What IS Comparative Effectiveness Research and Who Funds It?

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Professor and Director, Section of Health Systems Research and Policy
Division of General Internal Medicine and Geriatrics, MUSC
& Director, SCTR CER Initiative
Again ... what is CER?

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.

-- Congressional Budget Office
Core CER question......

Which treatments work best, for whom, and under what circumstances?
One Hundred Eleventh Congress of the United States of America
AT THE FIRST SESSION
Began and held at the City of Washington on Tuesday, the sixth day of January, two thousand and nine

The American Reinvestment and Recovery Act (ARRA) of 2009
Effective February 17, 2009
SEC. 3. PURPOSES AND PRINCIPLES.

(a) **Statement of Purposes.**—The purposes of this Act include the following:

1. To preserve and create jobs and promote economic recovery.
2. To assist those most impacted by the recession.
3. To provide investments needed to increase economic efficiency by spurring technological advances in science and health.
4. To invest in transportation, environmental protection, and other infrastructure that will provide long-term economic benefits.
5. To stabilize State and local government budgets, in order to minimize and avoid reductions in essential services and counterproductive state and local tax increases.
The measures are nominally worth $787 billion.

- Tax Relief: $288 B
- Education and Training: $53 B
- Protecting the Vulnerable: $81 B
- Energy: $43 B
- Health Care: $59 B
- Other: $8 B
- State and Local Fiscal Relief: $111 B
- Infrastructure and Science: $144 B

Tax incentives include $15 B for Infrastructure and Science, $61 B for Protecting the Vulnerable, $25 B for Education and Training, and $22 B for Energy, so total funds are $126 B for Infrastructure and Science, $142 B for Protecting the Vulnerable, $78 B for Education and Training, and $65 B for Energy.

State and Local Fiscal Relief prevents state and local cuts to health and education programs and state and local tax increases.
The American Reinvestment and Recovery Act (ARRA)

• ARRA contained $1.1 billion for comparative effectiveness research.
  – $300 million for the Agency for Healthcare Research and Quality (AHRQ)
  – $400 million for the National Institutes of Health (NIH)
  – $400 million at the discretion of the HHS Secretary

• The legislation called on the Institute of Medicine to recommend research priorities for the Secretary's funds
<table>
<thead>
<tr>
<th>Priority Setting Organization</th>
<th>Prevention</th>
<th>Acute Care</th>
<th>Care Coordination</th>
<th>Chronic Disease Care</th>
<th>Palliative Care</th>
<th>Patient Engagement</th>
<th>Safety</th>
<th>Overuse</th>
<th>IT Infrastructure</th>
<th>Impact of New Technology</th>
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<td>Institute of Medicine (2009)</td>
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<td>Federal Coordinating Council for Comparative Effectiveness Research</td>
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<td>Agency for Healthcare Research and Quality</td>
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<td>National Prevention, Health Promotion and Public Health Council</td>
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<td>National Priorities Partnership</td>
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Patient Protection and Affordable Care Act
March 23, 2010

Included:  Sec. 6301 Patient Centered Outcomes Research
SEC. 6301. PATIENT-CENTERED OUTCOMES RESEARCH.

(a) In General.—Title XI of the Social Security Act (42 U.S.C. 1301 et seq.) is amended by adding at the end the following new part:

"PART D—COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH

"COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH

"SEC. 1181. (a) Definitions.—In this section:

"(1) Board.—The term ‘Board’ means the Board of Governors established under subsection (f).

"(2) Comparative Clinical Effectiveness Research; Research.—

"(A) In General.—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, and items described in subparagraph (B).
Key Items in Sec. 6301

- Establishment of PCORI: Patient-Centered Outcomes Research Institute
- NOT an agency of the government
- Mission: to assist patients, clinicians, purchases, and policymakers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases...can effectively and appropriately be prevented, diagnosed, treated ... through research and evidence synthesis...and the dissemination of research findings...
Figure 1: Framework for Translation of PCORI National Priorities into the Research Agenda

<table>
<thead>
<tr>
<th>Priorities</th>
<th>PCORI Criteria</th>
</tr>
</thead>
<tbody>
<tr>
<td>Assessment of Options for Prevention, Diagnosis, and Treatment</td>
<td>▶ Impact on Health of Individuals and Populations</td>
</tr>
<tr>
<td></td>
<td>▶ Improvability via Research</td>
</tr>
<tr>
<td></td>
<td>▶ Inclusiveness of Different Sub-Populations</td>
</tr>
<tr>
<td></td>
<td>▶ Addresses Current Gaps in Knowledge/Variation in Care</td>
</tr>
<tr>
<td></td>
<td>▶ Impact on Health Care System Performance</td>
</tr>
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<td></td>
<td>▶ Potential to Influence Decision Making</td>
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<td></td>
<td>▶ Patient-Centeredness</td>
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<td></td>
<td>▶ Rigorous Research Methods</td>
</tr>
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<td></td>
<td>▶ Efficient Use of Research Resources</td>
</tr>
</tbody>
</table>

PCORI Research Agenda → PCORI Funding Announcement(s)
SEC. 3004. PATIENT-CENTERED OUTCOMES RESEARCH TRUST FUND

(a) CREATION OF TRUST FUND.—There is established in the Treasury of the United States a trust fund to be known as the "Patient-Centered Outcomes Research Trust Fund" (hereafter in this section referred to as the "PCORTF"), consisting of such amounts as may be appropriated or credited to such Trust Fund as provided in this section and section 9502(b).

(b) TRANSFERS TO FUND.—

(1) APPROPRIATION.—There are hereby appropriated to the Trust Fund the following:

(A) For fiscal year 2010, $10,000,000.
(B) For fiscal year 2011, $50,000,000.
(C) For fiscal year 2012, $150,000,000.
(D) For fiscal year 2013—

(ii) an amount equivalent to the net revenues received in the Treasury from the fees imposed under subchapter B of chapter 34 (relating to fees on health insurance and self-insured plans) for such fiscal year; and

(ii) $150,000,000.
(E) For each of fiscal years 2014, 2015, 2016, 2017, 2018, and 2019—

(ii) an amount equivalent to the net revenues received in the Treasury from the fees imposed under subchapter B of chapter 34 (relating to fees on health insurance and self-insured plans) for such fiscal year; and

(ii) $150,000,000.

The amounts appropriated under subparagraphs (A), (B), (C), (D)(ii), and (E)(ii) shall be transferred from the general fund of the Treasury, from funds not otherwise appropriated.
Funding of the PCORI

PCORI and CER Funding

Funding in Millions USD


- AHRQ*
- ARRA**
- PCORI Trust Fund
- PCORI Plan Contributions***

* AHRQ Funding FY2006-2010.
** American Recovery and Reinvestment Act: $1.1 Billion over 2009-2010; FY2009 HHS Report;
*** PCORI Trust Fund: $1/Covered Life in 2013; $2/Covered Life in 2014 with enhancement adjusted for inflation
<table>
<thead>
<tr>
<th>Priority</th>
<th>% Funding Allocation</th>
</tr>
</thead>
<tbody>
<tr>
<td>Assessment of Prevention, Diagnosis, and Treatment Options</td>
<td>Approximately 40%</td>
</tr>
<tr>
<td>Improving Healthcare Systems</td>
<td>Approximately 20%</td>
</tr>
<tr>
<td>Communication and Dissemination Research</td>
<td>Approximately 10%</td>
</tr>
<tr>
<td>Addressing Disparities</td>
<td>Approximately 10%</td>
</tr>
<tr>
<td>Accelerating PCOR and Methodological Research</td>
<td>Approximately 20%</td>
</tr>
</tbody>
</table>
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?”

Read more about the process for establishing the definition of ‘Patient-Centered Outcomes Research’

VIDEO: Dr. Sherine Gabriel highlights PCOR’s goal of producing high integrity research for patients and their caregivers
Overview

The Patient-Centered Outcomes Research Institute (PCORI) is an independent organization created to help people make informed health care decisions and improve health care delivery. PCORI will commission research that is guided by patients, caregivers and the broader health care community and will produce high integrity, evidence-based information.

PCORI is committed to transparency and a rigorous stakeholder-driven process that emphasizes patient engagement. PCORI will use a variety of forums and public comment periods to obtain public input throughout its work.

Provide Input

The Patient-Centered Outcomes Research Institute solicits and receives input from the public about its work, as part of its commitment to transparency, credibility and access.

Funding Opportunities

PCORI Announces $26 Million Pilot Projects Grant Program, seeks Patient, Stakeholder and Scientific Reviewers.
<table>
<thead>
<tr>
<th>Announcement</th>
<th>Letter of Intent and Application Deadlines</th>
<th>Cycle*</th>
<th>Total Direct Costs</th>
<th>Maximum Project Period</th>
<th>Funds Available Up To</th>
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</thead>
<tbody>
<tr>
<td>Patient-Centered Outcomes Research (PCOR) for Treatment Options in Uterine Fibroids: Developing a Prospective Multi-Center Practice-based Clinical Registry (P50)*</td>
<td>LOI Deadline: 11/15/2013 Application Deadline: 12/16/2013 *LOI is not required and is non-binding</td>
<td>One-time opportunity</td>
<td>Up to $4 Million total costs/year</td>
<td>5 Years</td>
<td>$20 million</td>
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## Active Funding Announcements: The LOI deadline has passed

<table>
<thead>
<tr>
<th>Announcement</th>
<th>Letter of Intent and Application Deadlines</th>
<th>Cycle</th>
<th>Maximum Budget</th>
<th>Maximum Project Period</th>
<th>Funds Available Up To</th>
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<tbody>
<tr>
<td>Clinical Trial of a Multifactorial Fall Injury Prevention Strategy in Older Persons</td>
<td>LOI Deadline: 10/13/2013 Application Deadline: 11/13/2013</td>
<td>One-time opportunity</td>
<td>$30 million</td>
<td>5 Years</td>
<td>$30 million</td>
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<tr>
<td>Assessment of Prevention, Diagnosis, and Treatment Options</td>
<td>LOI Deadline: 10/15/2013 Application Deadline: 1/21/2014</td>
<td>Winter 2014</td>
<td>$1.5 Million direct costs</td>
<td>3 Years</td>
<td>$32 Million</td>
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<tr>
<td>Improving Healthcare Systems</td>
<td>LOI Deadline: 10/15/2013 Application Deadline: 1/21/2014</td>
<td>Winter 2014</td>
<td>$1.5 Million direct costs</td>
<td>3 Years</td>
<td>$16 Million</td>
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<td>Communication and Dissemination Research</td>
<td>LOI Deadline: 10/15/2013 Application Deadline: 1/21/2014</td>
<td>Winter 2014</td>
<td>$1.5 Million direct costs</td>
<td>3 Years</td>
<td>$16 Million</td>
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<tr>
<td>Addressing Disparities</td>
<td>LOI Deadline: 10/15/2013 Application Deadline: 1/21/2014</td>
<td>Winter 2014</td>
<td>$1.5 Million direct costs</td>
<td>3 Years</td>
<td>$8 Million</td>
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<tr>
<td>Improving Methods for Conducting Patient-Centered Outcomes Research</td>
<td>LOI Deadline: 10/15/2013 Application Deadline: 1/21/2014</td>
<td>Winter 2014</td>
<td>$750,000 direct costs</td>
<td>3 Years</td>
<td>$8 Million</td>
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<tr>
<td>Project Description</td>
<td>Application Date</td>
<td>Close Date</td>
<td>Opportunity Type</td>
<td>Cost Breakdown</td>
<td>Duration</td>
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</table>
| Treatment Options for African-Americans and Hispanics/Latinos with Uncontrolled Asthma | 08/01/13         |            | One-time opportunity | Year 1: Up to $500,000 total costs  
Year 2: Up to $1.75 million total costs  
Year 3: Up to $1.75 million total costs | 3 Years         | $17 million   |
<p>| Improving Infrastructure for Conducting Patient-Centered Outcomes Research: Phase One—Clinical Data Research Networks | 06/19/13         |            | One-time opportunity | $7 million total cost/project** | 18 Months | $56 million   |
| Improving Infrastructure for Conducting Patient-Centered Outcomes Research: Phase One—Patient-Powered Research Networks | 06/19/13         |            | One-time opportunity | Up to $1 million total cost/project** | 18 Months | $12 million   |
| Assessment of Prevention, Diagnosis, and Treatment Options                          | 06/15/13         | August 2013|                       | $500,000 direct costs/year                                                   | 3 Years         | $32 million   |
| Improving Healthcare Systems                                                        | 06/15/13         | August 2013|                       | $500,000 direct costs/year                                                   | 3 Years         | $16 million   |</p>
<table>
<thead>
<tr>
<th>Table Title</th>
<th>Start Date</th>
<th>End Date</th>
<th>Annual Costs</th>
<th>Duration</th>
<th>Total Cost</th>
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<td>Communication and Dissemination Research</td>
<td>06/15/13</td>
<td>August 2013</td>
<td>$500,000 direct costs/year</td>
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<td>Addressing Disparities</td>
<td>06/15/13</td>
<td>August 2013</td>
<td>$500,000 direct costs/year</td>
<td>3 Years</td>
<td>$8 million</td>
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<tr>
<td>Improving Methods for Conducting Patient-Centered Outcomes Research</td>
<td>06/15/13</td>
<td>August 2013</td>
<td>$250,000 direct costs/year</td>
<td>3 Years</td>
<td>$8 million</td>
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</table>
3 Funding Cycles

PCORI has approved 197 awards totaling more than $273.5 million to fund patient-centered comparative clinical effectiveness research projects to date.

Avg: $273.5/197 ≈ $1.38m
**Pipeline to Proposal Awards – Tier I**

**Application Deadline:**
December 2, 2013 by 5:00 pm ET

**Summary:**
The research that PCORI funds must involve patients and other stakeholders from start to finish. We believe that by funding research that is shaped by and answers questions that are important to patients and other end users such as clinicians, we can achieve our mission of providing better information to patients as they make healthcare decisions. However, we recognize that it can be difficult for patients and other healthcare stakeholders to develop partnerships and to get involved in research. This funding opportunity is available to individual—or small groups of—patients, other stakeholders, and researchers who have an idea for a potential research project but need assistance and guidance to build it.

**RFP Announcement:**
Tier I Pipeline to Proposal Awards

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### Pipeline to Proposal Awards

![Pipeline Diagram]

**Tier I**
- Up to $15,000
- Up to 9-month term

**Tier II**
- Up to $25,000
- Up to 12-month term

**Tier III**
- Up to $50,000
- Up to 12-month term

**PCORI Funding Announcement**

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**Eligibility:**
Applications may be submitted by:
- Patients
- Other stakeholders
- Researchers

**Review Criteria:**
PCORI is interested in projects that:
- Involve community and capacity building and strengthen relationships
- Have a connection to potential research projects
- Include a strategy for communicating and sharing information
- Form a strong team and environment

**Applicant Resources:**
Applicant Training

**Other:**
*Deadlines are at 5:00 PM ET. If deadlines fall on a weekend or a federal holiday, the deadline will be the following Monday or the next day after the federal holiday.*
Methods of CER

- Systematic Reviews of Existing Research
- Analyses of Claims Records
- Medical Registries
- Randomized Controlled Trials
- Modeling

The Dissemination of Results

- Whatever types of results were produced, any new or augmented entity focused on comparative effectiveness would want to consider carefully how those results were communicated to doctors, patients, and other interested parties.
The Methods of Comparative Effectiveness Research

Harold C. Sox\(^1\) and Steven N. Goodman\(^2\)

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Keywords
propensity score, instrumental variable, sensitivity analysis, treatment-response heterogeneity, clinical prediction rule

Abstract
This review describes methods used in comparative effectiveness research (CER). The aim of CER is to improve decisions that affect medical care at the levels of both policy and the individual. The key elements of CER are \((a)\) head-to-head comparisons of active treatments, \((b)\) study populations typical of day-to-day clinical practice, and \((c)\) a focus on evidence to inform care tailored to the characteristics of individual patients. The review summarizes key critical issues in the methods used to answer these questions and offers integration of principles from related fields such as public health and general medical research.
Cost Research (okay?)

- CER provisions in the legislation *do not* prohibit comparative cost effectiveness
- Act *does* explicitly state that PCORI will not “develop or employ” a dollars per QALY approach to evaluation of interventions
.... In the legislation

“The Patient-Centered Outcomes Research Institute ... 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.”
What other organizations fund CER research?
Summary

- Exciting opportunities for improving effectiveness of care and care delivery
- Supportive legislation
- Possibility of more stable funding streams
- Greater transparency in research
- Appreciation of the role of stakeholders in this process
SCTR FUNDING OPPORTUNITY: 2011-2012 Call for Pilot Projects

SCTR Pilot Project funds assist faculty members at SCTR institutions compete successfully for major research funding.

- Rolling Pilot Grants for Health Disparities, Community/University Partnerships, Fast-Forward Seed Grants
- Matching Pilot Grants

SCTR would like to thank all pilot project applicants for its 1st cycle of 2011-12 funding. We received 110 Discovery pre-applications (including 20 from USC, 3 from Clemson, 1 from Claflin University) & 7 applications for other grant categories. Reviews & notifications will be made within 6-8 weeks.