traditional outcomes
What is a PRO?

• **Generic or Universal Health Status**
  – Appropriate for respondents with and without a condition
  – Examples: SF-36, PROMIS

• **Disease-Specific / Targeted**
  – Examples: FACT-Prostate, KCCQ, KOOS

• **Preference or Utility**
  – Define health states and then assign value to that state
    • Generate quality adjusted life years (QALYs)
  – Used in cost-effectiveness research
  – Examples: time trade-off, standard gamble
What is a PRO?

PROMIS® Adult Self-Reported Health

- **Physical Health**
  - Fatigue
  - Pain Intensity
  - Pain Interference
  - Physical Function
  - Sleep Disturbance

- **Mental Health**
  - Anxiety
  - Depression

- **Social Health**
  - Ability to Participate in Social Roles & Activities

PROMIS Profile Domains

- Dyspnea
- Gastrointestinal Symptoms
- Pain Behavior
- Pain Quality
- Sexual Function
- Sleep-related Impairment

PROMIS Additional Domains

- Alcohol
- Anger
- Cognitive Function
- Life Satisfaction
- Positive Affect
- Psychosocial Illness Impact
- Self-efficacy for Managing Chronic Conditions
- Smoking
- Substance Use

Global Health

HealthMeasures.net/PROMIS
What is a PRO?

Quantifies a characteristic that cannot be directly measured or observed (e.g., fatigue)

“How fatigued are you on average?”

Energetic

Not at all

FATIGUE

Very much

Severe
Why are PROs Important?
Why are PROs Important?

• Patient centered care is **better** care

• Physician compensation is tied to patient feedback
  – 25% of hospital VBP program
  – Public reporting
Why are PROs Important?

• Unique perspective on treatment effectiveness
  – Physiologic assessments often do not reflect how a patient functions or feels (e.g., FEV1)

• MORE reliable than informal interviews

• Clinicians are limited in ability to estimate outcomes

• Some treatment effects are known only to the patient (e.g., fatigue, depression, pain)
Why are PROs Important?

- PROs are actionable
- Funding
- Untapped area of research

Helping Patients Make Better Personal Health Decisions
The Promise of Patient-Centered Outcomes Research

- Barry MJ, JAMA 2011
Nuts and Bolts of Measurement

1) Measurement is not objective just because made by clinician

2) Subjective ≠ bad or useless

How can we facilitate **reproducible** and **credible** results from patient reports?
Nuts and Bolts of Measurement

• What we *want* in a measure
  – Psychometrically sound and interpretable
  – Brief, simple
  – Generalizable across groups, ages
  – Full range of any given trait (no floor or ceiling effects)
  – Cross cutting (many diseases/conditions)
  – Common scale / centered on a reference population
Nuts and Bolts of Measurement

• Classical test theory (CTT) vs. item response theory (IRT)
  – Different approaches to quantify domain
  – Assessed with reliability, validity
• CTT has limited adaptability, requires all items
• IRT allows for measurement using subsets of items (e.g., “short forms”)  
  – Requires calibration, centering on a population
Nuts and Bolts of Measurement

• Types of measures
  – CATs
    • Dynamic, precise, efficient
    • Requires platform
  – Off the shelf short-forms
    • Subset of items from “bank”
    • 4-10 items/ domain
  – Customized short-forms
    • User selection, need to assess calibration
  – Profiles
    • 4+ items / domain

www.HealthMeasures.net/PROMIS
Computerized Adaptive Testing

Question #1

- high physical function
- low physical function

Question #2

- Questionnaire
- with high precision
- AND a wide range

Question #3
Nuts and Bolts of Measurement

Choose your measure(s)

- Tailor to your interest
  - Select domain(s) vs. global
  - Select types of measures

- Ex: Abd surgery recovery
  - Pain, GI symptoms, physical function

www.HealthMeasures.net/PROMIS
Nuts and Bolts of Measurement

• Tailored assessments
  – What symptoms or outcomes do you expect to see / change?
  – Included in domains?
  – Age of population?
  – How much time available?
  – How will you collect the data?
    • Paper, tablet, computer?
  – How reliable?
    • Screening vs. primary outcome

www.HealthMeasures.net/PROMIS
Nuts and Bolts of Measurement

- Interpreting scores
- T-score $\rightarrow$ 50 is mean of the referent population, 10 is the standard deviation
  - 40 = one standard deviation lower than mean
  - 60 = one standard deviation higher than mean
- Function: high score = good
- Symptoms: high score = bad

www.HealthMeasures.net/PROMIS
Nuts and Bolts of Measurement

• Know the referent group
  – US general population? Clinical population?
• Scores 0.5-1.0 SD away from mean
  – **Mild** symptoms/ impairment
• Scores 1.0-2.0 SD away from mean
  – **Moderate** symptoms/ impairment
• Scores >2.0 SD away from mean
  – **Severe** symptoms/ impairment

www.HealthMeasures.net/PROMIS
Nuts and Bolts of Measurement

- What is a meaningful change?
  - No gold standard
  - Depends on context
  - Standards have been published for specific scenarios

- Used to make treatment decisions, determine which treatment is better, calculate sample size for trials

www.HealthMeasures.net/PROMIS
Nuts and Bolts of Measurement

• Goal: Many measures, but one metric
Research Examples
P: Thoracotomy for lung cancer (n=79)
I: PRO assessed twice weekly after discharge for 4 weeks. If reached predefined severity threshold, alert to clinical team
C: No alerts
O: Symptom threshold events (pain, distress, disturbed sleep, shortness of breath, constipation)

Cleeland CS et al, JCO 2010
Cleeland CS et al, JCO 2010

Automated Symptom Alerts Reduce Postoperative Symptom Severity After Cancer Surgery: A Randomized Controlled Clinical Trial


Fig 3. Mean symptom threshold events per patient.

19% vs. 8%
P: Consecutive patients initiating chemotherapy for metastatic solid organ tumors at MSKCC (2007-2011) (n=766)
I: Patient reported outcomes (self report 12 common symptoms at / between visits with email alerts to clinicians
C: Usual care
O: HRQL, overall survival, readmission

Basch E., JAMA 2017
• **OS**
  - 31.2 mo vs. 26.0 mo
  - HR 0.83 (95% CI 0.70-0.99)

• **Discussion**
  - Early responsiveness to symptoms?
    • Nurses responded to 77% of alerts
  - Continued chemo longer?
    • 8.3 mo vs. 6.3 mo

Basch E., JAMA 2017
Effect of Early Surgery vs Physical Therapy on Knee Function Among Patients With Nonobstructive Meniscal Tears
The ESCAPE Randomized Clinical Trial

Victor A. van de Graaf, MD; Julia C. A. Noorduyn, MSc; Nienke W. Willigenburg, PhD; Ise K. Butter, MSc; Arthur de Gast, MD, PhD; Ben W. Mol, MD, PhD; Daniel B. F. Saris, MD, PhD; Jos W. R. Twisk, PhD; Rudolf W. Poolman, MD, PhD; for the ESCAPE Research Group

van de Graaf VA, JAMA 2018

P: Noninferiority randomized trial of patients with meniscal tears (n=321)
I: Physical therapy protocol
C: Arthroscopic partial meniscectomy (APM)
O: Patient reported knee function on the International Knee Documentation Committee (IKDC) score from baseline over 24 months
• PT vs APM
  – 20.4 vs. 26.2 point improvement from baseline
• Overall between group difference
  – 3.6 points (p-value for non-inferiority 0.001)
Limitations
Real World Application

• Logistical issues
  – Reliable, efficient data collection requires resources, expertise and time
  – Clinical workflow concerns
    • Who, when, how?
  – Data interpretation
    • Defining clinically important changes
  – Intervention?
    • Who, when, how?
Real World Application

• Electronic health record integration
  – Systems need to be built and work seamlessly
    • Collaboration between health IT, informatics, institutional leadership, clinicians
  – Automation
  – Clinical alert triggers
  – Clinician interaction
Methodological Issues

- Matching domains to clinical scenario
  - Requires input from both clinicians and patients
- Must choose an instrument
- Linkage of measures
- Repeated measures (pre-post)
  - Time sensitive, particularly in surgery
- What about risk adjustment
  - Level playing field?
Summary

• What is a PRO?
  • Unfiltered patient experience

• Why are PROs important?
  • Oversight, improved care, academic success

• Nuts and bolts of measurement
  • Complicated, ask for help!

• Research examples
  • Cutting edge applications

• Limitations
  • Many…
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