

# The Policy Debate over Public Investment in Comparative Effectiveness Research

Eugene C. Rich MD

Scholar in Residence

Association of American Medical Colleges

Professor of Medicine

Creighton University School of Medicine

- The Current Role of Evidence in the US Health Care System

# High Hopes for Comparative Effectiveness research

- Rising health care costs are unsustainable
- Costs are driven by technology-related changes in medical practice
- When evidence is unclear geographic variations in care are high
- Some US communities achieve lower costs and high quality
- Therefore
  - production and use of better evidence MAY lower unnecessary costs
  - without reducing quality or access!

# Stakeholders agree on- need for more Comparative Effectiveness Research

- Advisors to Congress: CBO, MEDPAC, IOM
- Policy Experts: Republicans (eg Gail Wilensky, Mark McClellan) and Democrats (e.g. Tom Daschle, Jeanne Lambrew, Peter Orszag, )
- Health Plans: AHIP; BCBSA
- Employers: eg NBGH, Dow
- Drug and Device Manufacturers: PHARMA, ADVAMED, BIO
- Professional Associations: Academy Health, ACP, AAFP

# Stakeholders disagree on- a few details

- What is CER?
- Who should pay for it?
- How should it be directed?
- How should it be used?

# What is CER?

- Efficacy vs effectiveness
- Comparative effectiveness vs health services research about the delivery system
- Comparative effectiveness vs cost effectiveness
- Systematic reviews, practical clinical trials, observational studies

# The Role of Science in Fixing the US health care crisis

- “relatively little rigorous evidence is available about which treatments work best for which patients...”
  - Need for more **comparative effectiveness research (CER)**
- “...the financial incentives for both providers and patients tend to encourage the adoption of more expensive treatments...even if evidence of their relative effectiveness is limited.”
  - Need for more **health services research on delivery system reform**

# Cost effectiveness analysis

- CEA: an economic evaluation in which costs and health outcomes of different programs or interventions are evaluated on a common metric, often the ratio of net costs over net effectiveness
- E.g. \$/quality adjusted life year (quale)

# What is CER? Legislative Answers

- 2007 HR 3162: CHAMP Act (Section 904)
  - Research with respect to the outcomes, effectiveness, and appropriateness of health care services and procedures in order to identify the manner in which diseases, disorders, and other health conditions can most effectively and appropriately be prevented, diagnosed, treated, and managed clinically.
- 2008 S 3408: Comparative Effectiveness research Act of 2008
  - “The term ‘comparative effectiveness research’ means research evaluating and comparing the implications and outcomes of 2 or more health care strategies to address a particular medical condition”
- 2009 HR 1

# What is CER? Legislative Answers

## 2009 HR 1 Conference Report Language

- “...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”
- “... the conferees recognize that a "one-size-fits-all" approach to patient treatment is not the most medically appropriate solution to treating various conditions and include language to ensure that subpopulations are considered when research is conducted or supported with the funds provided in the conference agreement.”

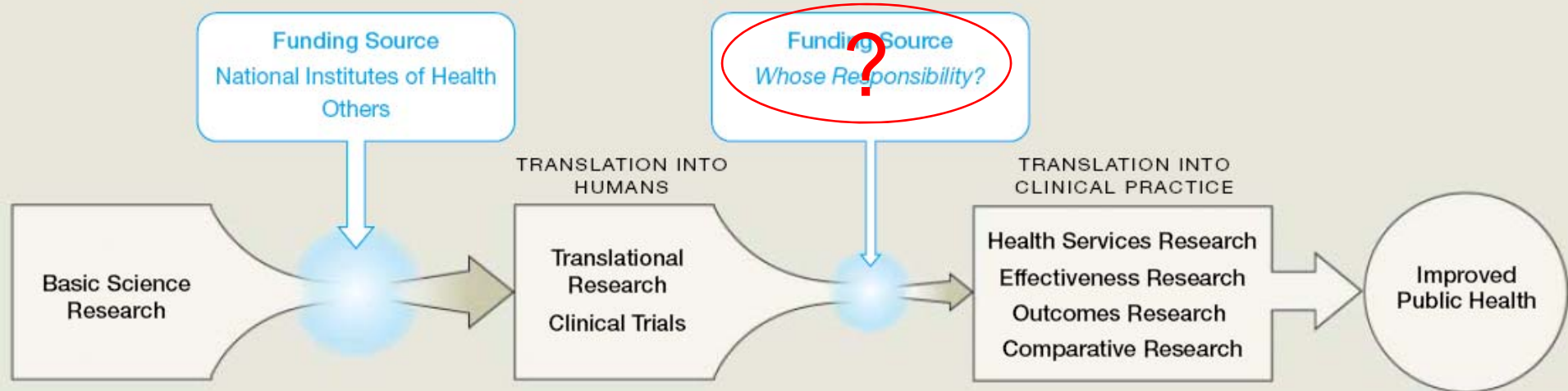
# Policy-maker definitions of CER

- Comparison of different health care service approaches to managing a specific clinical problem, condition or disease;
  - approaches can include diagnostics as well as therapeutics; surgery as well as drugs; watchful waiting as well as intervention;
- Treatment options must be applicable in the “real world”-
  - Services available in a variety of practice settings, and delivered by typically trained clinicians;
- Outcome measures must matter to patients making real-world decisions
  - mortality, major morbidity, symptom severity, quality of life

# Essential characteristics for policy-relevant CER

- CER involves comparison of different health care service approaches to managing a specific clinical problem, condition or disease;
- The options studied by CER must be applicable in the “real world”
- CER outcome measures must be clinically relevant; e.g. mortality, major morbidity, symptom severity, quality of life
- CER studies should provide information on which specific types of patients would benefit most from the interventions in question.
- CER studies should have sufficient statistical power to meaningfully inform clinicians and patients.

# Who should pay for CER?



Crowley WF, et al. JAMA 2004;291:1120-1126

# Why there isn't enough privately funded CER

- Health Economists (eg CBO)
  - “...the private sector generally will not produce as much research on comparative effectiveness as society would value..”
- Drug/device Manufacturer CEO:
  - “Why did we do a comparative clinical trial that just handed the market to our competition?”
- Health plan CFO:
  - “This study you medical directors want costs \$50 million; takes 5 years, and all our competitors can benefit from the information?”
- Consumer handbook publisher:
  - “I need to invest 100s of millions in research before I have a product to sell? And then do I sue patients using this “copyrighted” information without a subscription?”

# Why isn't there more CER at Federal Research Agencies?

- NIH Mission: science in pursuit of fundamental knowledge about the nature and behavior of living systems and the application of that knowledge to extend healthy life and reduce the burdens of illness and disability.
- CER questions and priorities rarely involve issues of fundamental knowledge.
- Agencies and Academe place a high value on investigator initiated research
- Federal Research Agencies rely on Appropriator enthusiasm, but CER creates losers who really HATE losing (ask AHCPR!)

# AHCPR and CER

- Established in 1989
- statutory responsibilities regarding outcomes research and practice guideline development
- Increased funding over former NCHSR : \$53 million 1989; \$97 million 1990; peak \$162 million 1995
- Patient Outcomes Research Teams (PORTs)
  - multidisciplinary centers focused on particular medical problems; review and synthesize available research, analyze practice variations and patient outcomes, disseminate the results, and evaluate the effects of dissemination.
- Back surgeon and device manufacturer hostility to PORT back pain research, and related guidelines
- Funding decline; loss of PORTs and Guidelines functions, name change 1999 (to AHRQ)

# Who Should Pay for CER?

## Legislative Answers

- 2007 HR 3162 (CHAMP Act): Section 904
  - Comparative Effectiveness Research Trust Fund: “In order to provide a consistent stream of public and private funding for comparative effectiveness research through a mechanism insulated from outside influence, the legislation will support the activities... through the Comparative Effectiveness Research Trust Fund... derived by fees charged to all those payers who will benefit from the efficiencies gained by comparative effectiveness research, Medicare and all forms of private health insurance”
  - \$90 M for 2008; \$100 M for 2009; \$110 M for 2010 from Medicare
  - 2011- and beyond: \$375 M per year (\$90 M from Medicare, up to \$2 pm per yr fee on pvt health insurance).
- 2008 S 3408: Comparative Effectiveness Research Act of 2008
  - Comparative Effectiveness Research Trust Fund: “ Provides revenues for the Trust Fund by transferring amounts from the General Fund and the Medicare Federal Hospital Insurance and Federal Supplementary Medical Insurance Trust Funds and by imposing a fee on certain accident or health insurance policies and self-insured health plans.”
  - \$ 5 M for 2009, \$25 M for 2010, \$75 M for 2011 from the general fund of the Treasury ,
  - 2012: est \$185 M (\$75 M from the Treasury and \$ 110 M from \$0.5 pm per yr fee on Medicare and pvt health insurance
  - 2013 to 2018: \$300+ M per year (\$75 M from the Treasury and \$ 225 M from \$1 pm per yr fee on Medicare and pvt health insurance)
- 2009 HR 1:
  - \$1.1 B appropriated over 2 years (FY 2009 and FY2010)
  - AHRQ would receive \$700 M for CER; AHRQ must transfer \$400 M to NIH to conduct or support CER.
  - Secretary would receive \$400 M to “accelerate the development and dissemination of CER

# How should CER be directed?

- IOM Roundtable on EBM: discussion of CER entity functions...
  - Priority setting
  - Budget allocation
  - Study selection
  - Research design/methods improvement
  - Study management
  - Study conduct (field)
  - Study certification
  - Study conclusions

# Hot Topics for CER are High Stakes!

## CT Coronary Angiograms for chest pain

- Key revenue/profit for manufacturers, hospitals, radiologists, physician owners of Angio-capable hi slice CT
- Advocates: manufacturers, hospitals, specialty groups, patient groups

# How can current clinical research not be enough?

- Medline search for articles published in the last year
- Humans, Clinical Trial, English, All Adult: 19+ years
  - 18,000+
- Humans, Randomized Controlled Trial, English, All Adult: 19+ years
  - 9,000+

# Doing CER- Not your typical NIH research grant

- Setting CER Research priorities
  - CER legislative proposals mandate broad representation of stakeholders in identifying priority research topics and specifying research questions.
  - Research questions- “what physicians and patients need to know to make bedside decisions”
  - This will NOT be an agenda directed by investigators, study sections, Research agency Councils or program staff
- Conducting CER projects
  - CER contracts with research questions and methods specified in the RFP
  - NOT as grants in response to PI-initiated proposals

# How Should CER be directed?

## HR 3162

### CER Center at AHRQ

- Conducts support, and synthesizes research and systematic reviews of clinical research
- Establishes standards for CER methods
- Encourages development and use of clinical registries clinical effectiveness research data networks from electronic health records, post marketing drug and medical device surveillance efforts, and other forms of electronic health data;

Advisory Commission oversight

# How Should CER be directed?

## HR 3162

### Advisory Commission Duties

- determine national priorities for research, consulting with patients and health care providers and payers;
- identify standards of evidence for CER and support forums to increase stakeholder awareness and permit stakeholder feedback on AHRQ efforts to advance CER methods
- appoint a clinical perspective advisory panel for each research priority to frame the specific research question to ensure that the information is relevant to decisions made by clinicians and patients
- review Center processes to ensure that the information produced is objective, credible, consistent with standards of evidence and developed through a transparent process that includes consultations with appropriate stakeholders;

17 Commission members “would represent a broad range of perspectives.”

- Director of the Agency for Healthcare Research and Quality
- Chief Medical Officer of the Centers for Medicare and Medicaid Services
- 15 additional members with 4 year appointments appointed by the Comptroller General of the United States, in consultation with the chairs of the committees of jurisdiction of the House of Representatives and the Senate.
  - representing “broad constituencies of stakeholders including clinicians, patients, researchers, third-party payers, consumers of Federal and State beneficiary programs.
  - “experience in epidemiology, health services research, bioethics, decision sciences, and economics.”
  - “At least one member would represent each of the following health care communities: consumers, practicing physicians--including surgeons, employers, public payers, insurance plans, and clinical researchers who conduct research on behalf of pharmaceutical or device manufacturers.

# How Should CER be directed?

## S3408

- Establishes the Health Care Comparative Effectiveness Research Institute, a private, nonprofit corporation
- Institute duties
  - establish a national agenda of research priorities based on the need for better evidence, disease burden, practice variations, the potential for improved care, and expenditures associated with a given health condition or care strategy.
  - establish a standing methodology committee
  - contract with federal agencies, such as AHRQ and appropriate private entities to conduct the research,
  - Research findings to be peer-reviewed and publicly disseminated in ways patients and healthcare providers can easily understand.
  - establish procedures to ensure that requirements for ensuring transparency, credibility, and access {to data} are met:
- 21 member Board of Governors

# How Should CER be directed?

## S3408

21 member Board of Governors

- Secretary of Health and Human Services (HHS)
- Directors of AHRQ and NIH
- 18 additional members with 6 year appointments appointed by the Comptroller General of the United States representing diverse public and private sector expertise and interests.
  - 3 members representing patients and health care consumers;
  - 3 members representing practicing physicians, including surgeons;
  - 3 members representing agencies that administer public programs (CMS, State Health programs, other Federal health programs (eg FEHBP, VA, IHS));
  - 3 members representing private payers;
  - 3 members representing pharmaceutical, device, and technology manufacturers or developers;
  - 1 member representing nonprofit organizations involved in health services research; 1 member representing organizations that focus on quality measurement and improvement or decision support; 1 member representing independent health services researchers.

# Principles for CER Entity

	HHS (AHRQ) Center	FFRDC (HHS private contractor)	NFP CER “Institute”
Public Accountability	↑	?	↓
Consistency	↑	?	↑
Use of existing capacity	↑	↑	↓
Political Feasibility	?	↑	↑
Objectivity	?	?	?
Responsiveness	?	↑	↑
Scientific rigor	?	?	?
Transparency	↑	↓	↓

# Other Considerations

- Coordination of Federally supported CE Research infrastructure (e.g. btwn AHRQ, NIH, VA, CDC, etc)

# Examples of CER Research Infrastructure

- AHRQ
  - 41 CER research centers nationwide
    - Evidence-Based Practice Centers;
    - Centers for Education and Research on Therapeutics (CERTs);
    - DEcIDE Centers (Developing Evidence to Inform Decisions About Effectiveness) -HIT Databases
      - Access to >120 different databases
- NIH
  - CTSA- 38 leading academic medical centers (\$500+ M annually)
  - Over 80 Illness-specific Clinical Research Networks
    - AIDS malignancies
    - Asthma
    - Leukemia
    - Drug-induced liver injury
    - Bariatric surgery
    - Maternal-fetal medicine
    - Alzheimer's disease
    - Drug abuse
    - And MANY more

# Coordination of Federal efforts

- CER research workforce development
- CER Methods development (Standards)
- Sample CER methods  
controversies/competencies
  - Systematic reviews and meta-analysis
  - Clinical trials
  - Outcomes measurement
  - Cost measurement
  - Role of observational studies
  - Cost-effectiveness analysis

# How Should CER be directed?

## Legislative Answers

### 2009 HR 1

- establishes the Federal Coordinating Council for Comparative Effectiveness Research (FCCCER)
- comprised of up to 15 representatives of federal agencies—at least half would have to be physicians or other experts with clinical expertise
- FCC-CER Purpose is to foster opportunities to assure optimum coordination of comparative effectiveness and related HSR (Reducing duplicative efforts, encouraging complimentary use of resources)
  - Council Duties:
    - assist federal agencies to coordinate the conduct or support of CER and related health services research,
    - advise the President and Congress on
      - Federal CER infrastructure needs.
      - organizational expenditures for CER by relevant Federal departments and agencies

# How should CER be used?

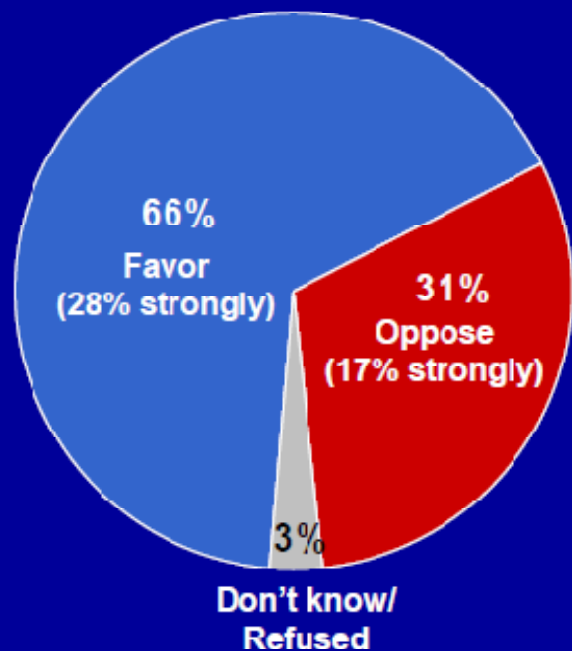
- CBO Score for CER in HR 3162
  - \$2.9 Billion spending on CER 2008-2017
  - Federal Health Care savings \$1.3 Billion-
    - Federal investments in CER would equal savings after 7 years
    - Assumes no changes in Medicare coverage rules
    - Anticipates evidence leads to changes in physician practice and patient choice
  - Private Health Care Savings \$5 Billion
    - Anticipates private insurers would use CER

# Commonwealth Report: savings from Federal investment in CER

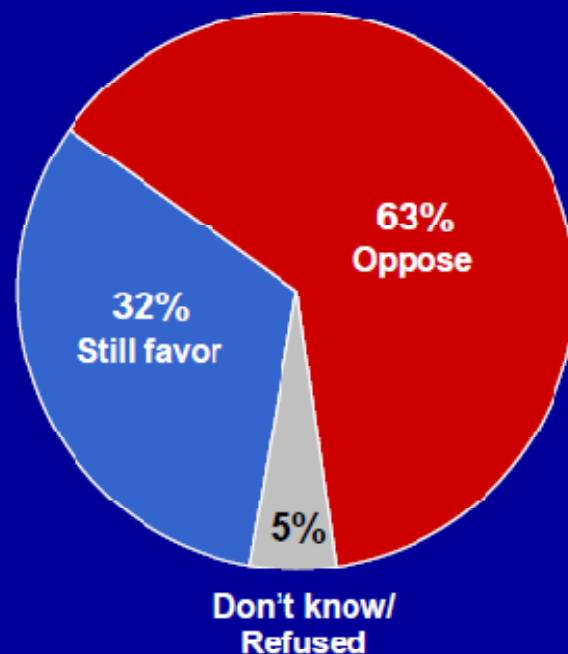
- Center for Medical Effectiveness and Health Care Decision-Making.
  - Invest in the knowledge needed to improve decision-making
  - incorporate information about the relative clinical and **cost-effectiveness** of alternative treatment options into **insurance benefit design**.
  - **payment and cost-sharing incentives for providers and consumers** to use CER
  - Potential health system savings of **\$368 billion over 10 years**, shared by all payers.

# A Federal Scientific Body to Evaluate Medical Technology

One way that health experts have proposed trying to slow the growth of health insurance premiums is to establish a new independent federal scientific body which would decide whether approved new medical technology, procedures, and drugs should be covered by insurance, based on whether they are proven to be more effective than existing, less expensive treatments. Would you favor or oppose this?



This might mean that in some cases, treatments or drugs recommended by a person's own doctor wouldn't be covered by their health insurance. Having heard that, would you favor or oppose having a new federal scientific body decide whether new treatments and drugs should be covered by insurance, or not?\*



\* Follow up question asked of those who initially favored. Responses shown in the pie chart on the right are based on total, where opposed includes those who initially opposed and those who opposed after follow up.

Source: KFF/HSPH *The Public's Health Care Agenda for the New President and Congress* (conducted Dec. 4-14, 2008)

# How should CER be used?

## Legislative Answers

- 2007 HR 3162 (CHAMP Act): Section 904
  - Commission is to “provide guidance or recommendations to health care providers and consumers for the use of information on the comparative effectiveness of health care services by consumers, providers and public and private purchasers; produced from such research to all interested parties” at least annually
- 2008 S 3408
  - Institute not permitted to “mandate coverage, reimbursement, or other policies for any public or private payer;
  - None of the reports submitted under this section or research findings disseminated by the Institute shall be construed as mandates, guidelines, or recommendations for payment, coverage, or treatment.”
- 2009 HR 1:
  - **Conference Report language** “The conferees do not intend {CER funding}... be used to mandate coverage, reimbursement, or other policies for any public or private payer. The funding ... shall be used to conduct or support research....”
  - **Legislative Language:** Rules of Construction re above

# Readings

- CBO
  - Technological Change and the Growth of Health Care Spending  
January 2008
  - Research on the Comparative Effectiveness of Medical Treatments:  
Issues and Options for an Expanded Federal Role  
December 2007
- IOM
  - Knowing What Works in Health Care: A Roadmap for the Nation; Jan  
2008
  - Comparative Effectiveness in Health Care; Sept 2007
- MedPAC
  - Chapter 5: Producing Comparative effectiveness information; in  
Reforming the Delivery System; June 2008
- ACP
  - American College of Physicians\*, . Information on Cost-Effectiveness:  
An Essential Product of a National Comparative Effectiveness Program.  
Ann Intern Med. 2008