Update on Comparative Effectiveness and Health Information Technology Legislation and Policy Developments, and Impact on Pharmacoeconomics
Presented by
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Update on Comparative Effectiveness Legislation and Considerations to Ponder
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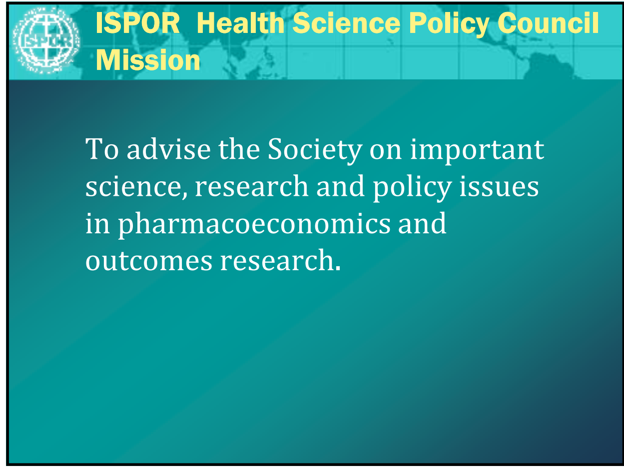
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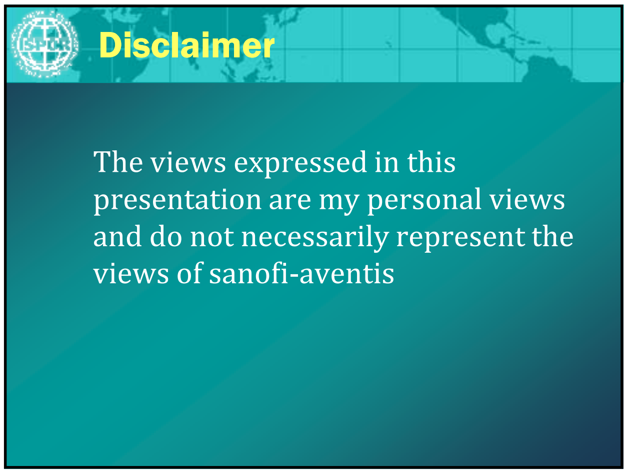
To advise the Society on important science, research and policy issues in pharmacoeconomics and outcomes research.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Talking Points
• Legislation
• Considerations

CE Past Legislative History

- CE offered as Frist-Clinton amendment to Medicare Modernization Act (MMA) (P.L. 108-173, Sec. 1013) in 2003
  - Purpose - syntheses of information to improve Medicare, Medicaid and State Children's Health Insurance Program (SCHIP) beneficiaries health care
- CER Act of 2008 – S.3408, Senators Baucus and Conrad, July 31, 2008
  - Purpose to advance evidence on how diseases, disorders and other health conditions can best be prevented, diagnosed, treated and managed

CE Past Legislative History

- 2009 American Recovery and Reinvestment Program – H.R. 1
  - Comparative Effectiveness Research
    - Purpose: "*Healthcare Research and Quality*" - AHRQ to use funding for CER under Section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act
  - Adoption and Use of Health Information Technology (HIT)
    - Purpose: The Secretary shall, using amounts appropriated under section 3018, invest in the infrastructure necessary to allow for and promote the electronic exchange and use of health information

Transforming the Health Care Delivery System: Proposals to Improve Patient Care and Reduce Health Care Costs

Senate Finance Committee - April 29, 2009

- Policy options for reducing costs and improving quality
  - Options would create incentives for health care providers to focus on high quality care and to closely coordinate with a patient's other doctors and providers

Infrastructure Investments: Tools to Support Delivery System Reform – Comparative Effectiveness Research

- Finding Out What Works in Health Care –
  - Establish a long-term or permanent framework to set national priorities and conduct comparative clinical effectiveness research (CCER)
  - Establish a private, non-profit corporation that would generate and synthesize evidence with CCERs
    - Institute governed by a multi-stakeholder board
    - Institute could contract with AHRQ, the NIH and other federal and private entities to conduct CCERs
    - Free standing Institute would help maintain objectivity and minimize political influence, it would be periodically audited by the GAO

Infrastructure Investments: Tools to Support Delivery System Reform – Comparative Effectiveness Research

- Ensuring Credible and Objective Research
  - Development of methods and standards for such research
    - Independent, expert committee charged with developing methodological standards for this type of research should be established.
    - Research could be guided by expert advisory panels or subject to a peer review process
- Transparency and Public Input
  - Public comment and input should be integral to CCERs
  - Options include:
    - Comment on research agenda, design, draft reports, priorities, and dissemination approaches
    - Peer-review of research designs and findings
    - Research findings publicly disseminated and easily understood

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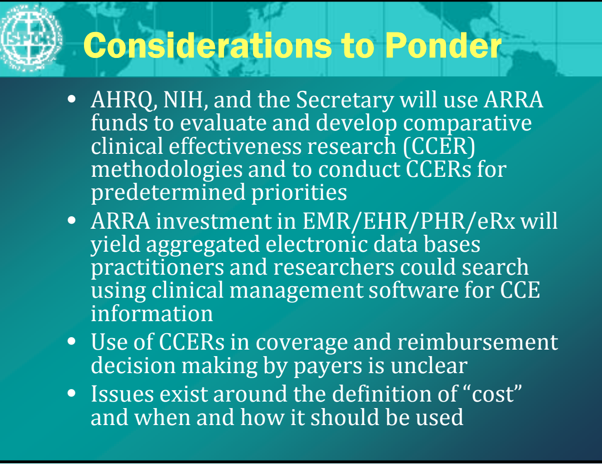
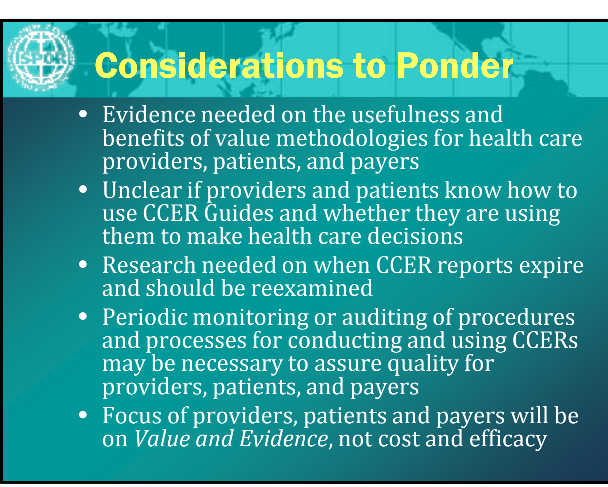
Infrastructure Investments: Tools to Support Delivery System Reform – Comparative Effectiveness Research

- Patient Safeguards -
  - Institute considers patient subgroup responses to different strategies when designing/approving studies
  - Institute disseminates findings but prohibited from issuing medical practice or coverage and reimbursement recommendations
  - Create limits on the use of the research by HHS
    - Process must be transparent
    - Relies on all available evidence
    - Considers effects on beneficiary populations
    - Allows for public comment on draft proposals using the (CER) information
  - This would prohibit HHS agencies from creating a fast-track process for automatically linking the research findings to coverage or reimbursement decisions in public programs.

Talking Points
Legislation
Considerations



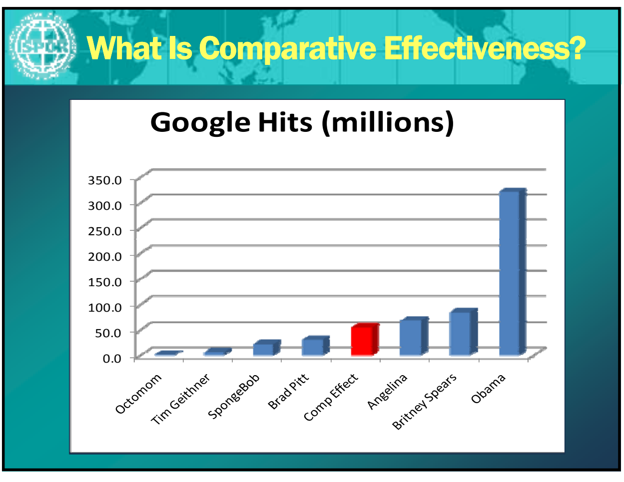
Considerations to Ponder
AHRQ, NIH, and the Secretary will use ARRA funds to evaluate and develop comparative clinical effectiveness research (CCER) methodologies and to conduct CCERs for predetermined priorities
ARRA investment in EMR/EHR/PHR/eRx will yield aggregated electronic data bases practitioners and researchers could search using clinical management software for CCE information
Use of CCERs in coverage and reimbursement decision making by payers is unclear
Issues exist around the definition of "cost" and when and how it should be used



Considerations to Ponder
Evidence needed on the usefulness and benefits of value methodologies for health care providers, patients, and payers
Unclear if providers and patients know how to use CCER Guides and whether they are using them to make health care decisions
Research needed on when CCER reports expire and should be reexamined
Periodic monitoring or auditing of procedures and processes for conducting and using CCERs may be necessary to assure quality for providers, patients, and payers
Focus of providers, patients and payers will be on Value and Evidence, not cost and efficacy

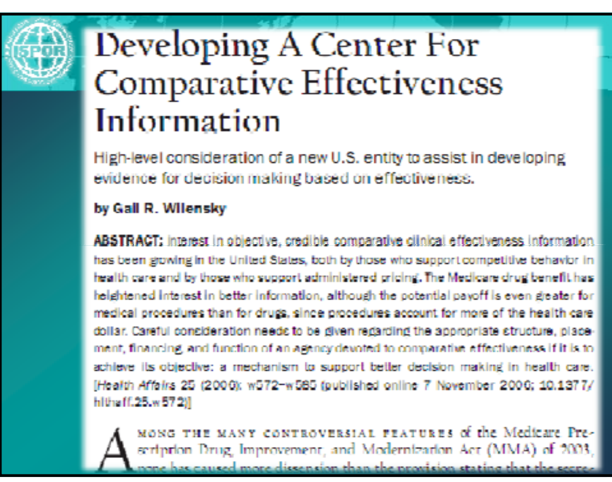
Summary
Policy Options
Considerations

What is Comparative Effectiveness, and Why Do We Need It?
Joel Hay PhD
Department of Pharmaceutical Economics & Policy
University of Southern California
Los Angeles, CA, USA



Evidence is Important

- We spend $2.7 trillion on health care annually
- Rand estimates that 1/3 of this medical spending is harmful or useless
- $900 billion per year! This is larger than the Obama stimulus package



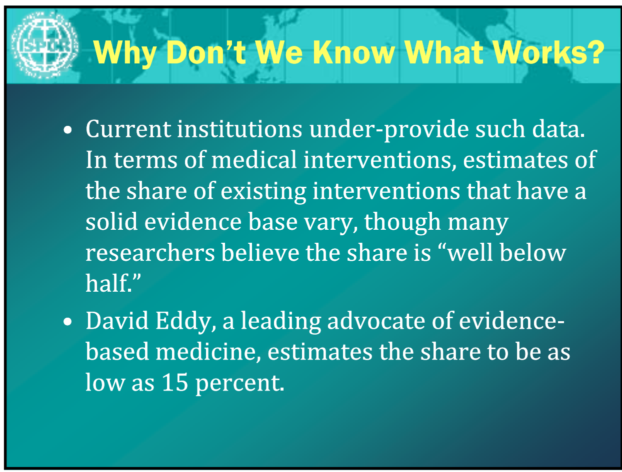
Developing A Center For Comparative Effectiveness Information

High-level consideration of a new U.S. entity to assist in developing evidence for decision making based on effectiveness.

by Gail R. Wilensky

ABSTRACT: Interest in objective, credible comparative clinical effectiveness information has been growing in the United States, both by those who support competitive behavior in health care and by those who support administered pricing. The Medicare drug benefit has heightened interest in better information, although the potential payoff is even greater for medical procedures than for drugs, since procedures account for more of the health care dollar. Careful consideration needs to be given regarding the appropriate structure, placement, financing, and function of an agency devoted to comparative effectiveness if it is to achieve its objective: a mechanism to support better decision making in health care. [*Health Affairs* 25 (2006): w572–w585 (published online 7 November 2006; 10.1377/hlthaff.25.w572)]

AMONG THE MANY CONTROVERSIAL FEATURES of the Medicare Prescription Drug, Improvement, and Modernization Act (MMA) of 2003,



The New York Times

March 19, 2009

Prostate Test Found to Save Few Lives

By GINA KOLATA

The PSA blood test, used to screen for prostate cancer, saves few lives and leads to risky and unnecessary treatments for large numbers of men, two large studies have found.

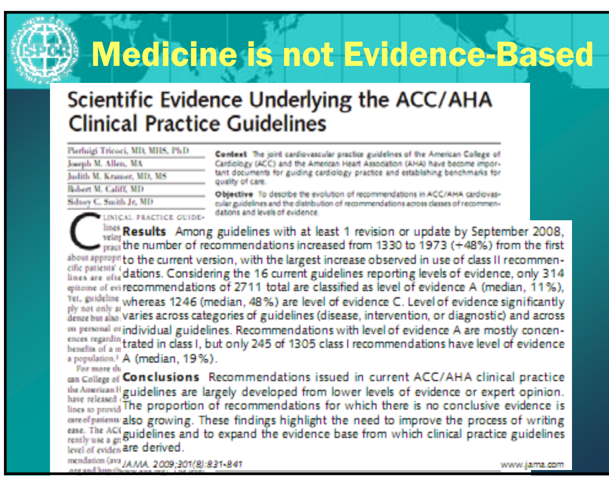
The findings, the first based on rigorous, randomized studies, confirm some longstanding concerns about the wisdom of widespread prostate cancer screening. Although the studies are continuing, results so far are considered significant and the most definitive to date.

The PSA test, which measures a protein released by prostate cells, does what it is supposed to do — indicates a cancer might be present, leading to biopsies to determine if there is a tumor. But it has been difficult to know whether finding prostate cancer early saves lives. Most of the cancers tend to grow very slowly and are never a threat and, with the faster-growing ones, even early diagnosis might be too late.

The studies — one in Europe and the other in the United States — are "some of the most important studies in the history of men's health," said Dr. Otis Brawley, the chief medical officer of the American Cancer Society.

In the European study, 48 men were told they had prostate cancer and needlessly treated for it for every man whose death was prevented within a decade after having had a PSA test.

Dr. Peter B. Bach, a physician and epidemiologist at Memorial Sloan-Kettering Cancer Center, says one way to think of the data is to suppose he has a PSA test today. It leads to a biopsy that reveals he has prostate cancer, and he is treated for it. There is a one in 50 chance that, in 2019 or later, he will be spared death from a cancer that would otherwise have killed him. And there is a 49 in 50 chance that he will have been treated unnecessarily for a cancer that was never a threat to his life.



Scientific Evidence Underlying the ACC/AHA Clinical Practice Guidelines

Pierluigi Tricoci, MD, MHS, PhD
Joseph M. Allen, MA
Judith M. Kramer, MD, MS
Robert M. Califf, MD
Sidney C. Smith Jr, MD

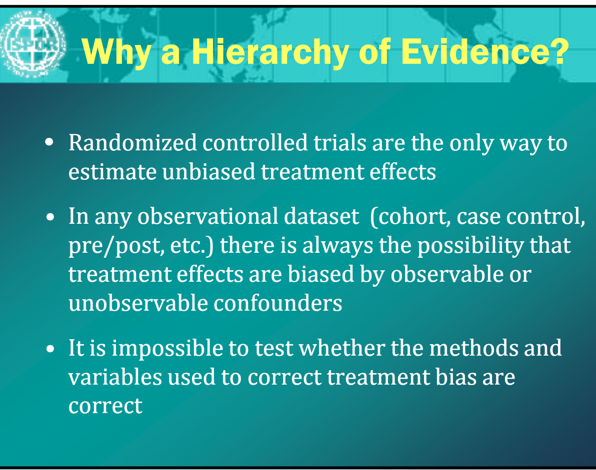
Context The joint cardiovascular practice guidelines of the American College of Cardiology (ACC) and the American Heart Association (AHA) have become important documents for guiding cardiology practice and establishing benchmarks for quality of care.

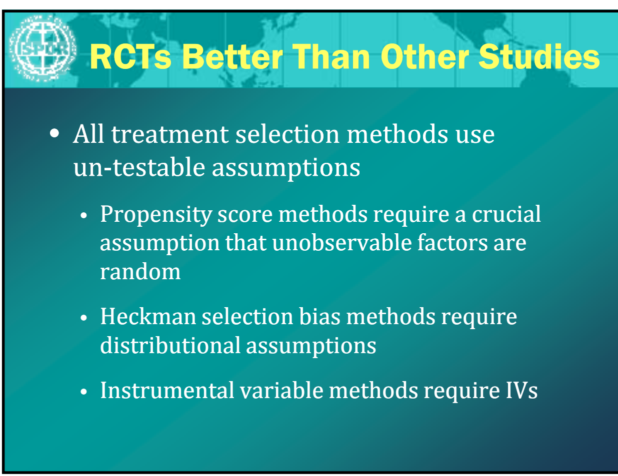
Objective To describe the evolution of recommendations in ACC/AHA cardiovascular guidelines and the distribution of recommendations across classes of recommendations and levels of evidence.

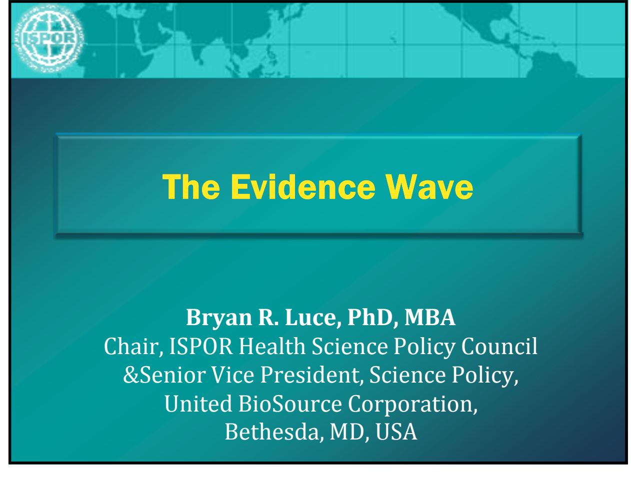
Results Among guidelines with at least 1 revision or update by September 2008, the number of recommendations increased from 1330 to 1973 (+48%) from the first to the current version, with the largest increase observed in use of class II recommendations. Considering the 16 current guidelines reporting levels of evidence, only 314 recommendations of 2711 total are classified as level of evidence A (median, 11%), whereas 1246 (median, 48%) are level of evidence C. Level of evidence significantly varies across categories of guidelines (disease, intervention, or diagnostic) and across individual guidelines. Recommendations with level of evidence A are mostly concentrated in class I, but only 245 of 1305 class I recommendations have level of evidence A (median, 19%).

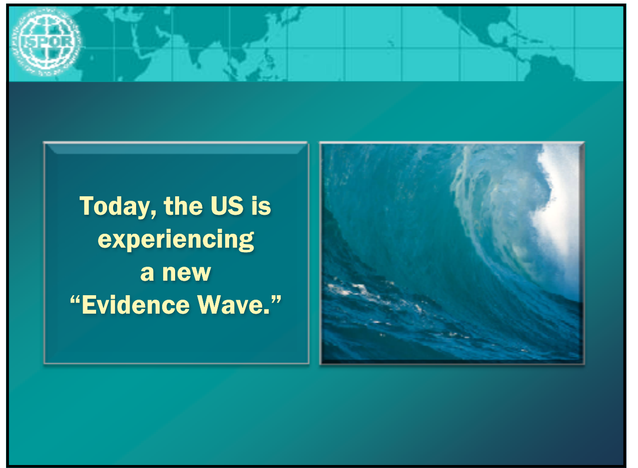
Conclusions Recommendations issued in current ACC/AHA clinical practice guidelines are largely developed from lower levels of evidence or expert opinion. The proportion of recommendations for which there is no conclusive evidence is also growing. These findings highlight the need to improve the process of writing guidelines and to expand the evidence base from which clinical practice guidelines are derived.

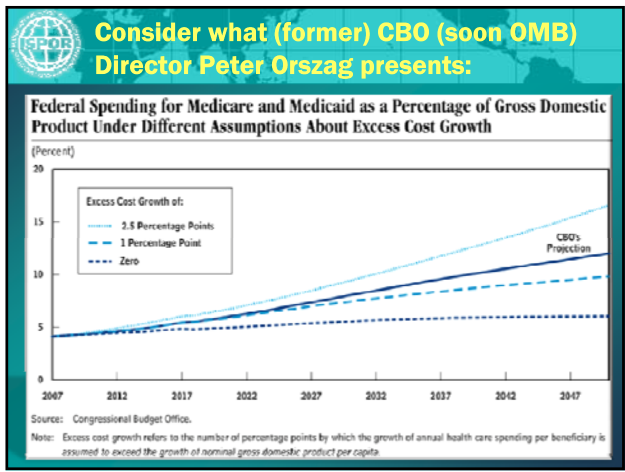
JAMA. 2009;301(8):831-841 www.jama.com



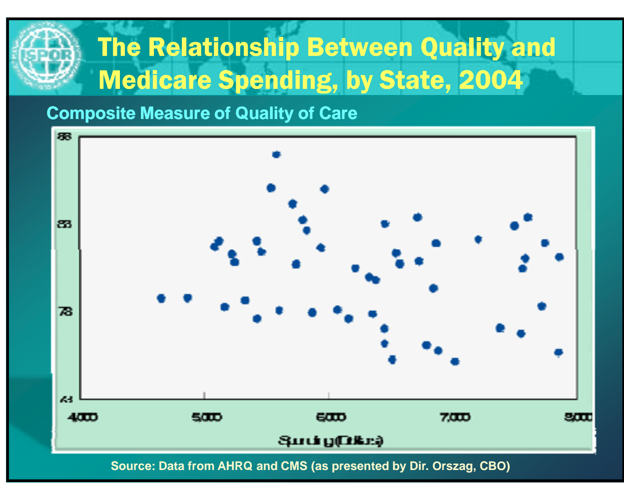






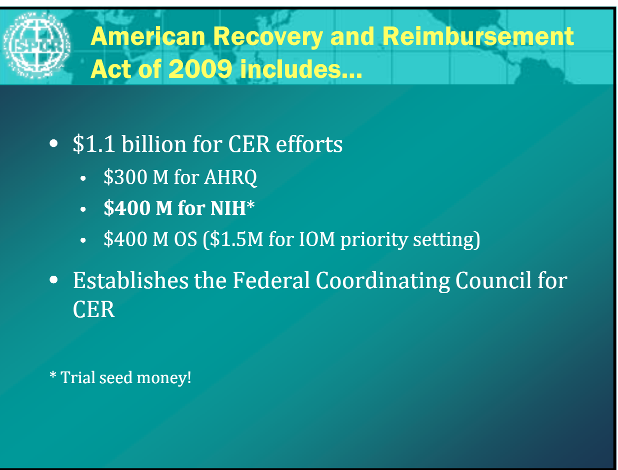


Consider what (former) CBO (soon OMB) Director Peter Orszag presents:
Federal Spending for Medicare and Medicaid as a Percentage of Gross Domestic Product Under Different Assumptions About Excess Cost Growth
(Percent)
20
15
10
5
0
Excess Cost Growth of:
2.5 Percentage Points
1 Percentage Point
Zero
CBO's Projection
2007 2012 2017 2022 2027 2032 2037 2042 2047
Source: Congressional Budget Office.
Note: Excess cost growth refers to the number of percentage points by which the growth of annual health care spending per beneficiary is assumed to exceed the growth of nominal gross domestic product per capita.



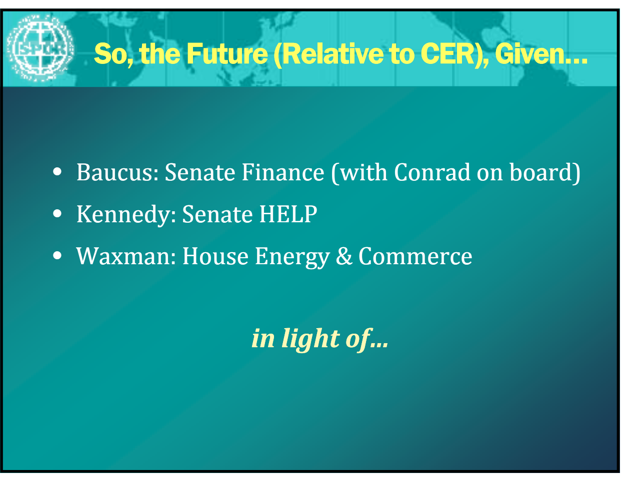
The Relationship Between Quality and Medicare Spending, by State, 2004
Composite Measure of Quality of Care
4,000
5,000
6,000
7,000
8,000
Source: Data from AHRQ and CMS (as presented by Dir. Orszag, CBO)

Stakeholders, Especially Health Plans, Call for a Centralized CER Entity
November 2006:
Wilensky HA Article
April 2007:
AHIP proposal
May 2007:
BCBS proposal
IOM recommendation
Allen Emerson H.R. 2184
June 2007:
MedPAC Proposal
W&M CER hearing
CBO Dir Orszag endorses CER entity
August 2007:
House passes CHAMP bill
February 2008:
President's FY09 Budget doubles funding for AHRQ's EHC Program
IOM "Roadmap" Rpt: CER central "program"
April 2008:
Sen Obama: "CER to improve quality of care;" endorses CER Institute
August 2008:
Sen Baucus and Conrad bill
November 2008:
Sen Baucus releases health care reform proposal
Pres Obama selects Orszag for OMB and Daschle for HHS; Rep. Waxman wins helm of E&C
January 2009:
AReRA includes $1.1 billion for CER
BCBS=Blue Cross and Blue Shield
AHIP=America's Health Insurance Plans
IOM=The Institutes of Medicine



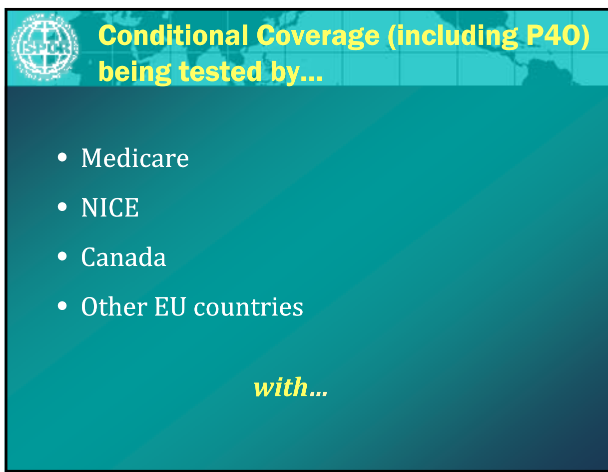
American Recovery and Reimbursement Act of 2009 includes…
$1.1 billion for CER efforts
$300 M for AHRQ
$400 M for NIH*
$400 M OS ($1.5M for IOM priority setting)
Establishes the Federal Coordinating Council for CER
* Trial seed money!

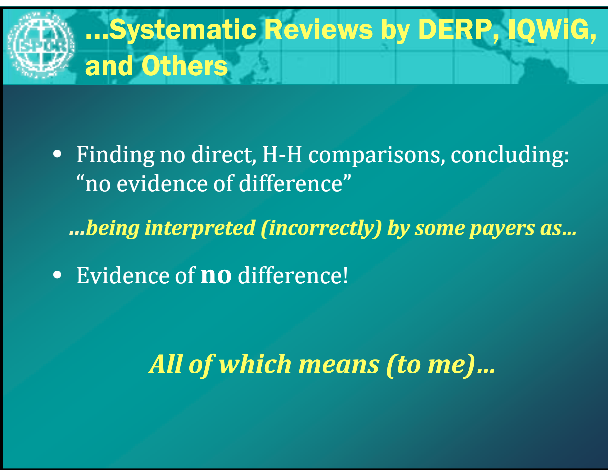
So, the Future (Relative to CER), Given…
IOM, Payers, Clinical Leaders, Academics, Politicians all calling for:
Investment in evidence (generally)
New CER Institute (specifically)
Obama as President
HHS on board
Orszag as OMB



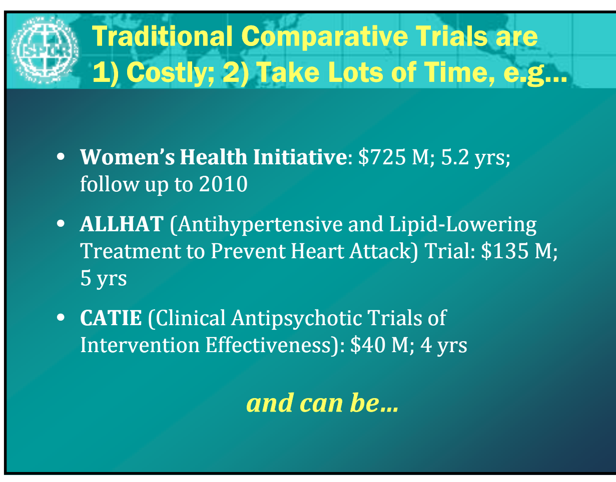
So, the Future (Relative to CER), Given…
Baucus: Senate Finance (with Conrad on board)
Kennedy: Senate HELP
Waxman: House Energy & Commerce
in light of…

The Financial Crisis, with…
Health Costs believed to big part of problem
Evidence generation believed to important element of the solution
Waning political opposition to new evidence policies
Traditional opponents somewhat co-opted as included stakeholders


Conditional Coverage (including P4O) being tested by…
Medicare
NICE
Canada
Other EU countries
with…


…Systematic Reviews by DERP, IQWiG, and Others
Finding no direct, H-H comparisons, concluding: "no evidence of difference"
…being interpreted (incorrectly) by some payers as…
Evidence of no difference!
All of which means (to me)…

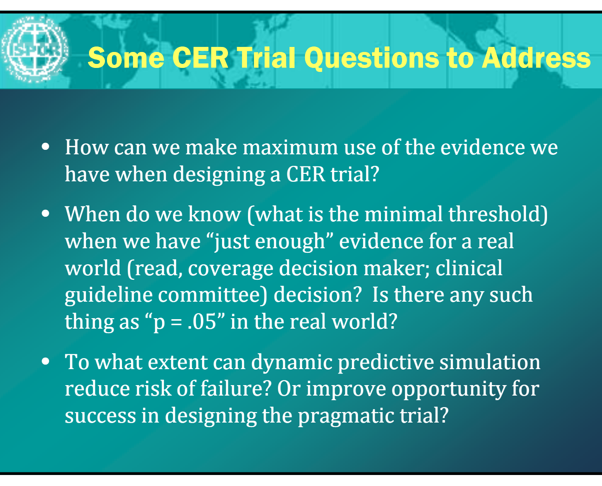
…that we will continue to see…
Sustained federal funding for CER to include
Federally-funded comparative trials
Investment in infrastructure (especially HIT, EMR)
More $ for traditional systematic reviews/HTA activity
Strengthening "conditional coverage" policies by CMS, and possibly major private payers
Increased CER trial activity by manufacturers to preempt the above
However…


Traditional Comparative Trials are 1) Costly; 2) Take Lots of Time, e.g…
Women's Health Initiative: $725 M; 5.2 yrs; follow up to 2010
ALLHAT (Antihypertensive and Lipid-Lowering Treatment to Prevent Heart Attack) Trial: $135 M; 5 yrs
CATIE (Clinical Antipsychotic Trials of Intervention Effectiveness): $40 M; 4 yrs
and can be…

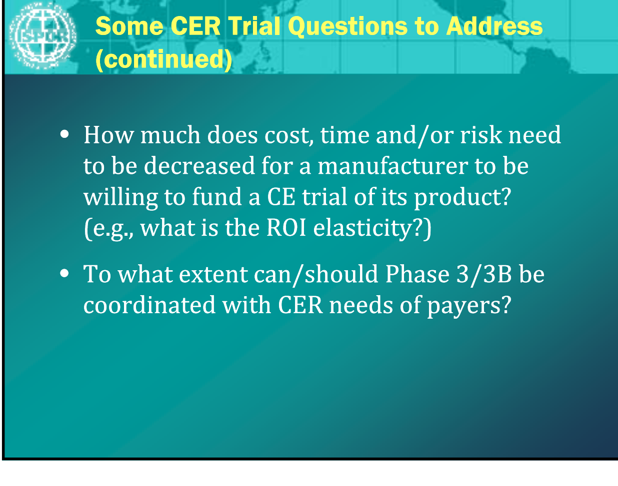
Risky Ventures for Manufacturers, e.g…
PROVE IT (Pravastatin or Atorvastatin Evaluation and Infection Therapy–Thrombolysis in Myocardial Infarction) trial*
ENHANCE (Effect of Combination Ezetimibe & High-Dose Simvastatin vs. Simvastatin Alone on the Atherosclerotic Process in Patients with Heterozygous Familial Hypercholesterolemia) trial
Neither trial concluded in favor of the sponsors' products.

Thus…
Although the new CER evidence wave is, in large part, an enlightened approach to…
• Improve health care decision-making
• Better target medical products and HC services
• Contain costs
…to my mind, CER trials are unsustainable without transformational change in generating evidence

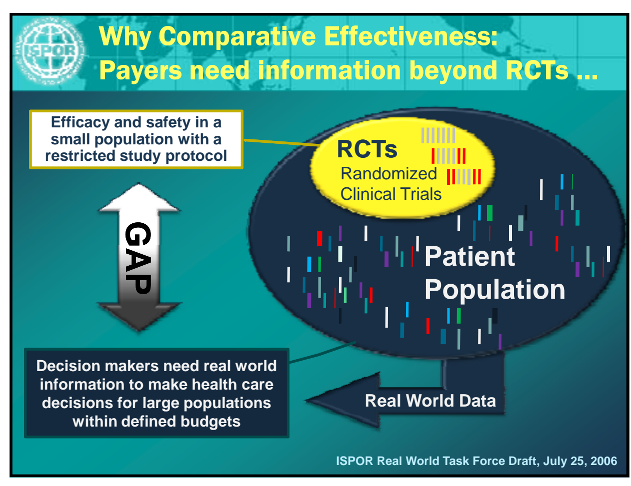
Three Potential Transformational CER Initiatives: CMTP, CTTI, PACE
• Center for Medical Technology & Policy (CMTP) (Collaborative stakeholder and proof of concept movement)
• Clinical Trial Transformation Initiative (CTTI) (Movement to streamline operations, logistics, and regulatory inefficiencies)
• Pragmatic Approaches to Comparative Effectiveness (PACE) Initiative (Streamlining analytical…including Bayesian adaptive… methods)

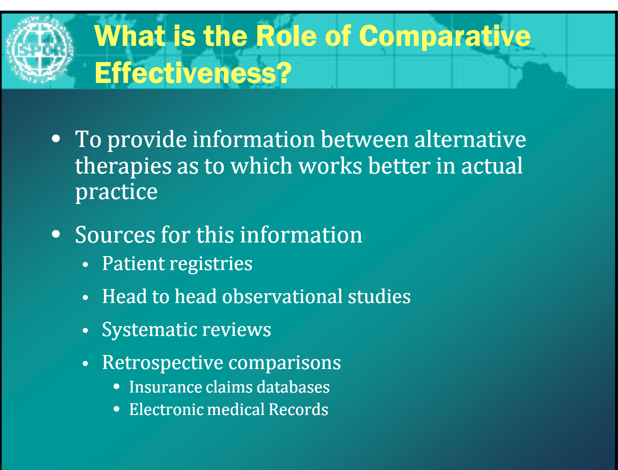

Some CER Trial Questions to Address
• How can we make maximum use of the evidence we have when designing a CER trial?
• When do we know (what is the minimal threshold) when we have "just enough" evidence for a real world (read, coverage decision maker; clinical guideline committee) decision? Is there any such thing as "p = .05" in the real world?
• To what extent can dynamic predictive simulation reduce risk of failure? Or improve opportunity for success in designing the pragmatic trial?

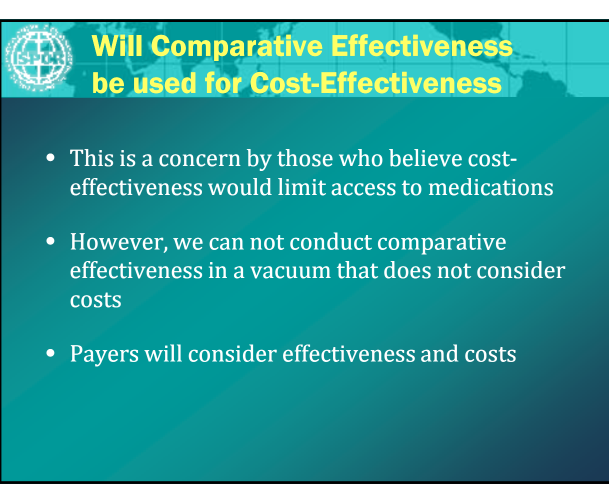
Some CER Trial Questions to Address (continued)
• Can comparative effectiveness trials be designed to evaluate heterogeneity of treatment effect; of patient preference, adherence, etc.? (Role for adaption process?)
• Can we design continuous learning trials to mirror IOM's "learning health care system" concept? For instance, can we adapt the trial design as we gain experience in a drug's place in therapy?


Some CER Trial Questions to Address (continued)
• How much does cost, time and/or risk need to be decreased for a manufacturer to be willing to fund a CE trial of its product? (e.g., what is the ROI elasticity?)
• To what extent can/should Phase 3/3B be coordinated with CER needs of payers?

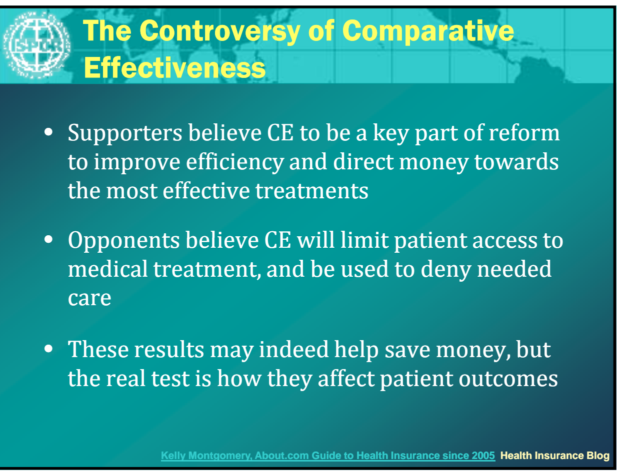
Comparative Effectiveness Studies: The Value of Research and Cost Analysis to Managed Care
Diana Brixner RPh, PhD
Executive Director, Pharmacotherapy Outcomes Research Center,
University of Utah, Salt Lake City, UT, USA

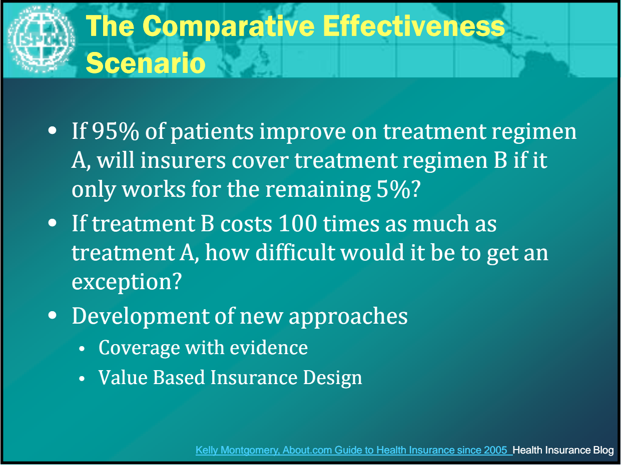

Why Comparative Effectiveness: Payers need information beyond RCTs …
Efficacy and safety in a small population with a restricted study protocol
RCTs
Randomized Clinical Trials
GAP
Patient Population
Decision makers need real world information to make health care decisions for large populations within defined budgets
Real World Data
ISPOR Real World Task Force Draft, July 25, 2006


What is the Role of Comparative Effectiveness?
To provide information between alternative therapies as to which works better in actual practice
Sources for this information
Patient registries
Head to head observational studies
Systematic reviews
Retrospective comparisons
Insurance claims databases
Electronic medical Records


Will Comparative Effectiveness be used for Cost-Effectiveness
This is a concern by those who believe cost-effectiveness would limit access to medications
However, we can not conduct comparative effectiveness in a vacuum that does not consider costs
Payers will consider effectiveness and costs

International Society of Pharmacoeconomics and Outcomes Research (ISPOR) Letter to CBO
"If we appropriately execute comparative effectiveness studies, then the cost-effectiveness analyses that are conducted also will improve based on a more accurate denominator."
- ISPOR Past, Current and Elected Presidents
http://www.ispor.org/workpaper/ispor_comments/index.asp

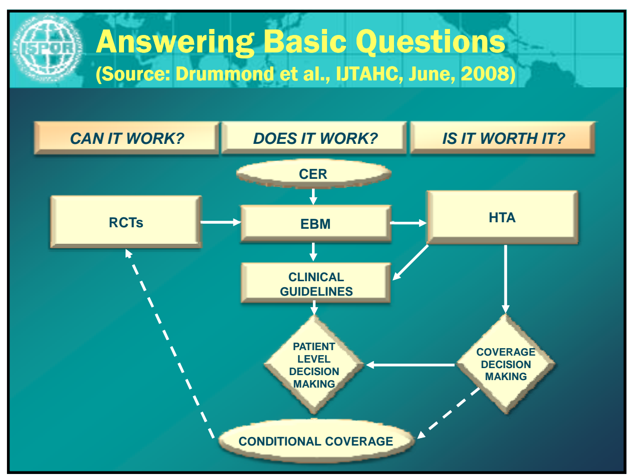

The Controversy of Comparative Effectiveness
Supporters believe CE to be a key part of reform to improve efficiency and direct money towards the most effective treatments
Opponents believe CE will limit patient access to medical treatment, and be used to deny needed care
These results may indeed help save money, but the real test is how they affect patient outcomes
Kelly Montgomery, About.com Guide to Health Insurance since 2005 Health Insurance Blog

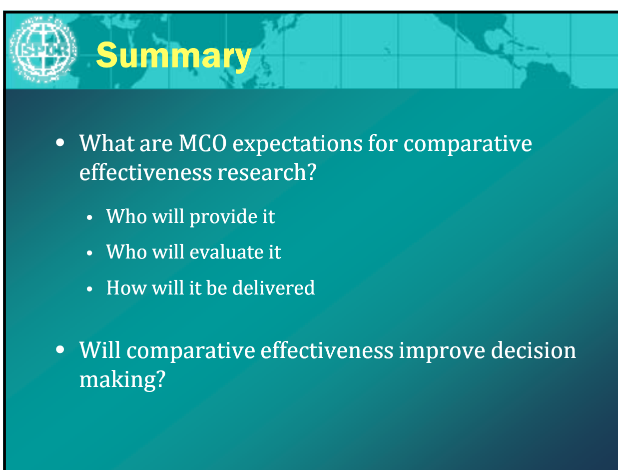


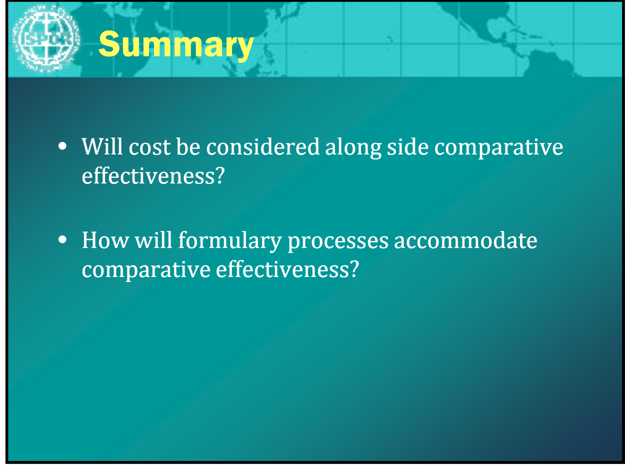
Push to Compare Treatments Worries Drug, Device Makers

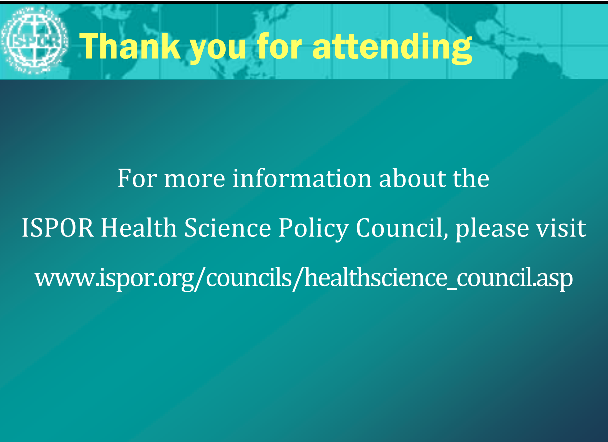
By JANE ZHANG

WASHINGTON -- Federal health-care agencies are getting $1.1 billion in economic-stimulus funds for research comparing the effectiveness of various treatments. But drug and medical-device makers, along with some members of Congress, say they are worried the findings will be used to limit patients' options.





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