From Efficacy to Comparative Effectiveness: Is This the Way Forward for Complementary and Alternative Medicine?
Ian D. Coulter Ph.D.

Professor, UCLA
RAND/Samueli Chair in Integrative Medicine &
Senior Health Policy Scientist,
RAND Corporation;
Adjunct Research Faculty, Southern California University of Health Sciences
Comparative Effectiveness Research

IOM DEFINITION (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
National Center for Complementary and Alternative Medicine

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

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
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.
The Problem

The Provider
Evidence means what works well for me in my practice. Clinical experience is the basis for deciding this.

The Researcher
Evidence means both what has efficacy & why and clinical experience is a very problematic source for this.
The Problem

The disconnect between research and practice. The research can be both rigorous and clinically useful, unfortunately often that which is rigorous (RCTs) is not useful and that which is useful (observation studies) is seldom rigorous.
CAM and the Challenge of Efficacy Studies

IOM Report 2005 CAM in the US

1. Bundles of therapies
2. Precise descriptions
3. Individualized treatment
4. Unique characteristics of the healer
5. Role of expectation effects and placebo
6. End points difficult to measure
7. Lack of professional boundaries
8. Ethical issues
“The characteristics of CAM therapies and modalities make it difficult to apply the traditional RCTs or treatment effectiveness studies used in conventional medicine.”
Two Paradigms: CAM /Biomedicine

These are two distinct constructions of reality, different & opposing views about illness, health, health care & healing. They are alternative philosophical paradigms.
The Metaphysics of CAM

- Vitalism/spiritualism - the healing power of nature, Taoist, Hindu, Buddhist, Theosophy, Metaphysics (chi, UI, dosha)
- Holism - mind, body, & spirit, non-reductionist
- Naturalism - the body is built on nature’s order, we should look to nature for the cure
The Philosophy of Health & Health Care in CAM

- Health is the natural state, the innate tendency of the body is to restore health, homeostasis (*vis medicatrix naturae*)
- Health is the expression of body, mind and spirit
- Health is unique for each person
- Health comes from within

- Disease vs. illness (dis-ease)
- Health is not just the absence of disease
- Treatment is not equal to care
- Treat the whole person
- The healer is a facilitator and an educator- “I can no more give you health than I can give you honesty”
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
The Weakness of CER

1. To the extent the provider is free to do what they want, it is difficult to know what was done

2. To the extent we do not know what was done we do not know what contributes to the outcome

3. To the extent we do not know what was done we do not know what to replicate or how to do so
Descriptive Studies of CAM

In the case of CAM we lack a body of descriptive studies that would tell us what the treatment & the health encounter includes. We are not even sure about how we might collect such data or what is important to collect. This is not just the therapy but the whole health encounter and the context.
### Solutions-Observation Studies

<table>
<thead>
<tr>
<th>Stroup et al.</th>
<th>Challenges</th>
</tr>
</thead>
<tbody>
<tr>
<td>“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”</td>
<td>1. No randomization</td>
</tr>
<tr>
<td></td>
<td>2. Cannot measure efficacy</td>
</tr>
<tr>
<td></td>
<td>3. Cannot assess bias</td>
</tr>
<tr>
<td></td>
<td>4. Cannot be pooled for analysis</td>
</tr>
<tr>
<td></td>
<td>5. Measures the wrong things</td>
</tr>
</tbody>
</table>
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

**HSR**
- Musculoskeletal specialists
- Narrow scope
- Manipulation
- Back problems

**Ethnographic Observations**
- Holistic
- Broad scope
- Wellness practitioners
Observation of practices

Descriptive research

• What are they doing?
• To whom are they doing it?
• What are they doing it for?
• When are they doing it?
• How often are they doing it?
• What results do they get from doing it?
• What settings are they doing it in?
• What are the features of the encounter?
IKEA HAS ANNOUNCED IT'S INTENTION TO TAKE OVER GM, AND TO SELL CARS.
Dedicated to Sir David Low and COLONEL BLIMP

“Gad, sir, reforms are all right as long as they don't change anything.'
References

• Zwarenstein M, Treweek S. What kind of randomized trials do we need? CMAJ May 12, 2009; 180(10): 998-1000.
References


