The Recovery Act Investment in Comparative Effectiveness Research:
Interim Assessment and Implications

Presentation at Health Care Policy Research Forum
Mathematica Policy Research
Washington, DC
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Welcome

Moderator

Eugene Rich, M.D.

Mathematica Policy Research
The Center on Health Care Effectiveness (CHCE) conducts and disseminates research and policy analyses that support better decisions at the point of care. Our focus is on the delivery systems and policy environments that help clinicians and patients make more informed decisions, using information on outcomes and effectiveness.
Today’s speakers

Dominick Esposito
Mathematica

Eugene Rich
Mathematica

Sheldon Greenfield
U.C. Irvine

Sanford Schwartz
Univ. of Pennsylvania

Bryan Luce
PCORI
Overview, findings, and implications

Dominick Esposito
Mathematica Policy Research
Context for the evaluation

Some CER RFAs/RFPs released, proposals submitted and reviewed

Other CER RFAs/RFPs released, proposals submitted and reviewed; All ARRA CER projects awarded

Short-term process metrics measurable

Long-term outcome metrics measurable

February 2009
ARRA passed

ARRA CER: Expansion of public investments in CER in context of overall effort at economic stimulus

June 2009
FCC and IOM CER reports released

Evolving environment for CER: Patient-centered outcomes research

March 2010
ACA passed

August 2010
Start of evaluation

September 2013
End of evaluation

Limited time for CER to affect society or stakeholders
Evaluation objectives and approach

• Purpose
  – Describe what was learned from the ARRA CER investments relative to FCCCER and HHS goals
  – Provide guidance for future CER investments (and evaluations)

• Focus on findings across entire portfolio
  – Not findings from specific projects

• Midstream evaluation using data collected while projects were ongoing
  – Redacted project proposals
  – Investigator survey
  – Discussions with select project officers, investigators, and other scholars
Questions addressed by the evaluation

• What types of investments were made with ARRA CER funds?

• How has the ARRA CER portfolio of investments begun to address strategic priorities for CER?

• What midstream findings were identified that can inform HHS?

• What are the implications for future federal investments in CER?

• What are the lessons learned for evaluating the long-term impact of ARRA (or other) CER investments?
Questions addressed by the evaluation

- What types of investments were made with ARRA CER funds?
- How has the ARRA CER portfolio of investments begun to address strategic priorities for CER?
Percentage of ARRA CER projects, by primary area of focus

- 46% Research on Comparative Effectiveness
- 23% Data Infrastructure
- 13% Dissemination and Translation
- 10% Research Training and Career Development
- 5% Methods Development
- 3% Other

The ARRA CER portfolio
Percentage of ARRA CER projects, by primary area of focus

- Research on Comparative Effectivness: 46%
- Data Infrastructure: 23%
- Research Training and Career Development: 13%
- Dissemination and Translation: 5%
- Methods Development: 3%
- Other: 10%
Many ARRA CER projects had multiple areas of focus

- Data Infrastructure primary, 15 projects
- Research primary, 194 projects
- Dissemination and Translation primary, 15 projects
- Research Training and Career Development primary, 38 projects
- Research secondary, 130 projects
- Data Infrastructure primary, 70 projects
- Other primary, 86 projects
- Methods Development primary, 13 projects
- Dissemination and Translation primary, 38 projects
- Research Training and Career Development primary, 13 projects

Total is 410 projects, which excludes 14 projects whose primary areas of focus were something other than Research, Data Infrastructure, Training, Methods, or Dissemination and Translation.
### Project characteristics: priority themes

#### The ARRA CER portfolio

<table>
<thead>
<tr>
<th>Priority themes and IOM topics addressed by ARRA CER projects</th>
<th>Percentage of projects by primary area of focus</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Number of projects</td>
</tr>
<tr>
<td>Any population, condition, or intervention</td>
<td>329</td>
</tr>
<tr>
<td>Priority population</td>
<td>192</td>
</tr>
<tr>
<td>Priority condition</td>
<td>260</td>
</tr>
<tr>
<td>Priority intervention</td>
<td>184</td>
</tr>
<tr>
<td>IOM priorities for CER</td>
<td>174</td>
</tr>
</tbody>
</table>
Project characteristics: IOM priority topics

The ARRA CER portfolio

Distribution of ARRA CER projects and funding, by IOM research areas

<table>
<thead>
<tr>
<th>IOM research area</th>
<th>Number of projects</th>
<th>Number of topics included</th>
<th>Total funding (in millions)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Research areas addressed by 10 or more projects</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Health care delivery systems</td>
<td>94</td>
<td>39</td>
<td>$221.9</td>
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<tr>
<td>Racial and ethnic disparities</td>
<td>25</td>
<td>18</td>
<td>$36.7</td>
</tr>
<tr>
<td>Cardiovascular and peripheral vascular disease</td>
<td>17</td>
<td>9</td>
<td>$58.8</td>
</tr>
<tr>
<td>Oncology and hematology</td>
<td>13</td>
<td>6</td>
<td>$36.7</td>
</tr>
<tr>
<td><strong>Research areas addressed by 5 to 9 projects</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Nutrition (including obesity)</td>
<td>9</td>
<td>6</td>
<td>$19.2</td>
</tr>
<tr>
<td>Endocrinology and metabolism disorders and geriatrics</td>
<td>7</td>
<td>10</td>
<td>$27.9</td>
</tr>
<tr>
<td>Kidney and urinary tract disorders</td>
<td>7</td>
<td>5</td>
<td>$15.5</td>
</tr>
<tr>
<td>Complementary and alternative medicine</td>
<td>6</td>
<td>4</td>
<td>$5.7</td>
</tr>
<tr>
<td>Alcoholism, drug dependency, and overdose</td>
<td>6</td>
<td>2</td>
<td>$14.7</td>
</tr>
</tbody>
</table>
Questions addressed by the evaluation

• What midstream findings were identified that can inform HHS?

• What are the implications for future federal investments in CER?
Context for HHS

• 20% of PCORTF monies allocated to HHS
  – 80% to AHRQ to train researchers and disseminate findings
  – 20% to Office of the Secretary of HHS to build data capacity

• NIH and AHRQ authority to conduct CER as funded through appropriations

• HHS responsibility for legal, regulatory, and policy guidance relevant to clinical research
ARRA CER projects: key findings and implications

- Research on Comparative Effectiveness
- Data Infrastructure
- Research Training and Career Development
- Dissemination and Translation
- Methods Development
ARRA CER projects: key findings and implications

- Research on Comparative Effectiveness
Research on comparative effectiveness: key findings

1. Team members from nonresearch organizations might be particularly important contributors

2. Stakeholder engagement can be important but also challenging and resource intensive

3. Multi-organizational collaborations were a prominent feature of Research projects

4. Achieving a shared understanding of project goals and expectations across project team was helpful

5. Deep understanding of differences among organizations and settings facilitated collaborations

6. Various strategies can facilitate multi-institutional CER projects
Research on comparative effectiveness: implications for HHS

1. Provide guidance to address varying local interpretation of privacy regulations and human subject protections
2. Study differences in private payer coverage for multisite trials
3. Consider reducing time between proposal submission and award notification
4. Identify best practices for managing multi-organizational collaborations
5. Support tools to facilitate cross-site data collection and data sharing
6. Identify best practices that facilitate stakeholder engagement in CER design
7. Support efforts to engage stakeholders in CER design
ARRA CER projects: key findings and implications

- Research Training and Career Development
CER training: key findings

1. Multidisciplinary support and mentorship are important to development of new CER researchers

2. CER training programs must employ strategies to accommodate diverse educational backgrounds and future research roles

3. CER is not a specific scholarly discipline but is rather a broad, multidisciplinary field of research

4. A variety of competencies are currently required by CER trainees

5. CER continues to evolve; advances might require additional competencies

6. Maintaining CER-specific curricula might require continuing support
CER training: implications for HHS

1. Promote strategies that prepare researchers with diverse educational backgrounds for the broad range of careers in CER

2. Update training curricula to incorporate new CER developments

3. Support engaging people with diverse disciplines and clinical perspectives in CER training

4. Support mentoring and trainee involvement in CER projects
ARRA CER projects: key findings and implications

- Methods Development
CER methods: key findings

1. Access to data with sufficient clinical detail was an important constraint for some *Methods* projects.

2. Opportunities to improve availability and usability of new CER methods.

3. CER *Methods* research teams required a broad range of skills and expertise.

4. Use of different terminology across disciplines is a barrier for methods development.

5. Projects examined a diverse array of topics, but many priorities remain for continued work.

6. Information needs of decision makers can inform future CER methods priorities.
CER methods: implications for HHS

1. Support efforts such as learning networks to increase dissemination and usability of CER methods

2. Support collaboration among CER methods and data infrastructure investigators

3. Support collaborations for development of methods curricula
ARRA CER projects: key findings and implications
1. Project-specific privacy and data security issues must be prospectively recognized and resolved

2. Investigators need access to rich, detailed patient data to support research on effectiveness for patient subpopulations

3. Effective cross-organizational collaborations that establish key roles and responsibilities for team members were a key element of projects

4. Several skills were commonly needed

5. Work was sometimes more difficult than anticipated; with experience, project teams were able to overcome challenges

6. Clinical data collection is greatly facilitated by reducing data collection burden at the site of care
CER data infrastructure: implications for HHS

1. Provide regulatory guidance for holders of sensitive data to facilitate use in CER

2. Support development of data infrastructure that observes the effectiveness of different treatments in diverse populations

3. Support development of data infrastructure that also serves complementary purposes that enhance value to providers

4. Support ongoing costs of maintaining data infrastructure
ARRA CER projects: key findings and implications

- Dissemination and Translation
CER dissemination and translation: key findings

1. Understanding local context, culture, and resource constraints is important

2. Projects developed a variety of approaches and tools using diverse technology and media

3. The additional skills required in *Dissemination and Translation* projects included implementation science and communications
CER dissemination and translation: implications for HHS

1. Support development and dissemination of decision tools

2. Support engaging end users of CER findings in planning dissemination efforts

3. Promote health care delivery system efforts to translate CER into practice
Lessons for future evaluations

Eugene Rich, M.D.
Mathematica Policy Research
Questions addressed by the evaluation

- What are the lessons learned for evaluating the long-term impact of ARRA (or other) CER investments?
Revised logic model for evaluating impact of CER investments

**ENVIRONMENT**
- FCC Strategic Framework
- Institute of Medicine CER Priorities and Recommendations
- Key Motivations for CER: Rapidly rising healthcare costs, little evidence of improved quality, and gaps in value
- Grantors/Funders: OS, NIH, and AHRQ
- Grantees/Contractors College, universities, and private-sector firms
- Key Stakeholders: Patients, providers, consumers, health care delivery organizations, payers, innovators, and others
- Context: Building momentum for CER among policymakers and research community
- Concurrent federal policy initiatives, such as HITECH, health reform, others

**ACTIVITIES OR PROJECTS**
- Data Infrastructure Projects to Support CER: Registries, claims databases, community-based networks, and other activities
- Human and Scientific Capital to Increase CER Capacity: Training grants, research methods development, and other activities
- Dissemination and Translation to Inform Decision Makers: AHRQ, Community Forum, academic detailing, and other activities
- Research Activities to Generate Knowledge: Original research, Research syntheses, Research networks

**PROCESS METRICS/OUTPUTS**
- New/updated data sets
- Improved integration of existing datasets
- More clinical data collected at point of care
- New or expanded CER training programs
- Advances in methods for observational CER studies
- More projects developing point-of-care decision resources
- Added resources for implementing CER in usual care settings
- Increased translation and dissemination strategies
- More CER projects addressing priority populations, conditions, and interventions
- New interim reports, briefs, presentations, and other products

**MEDIATING FACTORS**
- Accessibility of new data to CER researchers
- Delivery system support for CER data infrastructure
- Availability of funding for new or follow-on projects
- Dissemination of methodologies
- Availability of funding expectations on decision makers to use CER
- Motivation of delivery organizations to promote CER use
- Coordination of activities across federal agencies
- Identification of ongoing research priorities
- More stakeholder engagement in project design

**INTERMEDIATE OUTCOMES**
- Improvements in CER usability
- Increased use of new CER databases
- More and higher-quality investigators
- Increased application of advanced methods
- Changes in CER-related attitudes and opinions
- Endorsement of CER by professional associations, USPSTF, payers
- Reduced uncertainty in treatment options
- More peer-reviewed CER publications
- Increased knowledge of CER findings
- More CER relevant to decision making
- More CER findings salient to stakeholders
- Incorporation of CER into tools and quality measures

**LONG-TERM OUTCOMES**
- Improved health care decision making by providers and patients
- Development and change in policies reflecting understanding of CER
- Provision of more effective, higher-quality health care
- Less inappropriate, ineffective, or unnecessary care
- Reduced unwarranted variation in health care practices
- Reduced disparities in health
- Improved overall population health, including for priority populations

**Changes in External Factors That Shape the CER Landscape:** Changes to public opinion, provider attitudes and training, payers' CER implementation practices, law (e.g., ACA, PCORI, CMMI), political and economic climate, and other factors
Revised Logic Model for Evaluating Impact of CER Investments

Environment

FCC Strategic Framework

Institute of Medicine CER Priorities and Recommendations

Key Motivations for CER: Rapidly rising health care costs, little evidence of improved quality, gaps in value

Grantors/Funders: OS, NIH, and AHRQ

Grantees/Contractors: Colleges, universities, and private-sector firms

Key Stakeholders: Patients, providers, consumers, health care delivery organizations, payers, innovators, and others

Context: Building momentum for CER among policymakers and research community

Concurrent federal policy initiatives, such as HITECH, health reform, others
Revised logic model: process metrics/outputs

• Data infrastructure
  – New/updated data sets

• Human and scientific capital
  – New or expanded CER training programs
  – Advances in methods for observational CER studies

• Research on comparative effectiveness
  – New interim reports, briefs, presentations, and other products

• Dissemination and translation
  – More projects developing point-of-care decisions resources
Revised logic model: intermediate outcomes

- Increased use of new CER databases
- More and higher quality investigators
- Increased application of advanced methods
- More peer-reviewed CER publications
- Endorsement of CER by professional associations
- Incorporation of CER into tools and quality measures
Revised logic model: long-term outcomes

- Improved health care decision making by providers and patients
- Less inappropriate, ineffective, or unnecessary care
- Reduced unwarranted variation in health care practices
- Reduced disparities in care
- Improved overall population health
Revised Logic Model for Evaluating Impact of CER Investments

**Mediating Factors**

- Accessibility of new data to CER researchers
- Delivery system support for CER data infrastructure
- Availability of funding for new or follow-on projects
- Dissemination of methodologies

**Availability of funding**

- Expectations on decision makers to use CER
- Motivation of delivery organizations to promote CER use

**Coordination of activities across federal agencies**

- Identification of ongoing research priorities
- More stakeholder engagement in project design

**Changes in External Factors that Shape the CER Landscape**: Changes to public opinion, provider attitudes and training, payers’ CER implementation practices, law (e.g. ACA, PCORI, CMMI), political and economic climate, and other factors
Revised logic model: mediating factors

• Mediating factors
  – Availability of funding for new or follow-on projects
  – Delivery system support for CER data infrastructure
  – Expectations on decision makers to use CER
  – Motivation of delivery organizations to promote CER
  – More stakeholder engagement

• External factors
  – Public opinion, providers’ attitudes and training
  – Payers’ CER implementation practices
  – Laws (for example, ACA, PCORI, CMMI)
  – Political and economic climate
Policy questions for evaluating CER investments

• “The {CER} funding in the conference agreement shall be used to conduct or support research to evaluate and compare the clinical outcomes, effectiveness, risk, and benefits of two or more medical treatments and services that address a particular medical condition.” (ARRA)

• Near-term expected results:
  – CER research, training, and infrastructure projects

• What is the impact of the CER investment on long-term outcomes such as “overall population health”?  
  – It would require decades to determine this
Policy questions for evaluating CER investments

• What is the impact of the CER investment on improved health care decision making by providers and patients?

• Has there been increased application of advanced CER methods?

• Have CER findings been incorporated into quality measures?

• Can only be understood in context
  – What environmental factors facilitated or challenged the achievement of these outcomes?
  – What mediating factors were most influential on achieving or not achieving these outcomes?
Research questions relevant to the work of CER funders

• Are CER projects meeting stated program goals?
• What gaps remain in strategic priorities for CER?
• What opportunities remain for further investment?
• Which project or research team features facilitated CER project progress or success?
  – Sustaining collaboration in multi-institutional clinical trials
  – Engaging research mentors in training projects
  – Facilitating data infrastructure projects
  – Overcoming barriers to stakeholder engagement
Lessons learned regarding methods for evaluating CER investments

• Publications as an intermediate outcome metric

• Social network analysis (SNA) as a method to assess project team features that facilitate projects

• Investigator and stakeholder surveys in evaluating the impact of CER investments
Assessing intermediate outcomes: role of publications as an outcome metric

• Advantages
  – Widely understood outcome and relatively transparent
  – Can be discretely counted
  – Many aspects of publication search can be automated
Assessing intermediate outcomes: role of publications as an outcome metric

• Technical challenges
  – Ease of detecting publications varies substantially based on agency funding
  – Grant numbers not standardized
  – When multiple grants cited, unable to determine contribution of funds from a specific grant
  – Need to search gray literature
  – Could use investigator survey, but disadvantages include costs and threats to reliability and validity
Assessing intermediate outcomes: role of publications as an outcome metric

• Conceptual challenges after data are collected
  – Comparing absolute number of publications or impact factor might not be informative
    • Varying conventions about publication across disciplines
    • Different types of projects have varying motivation or opportunity to publish
  – How to enumerate outcomes such as statistical methodology code, usability of CER databases, or dissemination of electronic health record decision support
Discussant reactions and commentary

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Audience Q & A

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Save the date!

Join us for the next Health Care Policy Research Forum

June 27, 2014
12:00 – 1:30 PM ET

“Measuring Comprehensiveness of Primary Care: Past, Present, and Future”