Outsider Perspectives on the US Comparative Effectiveness Research Movement:
Real Progress or Real Disappointment?

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Panelists
- David Thompson, PhD (Moderator)
  - Executive Vice President & Senior Scientist, OptumInsight, Boston
- Michael Drummond, DPhil
  - Professor of Health Economics, Centre for Health Economics, University of York
  - Principal Consultant, OptumInsight
- Prof Federico Augustovski, MD, MSc, PhD
  - Director, Health Technology Assessment and Health Economic Dept, Institute for Clinical and Economic Effectiveness (IECS), Buenos Aires, Argentina
  - Professor of Public Health, Universidad de Buenos Aires
- Don Husereau, BScPharm, MSc
  - Adjunct Professor, Faculty of Medicine at the University of Ottawa
  - Senior Scientist, University for Health Sciences, Medical Informatics & Technology, Tirol, Austria
  - Formerly, Senior Advisor, Canadian Agency for Drugs and Technologies in Health, Ottawa, Canada
Panel Objectives

• Examine alternative definitions of CER and consider impact of Patient Centered Outcomes Research Institute (PCORI) with its emphasis on “patient-centeredness”

• Consider perspectives on CER from ex-US health policy experts:
  – Canada
  – Europe
  – Latin America

• Assess strengths & limitations of CER in terms of extent to which it can facilitate value-based decision making in US health-care system

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Defining CER …

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<tr>
<th>Organization</th>
<th>Definition</th>
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<tr>
<td>Congressional Budget Office</td>
<td>A rigorous evaluation of the impact of different options that are available for treating a given medical condition for a particular set of patients. Such a study may compare similar treatments, such as competing drugs, or it may analyze very different approaches, such as surgery and drug therapy. The analysis may focus only on the relative medical benefits and risks of each option, or it may also weigh both the costs and the benefits of those options. In some cases, a given treatment may prove to be more effective clinically or more cost-effective for a broad range of patients, but frequently a key issue is determining which specific types of patients would benefit most from it. Related terms include cost-benefit analysis, technology assessment, and evidence-based medicine, although the latter concepts do not ordinarily take costs into account.</td>
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TABLE 2-1 Definitions of CER

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<tr>
<td>Department of Health and</td>
<td>The comparison of one diagnostic or treatment option to one or more others. In this respect, primary comparative effectiveness research involves the</td>
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<td>Human Services (US)</td>
<td>direct generation of clinical information on the relative merits or outcomes of one intervention in comparison to one or more others, and</td>
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<td>secondary comparative effectiveness research involves the synthesis of primary studies to allow conclusions to be drawn. Secondary comparisons of</td>
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<td>the relative merits of different diagnostic or treatment interventions can be done through collective analysis of the results of multiple head-</td>
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<td>to-head studies, or indirectly, in which the treatment options have not been directly compared to each other in a clinical evaluation, and</td>
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<td>inferences must be drawn based on the relative effect of each intervention to a specific comparison, often a placebo.</td>
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TABLE 2-1 Definitions of CER

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<td>Medicare Payment Advisory Commission</td>
<td>Comparative-effectiveness analysis evaluates the relative value of drugs, devices, diagnostic and surgical procedures, diagnostic tests, and medical services. By value, we mean the clinical effectiveness of a service compared with an alternative. Comparative-effectiveness information has the potential to promote care of higher value and quality in the public and private sectors.</td>
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| Initial National Priority | Comparison of the impacts of different options for caring for a medical condition for a defined set of patients. The comparison may be between similar treatments, such as competing prescription medications, or for very different treatment approaches, such as surgery or radiation therapy. Or, the comparison may be between using a specific intervention and its routine (sometimes called watchful waiting). This report uses the terms "effectiveness," "clinical effectiveness," and "comparative effectiveness" interchangeably. |

Committee on Comparative Effectiveness Research Committee on Comparative Effectiveness Research Promulgation Based on Health Care Systems INSTITUTE OF MEDICINE OF THE NATIONAL ACADEMS THE NATIONAL ACADEMS PRESS Washington, D.C. www.iom.edu
## Defining CER …

### Table 2-1 Definitions of CER

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<td>Agency for Healthcare Research and Quality</td>
<td>Comparative effectiveness research that compares the results of one approach for managing a disease to the results of other approaches. Comparative effectiveness usually compares two or more types of treatment, such as different drugs, for the same disease. Comparative effectiveness also can compare types of surgery or other kinds of medical procedures and tests. The results often are summarized in a systematic review. The direct comparison of existing healthcare interventions to determine which work best for which patients and which pose the greatest benefits and harms. The core question of comparative effectiveness research is which treatment works best, for whom, and under what circumstances.</td>
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## Institute of Medicine Definition of CER …

**IOM National Priorities Committee Definition**

Proceeding from the definitions in Table 2-1 and the preceding considerations, the committee developed the following working definition of CER to guide its deliberations:

Comparative effectiveness research (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 the individual and population levels.
EBM, HTA & CER: Overlapping Terminology …

Patient Protection & Affordable Care Act, 2010

"(e) The Patient-Centered Outcomes Research Institute established under section 1181(b)(1) shall not develop or employ a dollars-per-quality adjusted life year (or similar measure that discounts the value of a life because of an individual’s disability) as a threshold to establish what type of health care is cost effective or recommended. The Secretary shall not utilize such an adjusted life year (or such a similar measure) as a threshold to determine coverage, reimbursement, or incentive programs under title XVIII."
PCORI’s Take on Things...

Patient-Centered Outcomes Research (PCOR) helps people and their caregivers communicate and make informed health care decisions, allowing their voices to be heard in assessing the value of health care options. This research answers patient-centered questions such as:

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 potential benefits and harms of those options?"
3. "What can I do to improve the outcomes that are most important to me?"
4. "How can clinicians and the care delivery systems they work in help me make the best decisions about my health and healthcare?"

To answer these questions, PCOR:

- Assesses the benefits and harms of preventive, diagnostic, therapeutic, palliative, or health delivery system interventions to inform decision making, highlighting comparisons and outcomes that matter to people;
- Is inclusive of an individual’s preferences, autonomy and needs, focusing on outcomes that people notice and care about such as survival, function, symptoms, and health-related quality of life;
- Incorporates a wide variety of settings and diversity of participants to address individual differences and barriers to implementation and dissemination; and
- Investigates (or may investigate) optimizing outcomes while addressing burden to individuals, availability of services, technology, and personnel, and other stakeholder perspectives. Note: this sentence was updated on 4/20/2012. The revision is explained below.
PCORI's Take on Things …

Comment: The definition should clarify that PCOR and CER are compatible; the definition should be about CER.

Response: No change is recommended.

The definition of PCOR includes many components of CER but is intended to be broader to also include other focuses and other research methodologies.

PCORI's Take on Things …

Comment: Comments related to cost, resources, value are not appropriate for the definition to include optimizing outcomes/addressing burdens/resources. Alternatively, comments also suggested that the definition should focus on costs but should not make reference to value.

Note: The following section was updated on April 20, 2012 based on stakeholder feedback to clarify wording that some might have found not to accurately reflect PCORI’s intent in the original revision.

Response: Proposed revision to response: A change to the definition is recommended, removing the phrase “resources” and substituting the phrase “availability or services, technology, and personnel” in this part of the definition.

The original definition did not mention “cost”, but several comments assumed that the term “resources” was being used to reflect costs. Resources refers to healthcare infrastructure, workforce, supplies and services. It is important to understand how the availability of these resources may influence clinical outcomes for patients as they consider different treatment strategies and make treatment decisions.

For example, when comparing strategy A (which involves a new technology, requires highly specialized clinicians to execute, and uses supplies that are in limited production) to strategy B (existing technology, not requiring specialists to execute and without limited supplies), PCORI should include studies that take into account the availability of the new technology, workforce capable of offering that technology and supply limitations. This resource availability may have direct bearing on how, from a patient's perspective, the two strategies compare.
Outsider Perspectives on CER: Real Progress or Real Disappointment?

• From the perspective of HTA in your region, what are your thoughts on the US CER movement?

• There’s lingering disagreement about the role of costs in CER—where do you stand on this?

• Does CER constitute a step in the right direction for the US, or is it a smokescreen that allows us to continue to avoid the value for money issue?

• What about the influence of PCORI—is “patient-centeredness” crucial in the era of personalized medicine?

• Should CER be considered a building block to more rigorous analyses of cost-effectiveness and value for money?

Thank You.

Contact Info:
David Thompson, PhD
david.thompson@optum.com
781-518-4034