The Role of Qualitative Methods in Comparative Effectiveness Research: New Paradigms for Research in the US Health Care System

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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**

CER

Explanatory	Practical/Pragmatic
Hypothesis driven and usually done with the hope of revealing the biological effect of a treatment or causal connections	Address practical questions about the risks, benefits, costs of an intervention as they would occur in routine clinical practice.

Institute Of Medicine

"CER is the 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 individual and population levels."

CER

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

Patient Protection and Affordable Care Act

SEC. 6302. FEDERAL COORDINATING COUNCIL FOR COMPARATIVE EFFECTIVENESS RESEARCH.

Notwithstanding any other provision of law, the Federal Coordinating Council for Comparative Effectiveness Research established under section 804 of Division A of the American Recovery and Reinvestment Act of 2009 (42 U.S.C. 299b–8), including the requirement under subsection (e)(2) of such section, shall terminate on the date of enactment of this Act.

EVIDENCED BASED PRACTICE

“the conscientious, explicit and judicious use of the current best evidence in making decisions about the care of individual patients ….means integrating clinical expertise with the best available external clinical evidence from systematic research”

Sackett 1998.

TRADITIONAL PRACTICE

Emphasis is placed on accumulated knowledge and experience, adherence to accepted standards and the opinion of experts and peers.

“practical, prudent, personal”

Niedrman, Badovinac 1999

Research and EBP

In science the researchers are going about this in several ways

1. **Basic research-mechanisms,processes, structures**
2. **Clinical research- efficacy, clinical trials (RCTs)**
3. **Health Services Research- effectiveness, costs, appropriateness, epidemiology**
4. **Behavioral Science (social sciences)**
5. **Systematic reviews-meta-analysis**

Hierarchy of Evidence

1. **Meta-analysis**
2. **Evidence from at least one well designed RCT**
3. **Evidence from a control trial that is not randomized**
4. **Evidence from a well design cohort or case controlled**
5. **Evidence from a multiple time series**
6. **Descriptive studies**
7. **Case studies**
8. **Opinions of experts**

The Good

1. **EBP and systematic reviews have forced the health professions to take note of the totality of the literature**
2. **Systematic reviews have transparency.**
3. **They clear the undergrowth away to highlight where the evidence is problematic, where there are gaps in the evidence, and where practice is clearly at odds with good evidence.**
4. **Isolating the state of the science (the deplorable state)**
5. **Stop the perpetuation of myths instead of medicine**
6. **They cannot be ignored & force a debate about both the evidence and the practice.**
7. **Those opposed to the findings are forced to challenge them.**

The Challenge

Despite great hopes for EBP there is little evidence that it results in better outcomes for patients.

Despite great hopes for EBP there is scant evidence that those educated in EBP in fact practice better medicine.

The Bad: Problems with RCTs

- Test therapy under ideal conditions
- Homogeneous populations
- Ethical issues may prevent some participating e.g. high risk patients
- Very low risk patients may not be included – need too big a sample
- Lack soft data needed for practice
- Provide average results

The Bad: 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/Patient

Evidence means what works well for me. Clinical experience and patient outcomes is the basis for deciding this.

The Researcher

Evidence means both what has efficacy & why (mechanisms) and clinical experience is a very problematic source for this.

Effectiveness and EBP

If we include effectiveness studies in EBP (real patients in real practices with real providers) then we have to focus on the impact of the health encounter and this involves all the elements in that encounter most of which are not captured by standard empirical methods and that requires qualitative methods.

The Problem

The disconnect between research and practice. Research can be both rigorous and clinically useful, unfortunately that which is rigorous is not useful and that which is useful is seldom rigorous. Quite frankly too little of scientific research is clinically useful (or even clinically oriented.)

Putting the P back into EBP

- Observation studies
- Include sub populations
- Include real practice
- Provide effectiveness not just efficacy
- Reject the privileging of RCTs over all other forms of evidence

Patient Protection and Affordable Care Act

FEDERAL COORDINATING COUNCIL FOR COMPARATIVE EFFECTIVENESS RESEARCH

The Center for Quality Improvement & Patient Safety. Research Functions of the Center :

Part II, Part S- National Strategy For Quality Improvement in Health Care

(iii) Address gaps in quality, efficacy, comparative effectiveness information and health outcome measures and data aggregation techniques

Subpart II- Health Care Quality Improvement Programs

SEC.993 Health Care Delivery Systems Research

(E) Support the discovery of processes for reliable, safe, efficient and responsive delivery of health care taking into account discoveries from clinical research and comparative effectiveness research

- **Patient-Centered Outcomes Research Institute (PCORI)**

Funding of comparative effectiveness research; systematic reviews; RCTs; any other methodologies recommended by the methodology committee.

PCORI

2010-2012, PCORI funding will amount to $210 million from general revenues; for 2013, PCORI funding will be general revenues of $150 million plus an annual $1 fee per Medicare beneficiary transferred from the Medicare Trust Fund plus a $1 fee per individual assessed on private health plans; for 2014-2019, PCORI funding will be general revenues of $150 million plus a $2 fee per Medicare beneficiary plus a $2 fee per privately insured individual. By 2015, total annual funding for the Institute will reach nearly $500 million

Patient-Centered Outcomes Research Institute PCORI

PUBLIC LAW 111–148—

MAR. 23, 2010

The terms ‘comparative clinical effectiveness research’ and ‘research’ mean research evaluating and comparing health outcomes and the clinical effectiveness, risks, and benefits of 2 or more medical treatments, services, or products.

Comparative Effectiveness Research

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**

“ The popular belief that only randomized, controlled trials produce trustworthy results and that all observational studies are misleading does a disservice to patient care, clinical investigations, and the education of health care professionals.” Concato, Shah and Horwitz 2000

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

Challenges

1. The comparability of the two groups before any intervention or exposure has occurred
2. Self selection means that characteristics of the patient determines who gets what therapy
3. Perfect matching is seldom possible and it assumes that the variables you matched on and not some other unknown variables are the important ones to control for

Challenges

4. Is the individual really a member of the group to which they are allocated (correctly diagnosed)
5. Control for other important variables

inequalities in susceptibility

6. You are not able to establish causation. They may establish correlations between outcomes and events, exposures etc. but not causation.
7. How do you know what was done, how do you replicate it?

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

Qualitative Observation Studies

Descriptive research

- **What are providers 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 it ?**

Chiropractic HSR vs. Social science Observation

HSR

- Musculoskeletal specialists
- Narrow scope
- Manipulation
- Back problems

Ethnographic Observations

1. Holistic
2. Broad scope
3. Wellness practitioners

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

Challenges

A negative outcome (effect) in an explanatory trial is thought to be a fatal blow since if there is no effect when the trial is done under ideal conditions it is highly unlikely that it would hold under less ideal conditions. A positive effect in an explanatory trial still leaves you with uncertainty about what would happen under less ideal conditions. In pragmatic trials however a negative effect leaves you unclear about whether it might work under more ideal conditions and a positive effect can inform you about how it works under normal conditions (effectiveness).

Complementary and Alternative Medicine (CAM) and the Challenge of Efficacy Studies

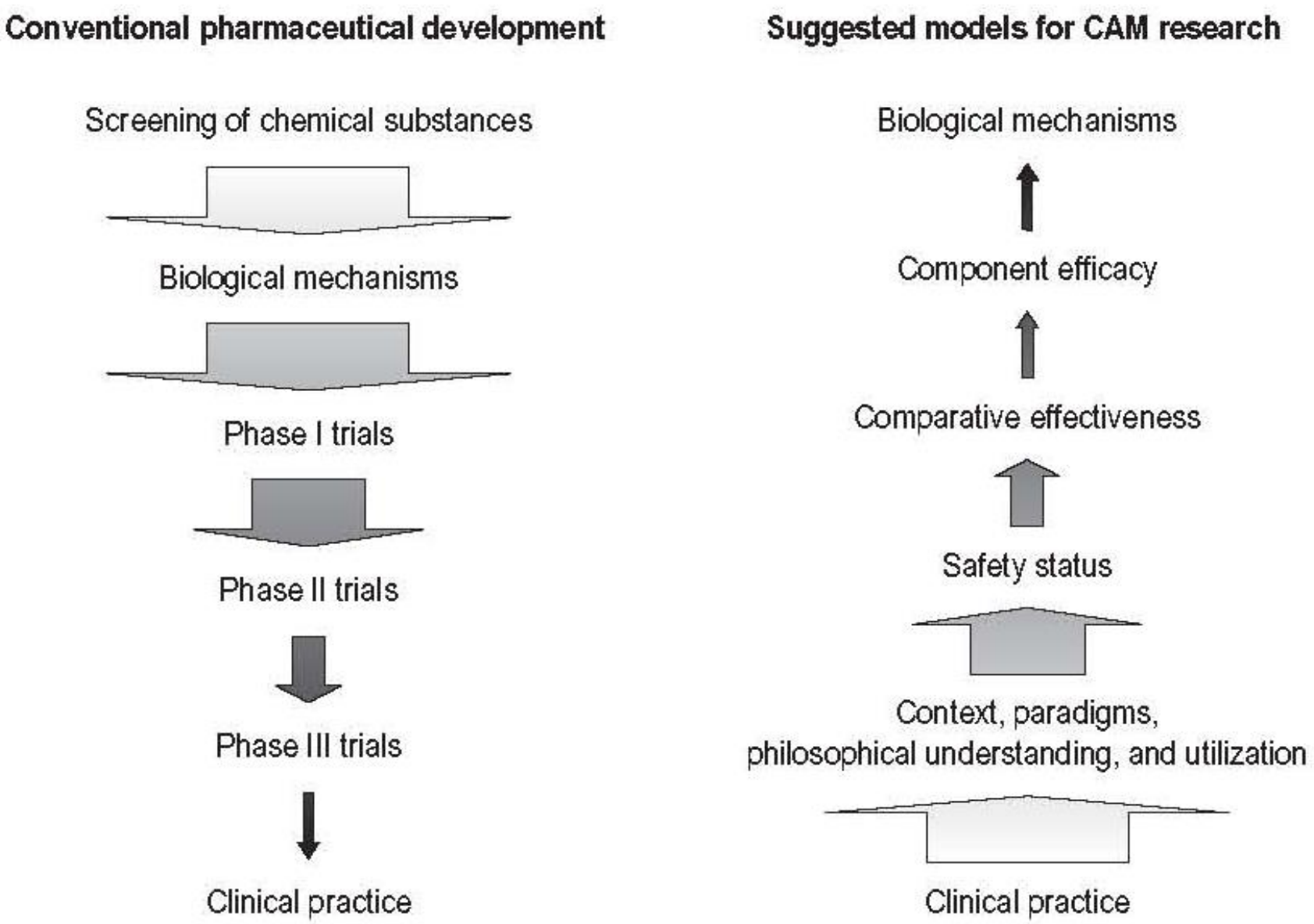
IOM Report 2005 CAM in the US

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

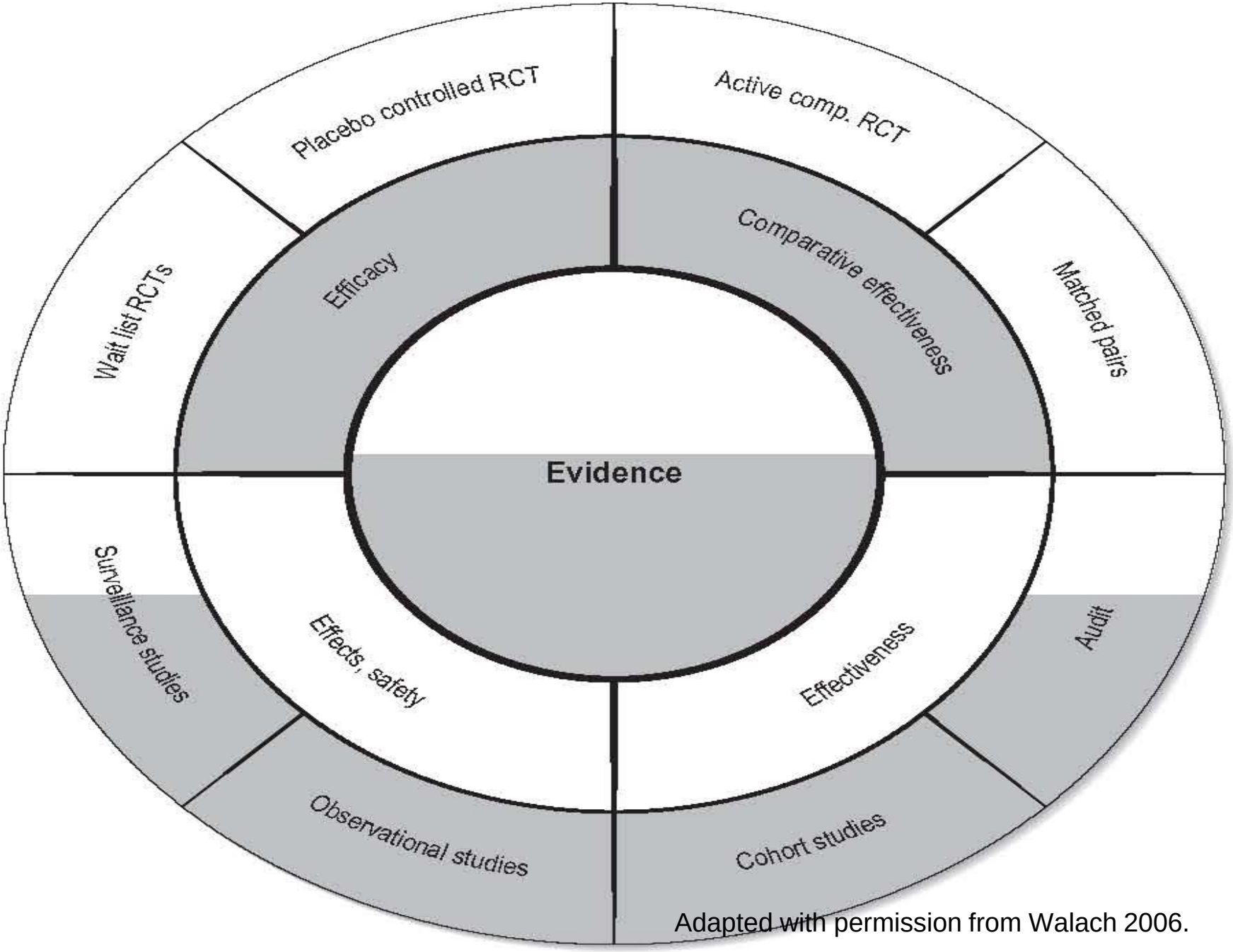
New Research Paradigm

- **Practice-centric research**
- **Patient-centric research**
- **Different model of evidence in which primacy is given to effectiveness not efficacy**
- **Different research methods**

The Reverse Phases Model

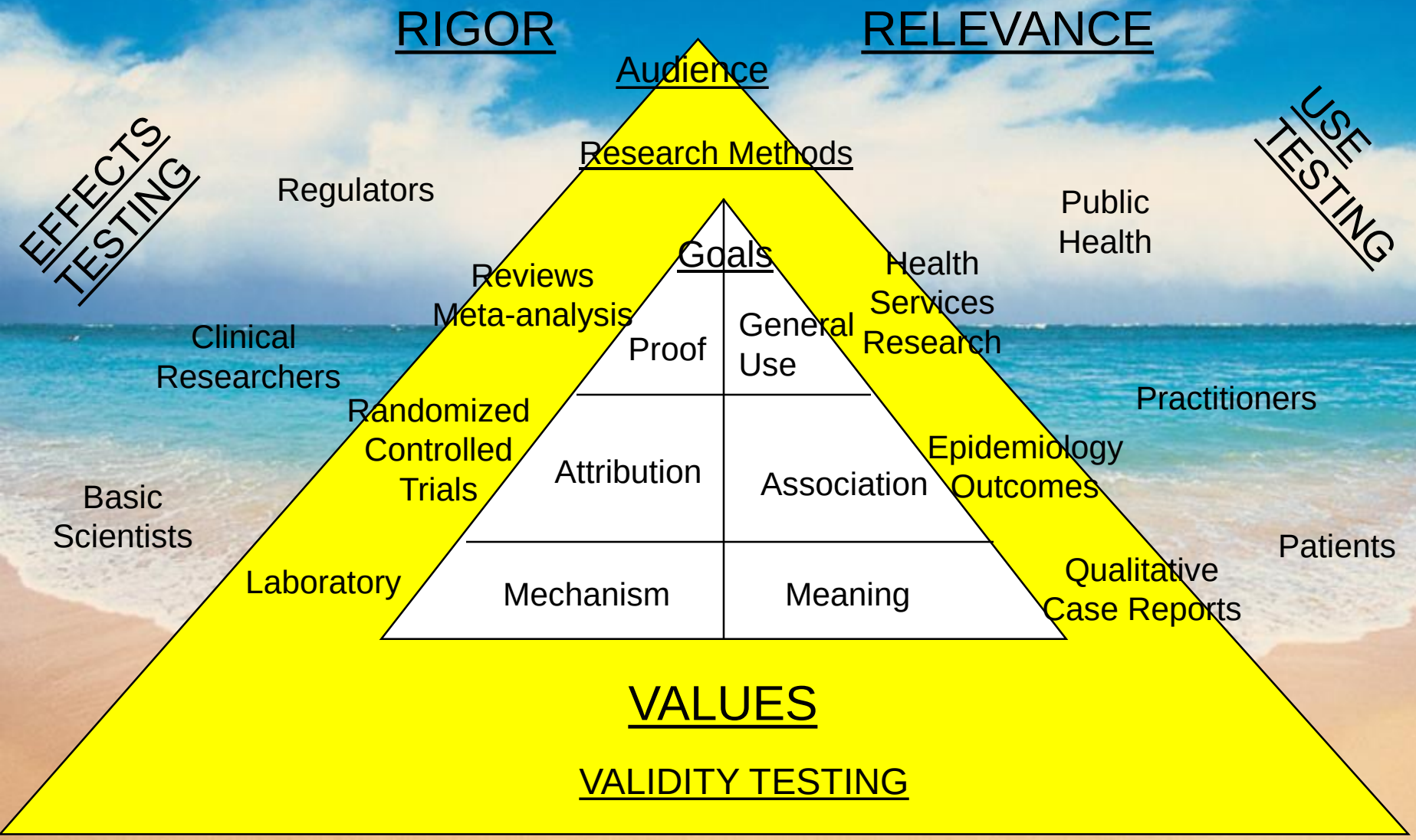


A Circular Model

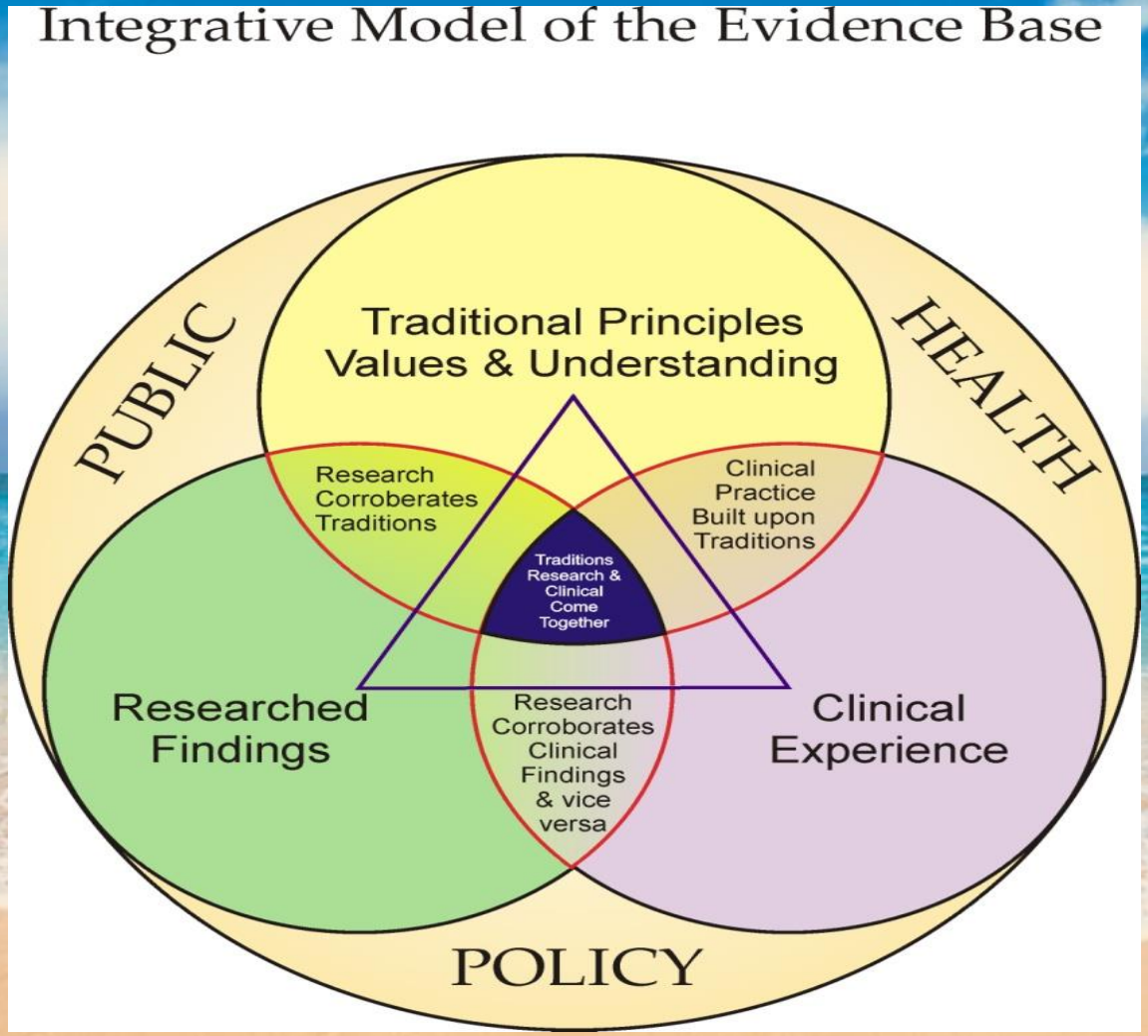


Adapted with permission from Walach 2006.

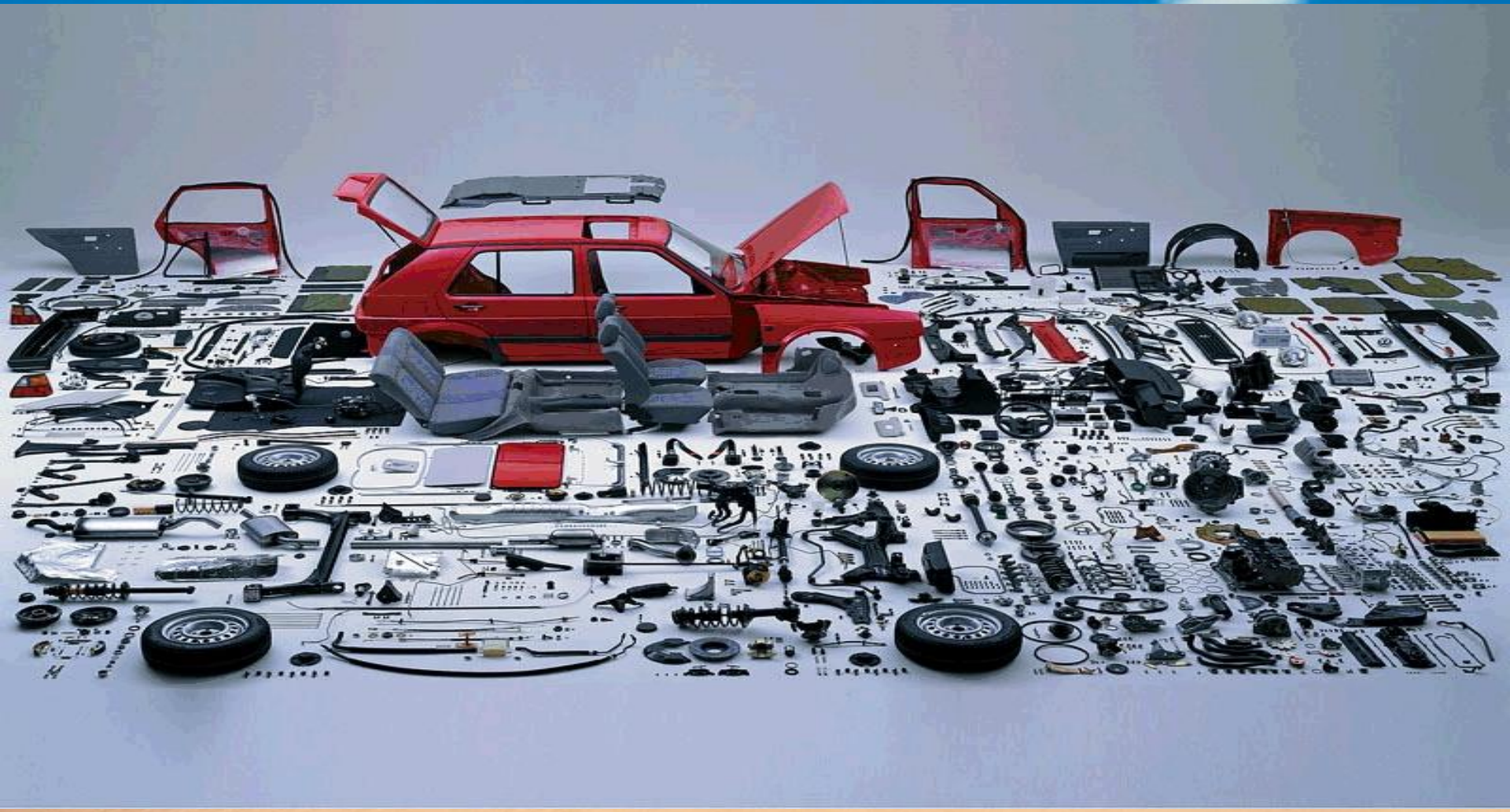
The Evidence House-W.Jonas



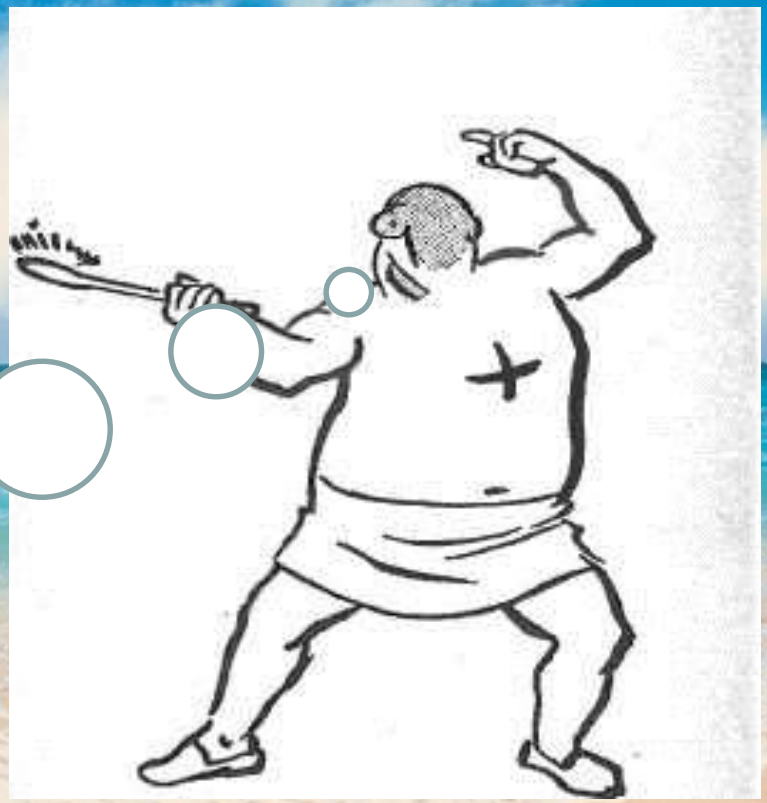
Phillip Cottingham -Wellpark College of Natural Therapies 2012



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.
- Luce BR, Kramer JM, Goodman SN, Connor J, Tunis S, Whicker D, Schwartz S. Rethinking Randomized Clinical Trials for Comparative Effectiveness Research: The Need for Transformational Change. Annals of Internal Medicine, August 4, 2009; 151(3): 206-209.
- Thorpe KE, A Pragmatic-Explanatory Continuum Indicator Summary (PRECIS). Dalla Lana School of Public Health. May 8, 2009.
- Sox H., Greenfield S. Comparative Effectiveness Research: A Report From the Institute of Medicine. Annals of Internal Medicine, 2009; 151: 203-205.
- Chiappelli F, Cajulis O, Newman M. Comparative Effectiveness Research in Evidence-Based Dental Practice. J Evid Base Dent Pract 2009; 9: 57-58.

References

- **Coulter ID**. Putting the practice back into evidence-based dentistry. *CDA Journal* 2007;35(1):45-9.
- **Coulter ID,** Khorsan R. Is Health Services Research the Holy Grail of CAM Research? (Review Article) Alternative Therapies Health Med 2007. Altern Ther Health Med. 2008 July/Aug;14(4):40-5.
- **Coulter ID**. Competing Views of Chiropractic: Health Services Research versus Ethnographic Observation. Chapter 3 IN: Oths KS, Hinojosa SZ (eds). *Healing by Hand*. *Manual Medicine and Bonesetting in Global Perspective.* AltaMira Press: Walnut Creek, CA, 2004.
- Linde K, **Coulter ID.** Systematic reviews and meta-analyses. IN Lewith G, Jonas W, Walach H (Eds.) Clinical Research in Complementary Therapies 2c. Elsevier; Oxford, England (In Press).
- **Coulter ID**. Comparative Effectiveness Research: Does the Emperor Have Clothes? Alternative Therapies in Health and Med 2001;17(2):8-15