

Comparative Effectiveness Research: *Opportunities and Challenges for Transforming Health Care*

March 18, 2011

Learning Objectives

- Understand the critical role of comparative effectiveness research (CER) to provide information on the costs and benefits of different treatment options as a guide to fiscal policy
- Identify some of the key data issues in comparative effectiveness research
- Learn about the funding sources and agencies involved in comparative effectiveness research



Outline

- AcademyHealth – An Overview
- Forces driving CER
- Definitions and funding priorities
- Summary of recent funding
- PCORI & lessons learned internationally
- CTSA Key Function Committee on CER

Improving Health & Health Care

- As the professional society for health services researchers and health policy analysts, our mission is three-fold:
 1. Support the development of health services research
 2. Facilitate the use of the best available research and information
 3. Assist health policy and practice leaders in addressing major health challenges

- We work to both “**push**” the production of research and promote the “**pull**” by decision makers



Programs Support the Mission

Methods seminars
EDM Forum
NLM HSRProj
RWJF PHSSR
Fellowships



Improving the field

RWJF's HCFO
Annual Research Meeting
Health Policy Orientation



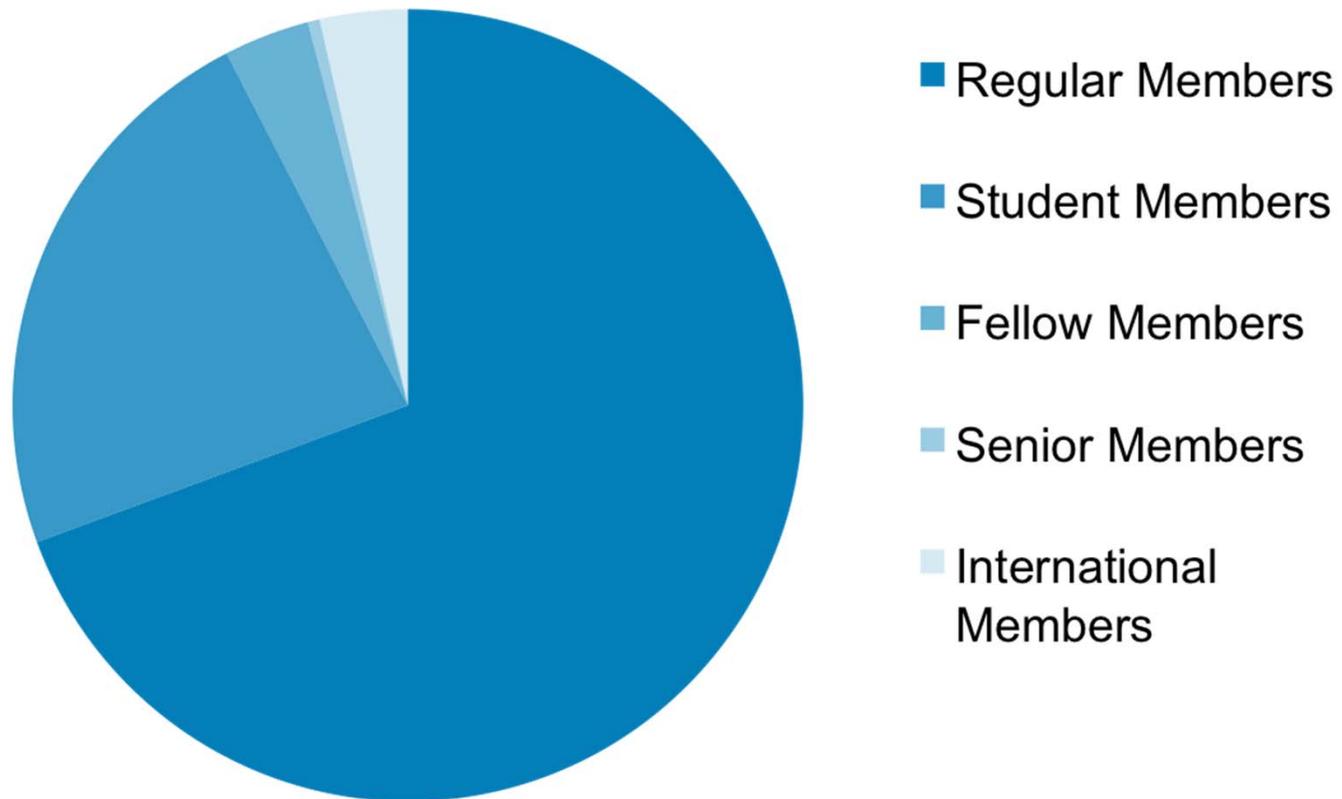
Using the best
available research
and information

SCI
AHRQ Medicaid Medical
Director's Learning Network
State Quality Improvement
Institute

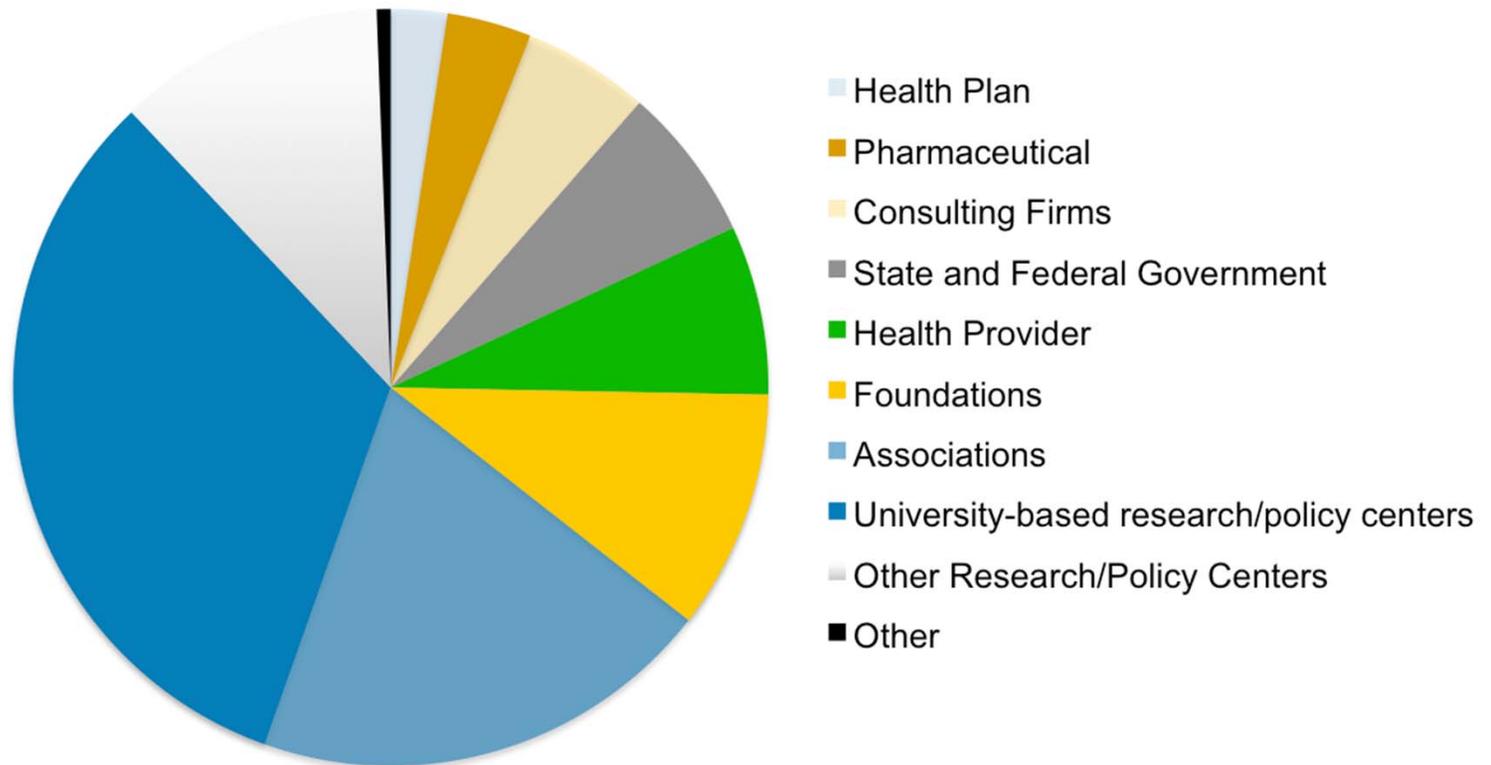


Assisting health policy
and practice leaders

Leveraging >4,000 Diverse, Expert Members



Organizational Affiliates Add Reach



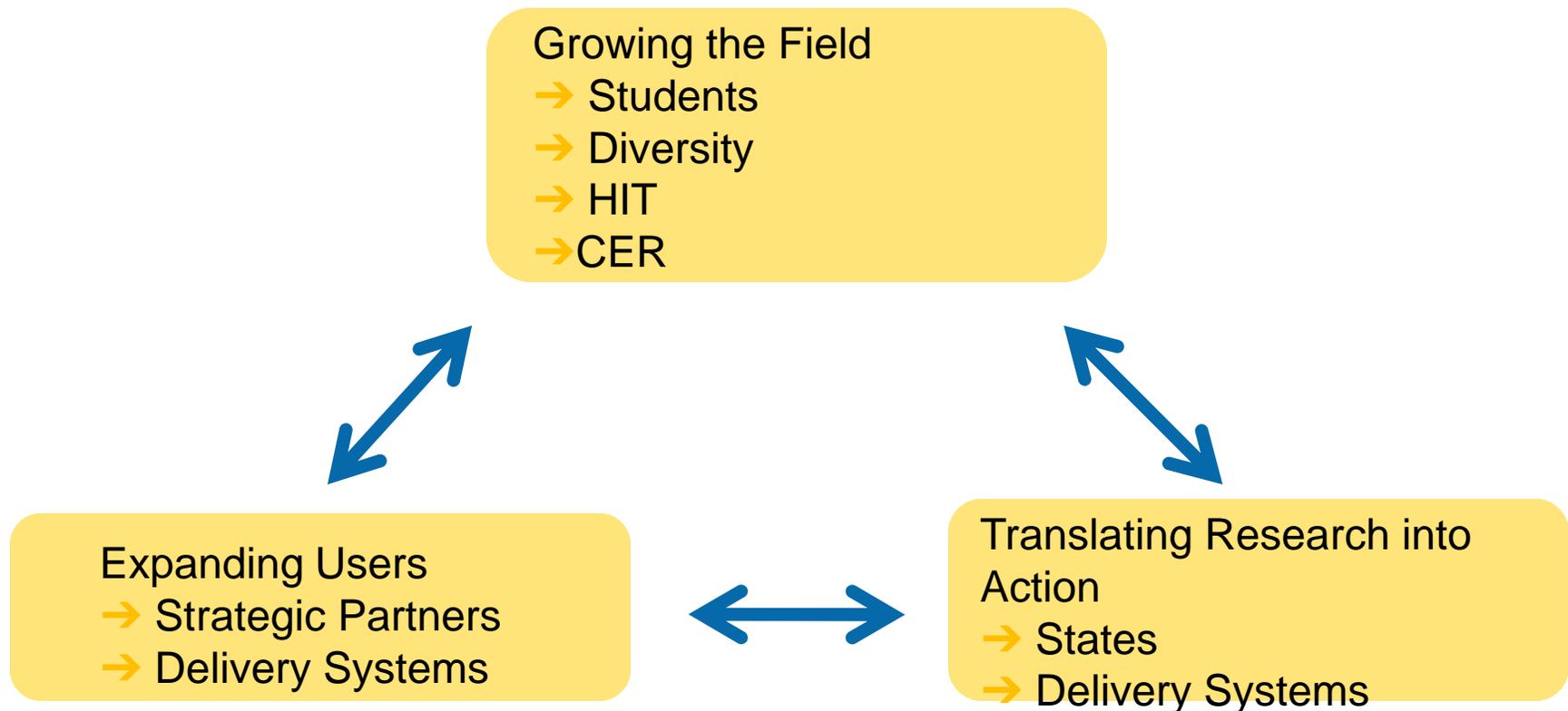
Interest Groups Add Value

- Behavioral Health Services Research
- **Child Health Services Research**
- Disability Research
- Disparities
- Gender and Health
- Health Economics
- Health Information Technology
- Health Policy Communications
- Health Workforce
- Interdisciplinary Research Group on Nursing Issues
- Long-Term Care
- Public Health Systems Research
- **Quality**
- Research Translation
- State Health Research and Policy

Stable Foundation & Broad Reach

- \$10 million budget
- 50 staff
- Reach:
 - Database of >80,000 research & policy experts
 - >4000 members
 - >2,000 at Annual Research Meeting
 - >800 at annual National Health Policy Conference
- Growth in last 2 years:
 - 10% per year in membership

2011 Priorities: Grow the Field, Expand Reach and Translation



At the Forefront of CER

- Electronic Data Methods Forum
- Multi-payer Claims Databases
- CER Inventory (Lewin)
- CER Institute (CMTP)
- CER at the ARM

EDM Forum: Aims

- Advance methods related to developing the infrastructure and methodology for collecting and analyzing prospective electronic clinical data for CER
- Serve as the learning forum and sustainable foundation for CER using prospective electronic clinical data
- Focus on a specific set of issues related to implementing CER with electronic clinical data in the following domains:
 - Scientific
 - Clinical
 - Technical
 - Organizational
 - Data Governance



CER Research Projects

- Prospective Outcome Systems using Patient-specific Electronic data to Compare Tests and therapies (PROSPECT) studies (n=6)
- Enhanced Registries for Quality Improvement and CER (n=2)
- Scalable Distributed Research Networks for CER grant programs (n=3)



Leadership: Sub-Committees

- Analytic methods for CER
- Clinical informatics
- Data governance (data stewardship & privacy, including HIPAA, IRB's, etc.)



Stakeholder Symposium: April 8th

- Designed to engage in dialogue with key CER stakeholders (both users and producers of CER).
- Key objectives:
 - Update the community on the work of the EDM Forum and the participating research projects (PROSPECT Studies, Enhanced Registries, and Scalable Distributed Research Networks).
 - Provide stakeholders with the opportunity to:
 - share innovative ideas and/or provide updates on projects they are involved in that have relevance for the EDM Forum; and
 - provide input on priority topics and activities the EDM Forum should undertake.
 - Encourage participant engagement with the EDM Forum online to provide feedback and share information.



Related Websites

- Research Portal (March 2011)
 - Secure online space to collaborate within and across research teams
- Stakeholder Public Website (April 2011)
 - Channel for promoting and disseminating EDM Forum products
 - Will allow stakeholders to submit commentary on commissioned papers and issue briefs

Research Team Site Visits

- Bay Area, CA: March 10-11
 - Comet (Kushida); SUPREME-DM (Selby)
- New York, NY: April 29
 - WICER (Wilcox)
- Cincinnati, OH: May 6
 - PEDSNet (Hutton)
- Seattle, WA: June 10
 - SCOAP (Flum)
- Denver, CO: June 28-29
 - SPAN (Steiner); SAFTINet (Schilling)



Emerging Topics: Analytic Methods

- Policy brief on major CER study designs and challenges presented by each
- Policy brief on the Best practices for incorporating patient-reported data into EHRs
- A two-paper series on assessing data (both data availability/missing data and data quality) across health systems
 - Paper 1: Describe the issues and different ways to assure quality when drawing data from various systems, outlining the lessons learned, and enumerating potential problems
 - Paper 2: Technical focus on approaches to assessing and handling missing data
- Methodological issues related to identifying cohorts (especially across systems and types of data – e.g. EMR and claims)
 - Two case examples will be used based on the diabetes and asthma teams (Selby and Lieu)



Emerging Topics: Clinical Informatics

- Policy brief on data warehousing v. distributed data approaches: understanding of the landscape and potential issues related to conducting CER. Those in the clinical informatics sub-committee have recommended writing the following two briefs or papers instead
- Paper on cloud computing and the risks of hosting technologies, including privacy and proprietary/data ownership issues
 - Ideally this paper would provide guidance for IRBs faced with making a security and privacy determination about the use of these technologies.
- A two-paper series on clinical informatics platforms:
 - Information platforms for comparative effectiveness research (e.g. i2b2, HMORN, OMOP, and homegrown systems such as RedX).
 - Criteria for selecting information platforms (see examples above).



Emerging Topics: Data Governance

- Policy brief that will provide a thorough review of state laws that are applicable to data sharing and re-use
- Technical brief on multi-site IRB approval for CER, with an overview of key issues “pathways to success” for seeking IRB approval
- Technical brief that includes a review of DUAs and ‘Round Robin’ approaches to multi-site approval
- A technical paper on de-identification that gets deeper into the range of technical issues/options for de-identifying data



Outline

- AcademyHealth
- Forces driving CER



Interest in comparative information is not new...

- In US, used for payment/coverage and cost containment and grows whenever interest in competition
- Many health plans have “in house” capabilities
- Internationally, long standing efforts
 - UK
 - Australia
 - Canada
 - Germany
- Focus on pharmaceuticals

Great Expectations for CER

- “Better information about the costs and benefits of different treatment options, combined with new incentive structures reflecting the information...is essential to putting the country on a sounder long-term fiscal path.”
 - Peter Orszag testimony, June 2007

ACA (*a.k.a. Health Reform*)

Patient Centered Outcomes Research Institute (PCORI)

- Non-profit organization to assist patients, clinicians, purchasers, and policy-makers in making informed health decisions
- carrying out research projects that provide quality, relevant evidence on how diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed.
- 19 member Board appointed by GAO & AHRQ and NIH Directors
- GAO to appoint 15 members to a Methodology Committee of PCORI



Outline

- AcademyHealth – An Overview
- Forces driving CER
- **Definitions and funding priorities**



IOM Committee's Definition of CER

The generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition **or to improve the delivery of care**. The purpose of CER is to assist consumers, clinicians, purchasers, and policy makers to make informed decisions that will improve health care at both the individual and population levels.



Federal Coordinating Committee CER Definition

- Comparative effectiveness research is the conduct and synthesis of research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat and monitor health conditions in “**real world**” settings.
- The purpose of this research is to improve health outcomes by developing and disseminating evidence-based information to patients, clinicians, and other decision-makers, responding to their **expressed needs**, about which interventions are most effective for which patients under specific circumstances.



ACA Definitions

→ CER:

- “research evaluating and comparing health outcomes and the clinical effectiveness, risks, and benefits of 2 or more medical treatments, services, and items.”

→ “medical treatments, services, and items”:

- defined as “health care interventions, protocols for treatment, care management, and delivery, procedures, medical devices, diagnostic tools, pharmaceuticals (including drugs and biologicals), integrative health practices, and any other strategies or items being used in the treatment, management, and diagnosis of, or prevention of illness or injury in, individuals.”



CER Defining Characteristics

- Objective of directly informing clinical or health policy decision
- Compares at least 2 alternatives
- Results at population and subgroup level
- Measures outcomes important to patients
- Methods and data sources appropriate for the decision of interest
- Conducted in real world settings

Sean Tunis, 2009

Categories of CER Methods

- Systematic reviews of existing research
- Decision modeling, with or without cost information
- Retrospective analysis of existing clinical or administrative data
 - Significant new investments in data infrastructure
- Prospective non-experimental studies, including registries
- Experimental studies, including randomized clinical trials (RCTs)

Sean Tunis, 2009

All methods have a role

- Inevitable tradeoff between internal validity and feasibility, generalizability, cost, time
- The nature of the research question, and the decision maker will influence best practices
- Experimental studies will have a crucial role in CER, and there is need for improving design and implementation
- Non-experimental methods hold great promise, particularly as methods are refined and data infrastructure is improved

Sean Tunis, 2009

CER Methods

- June 2010 supplement to *Medical Care* on CER Methods
- 22 original articles, special focus on:
 - Ways to enhance the inclusion of clinically heterogeneous populations in clinical and comparative effectiveness studies
 - Methods for implementing longitudinal investigations that capture longer term health outcomes, including patient
- Printed copies available free of charge through the AHRQ Publications Clearinghouse





TAILORING RESEARCH

By Lisa A. Simpson, Laura Peterso
Zhaoxia Ren, and Anne Zajicek

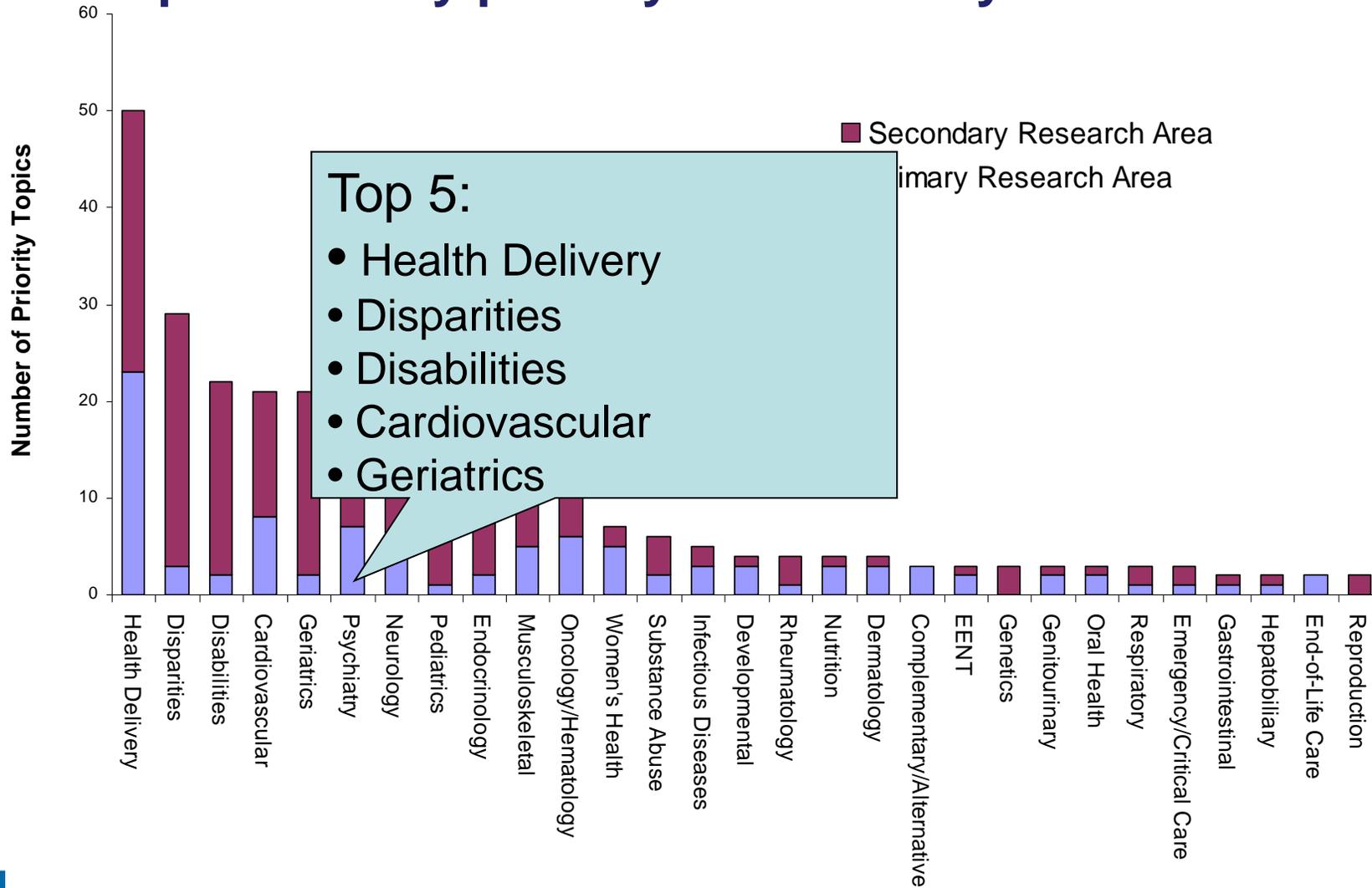
Special Challenges in Comparative Effectiveness Research On Children's And Adolescents' Health

Unique Dimensions

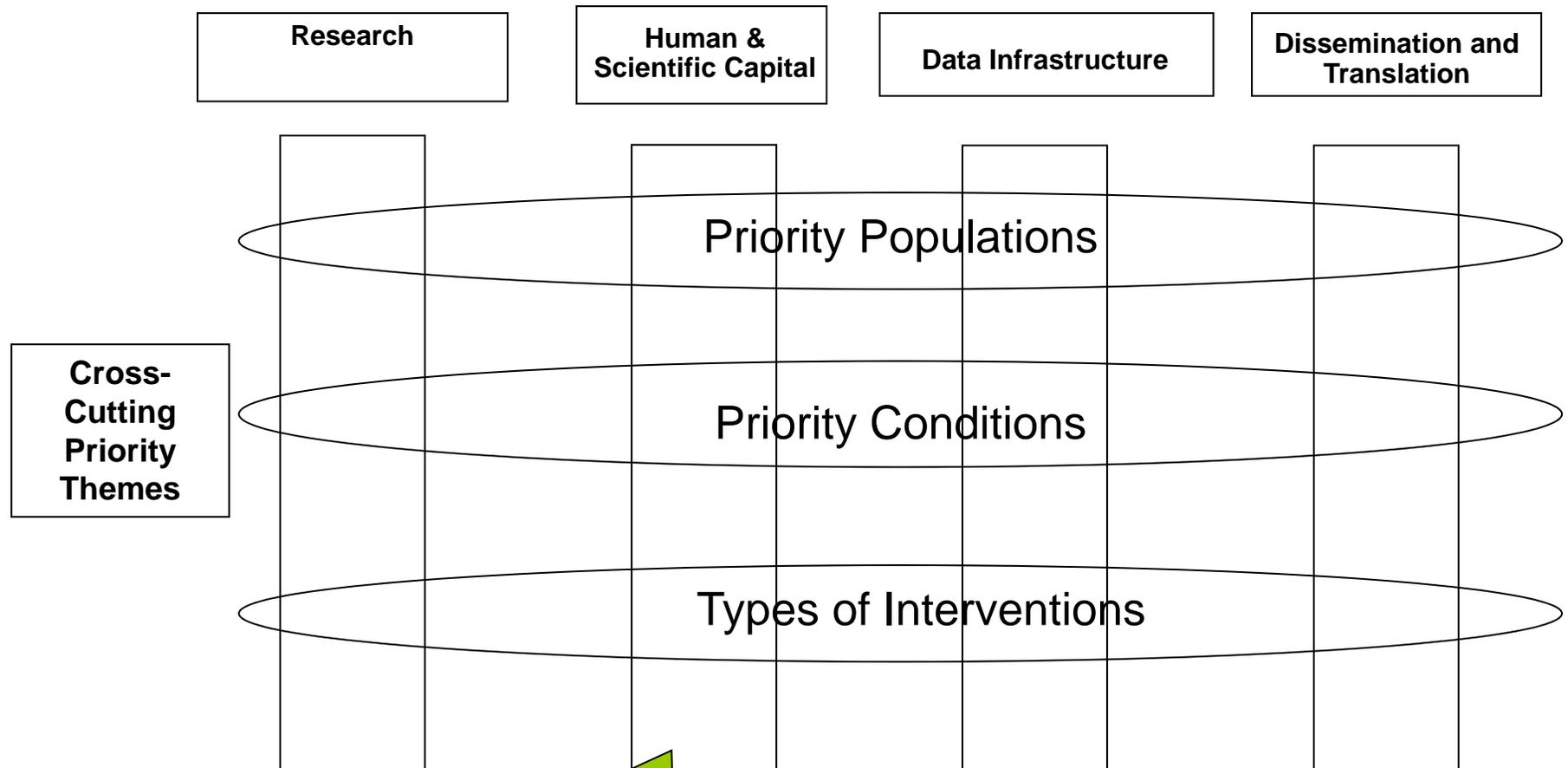
- Four “Ds”
 - Development
 - Differential epidemiology
 - Dependency
 - Demographics

IOM Priorities:

Distribution of the recommended research priorities by primary & secondary research areas



FCC Report: Strategic Framework for CER



Investments can be made in a single category and/or be cross-cutting in one of the themes

Outline

- Forces driving CER
- Definitions and funding priorities
- **Summary of recent funding**



Allocations for the \$1.1 billion in comparative effectiveness research funding in the American Recovery and Reinvestment Act of 2009:



- | | |
|---|----------------|
| ■ Research | ■ \$681M (62%) |
| ■ Data Infrastructure | ■ \$268M (24%) |
| ■ Dissemination and Adoption | ■ \$132M (12%) |
| ■ Administrative support, inventory, evaluation | ■ \$19M (2%) |



FCC and IOM Recommendations Guide ARRA Funding

FCC-CER

- Data infrastructure
- Dissemination and translation
- Human and scientific capital
- Real-world settings for subpopulations, priority conditions and interventions

IOM

- 100 top priority CER topics
 - 50% focus on health care delivery systems
 - Only three of the topics are narrowly focused on drug vs. drug

Select examples of AHRQ funding

- Enhanced State Data for Analysis and Tracking of Comparative Effectiveness Impact: Improved Clinical Content and Race-Ethnicity Data
- Registry of Patient Registries

Select examples of OS funding

- Electronic Data Methods (EDM) Forum for Comparative Effectiveness Research
- Enhanced Registries for Quality Improvement and Comparative Effectiveness Research



Data Infrastructure Investments: OS Spend Plan (\$210.5M)

Medicare Claims	CMS	\$35 million
Medicaid Claims	CMS	\$19.5 million
Clinically Enhanced State Data	AHRQ	\$8 million
Design and Implementation	ASPE/CMS	\$19 million
Distributed Networks	AHRQ	\$25 million
Community Health Applied Research Network	HRSA	\$10 million
Patient Registries	AHRQ	\$20 million
Cancer Registries	CDC	\$20 million
Registry of Registries	AHRQ	TBD
FDA: Medication and Device CER	FDA	\$20 million
Data and Research: Chronic Conditions	AHRQ/IHS	\$27 million
Pediatric Care Networks	HRSA	\$7 million

NIH Challenge Grants

- 8416 Challenge Grants funded under ARRA.
 - 116 projects focus on CER topics
 - 76 of which can be classified as HSR.
- This represents 9% of the ARRA Challenge Grants
- In total, NIH ARRA funding for Challenge Grants focused on HSR-related CER represents more than \$35.4 million in new investment in the field.



Figure 1. NIH ARRA Challenge Grant Awards on HSR-Related CER, by Institute, November 2009

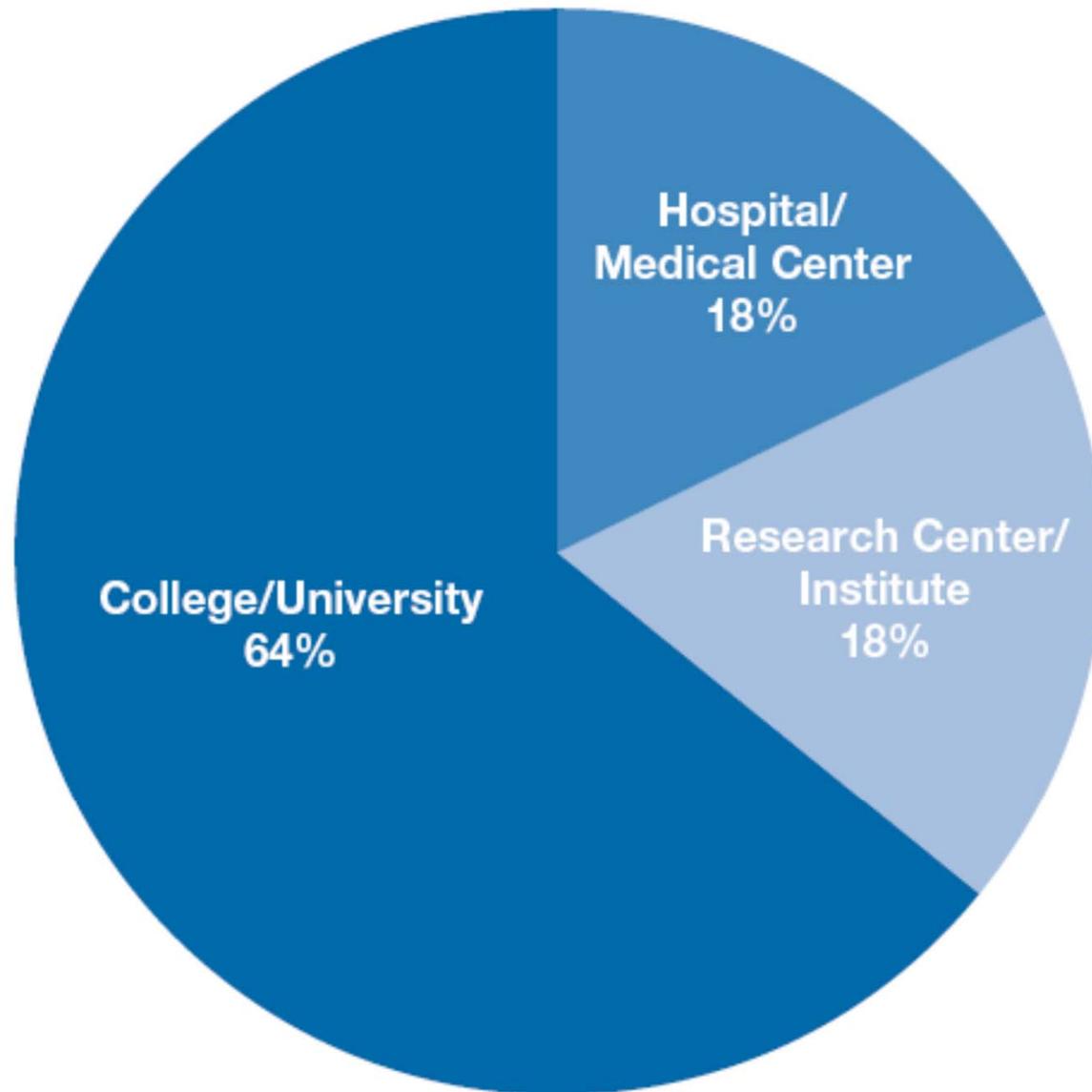
NIH Center/Institute	Number of Challenge Grants with Keywords "Comparative Effectiveness"	Number of HSR CER Challenge Grants	Funding (\$)
National Cancer Institute (NCI)	15	13	\$ 5,765,520
National Center for Complementary and Alternative Medicine (NCCAM)	4	2	\$ 995,326
National Center for Research Resources (NCRR)	5	4	\$ 1,915,783
National Center on Minority Health and Health Disparities (NCMHD)	9	7	\$ 3,316,135
National Eye Institute (NEI)	2	1	\$ 454,142
National Heart, Lung and Blood Institute (NHLBI)	13	9	\$ 4,446,708
National Institute of Allergy and Infectious Diseases (NIAID)	3	2	\$ 998,967
National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)	5	3	\$ 1,497,605
National Institute of Biomedical Imaging and Bioengineering (NIBIB)	6	3	\$ 1,488,120
National Institute of Child Health and Human Development (NICHD)	2	0	\$ 0
National Institute of Dental and Craniofacial Research (NIDCR)	4	4	\$ 1,882,347
National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)	5	2	\$ 986,399
National Institute of Mental Health (NIMH)	5	4	\$ 1,678,552
National Institute of Neurological Disorders and Stroke (NINDS)	6	3	\$ 1,494,999
National Institute of Nursing Research (NINR)	1	1	\$ 496,230
National Institute on Aging (NIA)	15	5	\$ 2,098,357
National Institute on Alcohol Abuse and Alcoholism (NIAAA)	6	6	\$ 2,609,113
National Institute on Drug Abuse (NIDA)	8	5	\$ 2,417,214
National Library of Medicine (NLM)	2	2	\$ 897,606
Total	116	76	\$ 35,439,123

Source: National Institutes of Health Research Online Portfolio Tool Expenditures and Report (RePORTER). Data shown are from records retrieved from RePORTER on November 13, 2009.

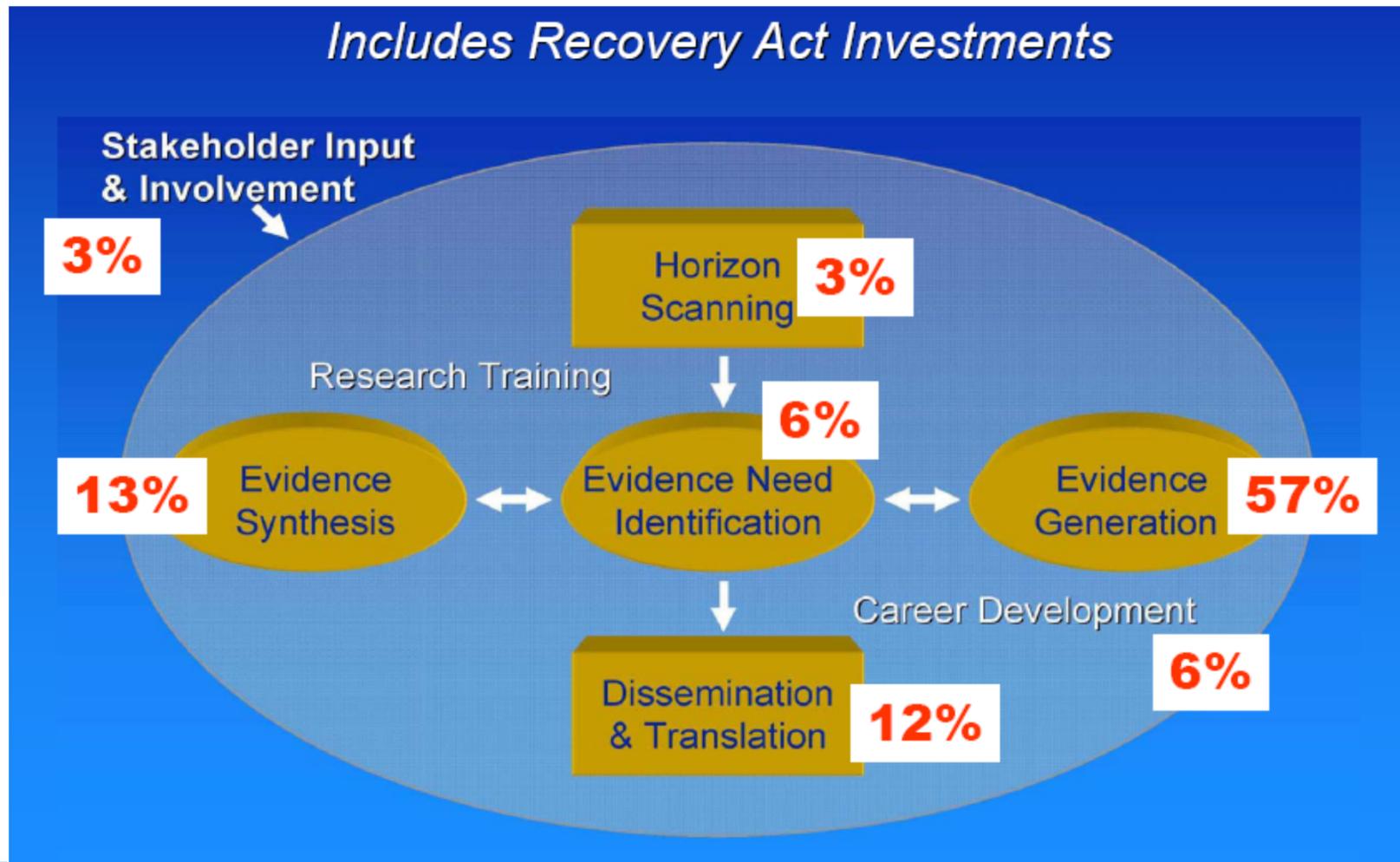
NIH Dissemination

<http://www.ncbi.nlm.nih.gov/pubmedhealth/>

Figure 2. Distribution of Challenge Grant Funding for HSR Projects Across Performing Organizations



AHRQ CER Investments



RFI for CER Inventory

- Sources for CER
- Encouraging participation/submission
- Categorization
- Data elements
- Features
- Sustainability
- Additional considerations

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- **PCORI & lessons learned internationally**



PCORI Funding

- Patient-Centered Outcomes Research Trust Fund (PCORTF)
- For FYs 2010-2012, appropriations:
 - \$10M, \$50M, \$150 M
- For FYs 2013-2019,
 - \$150 million in appropriations **PLUS**
 - Transfers = the average # of individuals enrolled in the Medicare, health insurance policies, and self-insured plans x:
 - \$1 for FY 2013;
 - \$2 for FY 2014;
 - \$2 for FYs 2015 through 2019 (w/inflation).

PCORI – International Lessons

- Independence from government is important
- Independent oversight boards are common
- Some CE entities set standards while others only identify and recommend standards
- Most CE entities are relatively small & operate with a modest budget



PCORI – International Lessons

- Prioritization process critical
- Acceptance depends on methodological rigor
- Applicability to different populations essential
- Different types of data used for recommendations
- Increasing use of conditional coverage & costs are considered
- Crucial elements:
 - Transparency
 - Timeliness
 - Appeal process

Future Challenges

- Concerns about role of CER in dampening innovation
- Desire to repeal ACA's "most egregious provisions" including:
 - Eliminating AHRQ
 - "AHRQ could be eliminated and no one would notice, except the academics and consultants who both set AHRQ's research agenda and get paid for the research."
- Eliminate:
 - Center for Medicare and Medicaid Innovation (CMS) (\$10 billion through 2019)
 - Patient-Centered Outcomes Research Institute (avoiding taxes on health plans of about \$500 million a year)
 - Funding for Shared Decisionmaking and Quality Measurement Development (\$75 million)
 - The new NIH National Institute on Minority Health and Health Disparities (NIMHD) (\$220 million)



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CTSA Consortium Executive Committee Priority Objective for SGC #4, vMarch 1, 2011

Develop a CTSA Consortium-wide framework for community-engaged and comparative effectiveness research that will support improvement in healthcare access, quality, efficiency, and patient-centered outcomes and will improve the health of diverse communities and the public

CER KFC REFERENCE IN THE SGC4 GOALS

(<https://www.ctsawiki.org/wiki//x/fIFOAQ>)

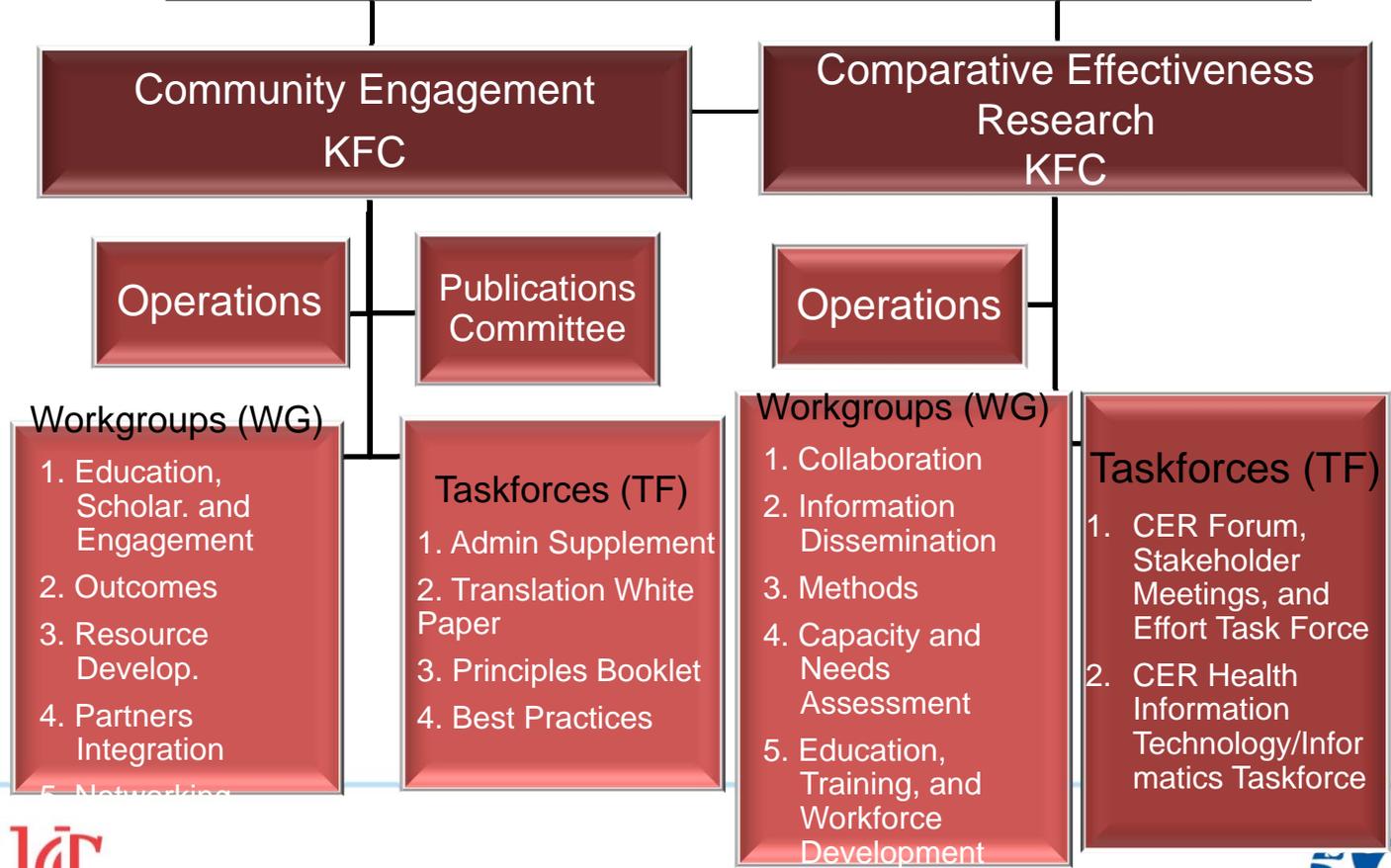
SGC4 Objective 1 (“CE”): Linking and facilitating collaboration among community based research networks to implement research best practices.

SGC4 Objective 2 (“CER”): Develop capacity and methods for the translation of research results into practice across the healthcare system, including but not limited to: health services and health policy research, comparative effectiveness research, and research into the generation and implementation of evidence-based medicine

Updated SGC4 Structure Merged from 4A and 4B

(Merged Winter/Spring 2019)

Strategic Goal Committee 4



Structure of Comparative Effectiveness Research KFC (CER KFC)

Co- Chairs: Jodi Segal and Tom Concannon

CER KFC PI Liaison of the SGC & CCSC, Harry Selker

CMG Liaison, Nick Gaich

NIH Coordinator, Rosemarie Filart

(NEW) CER HIT/Informatics Taskforce
Co-Leads: Joel Saltz and Bill Hersh

Sunsetted: CER Forum/Stakeholder Meetings
and Effort: co-Leads, Tom Concannon and Harry
Selker

Operations Group:
Co-Chairs, PI and CMG Liaisons, WG/TF
Leads, NIH Coordinator and BAH PM

**Collaboration
Workgroup**
Acting Lead: Tom
Concannon
KFC Chair: Tom
Concannon

**Information
Dissemination**
Lead: Paul Meissner
KFC Chair: Tom
Concannon

**(SGC4B WG Moved) CER
Methods Workgroup**
Lead: Mark Helfand; Chris
Schmid; Sean Tunis
KFC Chair: Tom
Concannon

**(SGC4B WG Moved) Capacity
and Needs Assessment**
Lead: Dan Ford and Harold
Pincus
KFC Chair: Jodi Segal

**(SGC4B WG Moved) Education/Training/
Workforce
Development
Workgroup**
Lead: Jodi Segal
KFC Chair: Jodi
Segal

All Members invited to participate in any group

CER KFC MISSION AND VISION STATEMENTS

[HTTPS://WWW.CTSAWIKI.ORG/WIKI/DISPLAY/CER/COMPARATIVE+EFFECTIVENESS+RESEARCH-+HOME](https://www.ctsawiki.org/wiki/Display/CER/Comparative+Effectiveness+Research-+HOME)

Mission Statement

The Comparative Effectiveness Research (CER) Key Function Committee builds the field of comparative effectiveness research (CER) and patient-centered outcomes research by creating a learning community across CTSA institutions, spurring the development of methods, expanding training and education, promoting community and public engagement, applying CER findings and sharing successes and lessons learned.

Vision Statement

Through collaborative work products, the Comparative Effectiveness Research (CER) Key Function Committee facilitates the generation and synthesis of evidence about alternative interventions that results in actionable findings for policymakers, clinicians, patients, and purchasers to use in improving the quality and outcomes of patient-centered health care.

Education/Training/Workforce Development Workgroup

Mission Statement:

To promote and facilitate incorporation of comparative effectiveness research skills into the training activities of early investigators at CTSA consortium institutions, and to promote activities that allow established investigators to develop skills of comparative effectiveness research and thereby broaden the available workforce

Vision Statement:

To advance comparative effectiveness research by expanding the workforce nationwide that has the requisite skills for conducting and using comparative effectiveness research.

Collaboration Workgroup

Mission/Vision Statement:

Support development of connections & collaborations to develop, execute, and apply CER at multiple levels:

- Within individual CTSA institutions
- Across CTSA CER investigators
- Between CTSA CER investigators and other investigators and stakeholders (e.g. AHRQ EPC institutions, DeCIDE network, CERTS, etc)

Methods Workgroup

Mission Statement:

To identify high priority methodological issues that would benefit from careful analysis and discussion within the CTSA program, with the objective of ensuring that the future CER work of the CTSA is consistently aligned with the nature and purpose of CER.

Vision Statement:

To improve public health by improving the methods used in comparative effectiveness research conducted by CTSA institutions.

Capacity and Needs Assessment Workgroup

Mission Statement

Through periodic assessments, provide data to academic institutions and funding organizations and other stakeholders about current capacities to conduct comparative effectiveness research and how the CTSA program supports CER.

Vision Statement

Academic organizations will organize their resources so that the CER research capacity, including training, will incorporate the priorities for CER as expressed by national groups such as IOM, federal agencies, professional societies, and consumer organizations as well as local stakeholders, providers and patients, health plans and purchasers.

Information Dissemination Workgroup

Mission Statement

To collect and disseminate CER information among interested CTSA Stakeholders in order to facilitate high impact CER outcomes and activities through the CTSA.

Vision Statement

- Identify information dissemination and implementation science theories and principles that are being applied in CER within and among the CTSA
- Identify best practices and methods in information dissemination and implementation science as related to CER within and among the CTSA
- Foster, facilitate and enable information dissemination, communications, and collaboration in the CTSA Consortium regarding CER

New
CER Health Information Technology/Informatics
Task Force

Task: Address Action Items of Dec. 1st CER Forum

Co-Leads: Joel Saltz and Bill Hersh

Cross Communication with Liaison Members

Committee	Liaison	CTSA
Biostatistics / Epidemiology / Research Design (BERD) KFC	<ul style="list-style-type: none"> • Frank Harrell • Chang Yu 	<ul style="list-style-type: none"> • Vanderbilt University • Vanderbilt University
CTSA Consortium Child Health Oversight Committee (CC-CHOC)	<ul style="list-style-type: none"> • Jill Herndon • Daphne Hsu 	<ul style="list-style-type: none"> • University of Florida • Albert Einstein
AHRQ DARTnet (Distributed Ambulatory Research in Therapeutics Network)	<ul style="list-style-type: none"> • Jeanne-Marie Guise • Doug Landsittel 	<ul style="list-style-type: none"> • OHSU • UPMC
Clinical Research Management (CRM) KFC	<ul style="list-style-type: none"> • Jeffrey Silverstein 	<ul style="list-style-type: none"> • Mount Sinai
Education/Training KFC	<ul style="list-style-type: none"> • Wishwa Kapoor • Pam Mitchell 	<ul style="list-style-type: none"> • UPMC • University of Washington
Community Engagement KFC – White Paper WG, Networking WG, etc..	<ul style="list-style-type: none"> • Lucy Savitz • Tom Concannon 	<ul style="list-style-type: none"> • Utah University • Tufts University
Community Engagement KFC-- Education, Scholarship and Engagement (ESE) Workgroup	<ul style="list-style-type: none"> • Mary Anne McDonald 	<ul style="list-style-type: none"> • Duke University
AHRQ CER T32 Program, 2010	<ul style="list-style-type: none"> • Tim Carey 	<ul style="list-style-type: none"> • University of North Carolina
Informatics KFC	<ul style="list-style-type: none"> • Stephen Pauker • Joel Saltz • Bill Hersh 	<ul style="list-style-type: none"> • Tufts University • Atlanta CTSI (GA Tech) • Oregon HSC
IOM UNIVERSITY OF Cincinnati	<ul style="list-style-type: none"> • Harry Selker • Lucy Savitz • Rosemarie Filart 	<ul style="list-style-type: none"> • Tufts University • Utah University 

Conclusion

- CER is growing in scope and funding
- CTSA family is engaging
- Opportunities for additional research & impact



Questions?

Lisa Simpson, MB, BCh, MPH, FAAP
President and CEO