COMPARATIVE EFFECTIVENESS RESEARCH: GOING BEYOND THE HYPE FOR MEANINGFUL USE

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ACMQ Presentation
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The content contained herein is based on publicly available information regarding CER.

Any opinions provided are my own and should not be interpreted to represent the position of any former or future employer.
US healthcare spending in 2013 - $3.8 trillion

Expenditures for healthcare are projected to increase each year for the next decade.

Healthcare expenditures likely to be 20% of the Gross Domestic Product (GDP) by 2021.
2009 - American Recovery and Reinvestment Act (ARRA)

2010 - Patient Protection and Affordable Care Act (ACA)
ARRA

$1.1 billion to DHHS for CER

Established coordinating council

Definition, prioritization, strategic framework

Coordinate CER in federal government

Recommendations for investment
  - Infrastructure!
  - High priority populations or interventions
IOM also asked to establish list of priorities

Set up 4 quartiles of priorities

As this research initiative progresses, the priorities will evolve as well. Ultimately, research on these and future topics will not yield real improvements unless the results are adopted by health care providers and organizations and integrated into clinical practice.”
Patient-Centered Outcomes Research Institute (PCORI)

Improve Quality

Increase Transparency

Increase Access to Better Healthcare

Methodologically Rigorous
OUTLINE

What?
Why?
Who?
When?
How?

Why should I care?
(Where?)
WHAT IS CER?

CER is the conduct and synthesis of systematic research comparing different interventions and strategies to prevent, diagnose, treat, and monitor health conditions.

Designed to inform health-care decisions by providing evidence on the effectiveness, benefits, and harms of different treatment options.

Purpose is to improve health outcomes through provision of evidence-based information.

Conway, NEJM, 2009
CER can be systematic reviews of **existing evidence** or **original research** where evidence is generated. In both cases, researchers are determining the effectiveness or comparative effectiveness of any test, procedure, or treatment.

<table>
<thead>
<tr>
<th>Existing Evidence</th>
<th>Original Research</th>
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<tbody>
<tr>
<td>Clinical Trials</td>
<td>Pragmatic Trials</td>
</tr>
<tr>
<td>Clinical Studies</td>
<td>Prospective Outcomes Studies</td>
</tr>
<tr>
<td>Systematic Reviews</td>
<td>Database Studies</td>
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<tr>
<td>Meta-Analysis</td>
<td>Costs Effectiveness</td>
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<tr>
<td>Network Meta-Analysis</td>
<td>Other New Comparisons of Healthcare Services/Products/Procedures</td>
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RCTs often provide information about whether a treatment is better than placebo for the average patient.

Patients and providers care more about which treatment is better for a specific patient.

CER informs care by identifying those who are most likely to benefit from (or be harmed by) a treatment.

Especially true for people with multiple chronic conditions, elderly, and racial/ethnic minorities who are poorly represented in RCTs.

CER has the potential to drive high-value innovation and to enable the practice of more personalized medicine based on subgroups of patients.
WHY IS CER NEEDED?

Patient-Specific

- Every patient is different — circumstances, history, values, conditions, etc
- Objective information for specific condition allows for evidence-based decisions on treatment
- Want to know which treatment is right for me

System Level

- Clarity on appropriate treatments
- Information on neglected diseases/populations
- Value for the money
WHO USES CER?

- Patient
- Providers
- Hospitals/Clinics
- Insurers/Payers
- Policy-makers
- Health Technology Assessors
- Governments
RAISES QUESTIONS...

What methods should be used to conduct the research?

Will physicians and other health care providers change what they do for patients based on CER findings?

How will patients and providers learn about the results?

Will the research be conducted openly and soundly enough that patients and providers will trust the outcomes?

Will private insurers and other payers use the research findings to make decisions on whether to cover treatments, and how much to pay for them?
WHEN IS CER CONDUCTED?

Depends on the stakeholder using the research...

Depends on the type of CER...

Depends on the location of the research...
TIMING OF CER

Newly marketed therapy — is it ‘better’ than other options?

Safety signal — is the signal ‘real’? is it ‘as safe as’ other options?

Specific population of interest — is it ‘better’ in this location? in this patient group?

Other new information or new interest
Again... it depends

Study Designs

Feasibility Info

Caveats
CER addresses a *specific comparative question*, including information on:

<table>
<thead>
<tr>
<th>Population</th>
<th>Outcome</th>
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<tbody>
<tr>
<td>Treatment</td>
<td>Timeframe</td>
</tr>
<tr>
<td>Comparison</td>
<td>Setting</td>
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</tbody>
</table>
STUDY DESIGNS

Qualitative and Quantitative

Surveys, observations, interviews, etc

RCTs, prospective and retrospective observational studies
INDIRECT COMPARISON

Study Population

Treatment A

Treatment B

Treatment C

Treatment D
CER

Adequate definitions of treatment and comparator

Check for adequate power given expected sample size

Assurance that each variable / measure is identifying the construct that it is intended to identify

Assessment of the potential for unmeasured confounding and plan for addressing it
Comparability between treatment and comparison groups

* Graphs from Walker, CER, 2013
Comparability between treatment and comparison groups

Utilization/treatment patterns — do they differ across subgroups?

Assess whether treatment choice or identifying outcome of interest is dependent on clinical work-up

Other biases as applicable to specific question
CAVEATS

Newly marketed therapies

Selective prescribing of patients

Early adopting physicians

Formulary position
Newly marketed therapies

Informed consent

Differences in decision-maker requirements for different treatment options
WHY SHOULD I CARE ABOUT CER?

... and, wasn’t this presentation supposed to include Meaningful Use?

To date, insurance claims predominant data for ‘real-world’ CER

But, richness of Electronic Medical Record (EMR) data is needed
Relevant and meaningful measures... including baseline measures

Claims uses ICD-9-CM; EHR is granular, has clearer information on ‘rule-out’

Can construct quality studies with EHR data
Monetary costs, resources, preference-based measures, QALYs/DALYs

Right resources to the right patients

Comparing utilization of resources between patients with different treatments

Example: DARTNet
DARTNet is the Distributed Ambulatory Research in Therapeutics Network

It is a network of electronic healthcare records (primary care clinics) which can be used as a platform for CER in real-world settings

Researchers wanted to know whether a survey at point of care could be implemented to obtain additional information within a study.
In a population of patients with type 2 diabetes, they were able to capture details from clinical encounters.

<table>
<thead>
<tr>
<th>Creatinine</th>
<th>Weight/BMI</th>
<th>Age</th>
<th>HbAlc</th>
</tr>
</thead>
<tbody>
<tr>
<td>Gender</td>
<td>Total physician visits</td>
<td>Triglycerides</td>
<td>All meds</td>
</tr>
<tr>
<td>Current smoking</td>
<td>Total cholesterol</td>
<td>Creatinine clearance</td>
<td>All diagnoses</td>
</tr>
<tr>
<td>Alcohol consumption</td>
<td>HDL</td>
<td>AST</td>
<td>Blood pressure</td>
</tr>
<tr>
<td>Weight and</td>
<td></td>
<td>ALT</td>
<td>LDL-C</td>
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In a population of patients with type 2 diabetes, they were able to capture details from clinical encounters. However, needed information was not available within the records. Through the survey, information on OTC medications, herbal supplements, and hypoglycemic events (past month) was collected. Paired with the clinical information in the electronic records, utilization and outcomes could be evaluated.
Using EHR data to conduct/supplement analysis at one’s own institution allows information from a study to be useful for the patients in the study.

Rapid-learning healthcare’

Example: intimacy concerns of colorectal cancer patients
Sexual intimacy is a concern for many cancer patients, but often undressed due to intensity of medical needs.

Gathered clinical data from EMR, supplemented with ePR of sensitive information from patients.

Skills-based telephone intervention along with education and coping info.

Able to address intimacy concerns at the point when patients identified them.

Abernethy, Medical Care, 2010
Map out course of care and identify interventions — CER principles can help to provide robust evidence development

Example: HIT intervention to improve adherence
HIT INTERVENTION

Pragmatic trial of patients with diabetes or CVD taking statins or antihypertensives

Gather clinical data from records

Automated call reminders to improve adherence to medications

65% of those ‘almost’ due for refill were reached; 48% with overdue refill

In first 10 months, 30% of calls transferred to automatic fill pharmacy line

2% more transferred to pharmacist

AHRQ  www.effectivehealthcare.ahrq.gov
PCORI  www.pcori.org
NICE  www.nice.org.uk
IQWiG  www.iqwig.de

GRACE Principles  www.graceprinciples.org
ISPOR Guidelines  www.ispor.org
I’ve really enjoyed learning about ACMQ -

Website and ‘Medical Quality Management: Theory and Practice’ info were particularly helpful.

Many thanks to you all for hosting these fora and inviting me to speak.

If you are interested in learning more...