Models of Comparative Effectiveness Research (CER)

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Topics for Discussion

• What is a model?
• Models used in comparative effectiveness research
  – Practice based models
  – Community based models
  – Mediational models
What is a Model

• Definition of a model:

A model is a system or object that stands in the place of another . . . a model is not exposed to refutation, but is used as long as any benefit can be derived from it. . . . A model can continue to be useful even though it yields many conclusions which are clearly wrong, provided only that it yields some conclusions that are correct (i.e., useful).

Rubenstein, Albert H., and Chadwick J. Haberstroh
What is a model?

• What does a model help you to do?
  SEEK THE TRUTH
  DRAW A PICTURE OF REALITY
  ESTABLISH CAUSE
What is a Model?

» MODELS IN THE HIERARCHY OF KNOWLEDGE

» TYPOLOGY (description)

» MODEL (causal relationships)

» THEORY (direction and explanation of cause – any negative findings refute theory)
Models in the Hierarchy of Knowledge

• Typologies help us to describe but:

Usually, although not always, one can find numerous implicit hypotheses buried among the comparisons of the several types, but it is my own observation that typology construction, for some reason, does not lend itself to an explicit focus on propositions and their interrelationships.

Blalock, Hubert M., Jr.
What is a model?

A different view of the theory-model relationship would seem to hold that a theory might include any conceptual scheme suggesting the relationship of two or more concepts ranging, according to Webster’s New Collegiate Dictionary, from “a speculation” or “guess” to “a body of theorems presenting a clear, rounded and systematic view of a subject.” In this view, the process of “modeling” is providing empirical reference to the concepts that allow the possibility of rejecting the proposed relationship or relationships.

- Ronald Andersen and Odin W. Anderson
What is a model?

• Successful model implementation requires accurate measurement of variables & causal imputation
• Accurate measurement requires:
  Reliable variables
  Valid variables
Accurate causal imputation requires:
  Internal validity
  External validity
What is a model?
An international health systems example (1)
What is a model? Reference (1) for international comparisons

Models used in CER

Comparative effectiveness research (CER) is the conduct and synthesis of systematic research comparing different interventions and strategies to outcomes to prevent, diagnose, treat and monitor health conditions. CER models attempt to show the causal link between interventions and outcomes.
Models used in CER

- Practice based models
- Community based models
- Mediational models
Practice based model

- Practice based evidence ((PBE) study designs address comparative effectiveness by creating a comprehensive set of patient, treatment, and outcome variables, and analyzing them to identify treatments associated with better outcomes for specific types of patients. PBE studies are an alternative to randomized controlled trials, well suited to determine what works best for specific patient types, and provide clinicians with a rational basis for treatment recommendations for individual patients. They provide a holistic picture of patients, treatments, and outcomes, with no preset limits to the number of variables that can be included. Such an approach is needed for high quality comparative effectiveness research.
Practice based model

Table 1

Table 1. Examples of Patient, Process, and Outcome Variables From Recent PBE Studies

Practice-Based Evidence: Incorporating Clinical Heterogeneity and Patient-Reported Outcomes for Comparative Effectiveness Research
Horn, Susan D.; Gassaway, Julie
doi: 10.1097/MLR.0b013e3181e57473
Practice based model reference

Community based model

- Translating Research Into Action for Diabetes (TRIAD)
- TRIAD is a national, multicenter prospective study that provides information about effective treatments and better care for people with diabetes in managed care settings. TRIAD was launched in 1998 to evaluate whether managed care organizations’ structures and strategies affect the processes and outcomes of diabetes care among adults, and to identify the barriers to and facilitators of high-quality care and optimal health outcomes.
The TRIAD study group comprises 6 translational research centers (Figure 1) and their 10 health plan partners. When TRIAD began, these health plans contracted with 68 provider groups to deliver primary and specialty care to more than 180,000 adult enrollees aged 18 years and older with diabetes. TRIAD is funded by a cooperative agreement from the Centers for Disease Control and Prevention (CDC) and the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK).
Community based model

- **Health system factors**
- By using Donabedian’s paradigm (Figure 2), TRIAD characterized and examined both managed care structural characteristics and disease management strategies. In Donabedian’s paradigm, system factors are hypothesized to influence patient care processes, which, in turn, influence patient outcomes.
Community practice model

Figure 2. TRIAD conceptual model of relationships among system-level factors, processes, and outcomes of care
Figure 3. TRIAD conceptual model of relationships among patient factors, patient-system interactions, processes, and outcomes of care

**Fixed patient factors**
- Demographics, socioeconomics, environment, disease severity, comorbidities

**Patient-physician system interactions**
- Communication, trust, access to care, out-of-pocket costs

**Care processes**
- HbA1c, blood pressure, LDL-cholesterol testing, nephropathy screening, dilated eye exam, foot exam, aspirin, smoking cessation advise

**Psychosocial factors**
- Depression, hopelessness, self-efficacy, social support

**Behavior**
- Self-management, adherence, physical activity, diet, smoking

**Outcomes**
- HbA1c, systolic blood pressure, LDL-cholesterol
Managed care systems should emphasize the development and reporting of care processes known to be closely linked to improved outcomes. Increased system-level attention to monitoring and improving treatment intensification rates may help improve intermediate outcomes. Specific areas for research and possible interventions that may improve the health of people with diabetes include the following:

- Redesigning benefits to lessen the cost burden of medicine on patients will ensure more people with diabetes take the prescribed medications.
- Increase cardio-metabolic control and behavioral and medical interventions to treat depression.
- Improve efforts to encourage provider communication and increase patient trust.
Community centered model reference

- **CDC - Translating Research Into Action for Diabetes (TRIAD ...**
- May 20, 2011 – Natural Experiments for Translation in *Diabetes*
Mediational models

• The advent of accessible structural equation modeling (SEM) programs (e.g., AMOS, MPlus, EQS, and LISREL) in combination with the focus on theory testing and the mechanisms of behavior change of the HIV/AIDS field, has caused an explosion in the use of SEM to test theory-based mediational questions. The result has been that researchers who may never have had training in mediational analysis or SEM per se are not only being asked to read and understand such analyses but are increasingly being asked to produce these analyses themselves.
Mediational models

• Within the context of a larger study testing the distal effect of alcohol on condom use, Theory of Planned Behavior (TPB) constructs were measured longitudinally in a sample of 300 adolescents involved with the Denver metro area juvenile justice system
Mediational models
Mediational models
Mediational model

• As shown, the hypothesized relationships were supported, such that attitudes, norms, and self-efficacy all predicted intentions to use condoms while both intentions and self efficacy predicted behavior. The model accounted for 29% of the variance in intentions, and 25% of the variance in behavior.
Mediational model reference

Postscript: taking arrows seriously in the CER models – causal inferences

- Experimental design: gold standard
- Quasi-experimental design: approximations – more or less?
- Instrumental variable analysis: to combat the two headed arrow?
- Propensity scores: getting rid of selection bias?
- Multi-level analysis: Cleaning the arrow from contextual level to individual level?
- Structural equation modeling: When there are many arrows to follow