Health Outcomes / Patient Reported Outcomes

Ted Ganiats, MD
Health Services Research Center
University of California San Diego
tganiats@ucsd.edu
Conflict of Interest

• None
Bottom line

• I love PROs

• PROs give you a more complete picture of person & disease

• PROs are limited in some circumstances

• Knowing these limitations helps you “choose wisely” and sets research agenda
What is *Value* in health?

• In part: Quality outcomes
What is *Quality*?

- National Academy of Medicine
  - *Safe, effective, patient-centered*, timely, efficient, equitable
- Good clinical outcomes
How measure clinical outcomes

• Disease-specific outcomes

• Patient-reported outcomes

• (I don’t think “clinical outcomes” are only disease-specific outcomes)
Disease-specific outcomes

• Examples: Blood test, xray

• Benefit: Objective, “Easy”
  • Interpretation standardized
  • Same for clinical and policy work

• Disadvantages
  • Often not correlated with patient condition
  • Often intermediate (e.g., serum cholesterol or A1c)
    • Therefore often poor window into prognosis
Review

• Up to this point of talk....

VALUE -> OUTCOME -> HEALTH OUTCOMES -> DISEASE-BASED
Assessing Patient-reported Outcomes

• Importance
  • Don’t forget symptom
  • Learn new symptom or use

• Types of Assessment
  • Informal

  • Formal
Patient-reported Outcomes

• Informal
  • How are you doing?
  • Are you having any problems?

• Formal – Generic
  • QWB, EuroQol, HUI

• Formal – Disease-specific
  • RAPS, MLHFQ, NYHA
Use of PROs (outside trials)

• Clinical

• Learning healthcare system

• Policy (patient-centredness)
Not “easy”

• “Assess the things we value rather than valuing the things that are the most easily assessed”

• As a rule, if you are interested in policy or scoring a PRO...you can’t
  • take a PRO assessment tool off shelf and use indiscriminately
  • sit down and create new PRO by simply writing some questions
Not “easy”

• To learn more....

• Sample references from ISPOR* in *Value in Health*
  • Overall: Rothman et al., 2009;12(8):1075-83
  • Rare diseases: Benjamin et al., 2017;20:238-55

*International Society for Pharmacoeconomics and Outcomes Research
PRO: HUGE Advantages

• Looking at what matters (time horizon caveat)
• Looking at what matters
• Looking at what matters

This lets us work (clinical, policy) with patients that have a disease to improve patient-centered AND disease-centered outcomes (and not looking to improve the disease)
PRO: Significant Disadvantages

• Snapshot of today
  • Therefore often poor window into prognosis

• Development important (Group B strept)

• Fit for purpose?
  • Clinical vs policy
PRO limitation

• A back pain treatment reduces pain but causes occasional slurred speech.

• 2 patients with back pain are treated.

• PRO assessment pre- post-treatment shows both people improved by 45 points (pain improved but speech occasionally slurred).
PRO Limitation

HOWEVER,

• The mail carrier’s improved back pain offers her some improvement in quality of life and allows her to return to work. The slurred *speech is a nuisance* and she *continues the treatment*.

• The politician’s improved back pain offers her some improvement in quality of life and she is able to return to work. The slurred *speech is a major problem* and she stops the treatment.

• Same PRO score; different outcomes
PRO Limitation

• A PRO with generic scoring may be misleading at the individual level

• Limits clinical usefulness if generic scoring
One Special PRO: Satisfaction

• Satisfaction is a PRO

• It is subjective

• CAVEAT: comparing scores
  • (Why can a 2-star hotel get better reviews than a 4-star hotel?)
Bottom line

• I love PROs

• PROs give you a more complete picture of person & disease

• PROs are limited in some circumstances

• Knowing these limitations helps you “choose wisely” and sets research agenda
Review

- Up to this point of talk....

VALUE -> OUTCOME -> HEALTH OUTCOME -> PRO

-> COST OUTCOMES

tganiats@ucsd.edu