Module 3: Comparative Effectiveness Research (CER) A Complete Curriculum

January - February - March 2014
Times and locations vary – see sessions following

Coordinator: Martin Shapiro MD PhD
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<td>Martin Shapiro</td>
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<td>Effectiveness Research (CER)</td>
<td>Ronald Andersen</td>
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<td>2 Patient-Centered Outcomes of Health Care</td>
<td>Ron Hays</td>
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<td>Mohsen Bazargan</td>
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<td>3 Access, Barriers, Disparities</td>
<td>Ronald Andersen</td>
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<td>Neil Wenger</td>
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<td>Jack Needleman</td>
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<td>Jerry Kominski</td>
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<td>Michael Weisman</td>
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<td>9 Implementation Science</td>
<td>Brian Mittman</td>
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<td>Douglas Bell</td>
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<td>10 Opportunities for Research on Evidence Based</td>
<td>Carl Stevens</td>
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Comparative Effectiveness Research: Overview of HSR and CER

- Module 1
- January 07, 2014, 10:30-12:30pm
- Instructors:
  - Martin Shapiro MD PhD (Geffen School of Medicine, GIM-HSR) and Ronald Andersen PhD (Fielding School of Public Health, Health Policy & Management)
OBJECTIVES OF MODULE 3A

• To be able to define CER and consider it as part of health services research
• To gain some appreciation for the precursors and current popularity of CER
• To understand research models as a SPECIAL tool for doing CER
• To be familiar with different types of CER research models and their application to help answer particular research questions
• To critically consider whether CER is worth adding to your arsenal for doing research and advancing your career
Questions You Might Consider when Reviewing the Module

- Is CER a new vintage of research wine or just old research wine in new bottles?

- What did CER take from the Outcomes Movement? Did CER add anything new?

- What does it mean to “take the arrows in research models seriously”?

- What advantages does CER have over an experimental design sometimes considered to be the “gold standard?”

- Of the various CER Models suggested (practice based, community based, mediational, nation based) which, if any, would you think interesting to apply as a research tool?
• DEFINITION OF HSR:

The multidisciplinary field of scientific investigation that studies how social factors, financing systems, organizational structures and processes, health technologies, and personal behaviors, affect access to healthcare, the quality and cost of healthcare, and ultimately our health and well-being. Its research domains are individuals, families, organizations, institutions, communities, and populations. (Lohr and Steinwachs, Health Services Research, 2002, pp. 7-9) The policy goals of HSR are: to control healthcare costs and improve efficiency; improve the quality of healthcare; improve access to healthcare for disadvantaged and uninsured populations; and reduce disparities in healthcare received by racial and ethnic minorities compared to the rest of the population.
Definition of CER (1):

Comparative effectiveness research (CER) is the conduct and synthesis of systematic research comparing different interventions and strategies to prevent, diagnose, treat and monitor health conditions. CER models attempt to show the causal link between interventions and outcomes.
Definition of CER (2):

Generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care. The purpose of CER is to assist consumers, clinicians, purchasers, and policy makers to make informed decisions that will improve health care at both the individual and population level.

-Institute of Medicine
Comparative Effectiveness Research Definition: Reference

- Medical Care:
- June 2010 - Volume 48 - Issue 6 - pp S7-S8
- doi: 10.1097/MLR.0b013e3181da3709
- Comparative Effectiveness
- Defining Comparative Effectiveness Research: The Importance of Getting It Right
- Sox, Harold C. MD, MACP
Prior Issues Stimulating “Comparative Effectiveness Research”

- The outcomes movement
- Reducing geographic variations
- Measuring and assuring quality
- Eradicating medical errors
- Eliminating health disparities
- Promoting cost-effectiveness in health care
- Source: Martin Shapiro, Comparative Effectiveness Research Program (CERP), UCLA, November 14, 2011
The Outcomes Movement

- 1969 President Nixon declared a “crisis in health care” – increasing costs, dissatisfied patients, doubts about expensive treatment efficacy
- Federal health policy response – market regulation, incentive payment, merging providers/HMO’s
- Crisis continues
- Problem – inability to measure outcome/effects of treatment
- Result – Out comes Movement – developing standards & guidelines for intervention, systematic measurement of patient function and clinical outcome, pool clinical & outcome data, analyze and distribute results, in 1986 congress allocates funds to AHCPR for patient outcomes research
The Outcome Movement References:


Geographic variation in commercial HEDIS® cardiac care performance (reducing geographic variation)

Commercial HEDIS® rates for diabetes care are higher in the New England and the Middle Atlantic. The quality scores are lowest in the South Central region.

Source: NCQA’s “State of Health Care Quality 2009”
Disparities in quality of care (measuring quality and reducing disparities)

For sizable proportions of measures, minorities and the poor receive lower quality care compared to Whites and those with high incomes respectively.

Hispanics and low income individuals receive lower quality care on about 60% of core measures.

Source: Agency for Healthcare Research and Quality, “2008 National Healthcare Disparities Report” (March 09)
Risk adjusted rates of adverse events/complications of care per 10,000 patients (eradicating medical errors & reducing health disparities)

Compared to White elderly patients:
- Minority patients were more likely to acquire infections in the hospital.
- Black patients were more likely to suffer blood clots in their legs or lungs following surgery.
- Black and Hispanic patients were more likely to develop pressure sores.

### Results Assuming All Care Provided at the Level of the Top 10% of Health Plans

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<tr>
<th>Selected measures</th>
<th>Avoidable Deaths</th>
<th>Avoidable Hospital Costs</th>
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<tr>
<td>Blood pressure control</td>
<td>14,000-34,000</td>
<td>$425 million - $1.8 billion</td>
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<tr>
<td>Smoking Cessation</td>
<td>7,000-11,000</td>
<td>$712 - $783 million</td>
</tr>
<tr>
<td>HbA1c control - diabetes</td>
<td>3,100-12,000</td>
<td>$1.3 - $1.7 billion</td>
</tr>
<tr>
<td>Breast Cancer Screening</td>
<td>500 - 1,900</td>
<td>$212 - $232 million</td>
</tr>
<tr>
<td>All Selected Quality Measures (Top 11)</td>
<td>38,000-88,900</td>
<td>$1.9 - $3.5 billion</td>
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</table>

Source: NCQA’s “State of Health Care Quality 2009”
Consider:

• Limitations of the RCT
  – Health Insurance Experiment

• The impact of research on policy discussions and public perception
  – Oregon Medicaid
“The philosophers have only interpreted the world in various ways; the point is to change it.”

-Karl Marx
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)
Models Used in CER

- 1. Practice based models
- 2. Community based models
- 3. Mediational models
- 4. Nation based models
Practice Based Models

• 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.
Community Based Models

- **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.
Community Based models

• **Health system factors**
• By using Donabedian’s paradigm 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.

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

**System factors**
- Health system structure.
- Disease management strategies.
  - Performance feedback.
  - Physician reminders.
  - Guideline use.
  - Formal case management.
  - Patient education resources.
- Management of referral care.
- Clinician payment, incentives.
- Cost-containment strategies.
- Data systems.

**Processes of care**
- Periodic HbA1c testing.
- Periodic lipid testing.
- Retinal examinations.
- Periodic microalbuminuria testing.
- Periodic foot examinations.
- Smoking cessation counseling.
- Aspirin prescription/advise.

**Health outcomes**
- Glycemic control.
- Blood pressure control.
- LDL-cholesterol control.
- Cardiovascular disease.
- Nephropathy/end stage renal disease.
- Retinopathy.
- Mortality.
- Health status.
- Symptoms.
- Utilization and costs.
Community Based Models 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.
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: Reference

Nation Based Models

• Often not included as “comparative effectiveness research”
• Can be viewed as extension of the other comparative effectiveness models
• Unit of observation extended beyond “practice”, beyond “community” to “nation”
• “Mediation” particularly important due to multiple levels for analysis and to explain why nations might differ in outcomes
Why do Nation based comparative effectiveness research?

• Can be used in health policy debate
• Can be used for strategy development at the national level
• National goals based on achievements in at least nation are at least reality based
• Can reveal successful elements of one system that might be applied to another and also generic system problems common to all
• Illustrates how culture history and economics prevent the application of some successful policies from one system to another.
The Original Health Systems Comparative Model (1960-1970)

**SYSTEM RESOURCES**
(national and local)

- Expenditures
- Personnel
- Facilities

**POPULATION CHARACTERISTICS**

- Predisposing
- Enabling
- Need

**OUTCOMES**

- Mortality

**Efficiency (1)**

**Equity (2)**

**SERVICES**

- Medical care

**Effectiveness (3)**
System Components of the Original Nation Based Model

- System Resources (national and local/amount and distribution)
- Population Characteristics (predisposing, enabling and need)
- Services (medical care)
- Outcomes (mortality)
Measures of System Performance in the Original Nation based Model

- Determined by linkage of system components
- Linkages show three types of performance:
  - **Efficiency (1)** medical care received/resources consumed
  - **Equity (2)** medical care received determined by needs of population
  - **Effectiveness (3)** medical care received reduces mortality rates
The Developed Health Systems Comparative Model (1970-2011)

* Additions emphasized in the Developed model marked with asterisk
• System resources: health policy/policy implementation
• Population characteristics: health beliefs, genetics, evaluated need
• Services: responsiveness, personal & public health practices
• Outcomes: morbidity, disability and functioning, satisfaction, quality of life
Additional Measures of System Performance in the Developed Nation Based Model

- **Equity (4)** System resources are distributed according to population needs

- **Equity (5)** Outcomes are independent of predisposing social and enabling population characteristics

- **Efficiency (6)** Positive outcomes are maximized per resource unit consumed
Conclusion: the Positive State of CER at the National Level

• Collection of comparable data on multiple countries by WHO, OECD, Eurostat, Commonwealth Fund, NOMESCO
• More comprehensive and sophisticated models
• More and improved measures of health care system components
• Advanced statistical analyses
• Inclusion of health policy and politics in the models
Conclusion: Continuing Challenges to CER at the National Level

- Expense of collecting international data and policies of some funding agencies against funding international work
- Gathering comparable data across countries
- Developing valid and reliable measures of comprehensive health care systems
- Determining the relative contribution of health care systems and other determinants of population health
- Political sensitivities of countries to ranking them on the health services and outcomes of their systems
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?