

# The Rocky Road from Efficacy to Effectiveness: New Research Directions in CAM in the US.

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## CER

Comparative Effectiveness Research (CER)

IOM DEFINITION

Effectiveness Research (CER)

Comparison of effectiveness of interventions among patients in a typical patient care setting with decisions tailored to individual need.

- > Pragmatic trials (as opposed to explanatory)
- > Head to head trials

What is CER



## Efficacy Vs Effectiveness

Efficacy Tests a therapy under ideal conditions using the RCT. But practice ultimately needs therapy that works under normal practice i.e. effectiveness studies. A therapy that has efficacy may not be effective and those of equal efficacy may not have equal effectiveness. Effectiveness must take into account the total health encounter and must be grounded in what actually occurs in the encounter.

## NIH

The American Recovery and Reinvestment Act of 2009 (generally referred to as the stimulus package), has allocated \$1.1 billion “down payment” to fund comparative effectiveness research

So that is real money



So how is this money to be spent?

\$300 million for the Agency for Healthcare Research and Quality (AHRQ), \$400 million for the National Institutes of Health, and \$400 million for allocation at the discretion of the Secretary of Health and Human Services



### Agency for Health Research Quality

Effective Health Care Program whose purpose is to fund research that provides reliable and practical data that can inform decisions in clinical practice.

AHRQ



### National Center for Complementary and Alternative Medicine

Request for research proposals for Comparative Effectiveness Studies of Complementary and Alternative Medicine.

NCCAM

Observational studies or secondary data analyses to compare the effectiveness or cost-effectiveness of:

- 1) CAM used in addition to standard conventional care
- 2) CAM or integrative health care versus standard conventional care
- 3) one CAM therapy to another



### The Good

- The move away from privileging RCTs above all other evidence
- Recognition that RCTs do not answer questions of effectiveness
- Placing the interests of patients and providers above or equal to that of scientists
- A recognition of the role of observational data
- Solves some of the ethical issues around RCTs
- Solves some of the methodological challenges of RCTs in CAM
- Average patients with average providers in average clinic
- Moves us towards whole systems research

Puts the "P" back into "EBP"



### The Bad

- Still a bit focused on "trials"
- It is not real clear if much of CAM will meet the inclusion criteria
- Not clear if it includes adjunctive therapy



### The Ugly

- Does not resolve the ethical challenges e.g. in the areas of CAM and cancer
- Does not go far enough
- Does not get at the health encounter



So what can we do?

- 1) Health Services research on effectiveness
- 2) Observation studies



### Health Services Research

**“Investigation of the relationship between social structure, process and outcomes for personal health services, a transaction between a client and a provider to promote health”**

**Institute for Medicine 1979. HSR focuses on the total organization of health care.**

### Heath Services Research

- Links structure, process & outcomes
- Measure quality of care
- Evaluates access, cost, utilization & services
- Measures health care need and risks
- Determines patient values & satisfaction & HRQOL
- Determines appropriateness of care
- Determines effectiveness of care
- Uses methods beyond RCTs – e.g. program evaluation
- Is multi-disciplinary

HSR with its focus on linking outcomes to structure and process, its work on cost and cost benefit introduces a badly needed dose of realism into the EBP movement.

### Observation Studies

<p><b>Stroup et al.</b> “an effectiveness study using data from an existing data base, a cross sectional study, a case series, a case control design, a design with historical controls, or a cohort design”</p>	<p><b>Challenges</b></p> <ol style="list-style-type: none"> <li>1. No randomization</li> <li>2. Cannot measure efficacy</li> <li>3. Cannot assess bias</li> <li>4. Cannot be pooled for analysis</li> </ol>
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### Sociological Anthropological Observation Studies

- Participant observation studies
- Rapid ethnographic observation
- Contextual analysis
- Social/cultural context
- Negotiation
- Meaning
- Health Encounter as the unit of analysis and as a contributor to outcomes
- Provide understanding for effectiveness
- Chiropractic HSR vs Social science observation

### Conclusion

- The money is great
- The focus is better
- The promise is good but we are still not sure about the delivery
- On paper it is good news for CAM/IM researchers
- But it needs to go further



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