Considerations for Patient Outcomes Research in Health Professions Education

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Bottom line
• Patient outcomes are important
• They are not the Holy Grail
• Excessive focus could make things worse
• Researchers commonly make serious errors
  • We need to strike a balance

Reconsidering the focus on "outcomes research" in medical education: a cautionary note.
David A. Cook, Colin P. West
Acad Med. 2013;88:162

A few basic principles

1. Taxonomy for outcomes:
   - Kirkpatrick’s hierarchy
   - Results (patient, society)
   - Behavior (practice)
   - Learning (knowledge, skill)
   - Reaction (satisfaction)
   - Surrogate outcomes
   - Soft (meaningful)
   - Patient outcomes

2. Assessments are diagnostic tests
   - Pulmonary embolism?
     • History
     • Exam
     • CBC
     • D-dimer
     • Chest x-ray
     • CT angiogram
   - Competent physician?
     • Rotation shelf exam
     • OSCE
     • Simulation procedural assessment
     • Certifying exam
     • Workplace observation

Integration → Decision → Action

3. Validity: the validity argument
   - Clearly state hypothesis (proposed inference)
   - Test weakest assumption(s)
   - Review evidence
   - Revise hypothesis if needed
   - Plausible (accept)
   • Content
   • Response process
   • Internal structure
   • Relations with other variables
   • Consequences

Cook, Adv Health Sci Educ 2013
Validity evidence: Consequences of the program/decisions

**Screening PSA**
- Detects cancer at earlier stage
- Cost
- False positives → unneeded biopsies, indolent cancers
- False negatives → unjust. reassurance

**Credentialing**
- Detects incompetent providers *(safety)*
- Cost
- False positives → unnecessary delay, remediation
- False negatives → unjust. reassurance

### Challenges and pitfalls

What will happen if we *focus* on patient outcomes?
- Neglect other important outcomes
- Restrict questions that can be answered
- Limit research (feasibility)
- Limit research (strength of evidence)

### Problem 1: Dilution

- Transfer
- Supervisor
- Team
- Systems, Policies
- Patient Factors
- Patient Effects

- Intrinsic to learner (controllable)
- Extrinsic (not controllable)

Hierarchy of outcomes

- Easy
- Difficult
- Soft
- Hard

Results *(patient, society)*

- Behavior *(practice)*
- Learning *(knowledge, skill)*
- Reaction *(satisfaction)*

Feasible? *Wise?*
Dilution: Solutions … or are they?

• Large expected effect
  • Comparison no intervention
    • \( \rightarrow \) No comparative effectiveness
    • Multifaceted (strong) intervention vs weak
      • \( \rightarrow \) Confounded

• Very large sample size
  • Lose individual signal

Problem 2: Feasibility: Sample size

• Size
  • MERIT-HF – 3991 patients
  • HOPE trial – 9541 patients
  • CAPRIE – 19,185 patients

• How many physicians required to show benefit of education on mortality????

Problem 2: Feasibility: Sample size

<table>
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<th>Publication</th>
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</table>

- Typical no-intervention comparison
  - Effect size 0.8 \( \rightarrow \) req’d sample N=52

Problem 3: Causal link: design control

- Cross-sectional
  - 1-group pre-post
  - Case-control
  - Historical control
  - Prospective, nonrandomized
  - Randomized

- Typical comparative effectiveness
  - Effect size 0.4 \( \rightarrow \) req’d sample 200 trainees

Problem 3: Causal link: multifaceted interventions
Problem 3: Causal link: patient populations

Problem 3: Causal link

Problem 4: Outcome selection

Problem 4: Outcome selection

Problem 4: Outcome selection

Problem 5: Teaching to the test

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Problem 5: Teaching to the test

- Select for instruction
- Influence outcomes
- Clear evidence for benefit
- All relevant topics

Pitfalls in published research …

Pitfall 1: Inadequate power

Fabricated: visualize the CI

Actual prevalence

- No intervention (N=609)
- Comp. effectiveness (N=289)

Cook et al., JAMA 2011
Cook et al., Med Teach 2013

\[ -4\% \text{ (CI 1 - 6) of 100} \]

Pitfall 2: Errors of analysis

- Patients are clustered in trainees
- Failure to account for clustering inflates power

Simulation Educ. (N=50)

Cook et al., J Gen Intern Med 2013

Final thought: Are patient outcomes REALLY better?

- “The primary customer of education is the student” – J. Shea
  - Seek value, efficiency, good product
  - All else equal, higher satisfaction is good

- Process measures inform implementation

- Are students cogs in the machine?

Summary: Challenges and pitfalls

- Dilution
- Feasibility (sample size)
- Causal link
- Biased selection
- Teach to the test

Pitfalls
- Underpowered
- Analysis errors

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Solutions and pearls

1. Don’t start with the outcome

   Objective (topic, question) → Outcome → Instrument

   No outcome is “most important”

2. Use the right label

   Test setting | Patient care
   --- | ---
   Skill time | Behavior time
   Skill process | Behavior process
   Skill product | Patient effect

   • Happen to patient
   • Happen within patient

3. Strengthen the link: Surrogates

   Study A
   Controlled trial
   Intervention
   Patient Effect

   Study B
   Validation of surrogate
   Intervention
   Surrogate
   Patient Outcome

   Cook et al, Acad Med 2013

3. Potential surrogate outcomes

   Transfer → Supervisor → Team
   Systems, Policies, Patient Factors
   Knowledge, Skills, Attitudes
   Behaviors

   Intrinsic to learner (controllable)
   Extrinsic (not controllable)

3. Validating a surrogate

   • 1. Does the surrogate correlate with the outcome of interest?
   • 2. Does the surrogate change when the outcome of interest changes?
     • e.g., after an intervention or over time

   Fleming & DeMets, Ann Intern Med 1996
   Bucher et al, JAMA 1999
   Rother et al, JAMA 1999
3. Validating a surrogate

- 1. Does the surrogate correlate with the outcome of interest?
- 2. Does the surrogate change when the outcome of interest changes?
  - e.g., after an intervention or over time
- 3. Other validity evidence (as per routine)

3. Cautions in the use of surrogate

- Causal pathways may differ
- Scales not uniformly responsive
- Correlated factors may explain shared variance
- Dominate when part of a composite endpoint

4. Stepwise progression

- Large-scale implementation
- Behavior, patient effect
- Test in practice
- Knowledge, skill, behavior
- Test in “lab”
- Reaction, kldg, skill
- Test with non-health learners
- Foundations, theory

5. Consider trainee level and team

- Less dilution with practitioners (CPD) and team interventions
  - “Educationally sensitive patient outcomes”
    - Interface between behavior and patient effect
    - Patient motivation to change
    - Team function

![Diagram of patient outcomes and transfer]

- Intrinsic to learner (controllable)
- Extrinsic (not controllable)

![Diagram of CPD transfer]

- Intrinsic to learner (controllable)
- Extrinsic (not controllable)
6. Beware design and analysis errors

- Use strongest design possible
- Plan appropriate power (power for trainees, not patients)
- Account for clustering (unit of analysis)
- Report patient demographics

Summary: Solutions and pearls (research)

1. Select objective first, then outcome
2. Terminology:
   - Skills in test setting
   - Behaviors and patient effects in patient care
3. Judicious use of surrogates (caution in validation)
4. Stepwise progression (what % “phase 3”?)
5. Teams and “educationally sensitive” outcomes
6. Beware design and analysis errors

Bottom line

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