Knowing What We Need to Know for Evidence-Based Healthcare: Getting the Outcomes Right

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Do you believe that the health care you receive is evidence-based?

*It might not be!*
What is evidence-based healthcare?

“The integration of best research evidence with clinical expertise and patient values.”

Sackett D et al, 2000
Evidence-based healthcare paradigm

Clinical Trials → Systematic Reviews → Clinical Practice Guidelines → Evidence-based Healthcare
What is an outcome?

In clinical trials, an outcome is a measure or event that are used to assess the effectiveness and/or safety of clinical interventions.

Five elements of a completely specified outcome:

1. **Domain**
   - Example: Blood pressure

2. **Specific measurement**
   - Example: Sphygmomanometer

3. **Specific metric**
   - Example: Value at a time-point

4. **Method of aggregation**
   - Example: Mean

5. **Time-points**
   - Example: 6 months

Houston
we've had
a problem

(Actually, two problems) 😞😞
Evidence-based healthcare paradigm

Clinical Trials → Systematic Reviews → Clinical Practice Guidelines → Evidence-based Healthcare

Problem 1
Story 1 – Gabapentin for neuropathic pain

21 clinical trials

4 outcome domains

Story 1 – Gabapentin for neuropathic pain

PROBLEM 1: Way too many outcomes in clinical trials!

21 clinical trials

4 outcome domains

214 different outcomes!

Story 2 – Quetiapine for Bipolar Depression

PROBLEM 1: Way too many outcomes in clinical trials!

Evidence-based healthcare paradigm

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Problem 2
A simple thought experiment

% of trials reporting the outcome

% of reviews reporting the outcome
Some hypothetical scenarios

Outcomes popular among trialists

% of reviews reporting the outcome

% of trials reporting the outcome
Some hypothetical scenarios

Outcomes popular among trialists

Outcomes popular among reviewers

% of reviews reporting the outcome

% of trials reporting the outcome
Some hypothetical scenarios

Outcomes popular among trialists

Outcomes popular among reviewers
Some hypothetical scenarios
What did we find in eye research?

**Age-related macular degeneration**

**Cataract**

**Diabetic retinopathy**

**Glaucoma**

**PROBLEM 2:** Way too little overlap in clinical trial and systematic review outcomes!

What did we find for HIV/AIDS?

**Clinical management**

**Biomedical prevention**

**Behavioral prevention**

**Health services**

**PROBLEM 2:** Way too little overlap in clinical trial and systematic review outcomes!

Saldanha IJ, et al. *Journal of Clinical Epidemiology* 2017
But, **WHY** are these problems for **YOUR** health care?

**Clinical Trials** → **Systematic Reviews** → **Clinical Practice Guidelines** → **Evidence-based Healthcare**

Problem 2

Problem 1
Back to our hypothetical scenarios

Outcomes popular among trialists

Outcomes popular among reviewers

% of reviews reporting the outcome

% of trials reporting the outcome
Back to our hypothetical scenarios

Outcomes popular among trialists

Outcomes popular among reviewers
Back to our hypothetical scenarios

Need for core outcome sets!

“A core outcome set is an agreed-upon minimum set of outcomes that should be measured and reported in a given disease area”
Advantages of core outcome sets

- Increases consistency across trials
- Maximise potential for trial to contribute to systematic reviews of these key outcomes

For rheumatoid arthritis
1. Tender joints
2. Swollen joints
3. Pain
4. Physician global assessment
5. Patient global assessment
6. Physical disability
7. Acute phase reactants
8. Radiographic structures of joints

Kirkham JJ, et al. BMJ 2017

NOT a pie in the sky!
Multi-stakeholder involvement in Core Outcome Set development

- Patients and the public
- Doctors
- Clinical trialists
- Systematic reviewers
- Guideline developers
- Research funders
- Drug and device manufacturers
- Journal editors
- Regulators (e.g., FDA)
Why should **YOU** care?

1. **Economics**

2. **Ethics**

3. **Your health!**

   - **Relevant** to decision-makers – your doctor, you!
   - **Consistently** examined (common yardsticks)
Do you believe that the health care you receive is evidence-based?

It might not be!

Let’s make sure it is!
What can **YOU** do?

- Get further informed
  - [http://www.comet-initiative.org](http://www.comet-initiative.org)
  - [http://www.ichom.org](http://www.ichom.org)
  - [http://omeract.org](http://omeract.org)

- Get involved
  - Core outcome set development efforts
  - Advocate for core outcome set adoption

- Get in touch
  - [ian_saldanha@brown.edu](mailto:ian_saldanha@brown.edu) (@saldanha_ian)
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Thank you!

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