2013 PIPC Annual Forum

Changing the Culture of Research: From CER Prioritization to Dissemination

November 5, 2013
12:00 pm – 2:00 pm

Reserve Officers Association
Washington D.C.
Panelists

• Honorable Tony Coelho, Chairman, PIPC (Moderator)
• Dr. Bryan Luce, Chief Science Officer, PCORI
• Dr. Hal Sox, Senior Advisor to PCORI
• Mr. Marc Boutin, Executive Vice President and COO, National Health Council
• Dr. Darius Tandon, Co-Chair, PCORI Advisory Panel on Patient Engagement
• Dr. Tanisha Carino, Executive Vice President, Avalere
The Honorable Tony Coelho
Chairman, PIPC

We all need good information to make good decisions about our health, and our health care. Comparative clinical effectiveness research can help us make good health care choices. But it is important to make sure this research isn't misused to make these decisions for us. The Partnership to Improve Patient Care exists to ensure that comparative clinical effectiveness research is focused on giving providers and patients the information they need, improving health care quality and supporting continued medical progress.
Hal Sox, MD
Senior Advisor to PCORI

“PCORI should plan its research agenda strategically, so that it addresses research questions that comparative effectiveness research could answer quickly and decisively.”

- Dr. Hal Sox, *Health Affairs*, 2012
Targeted research: priority-setting at PCORI

Harold C. Sox
Professor of Medicine
Geisel School of Medicine
Senior Advisor, PCORI
Conflicts of Interest

Dr. Sox is a part-time paid consultant to PCORI. He is not representing PCORI at this Forum.

No other conflicts.
Two approaches to funding research

- Investigator-initiated research
- Sponsor-initiated research (targeted funding)
Investigator-initiated research

• Agency announces a funding opportunity.
• Researchers who are expert in the field propose research that is based on their scientific interests.
• Agency peer reviewers rank the topics in order of scientific interest.
• The highest ranked proposals get funded.
• Who sets research priorities?
  – The investigators
  – The peer reviewers
Research programs at PCORI

• Investigator-initiated projects
  – Comparative Effectiveness Research
  – Improving Healthcare Systems
  – Communication and Dissemination Research
  – Addressing Disparities
  – Accelerating Patient-Centered Outcomes Research and Methodological Research

• Targeted Research
### PCORI Clinical Effectiveness Program slate of funded projects

<table>
<thead>
<tr>
<th>Cycle/Submission date</th>
<th>Cycle I</th>
<th>Cycle II</th>
<th>Cycle III</th>
<th>Aug 2013</th>
</tr>
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<tbody>
<tr>
<td><strong>Approx. award date</strong></td>
<td>January 2013</td>
<td>July 2013</td>
<td>September 2013</td>
<td>January 2014</td>
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<tr>
<td><strong>Maximum budget/yr (direct costs)</strong></td>
<td>$500K</td>
<td>$500K</td>
<td>$500K</td>
<td>$500K</td>
</tr>
<tr>
<td><strong>Max project period</strong></td>
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<td>3 years</td>
<td>3 years</td>
<td>3 years</td>
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<tr>
<td><strong>Funds available</strong></td>
<td>$32M</td>
<td>$34M</td>
<td>$48M</td>
<td>$32M</td>
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<tr>
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<td>8</td>
<td>8</td>
<td>8</td>
<td>5</td>
</tr>
<tr>
<td><strong># awards made</strong></td>
<td>9</td>
<td>22</td>
<td>24</td>
<td>TBD</td>
</tr>
<tr>
<td><strong>Total funding</strong></td>
<td><strong>$53.1M</strong></td>
<td><strong>$45.7M</strong></td>
<td>TBD</td>
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</table>

Courtesy of PCORI
Research programs at PCORI

• Investigator-initiated projects
  – Assessment of Prevention, Diagnosis, and Treatment Options
  – Improving Healthcare Systems
  – Communication and Dissemination Research
  – Addressing Disparities
  – Accelerating Patient-Centered Outcomes Research and Methodological Research

• Targeted Research
Sponsor-initiated research

• The sponsor sets priorities for research questions to address.
  – PCORI Advisory Panels set priorities.

• The sponsor develops a research project agenda → issues a RFA.
  – A package of research projects within a budget.
  – Expert advice on study design → RFA specifies the project design
  – Peer review → award a contract to perform the research.

VanLare et al. New Engl J Med. 2010;362:970-973
Priority-setting: IOM and PCORI

Clinical topic proposed by stakeholder

Priority-setting for research question

IOM priority setting

Limited information

PCORI priority setting

Priority-setting for clinical topic

Priority-setting for research question

Research question = clinical topic + intervention + outcome measure
PCORI: Topic Prioritization for CER

1,300+ Research Topics Received

841 Topics Accepted

594 Assigned to Assessment of Options

- Program director screened and consolidated topics
- Topics scored on 4 criteria

20 High Scoring Topics Considered

- In April 2013, Advisory Panel reviewed and prioritized 20 topics

4 Priority Topics Selected

- (1) Bipolar Disorder
- (2) Ductal Carcinoma in situ
- (3) Migraine Headache
- (4) Osteoarthritis

Courtesy of PCORI
4 criteria used to score topics

• 1) Patient-centeredness
• 2) Burden.
• 3) Potential for improving healthcare practice.
• 4) Timeliness.
From priority topic to research question: an overview

1. Identifying Known Evidence Gaps
2. Creation of Expert Stakeholder Group
3. Expansion of Evidence Gaps
4. Analytic Framework
5. Stakeholder Prioritization
6. Horizon Scan
7. Study Design Considerations
**From a high priority clinical topic to key research questions**

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<th>Step</th>
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<th>Information</th>
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<td>Analytic framework</td>
<td>Duke University researchers</td>
<td>Clinical knowledge</td>
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<tr>
<td>Identify evidence gaps → topic briefs</td>
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<td>Systematic reviews, guidelines</td>
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<td>Expansion of evidence gaps</td>
<td>Clinical experts + stakeholders</td>
<td>Duke U topic brief</td>
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<tr>
<td>Prioritization of evidence gaps</td>
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<td>List of evidence Gaps + topic brief</td>
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<td>Horizon scan “what’s out there?”</td>
<td>Duke University researchers</td>
<td>Pub Med + clinical trials registry</td>
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Analytic framework and key questions (KQs)
## From a high priority clinical topic to key research questions

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**Steps:**
- Analytic framework
- Identify evidence gaps
- Expansion of evidence gaps
- Prioritization of evidence gaps
- Horizon scan

**Responsible Parties:**
- Duke University researchers
- Clinical experts + stakeholders
- Advisory Panel for Clinical Effectiveness

**Information Sources:**
- Clinical knowledge
- Systematic reviews, guidelines
- Duke U topic brief
- List of evidence Gaps + topic brief
- Pub Med + clinical trials registry
From disease to research question: an overview

1. Identifying Known Evidence Gaps
2. Creation of Expert Stakeholder Group
3. Expansion of Evidence Gaps
4. Analytic Framework
5. Stakeholder Prioritization
6. Horizon Scan
7. Study Design Considerations
Bryan Luce, PhD, MBA
Chief Science Officer, PCORI

The Patient-Centered Outcomes Research Institute (PCORI) is an independent, non-profit organization authorized by Congress in 2010. Its mission is to fund research that will provide patients, their caregivers and clinicians with the evidence-based information needed to make better-informed health care decisions. PCORI is committed to continuously seeking input from a broad range of stakeholders to guide its work.
Marc Boutin
Executive VP/COO, National Health Council

“However, a study with high methodological quality is not necessarily one that patients and providers will find usable for their decision making.”

Establishing CER Usability Criteria:

- Research question is one that patients and providers want answered
- When placed in the context of the full body of evidence, study expands clinical understanding
- Research methods are transparent
- Limitations and implications for future research are discussed
- Presentation of study data is balanced
- Factors that led to the development of the study are disclosed
- Study provides clarity on the extent to which findings can be applied in individual contexts
According to its charter, the Patient Engagement Panel will:

• Advise on processes to identify research topics and priorities that are important to patients;

• Advise on all aspects of stakeholder review of applications for PCORI funding;

• Provide general recommendations to PCORI and externally on the conduct of patient-centered research;

• Advise on methods to evaluate the impact of patient engagement in research;

• Assist and advise PCORI on communications, outreach, and dissemination of research findings; and

• Provide advice on other questions and areas of interest that may arise relevant to PCORI’s mission and work.
Dr. Tanisha Carino, PhD.
Executive Vice President, Avalere

Tanisha Carino oversees Avalere’s strategic advisory and research services for the nation's leading life sciences companies. For close to a decade, Tanisha has worked with senior leadership in Fortune 500 companies to establish organizational goals and align their internal functions to capture opportunities and mitigate challenges related to evolving regulatory, evidence, and commercial trends.
Trends in Comparative Effectiveness Research (CER)

Tanisha Carino, Ph.D.
Executive Vice President, Avalere Health

November 5, 2013
avalerehealth.net
The Current Healthcare Environment Has Driven Demand for More Evidence

**Rising Costs**
- Costs of new treatments are rising
- Businesses find it harder to pay for healthcare
- Patients are paying more out of pocket

**Misaligned Payment**
- Payment is based on volume, not quality
- Payment system comes with perverse incentives

**Variable Treatment**
- Insufficient data on what works best
- Treatment varies; providers are not always following best practices
- Quality is suboptimal (preventable errors, safety concerns, overuse)

**Lack of Information**
- Minimal information about quality and cost of healthcare services
- Incomplete data at point of care to support good decision making
- Lack of data infrastructure to gather/share information
A Variety of Stakeholders Aim to Use CER to Answer Distinct Questions

- Employers
  - Is the use of this treatment affecting premiums and/or productivity?

- Health Plans & Government Payers
  - Is this treatment or service appropriate for my beneficiaries?

- Patients
  - Is this treatment right for me? How does it impact my quality of life?
  - Is it as good as alternatives for what it costs? What is the recommended use of this treatment / provision of this services?

- Life Sciences Industry
  - How can CER support my value proposition?

- Hospitals/Systems & Providers
  - What does the evidence say about the use of this treatment or service?

- Professional societies
The Federal Government’s Investments in CER Have Grown Over the Years

Agency for Healthcare Research and Quality (AHRQ)
Office of the Secretary, Health and Human Services
National Institutes of Health
Patient-Centered Outcomes Research Institute

$1.1B
(2 years)

$15M-$30M*

2003 MMA
2009 ARRA
2010 ACA

~$4-5B**
(10 years)

*AHRQ CER budget was initially $15M and has since been increased to $30M
**2010 – 2012: appropriations of $10M, $50M, and $150M respectively; mix of public and private funds thereafter
HHS: Health and Human Services; NIH: National Institutes of Health; PCORI: Patient-Centered Outcomes Research Institute
At the State Level, CER is Growing in Use As Well

DESPITE THE PROLIFERATION OF FEDERAL CER, ENVIRONMENTAL TRENDS AND GAPS IN THE EVIDENCE HAVE LED TO THE RISE IN STATE-LEVEL CER

<table>
<thead>
<tr>
<th>Environmental Trends</th>
<th>Gaps in Federal CER</th>
<th>The Rise in State CER</th>
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<tbody>
<tr>
<td>• Locus of decision-making shifting to states</td>
<td>• Decision-makers do not exclusively rely on federal CER</td>
<td>• Need to adapt CER to make it meaningfully locally</td>
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<tr>
<td>• Demand for payers and providers to demonstrate evidence-based, high-value care</td>
<td>• Federal CER is typically not effectively translated</td>
<td>• State-created HTAs emerging</td>
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<tr>
<td>• Federal CER investments in state and regional CER</td>
<td></td>
<td>• Academic detailing translation and dissemination efforts on a local level</td>
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HTA: Health Technology Assessment
A Number of Organizations Are Already Using CER to Make Decisions

<table>
<thead>
<tr>
<th>Advisory Support</th>
<th>Binding Decisions</th>
<th>Treatment Decisions</th>
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NIH: National Institutes of Health
USPSTF: United States Preventive Services Task Force
NQF: National Quality Forum
NCQA: National Committee for Quality Assurance

CMS: Centers for Medicare & Medicaid Services
FDA: U.S. Food and Drug Administration
Spotlight on USPSTF

USPSTF FORMALIZES EPC-GENERATED CER IN ITS CLINICAL RECOMMENDATIONS

CASE STUDY: HEPATITIS C VIRUS (HCV) SCREENING RECOMMENDATION, JUNE 2013

Evidence Base
- RCTs and cohort, case–control, and cross-sectional studies that assessed yield or outcomes of screening; studies reporting harms from HCV screening; and large series reporting harms of diagnostic liver biopsies
- Oregon EPC’s “Screening for Hepatitis C Virus Infection in Adults” CER report is a supporting document
- Consistent with AHRQ’s Methods Guide for CER, this recommendation received a Grade B recommendation*

Collaboration with AHRQ
- USPSTF recommends screening for HCV infection in persons at high risk for infection. The USPSTF also recommends offering 1-time screening for HCV infection to adults born between 1945 and 1965

Recommendation
- USPSTF concludes evidence is lacking on the comparative effects of current antiviral treatments on long-term clinical outcomes
- This may shape future CER focus

Future Research Needs

Given USPSTF’s positive recommendation, it is likely that CMS will expand Medicare coverage for HCV screening in adults through the NCD it initiated on September 5

*Under the Affordable Care Act, Medicare and private payers are required to cover services recommended by the USPSTF with a grade of A or B with no cost-sharing
EPC: Evidence-based Practice Center
CMS: Centers for Medicare & Medicaid Services
NCD: National Coverage Determination

Avalere
As CER Investments Grow, Focus is Growing on Usability of CER

THE NATIONAL HEALTH COUNCIL IS LEADING AN EFFORT TO CREATE CRITERIA THAT COULD BE APPLIED TO EVALUATING CER USABILITY

Potential Applications of Usability Criteria

- Refine and prioritize research questions
- Help patients and providers distill evidence to identify findings that are most applicable to their decision making

Based on discussion at a Dialogue meeting held on July 10, 2013, hosted by Avalere and the National Health Council
Key Takeaways

1. CER-informed clinical recommendations increasingly impact patient access

2. Monitoring state-level CER activities will be important to understanding the evidence-base for local decision-making

3. As the number of entities that communicate information expands, consistency in communication will grow in importance
Questions?

Tanisha Carino, Ph.D.
Executive Vice President, Avalere Health
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