

University of Pittsburgh

ctsi

CLINICAL +
TRANSLATIONAL
SCIENCE
INSTITUTE



Institute for Clinical Research Education
Promoting Education and Research in Clinical and Translational Research

Patient-Centered Outcomes and Comparative Effectiveness Research



Douglas Landsittel, PhD

Primary Objectives:

- Comparative effectiveness research (CER)
 - What is it and why is it needed?
 - Where is it going?
- Patient-centered outcomes research (PCOR)
 - Evolution from CER
 - Where we are now
- Potential impact on medical practice

What's usually done in clinical research?

- Assign people to treatment (or observe)
 - Follow and collect outcomes
- Compare treatments to each other, placebo or practice as usual
- Statistical results
 - Estimate of the average treatment effect
- Disseminate study results
- Synthesize across studies

What is Comparative Effectiveness Research (CER)?

Slutsky and Clancy (*Am J Med Qual* 2009) define CER as

“Which treatment works best, for whom, and under what circumstances?”

Look familiar?

A little more about CER

The Institute of Medicine describes CER is...

- 2+ treatments
- Usual practice
- Harms and benefits
- Informs clinical decisions
 - Population and individual

Emphasis of CER – why is it different?

CER:

2+ treatments

Usual practice

Harms and benefits

Informs decisions

Population & individual

= effectiveness

Typical Clinical Trials:

Versus placebo

Ideal setting

Intermediate changes

May inform decisions

Average person

= efficacy

CER is not new, but the emphasis is very different.

Why do we need CER?

- Insufficient evidence
- Evidence is about the average patient
 - Exclusive population
- Uncontrollable costs
- Inconsistent approaches to healthcare

Problem of insufficient evidence

- IOM: “more than half of the treatments delivered today without clear evidence of effectiveness.”
- Highly prevalent conditions, like diabetes, lack evidence on the best treatment
 - NIH spends > \$1 billion/year in diabetes
 - “numerous reviews on ... type 2 diabetes have been published ... practitioners are often left without a clear pathway of therapy to follow.”
(Diabetes Care. 2006;29(8):1963-1972)

Rethinking Randomized Clinical Trials for Comparative Effectiveness Research: The Need for Transformational Change

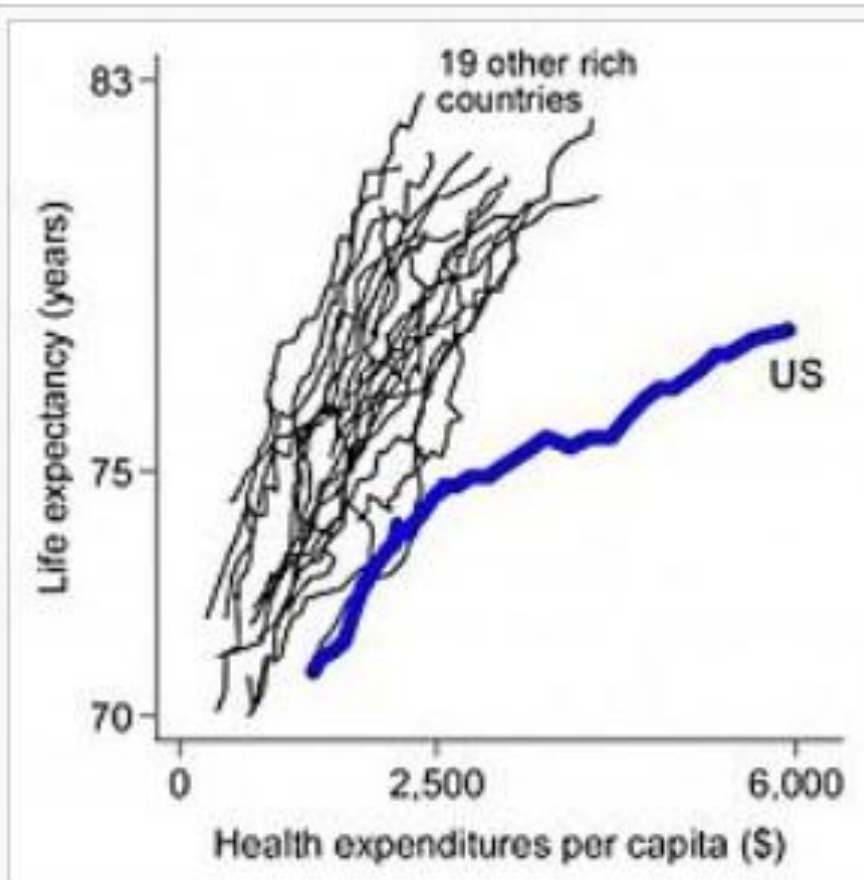
Bryan R. Luce, PhD, MBA; Judith M. Kramer, MD, MS; Steven N. Goodman, MD, MHS, PhD; Jason T. Connor, PhD; Sean Tunis, MD, MSc; Danielle Whicher, MHS; and J. Sanford Schwartz, MD

While advances in medical science have led to continued improvements in medical care and health outcomes, evidence of the comparative effectiveness of alternative management options remains inadequate for informed medical care and health policy decision making. The result is frequently suboptimal and inefficient care as well as unsustainable costs. To enhance or at least maintain quality of care as health reform and cost containment occurs, better evidence of comparative clinical and cost-effectiveness is required (1).

ENHANCING STRUCTURAL AND OPERATIONAL EFFICIENCY

As currently conducted, RCTs are inefficient and have become more complex, time consuming, and expensive. More than 90% of industry-sponsored clinical trials experience delayed enrollment (4). In a study comparing 28 industry-sponsored trials started between 1999 and 2002 with 29 trials started between 2003 and 2006, the time from protocol approval to database lock increased by a median of 70% (4).

Challenge of healthcare costs

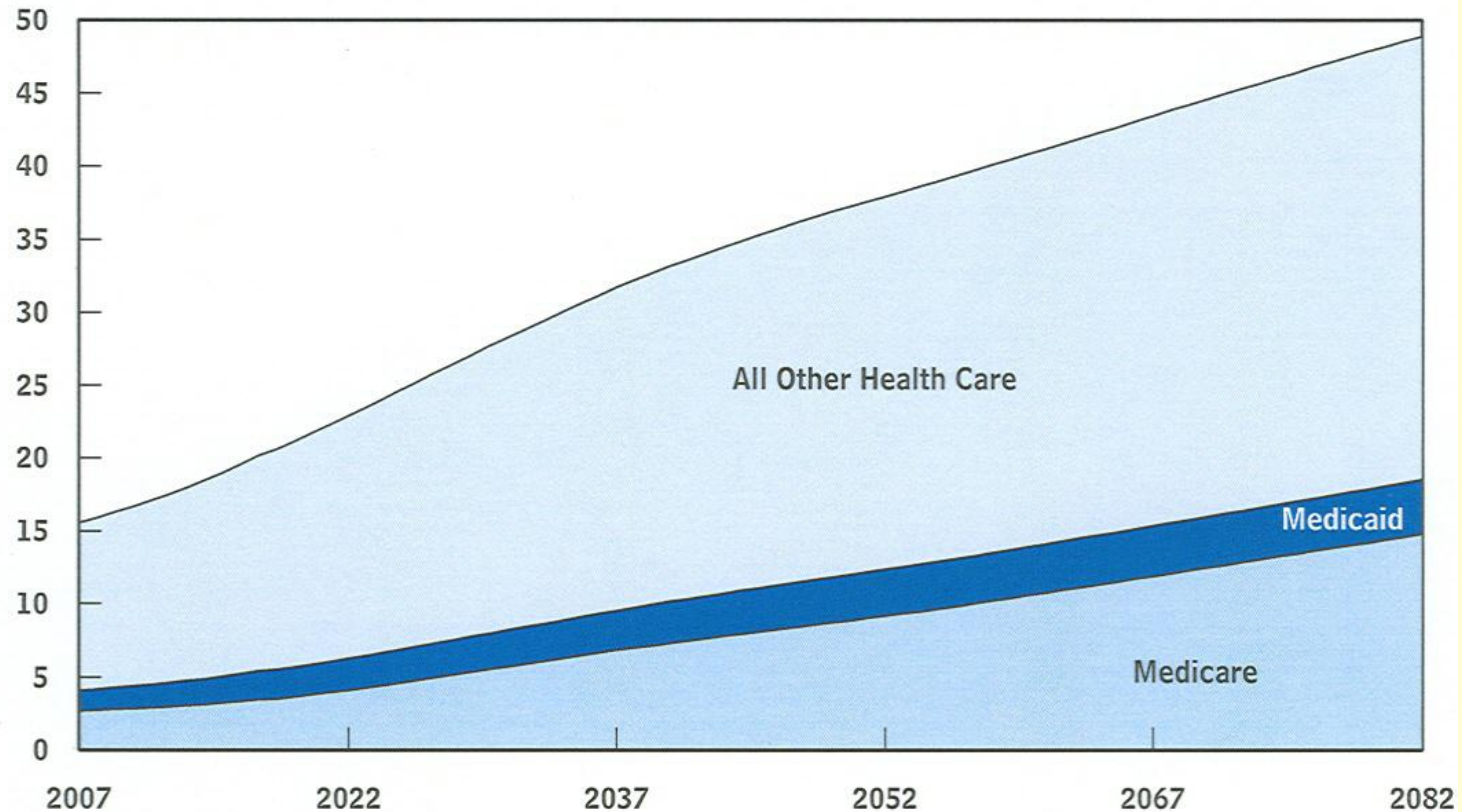


Life expectancy compared to healthcare spending from 1970 to 2008, in the US and the next 19 most wealthy countries by total GDP. ^[63]

Kenworthy, Lane (July 10, 2011). ["America's inefficient health-care system: another look"](#). *Consider the Evidence* (blog). Retrieved September 11, 2012.

Projected Spending on Health Care as a Percentage of Gross Domestic Product

(Percent)



Source: Congressional Budget Office.

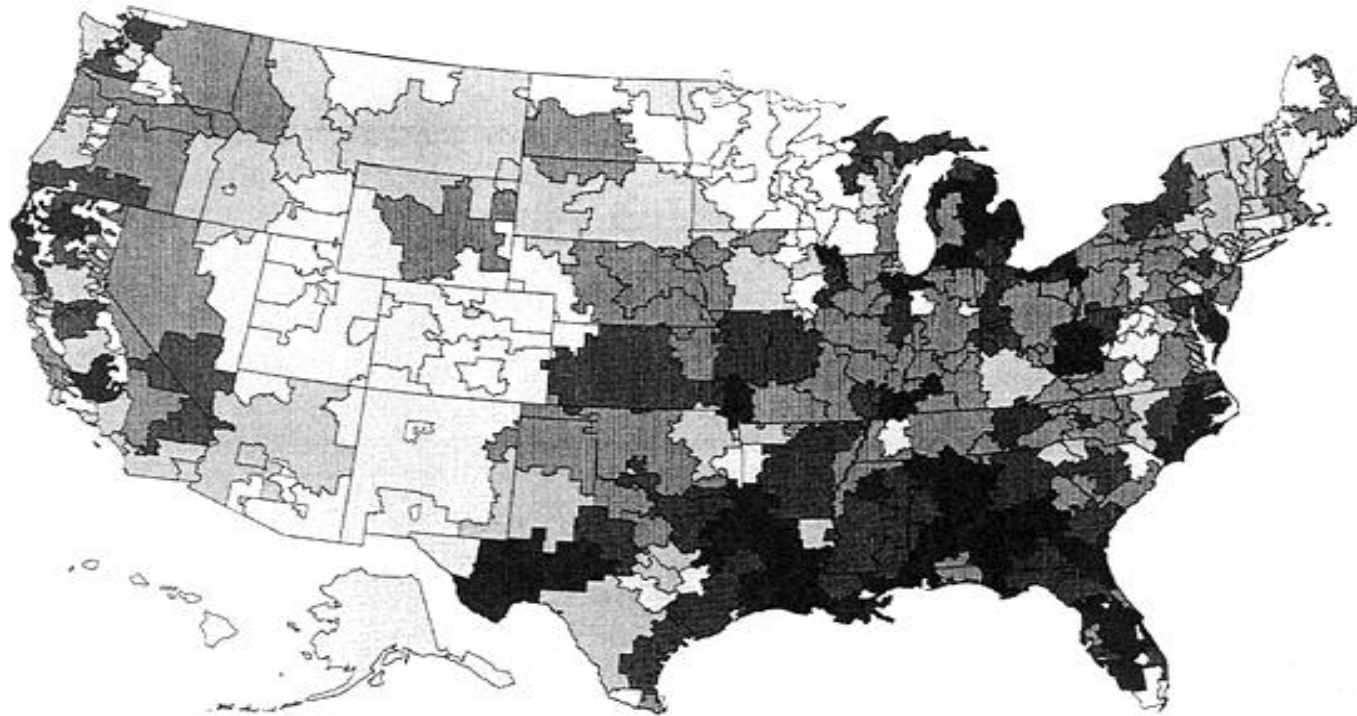
Note: Amounts for Medicare are net of beneficiaries' premiums. Amounts for Medicaid are federal spending only.

“Seriously, we basically have to solve the health cost problem, or nothing else matters.”

Paul Krugman

NY Times blog on restoring a healthy US economy,
September 28, 2009

Clinical practice varies by region



Ratio of HRR Rate of Carotid Endarterectomy to U.S. Average (1994-95)

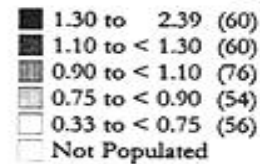


Fig 1. Map shows variation in rates of carotid endarterectomy across the 306 HRRs of the United States. (From the *Dartmouth Atlas of Health Care*.⁶)

Where is CER going?

- Recent momentum for CER
- Shift to patient-centered outcomes research
- Establishment of a PCOR institute

Momentum for CER

- In 2006 Senate takes up Healthcare reform
 - CER as tool to improve quality and efficiency
- In 2008 Obama calls CER “key to eliminating waste and missed opportunities”
 - Calls for an independent institute
- McCain backs CER and “the development of national standards for measuring and recording treatment and outcomes”

Building infrastructure for CER

- Institutes of Medicine
 - Prioritized 100 topics
 - Systematic review on healthcare
- American Recovery and Reinvestment Act
 - \$1.1 billion for CER
- Patient Protection and Affordable Care Act
 - Establishment of the PCOR Institute (PCORI)

Criticisms of CER and shift to patient-centered outcomes research

- CER may restrain innovation
- Potential connection to healthcare rationing
- Calls for an independent institute that separates coverage issues from the science (Wilensky, Health Affairs 2006)
- Focus on what matters most to the patient
- Input from stakeholders

The Patient-Centered Outcomes Research Institute (PCORI)

- Established by the PPACA
- Funded by the Treasury and a \$1 - \$2 fee on health insurance plans
- Prioritizes topics and outcomes that matter to patients and stakeholders
- Cannot use cost to measure effectiveness
- Potentially expires in 2019

Patient-centered outcomes research

Answers 4 questions:

1. “Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”
2. “What are my options and what are the benefits and harms of those options?”
3. “What can I do to improve the outcomes that are most important to me?”
4. “How can the health care system improve my chances of achieving the outcomes I prefer?”

(http://www.pcori.org/images/PCOR_Rationale.pdf)

Emphasis on big issues

“PCORI should plan its research agenda strategically, so that it addresses research questions that comparative effectiveness research could answer quickly and decisively. To date, the institute has not chosen this path. ... agenda that conveys a sense of urgency and strategic direction.”

H Sox. Health Aff. 2012 Oct;31(10):2176-82.

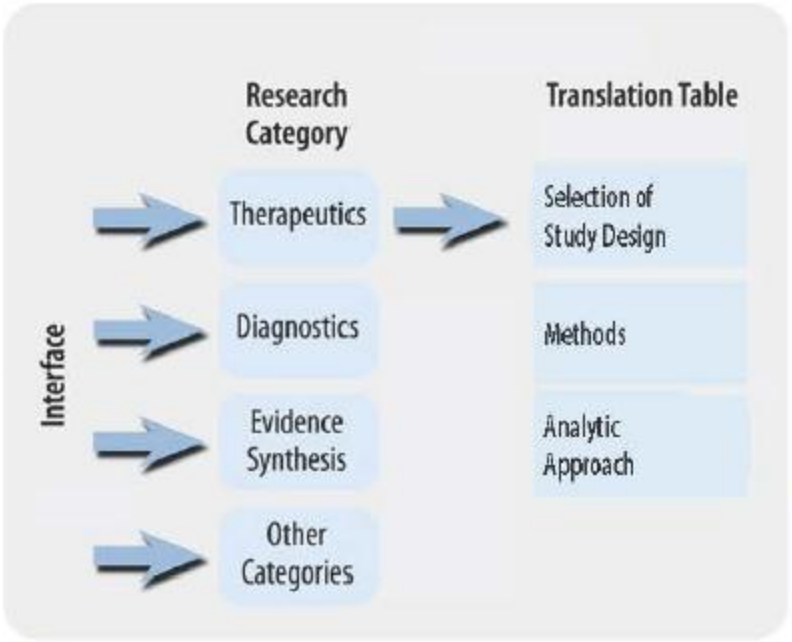
Where are we now?

- Concentrated focus on research that affects practice now
- Results that are directly interpretable for a given patient
- Urgency with PCORI potentially ending in 5 years

CER and PCOR should influence practice – doing so is a complex process.

Incorporate:

- Prior evidence
- Intent of research and decisions to be made
- Stakeholder perspectives



Specify elements:

- Patient population
- Intervention
- Comparator
- Outcomes
- Timing
- Setting



Prioritize study characteristics:

Intrinsic

- Internal validity (bias)
- External validity
- Precision
- Heterogeneity
- Ethical considerations
- Others

Extrinsic

- Timeliness
- Logistical and resource constraints
- Data availability, quality, and completeness
- Others

Impact on medical practice

- More available evidence
- Evidence on effectiveness more than efficacy
- Evidence that stresses the patient and the stakeholder (e.g. you!)
- More emphasis on evidence-based medicine and changing practice

Impact on medical practice

- Been more focus on high cost conditions and modalities
 - Imaging and diagnostics
 - Cardiovascular disease
- Overarching effect across healthcare
- Ultimately CER and PCOR must bend the cost curve

Finish with two questions:

- How might your questions, about selecting the most effective dental procedures, relate to CER and PCOR?

And...

- How is your perspective different from potential research studies, and/or patient perspectives?