MATHEMATICA
CENTER ON
HEALTH CARE EFFECTIVENESS

The Recovery Act Investment in Comparative Effectiveness Research:

Interim Assessment and Implications

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

May 29, 2014

Welcome

Moderator

Eugene Rich, M.D.

Mathematica Policy Research

About CHCE

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

Sheldon Greenfield
U.C. Irvine

Eugene Rich
Mathematica

Sanford Schwartz
Univ. of Pennsylvania

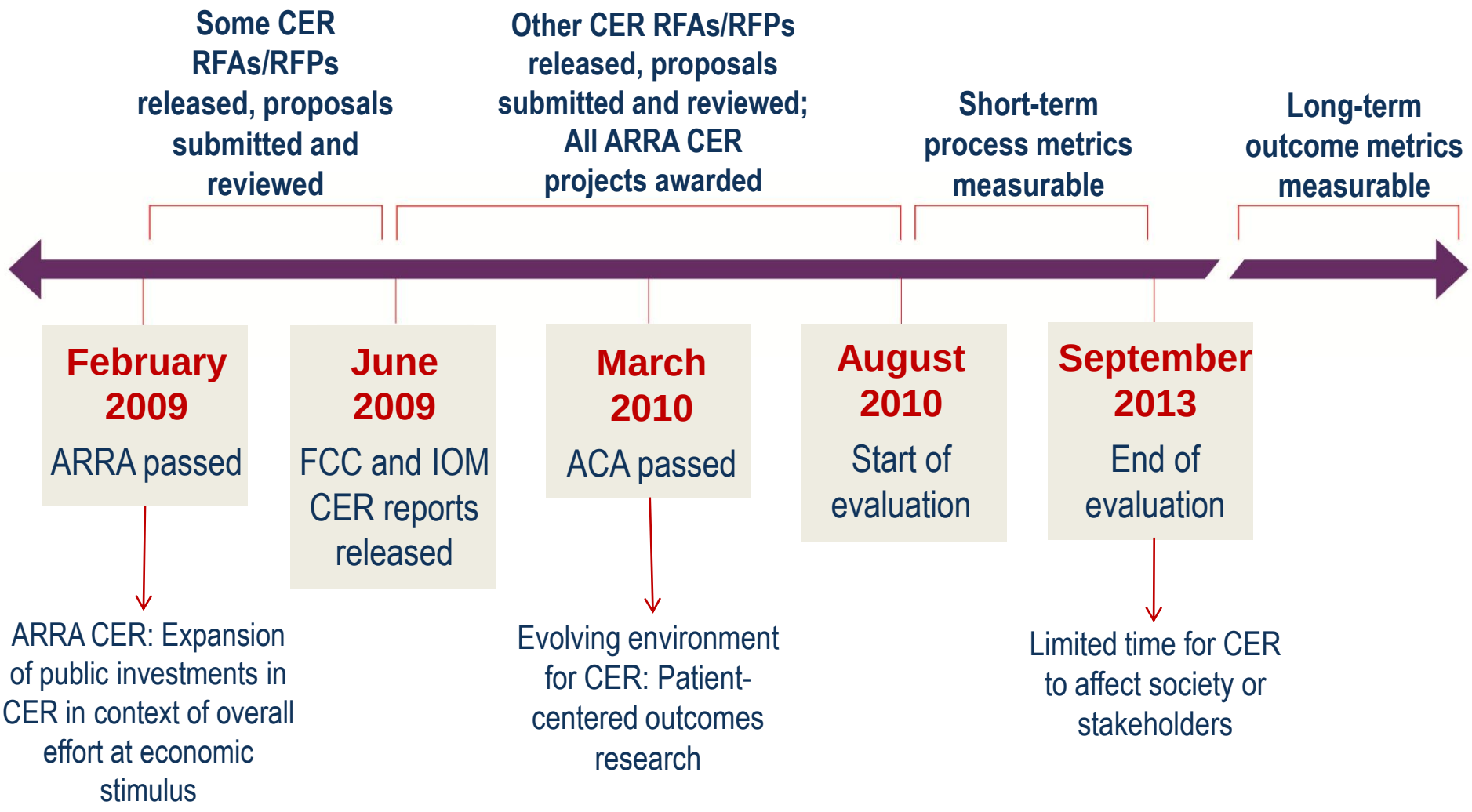
Bryan Luce
PCORI

Overview, findings, and implications

Dominick Esposito

Mathematica Policy Research

Context for the evaluation



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

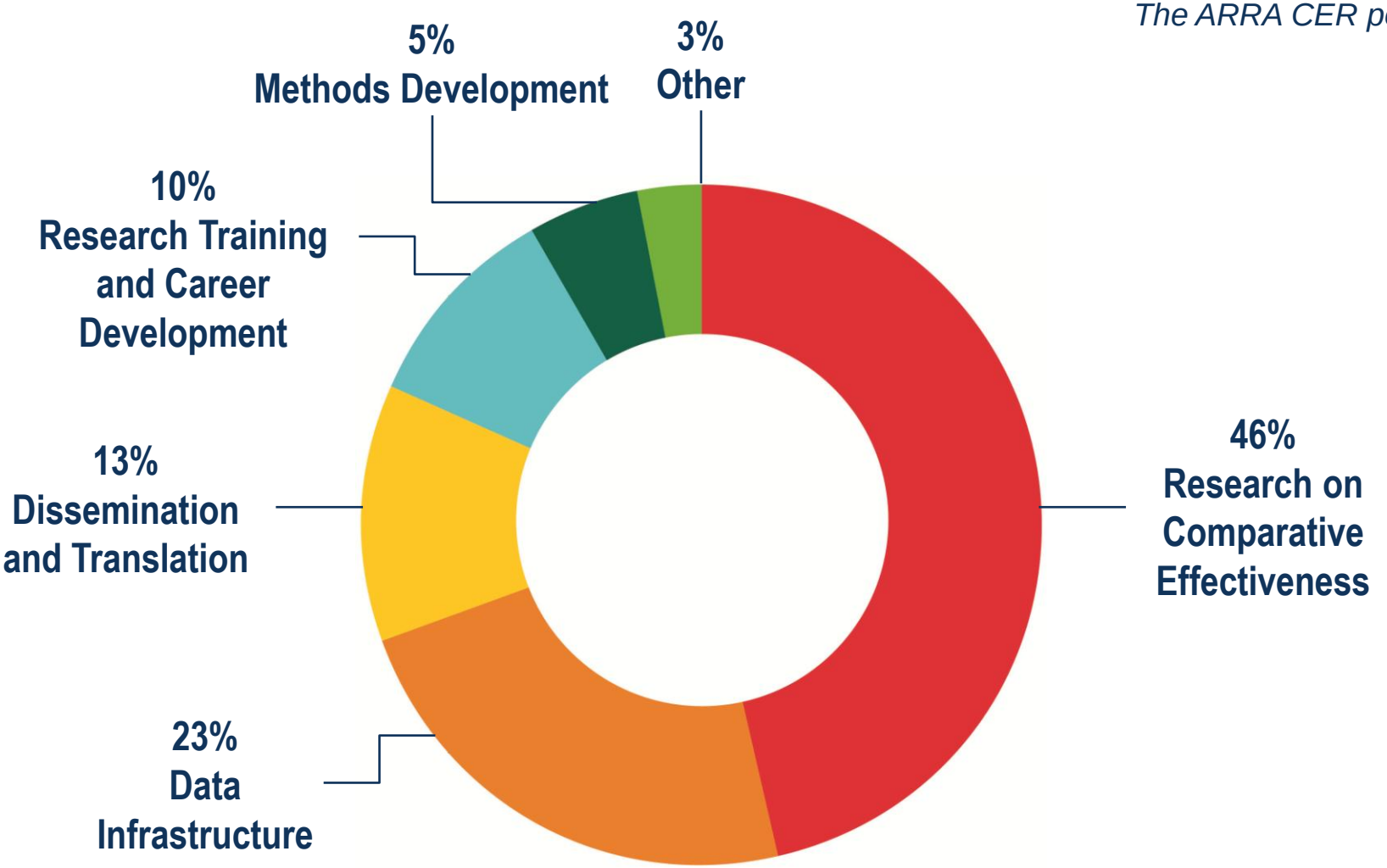
- What types of investments were made with ARRA CER funds?
- How has the ARRA CER portfolio of investments begun to address strategic priorities for CER?
- What midstream findings were identified that can inform HHS?
- What are the implications for future federal investments in CER?
- What are the lessons learned for evaluating the long-term impact of ARRA (or other) CER investments?

Questions addressed by the evaluation

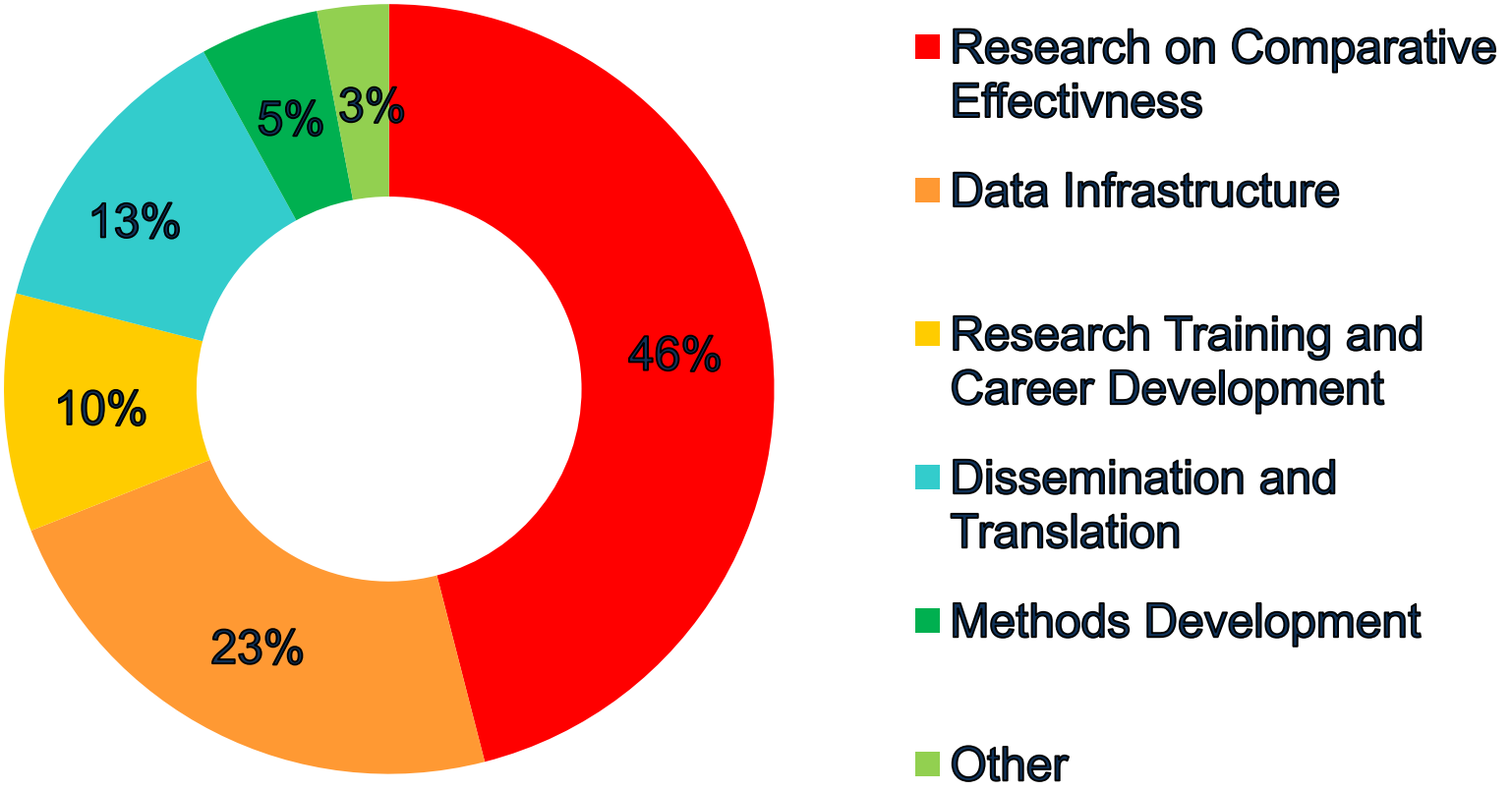
- What types of investments were made with ARRA CER funds?
- How has the ARRA CER portfolio of investments begun to address strategic priorities for CER?

Percentage of ARRA CER projects, by primary area of focus

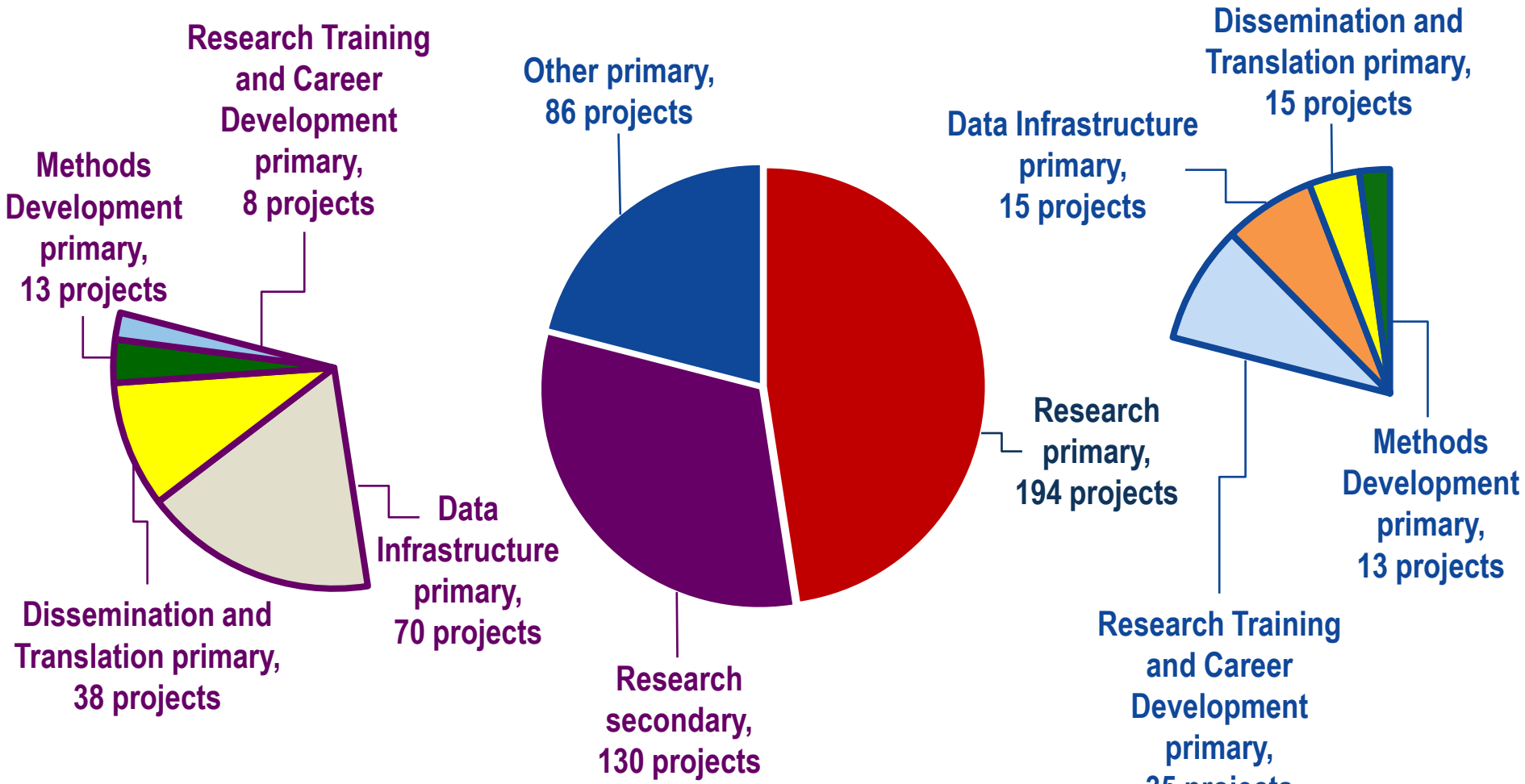
The ARRA CER portfolio



Percentage of ARRA CER projects, by primary area of focus



Many ARRA CER projects had multiple areas of focus



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

Priority themes and IOM topics addressed by ARRA CER projects

	Number of projects	Percentage of all projects	Percentage of projects by primary area of focus			
			Research	Data infrastructure	Human and scientific capital	Dissemination and translation
Any population, condition, or intervention	329	77.8	93.3	79.4	40.0	83.0
Priority population	192	53.3	50.3	56.7	12.7	56.6
Priority condition	260	61.3	78.6	49.5	28.6	71.7
Priority intervention	184	43.4	65.0	21.6	6.3	60.3
IOM priorities for CER	174	41.0	57.9	17.5	1.6	79.2

Project characteristics: IOM priority topics

The ARRA CER portfolio

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

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

Questions addressed by the evaluation

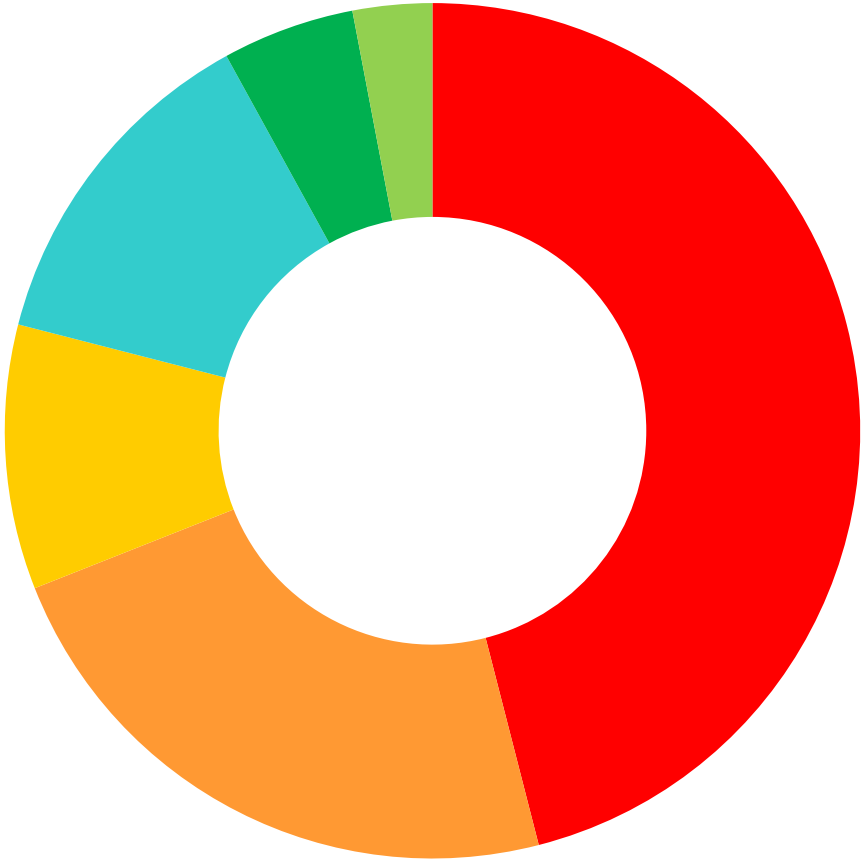
- What midstream findings were identified that can inform HHS?
- What are the implications for future federal investments in CER?

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 Effectivness
- Data Infrastructure
- Research Training and Career Development
- Dissemination and Translation
- Methods Development



ARRA CER projects: key findings and implications

- Research on Comparative Effectivness

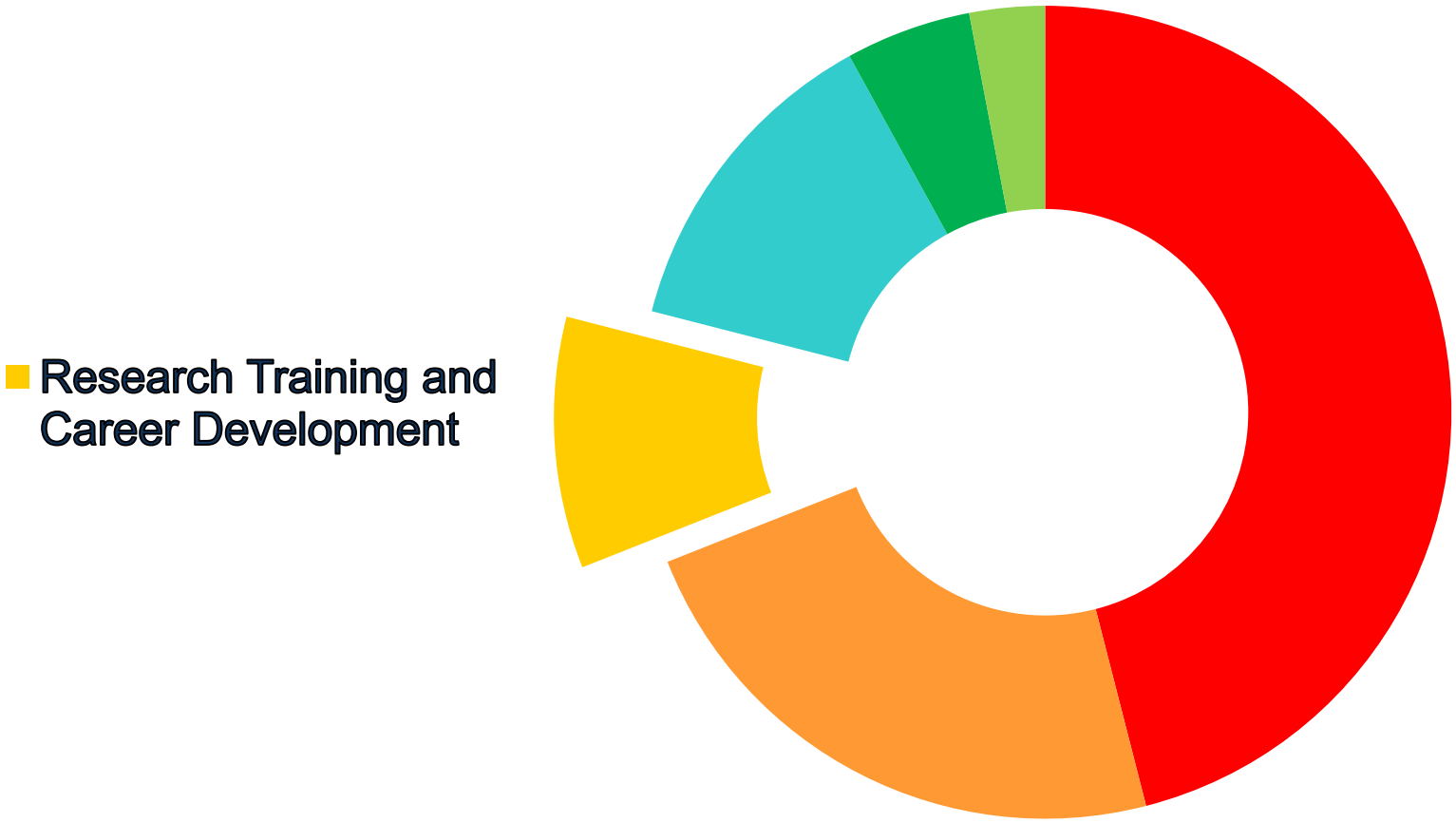
Research on comparative effectiveness: key findings

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



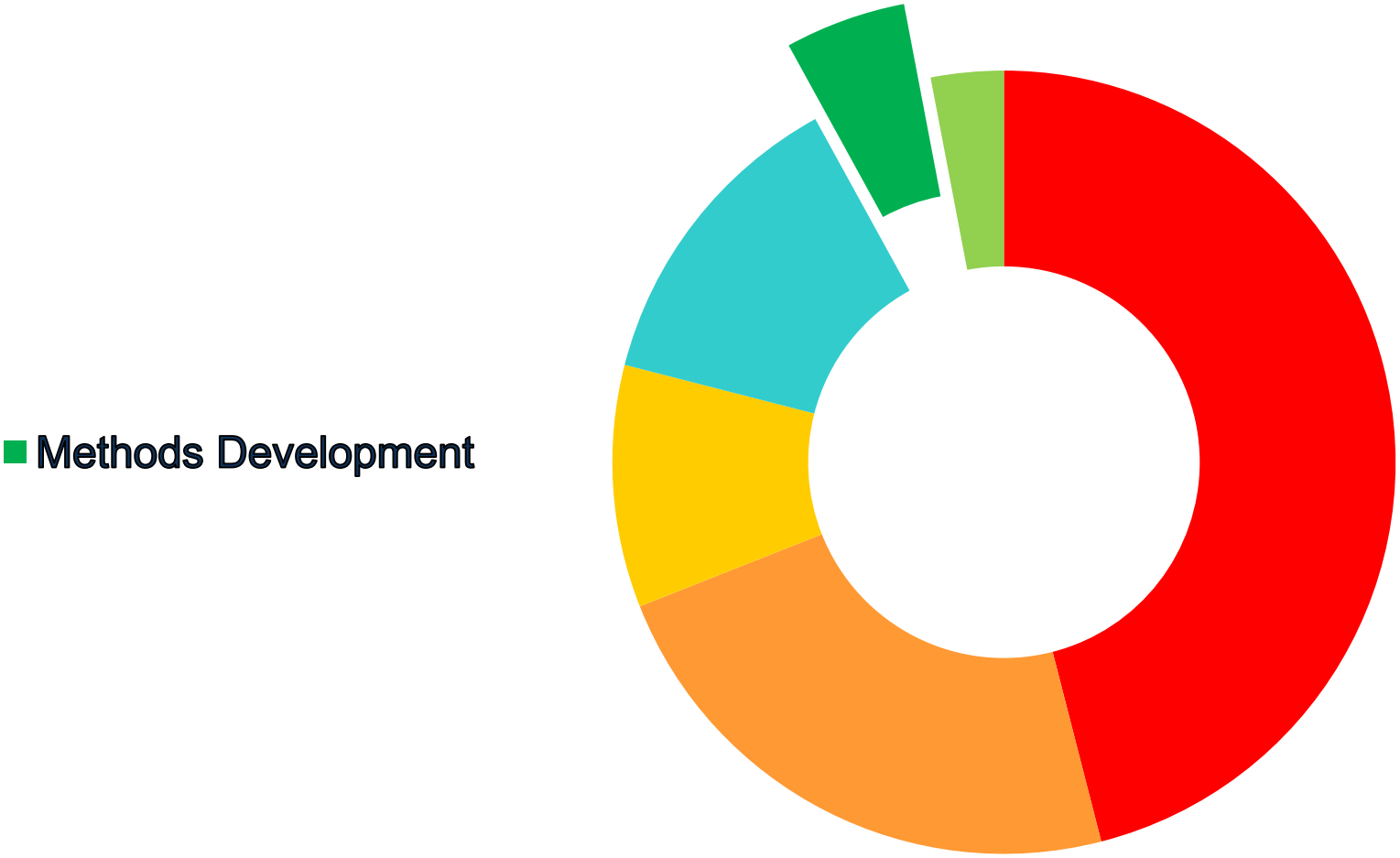
CER training: key findings

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



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

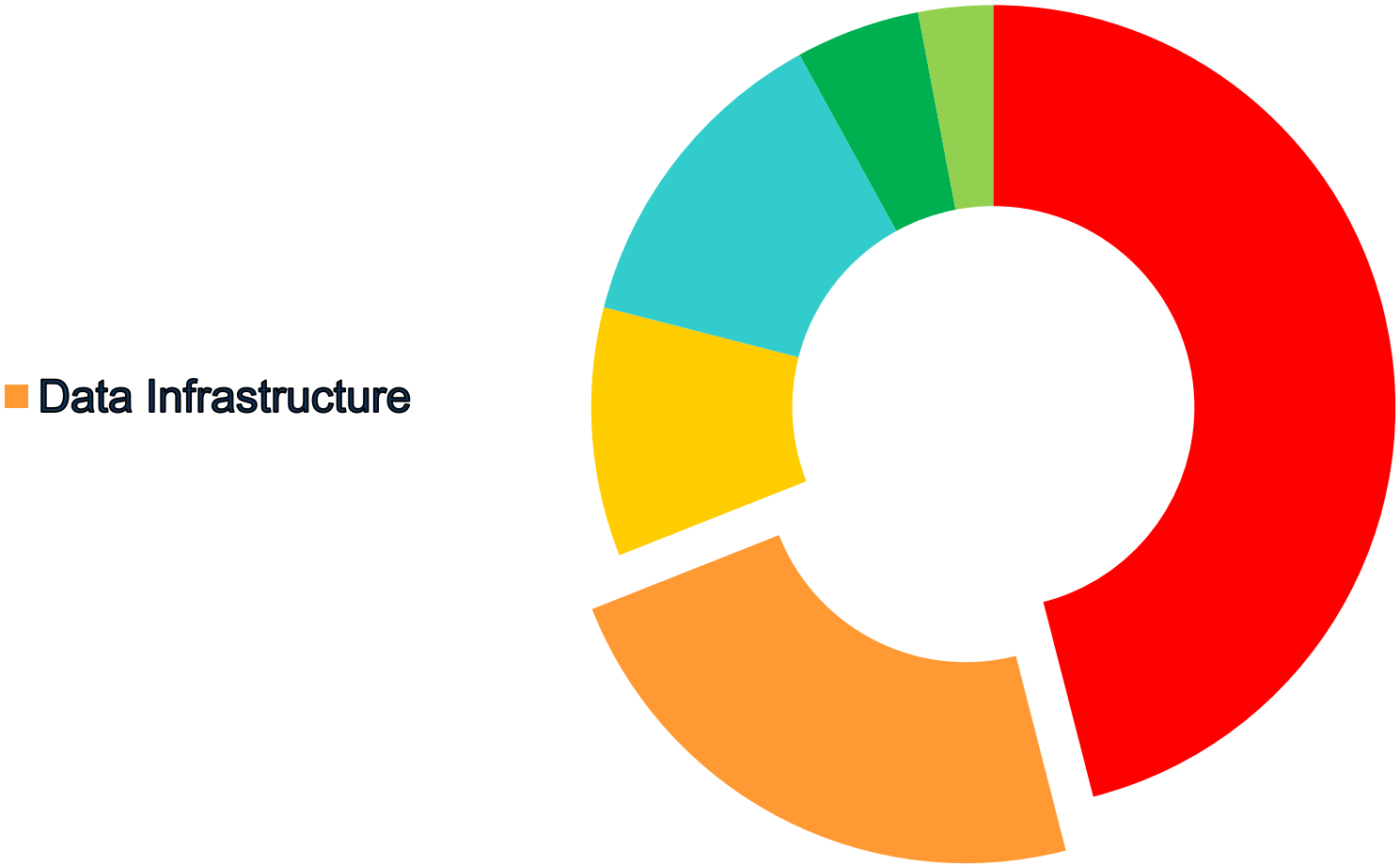
CER methods: implications for HHS

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



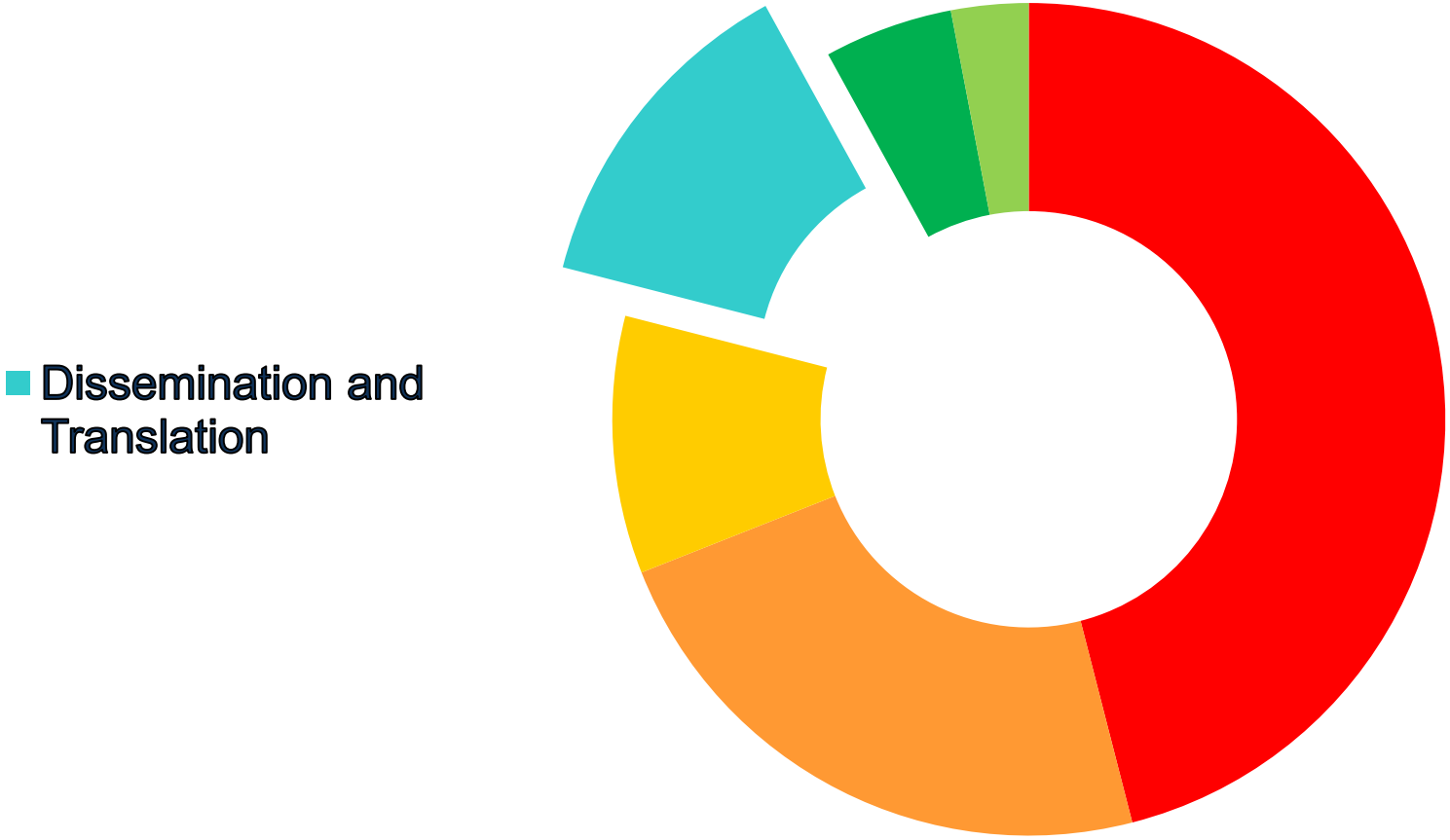
CER data infrastructure: key findings

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



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

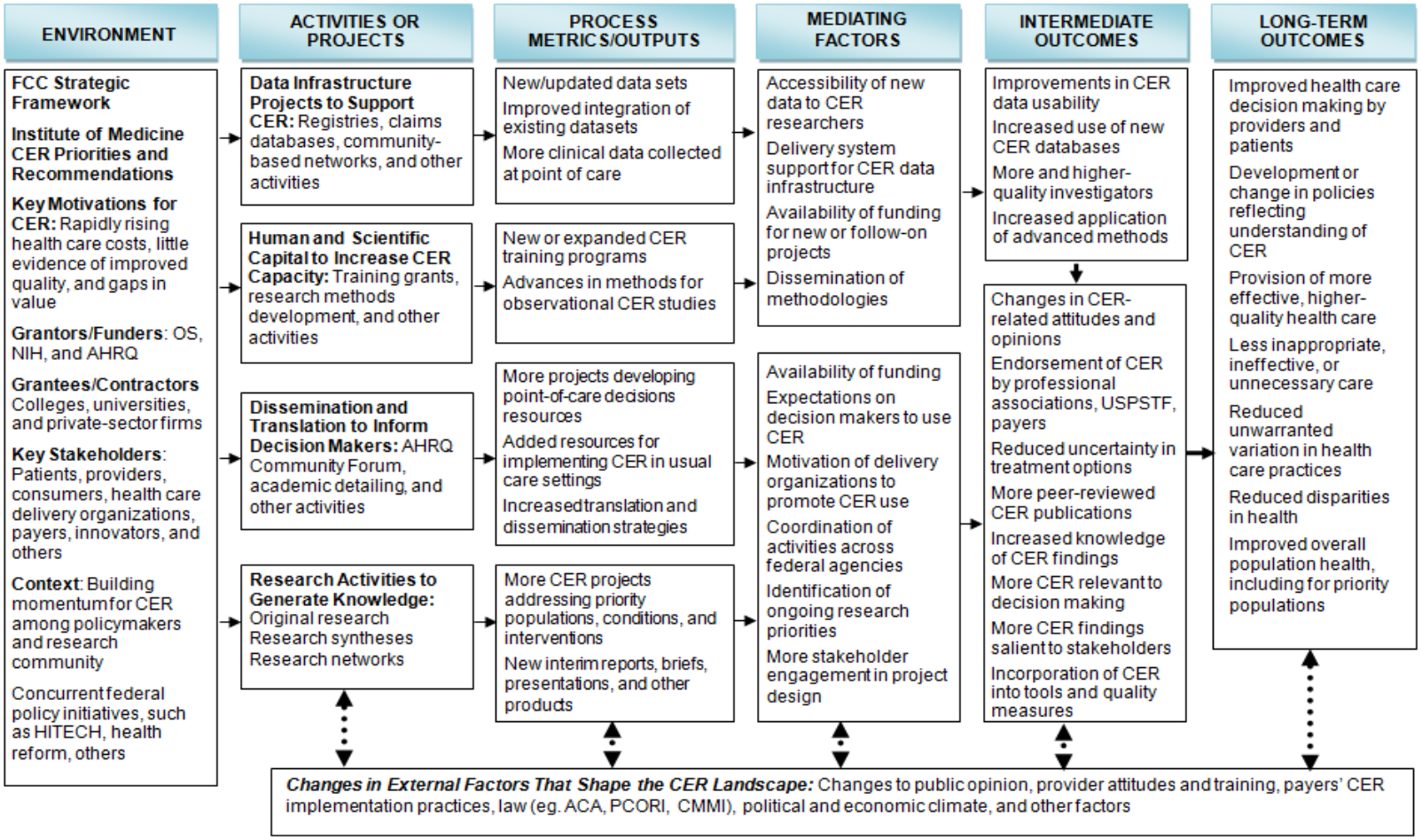
Eugene Rich, M.D.

Mathematica Policy Research

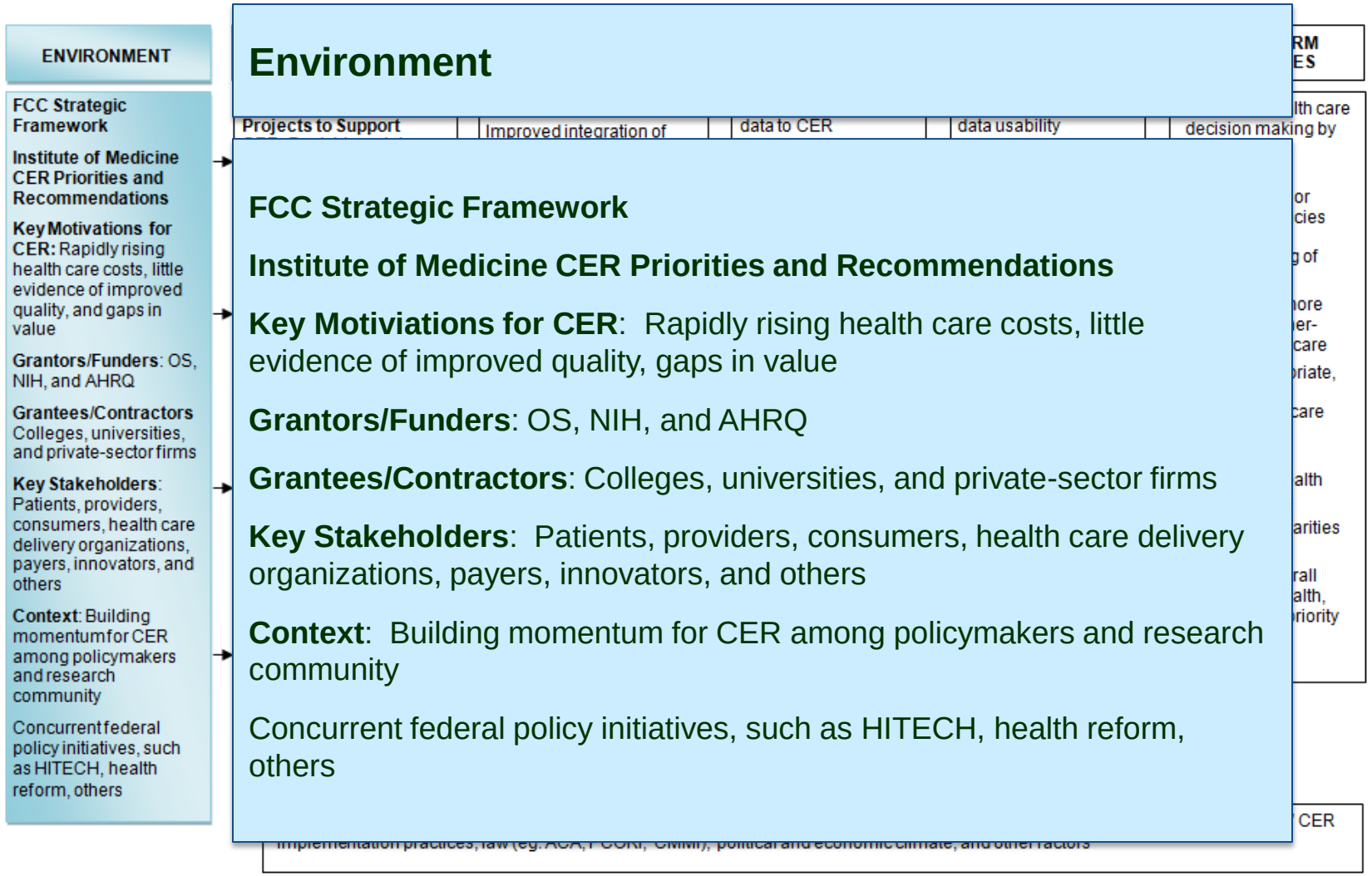
Questions addressed by the evaluation

- 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



Revised Logic Model for Evaluating Impact of CER Investments



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

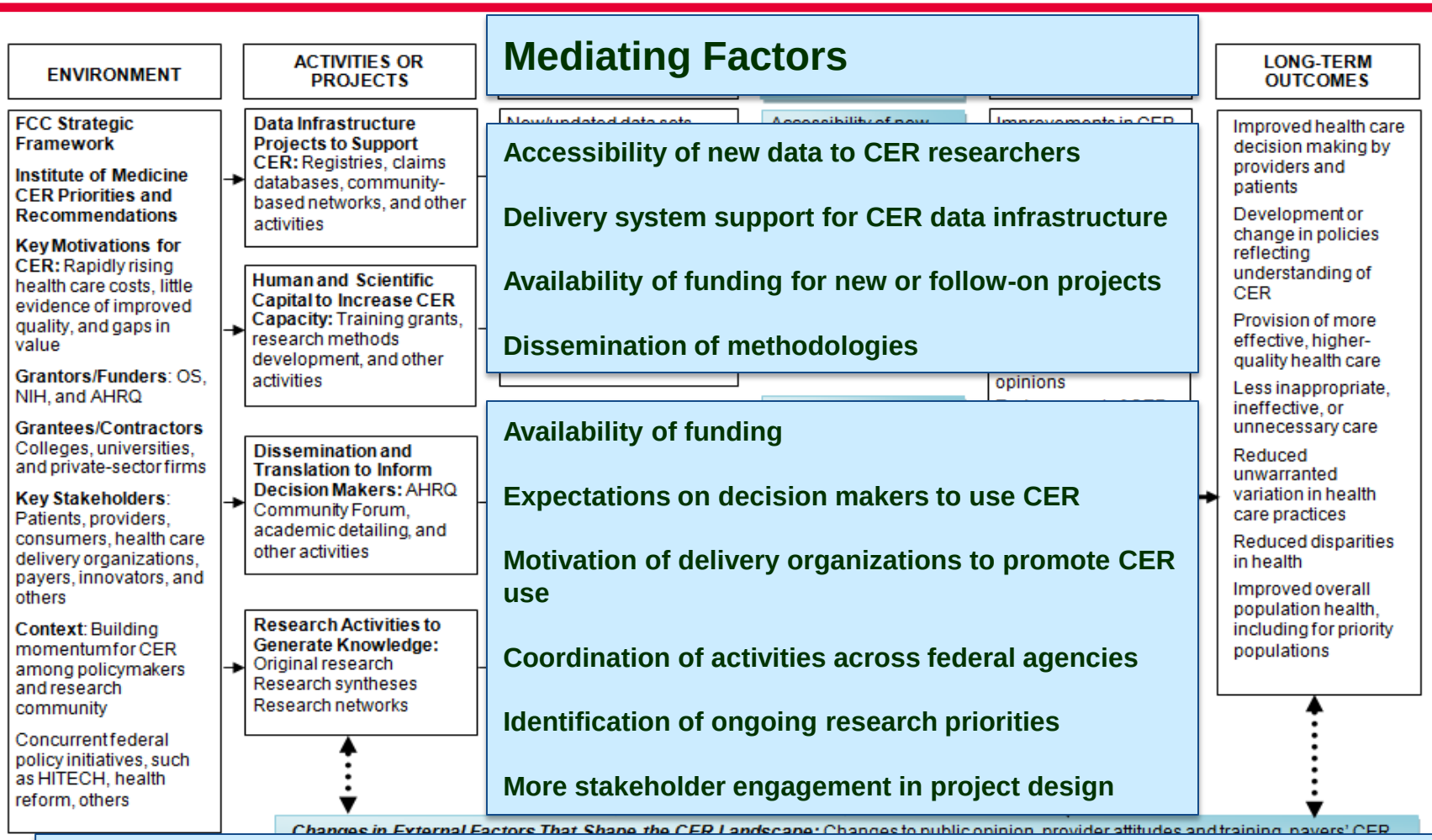
Revised logic model: intermediate outcomes

- 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



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

Sheldon Greenfield
U.C. Irvine

Sanford Schwartz
Univ. of Pennsylvania

Bryan Luce
PCORI

Discussant reactions and commentary

Sheldon Greenfield
U.C. Irvine

Sanford Schwartz
Univ. of Pennsylvania

Bryan Luce
PCORI

Discussant reactions and commentary

Sheldon Greenfield
U.C. Irvine

Sanford Schwartz
Univ. of Pennsylvania

Bryan Luce
PCORI

Audience Q & A

Dominick Esposito
Mathematica

Sheldon Greenfield
U.C. Irvine

Eugene Rich
Mathematica

Sanford Schwartz
Univ. of Pennsylvania

Bryan Luce
PCORI

For more information

Dominick Esposito: desposito@mathematica-mpr.com

Eugene Rich: erich@mathematica-mpr.com

CHCE: http://www.mathematica-mpr.com/chce/

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”