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

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Disclosures

- Nothing to disclose

Outline

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

What is a PRO?

AAS
Association for Academic Surgery

"***Any report of the status of a patient's health condition that comes directly from the patient, <u>without interpretation</u> of the patient's response by a clinician or anyone else***"

- U.S. Department of Health and Human Services

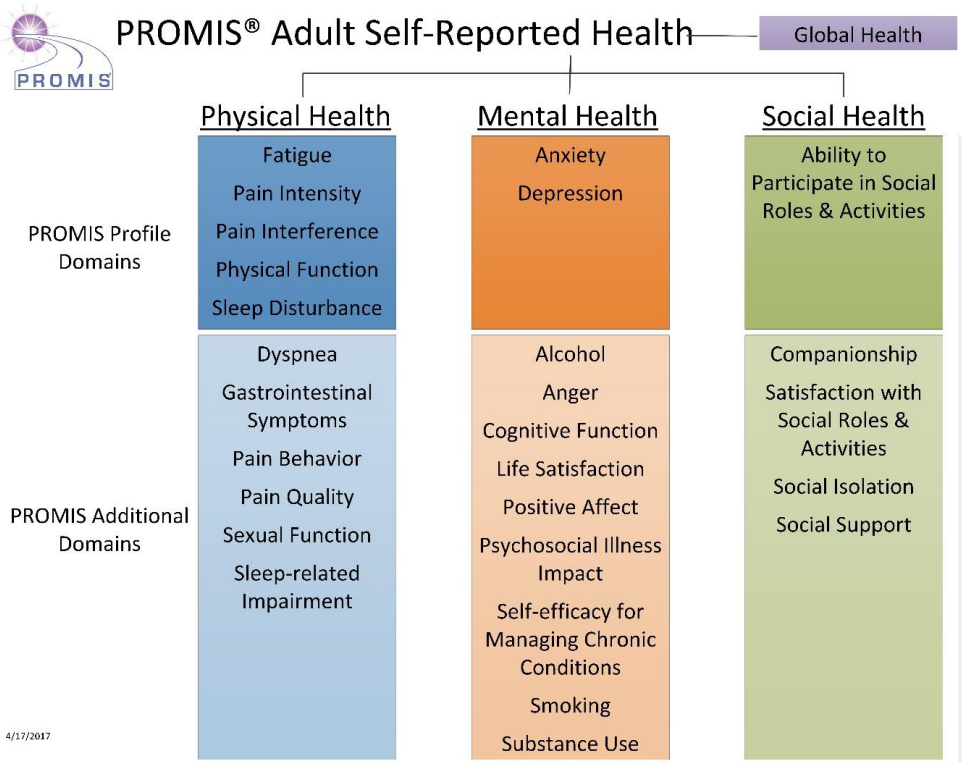
What is a PRO?

- 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?



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?"

Not at all ***Very much***

Energetic **Severe**

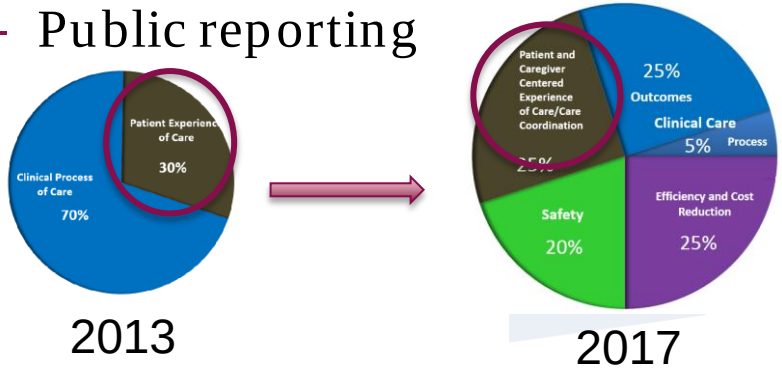
FATIGUE

Why are PROs Important?

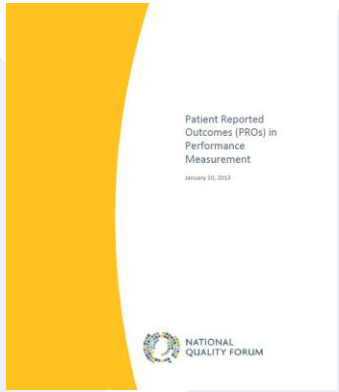
Why are PROs Important?

- Patient centered care is *better* care

- Physician compensation is tied to patient feedback
  - 25% of hospital VBP program
  - Public reporting



2013 2017



CMS
CENTERS FOR MEDICARE & MEDICAID SERVICES

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

JAMA

The Promise of Patient-Centered Outcomes Research

-Barry MJ, JAMA 2011

Nuts and Bolts of Measurement

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

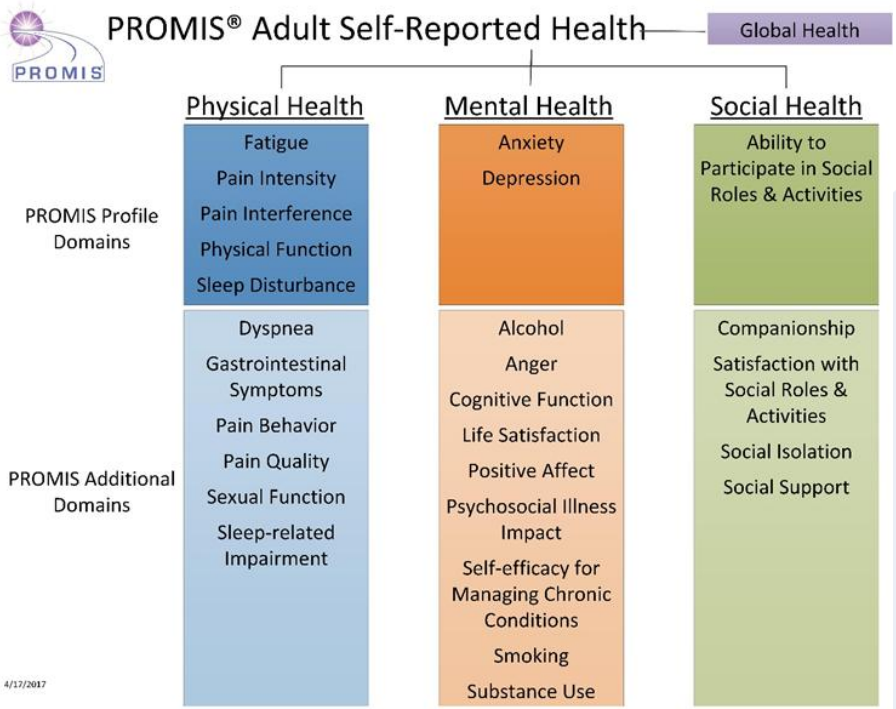
- What we *want* in a measure
  - Psychometrically sound and interpretable
  - Brief, simple
  - Generalizable across groups, ages
  - Full range of any given trait (no floor or ceiling effects)
  - Cross cutting (many diseases/ conditions)
  - Common scale / centered on a reference population

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

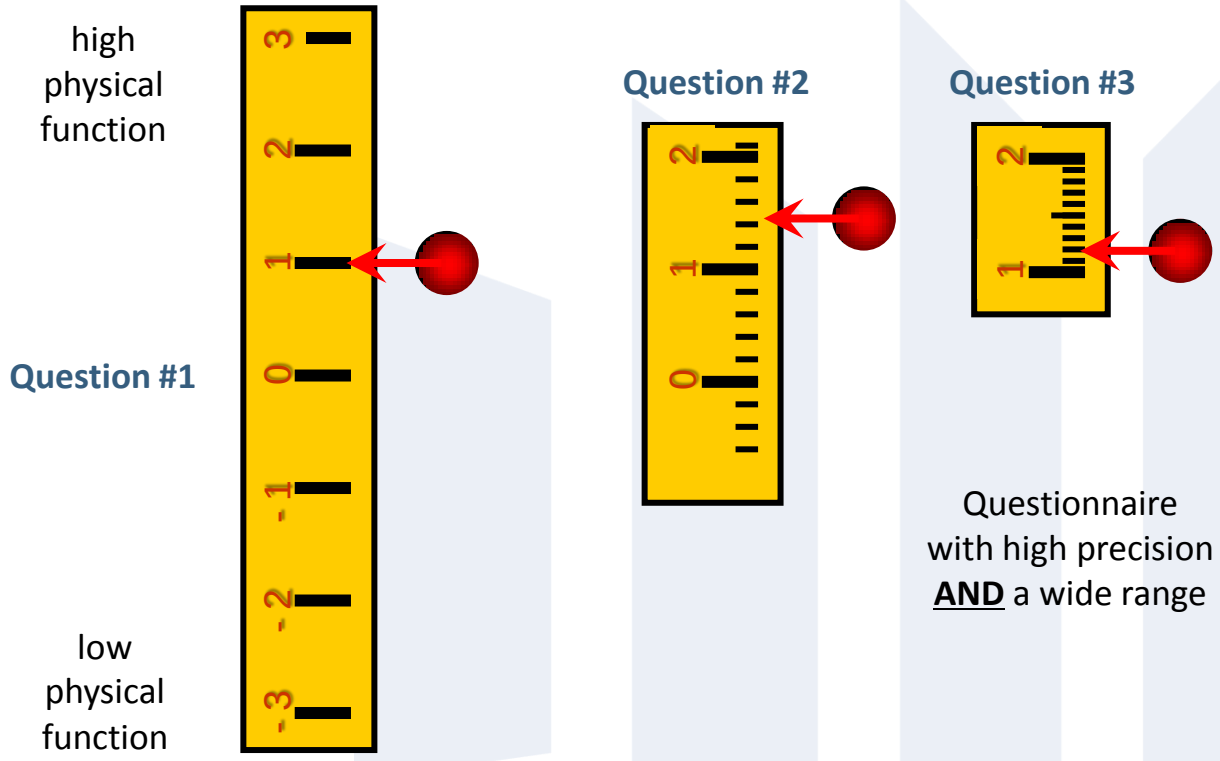
Nuts and Bolts of Measurement

- 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

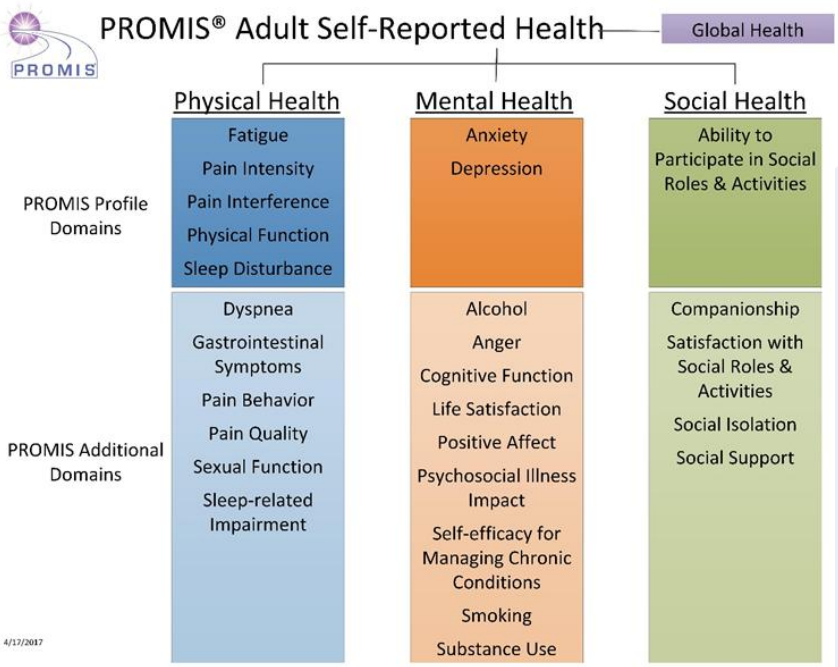
Computerized Adaptive Testing



Nuts and Bolts of Measurement

Choose your measure(s)

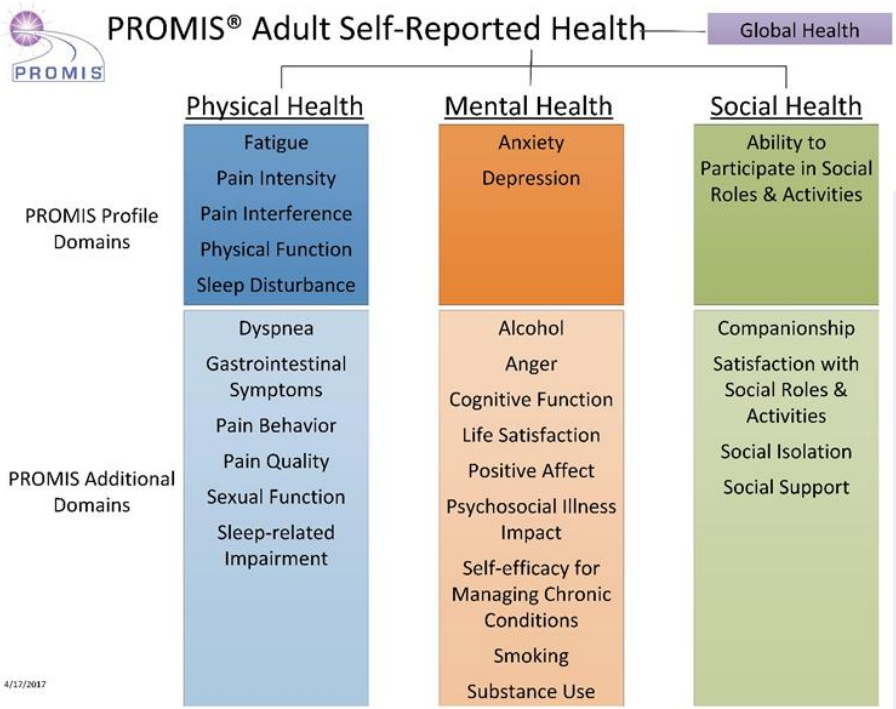
- Tailor to your interest
  - Select domain(s) vs. global
  - Select types of measures
- Ex: Abd surgery recovery
  - Pain, GI symptoms, physical function



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



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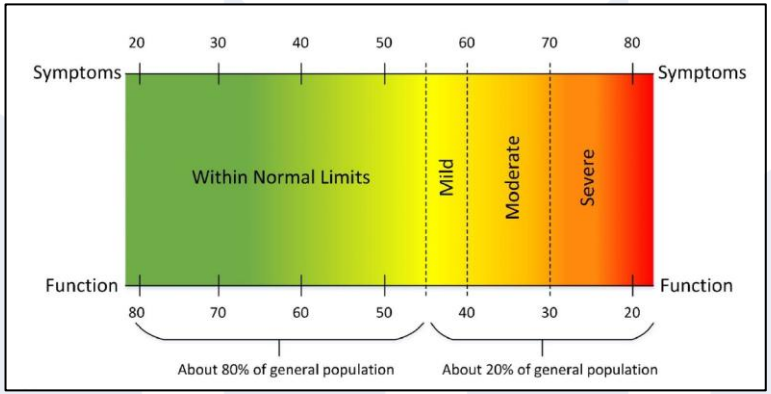
Nuts and Bolts of Measurement

- Interpreting scores
- T-score → 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

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



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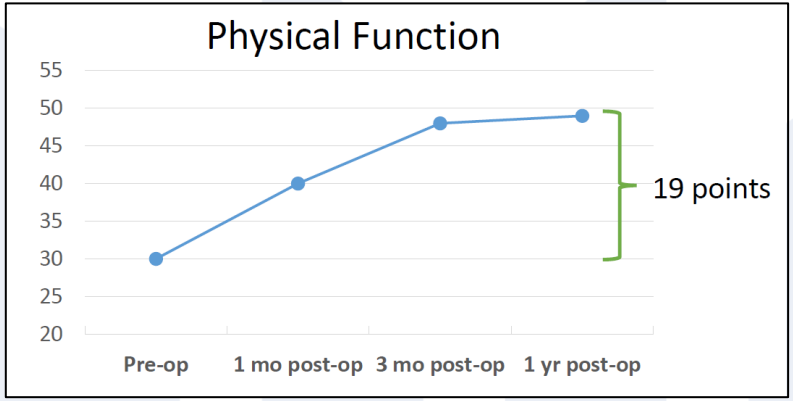
Nuts and Bolts of Measurement

- 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



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Nuts and Bolts of Measurement

- Goal: Many measures, but *one metric*

Research Examples

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

Charles S. Cleeland, Xin Shelley Wang, Qiuling Shi, Tito R. Mendoza, Sherry L. Wright, Madonna D. Berry, Donna Malveaux, Pankil K. Shah, Ibrahima Gning, Wayne L. Hofstetter, Joe B. Putnam Jr, and Ara A. Vaporciyan

AAS Association for Academic Surgery

JOURNAL OF CLINICAL ONCOLOGY

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

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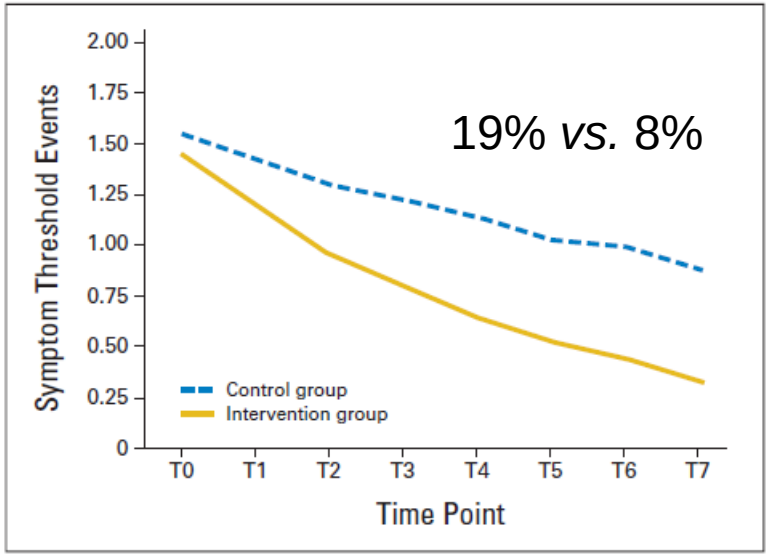

Fig 3. Mean symptom threshold events per patient.

Cleeland CS et al, JCO 2010

RESEARCH LETTER

Overall Survival Results of a Trial Assessing Patient-Reported Outcomes for Symptom Monitoring During Routine Cancer Treatment

JAMA

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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

RESEARCH LETTER

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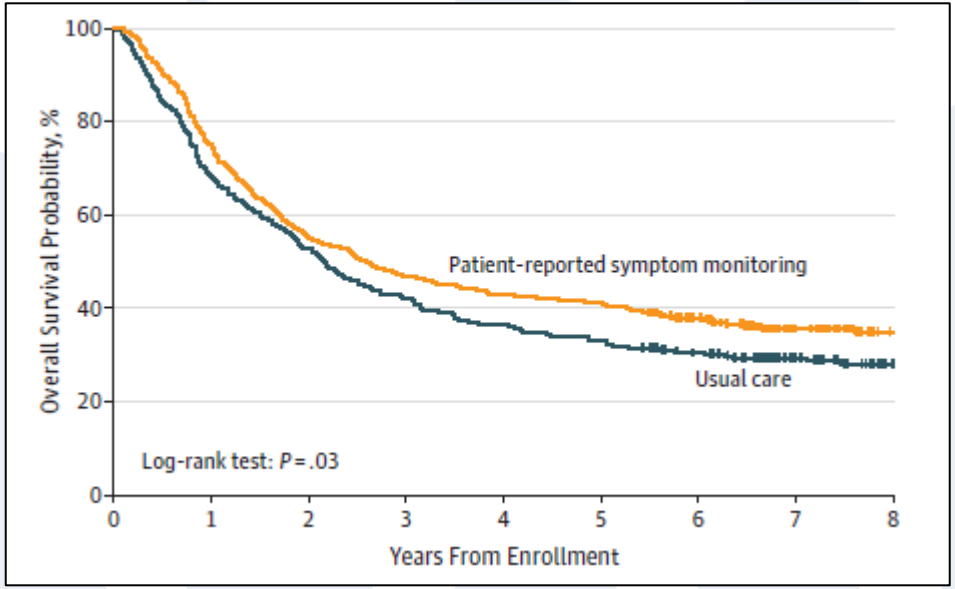
- OS
  - 31.2 mo vs. 26.0 mo
  - HR 0.83 (95% CI 0.70-0.99)
- Discussion
  - Early responsiveness to symptoms?
    - Nurses responded to 77% of alerts
  - Continued chemo longer?
    - 8.3 mo vs. 6.3 mo



Basch E., JAMA 2017

JAMA | Original Investigation

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

JAMA

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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

van de Graaf VA, JAMA 2018

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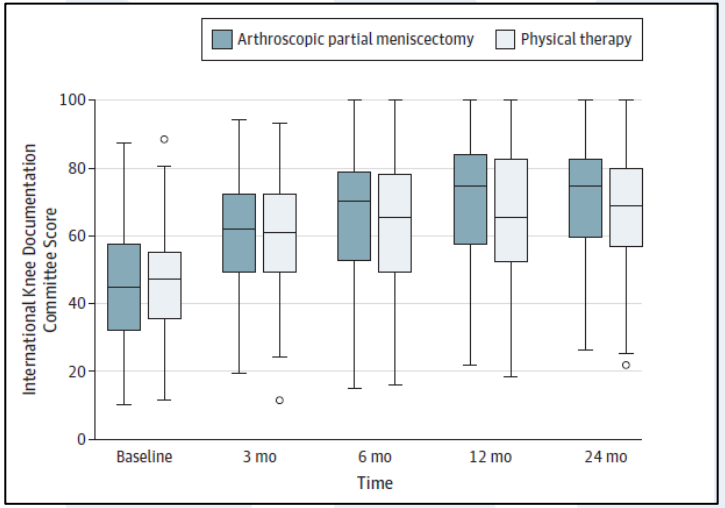
JAMA

- 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)



van de Graaf VA, JAMA 2018

Limitations

Real World Application

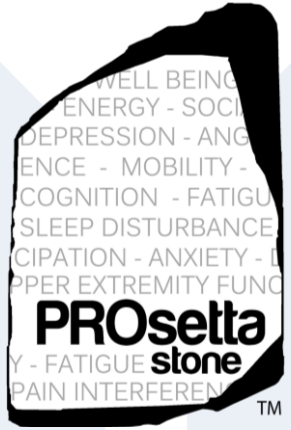
- Logistical issues
  - Reliable, efficient data collection requires resources, expertise and time
  - Clinical workflow concerns
    - Who, when, how?
  - Data interpretation
    - Defining clinically important changes
  - Intervention?
    - Who, when, how?

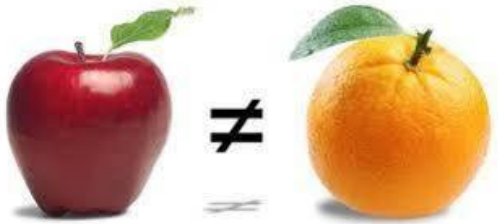
Real World Application

- 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

Real World Application

- 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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