Health Services Research (IOM)

"Examines the use, costs, quality, accessibility, delivery, organization, financing, and outcomes of health care services to increase knowledge and understanding of the structure, processes, and effects of health services for individuals and populations."


Evidence-Based Medicine

If all you have is a hammer, everything looks like a nail

“Clinical decisions should, as far as possible, be evidence based. So runs the current clinical dogma. We are urged to lump all the relevant randomized controlled trials into one giant meta-analysis and come out with a combined odds ratio for all decisions. Physicians, surgeons, nurses are doing it; soon even the lawyers will be using evidence based practice. But what if there is no evidence on which to base a clinical decision?”


My Presentation Objectives

- Define research study design options
- Differentiate clinical research from health services research
- Discuss the basics and importance of
  - Interrupted time series design and segmented regression
  - Patient-reported outcomes
  - Dissemination & implementation science

Katie – Bar the Door!

- “The key problems of today are ‘wicked’ problems that are multilevel, multiply determined, complex, and interacting.”
- “The RCT designs and hegemony around systematic reviews have worked well to create an initial body of research but have not worked for producing replicable results that matter or translate.”
- “We propose a 10-year moratorium on efficacy RCTs in health and health services research.”

Three Tough But Crucial Questions

• What does it cost?
  – Cost questions are important and complex, and cost data must be a primary focus rather than an afterthought or “add-on.”
• How many and what types of people will participate?
  – The second “field of dreams” question “if we offer it, will people come” is especially challenging to answer using traditional RCT designs.
  – More real-world relevant designs...include preference designs in which all or a subset of participants are allowed to choose among alternative interventions, as is more typically done in real-world settings.
• How do I know this will work in our setting?
  – Raises critical issues for translation and dissemination that are seldom addressed in traditional RCT designs.


Three Basic Questions That Can Be Asked in Assessing a New or Existing Treatment or Other Intervention

EFFICACY: Can it work?
Clinical benefits demonstrated under ideal or optimal circumstances in a randomized controlled trial (RCT)

EFFECTIVENESS: Does it work?
Clinical benefits observed under “real-world” conditions in a more typical population with a pragmatic RCT or cohort study

EFFICIENCY: Is it worth it?
Clinical benefits relative to costs determined via a cost-benefit analysis, cost-effectiveness analysis, or cost-utility analysis

Uncontrolled Before and After Study

• Only measures performance before and after the introduction of an intervention in the same study population and at the same care delivery site(s)
• Any observed before and after differences in the quality or key performance indicators are assumed to be due to the intervention itself.
• May fail to consider the effect of an underlying or background temporal trend, thus reducing the validity of attributing the observed change to the intervention
• May thus overestimate effects of a process improvement intervention or quality improvement program

Explanatory versus Pragmatic Trials

In 1967, Schwartz & Lellouch noted limited applicability of many trial results beyond the artificial, “laboratory” environment of the trial.

Explanatory Trials
  • Aimed at confirming a physiological hypothesis, precisely specified as a causal relationship between administration of an intervention and some physiological outcome

Pragmatic Trials
  • Aimed at informing a clinical, health service or policy decision, where this decision involves the choice between two or more interventions

Research Study Design Classification System or Taxonomy

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Sedgwick P. Before and after study designs. BMJ. 2014;349:g5074.
Interrupted Time Series (ITS)

- Considered the strongest, quasi-experimental research design for evaluating longitudinal effects of interventions
- Appropriate for evaluating the effects of a wide-scale, system-wide guideline implementation or process change—situations in which it is difficult to identify a valid control group or to randomize study participants
- Determines if the intervention had an effect greater than the underlying temporal or background trend
- Data are collected at 20 or more, nearly equal time points before and then at 20 or more, nearly equal time points after the intervention

Segmented Regression and ITS

- ITS design may not adequately compensate for effects of other known or unknown interventions or events occurring concurrently with study intervention, which might also affect quality/performance outcome measures.
- Segmented (hockey-stick, piecewise, or broken-stick) regression can mitigate this internal validity risk, and its application is recommended when there is an ITS design to use regression to determine the effect of the intervention.
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Segmented Regression Analysis

The level change is an estimate of the change in level that can be attributed to the intervention, observed between the time points immediately before and immediately after the intervention...but it is not actually so simple.

Rate = Su + 6*time.  A study program implementation + 6*time after program.
Health-Related Quality of Life

- **Health-related quality of life (HRQoL)** is a multi-dimensional concept that includes domains related to physical, mental, emotional and social functioning.
- It goes beyond direct measures of life expectancy and causes of death, and focuses on the impact health status has on quality of life.

Dissemination and Implementation (D&I) Research

- **Historical lack of timely incorporation of new, beneficial diagnostics and therapeutics into routine practice**
- **Dissemination**: Targeted distribution of materials to a specific clinical audience to spread knowledge about evidence-based practices and sustain in routine care
- **Implementation**: Fosters adoption and integration of evidence-based health interventions so as to change specific practice patterns
- **D&I Research**: Examines processes for transferring interventions into local settings

Patient-Reported Outcomes (PROs)

- **Patient-reported outcome (PRO) data are defined by the FDA as “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.”**

Patient Reported Outcomes Measurement Information System (PROMIS)

- Funded by the National Institutes of Health (NIH)
- System of highly reliable, valid, flexible, precise, and responsive assessment tools that measure patient-reported health status
- **PROMIS Global Health Measure** – 10-items: Assesses global physical, mental and social HRQoL through questions on self-rated health, physical HRQoL, mental HRQoL, fatigue, pain, emotional distress, social activities, and roles

Three Distinct Types of Research

- **What, how, and why of the dissemination and implementation of complex interventions**
- **Facilitators and barriers to successful dissemination and implementation**
- **Highly interdisciplinary**
- **Focus on real-world complex problems**
- **Many investigators in these fields also have extensive training as health care providers**
IF THIS IS NEW GROUND FOR YOU…
Find a local epidemiologist, health policy expert, healthcare economist, mixed methodologist, psychometrician, or whoever…and take them to lunch!