Comparative Effectiveness Research:
The Fantastic Voyage Through Policy And Politics

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Presentation to the National Comparative Effectiveness Summit
September 17, 2013
This presentation at a glance

- The role of evidence in health care and the concept of “comparative effectiveness (CER)”
- How CER first came into the policy arena
- The tangled politics behind comparative effectiveness research over the years – a truly “fantastic voyage”
- Ongoing themes: tension between desire to have best evidence and fears of providers, patients, producers and politicians that use of the evidence in care decisions will contravene their interests in some way
- Has led to comparative effectiveness being supplanted in the policy and political environment “patient centered” outcomes research
But first... a story
Imagine a country...

With an economy the size of France: $2.7 trillion...

With tens of millions of unhealthy people – and life expectancy below that of 28 of the world’s richest countries...

Where every day, a group of the natives “experiment” on others by subjecting them to “medical care,” about half of which has no evidence suggesting that it works...

Where what you get in “medical care” varies from doctor to doctor and place to place, such that geography really is destiny....

Where tens of millions don’t get much if any care, experimental, variable or not...

And partly because of the cost of the flawed care it does provide, the country is possibly going broke!
What would you do with this country?

- Send in the Marines?
- Send in the International Monetary Fund?
- Send in Amnesty International?
- Other?
What The US Did In 2010...
Enact The Affordable Care Act
Comparative Effectiveness Research

PCORI
PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE
Where did this concept come from?

What are the tangled politics that brought PCORI about?
The Evolutionary History of Comparative Effectiveness
Definitive text
The role of evidence in health care

Because of advances in research over past 60 years, clinical scientists can estimate with considerable precision whether a particular intervention will lead to net benefit over harm in groups of individuals possessing certain characteristics.


“What research evidence exists is predominantly of poor quality.”

Key health care leaders who advanced the field

- 1964 - Alvan Feinstein, MD (1925-2001) argues that practice of clinical medicine needed to adopt more rigorous scientific methods than were being employed.

- 1981 – David Sackett, MD (1934- ) develops “critical appraisal of the literature,” eventually developed into Users’ Guides in the *Journal of the American Medical Association*. 
Key health care leaders who advanced the field

- 1970s - Archibald L. Cochrane (1909-1988) writes *Effectiveness and Efficiency*, arguing that a treatment should not be considered effective until proven to cause more good than harm (preferably through a randomized trial)

- 1992 – the first Cochrane Centre is opened in Britain; eventually others follow in Canada, the US, other European countries, Australia, Brazil, South Africa
Cochrane Collaboration

- International network of more than 31,000 people in more than 120 countries
- Database of “systematic reviews” – critical summaries by specialty or subspecialty of all relevant research
- More than 5,000 reviews now in database
- About 3,000 more in progress
Agency for Health Care Policy and Research (now Agency for Healthcare Research and Quality)

- 1980s: members of Congress became aware of research showing wide variations in clinical practice and widespread inappropriate use of common surgical procedures

- Bipartisan support, including from George H.W. Bush administration

- Created AHCPR amid little opposition in 1989 to conduct outcomes research and practice guideline development

- Source: BH Gray et al, Health Affairs, June 2003
Agency for Health Care Policy and Research (now Agency for Healthcare Research and Quality)

- Narrowly escaped being eliminated in 1995 amid several forces of opposition

- E.g., an association of back surgeons disagreed with conclusions of Patient Outcome Research Teams on value of back surgery and practice guidelines that followed; appealed to House Republicans to kill agency

- Agency survived and was renamed Agency for Healthcare Research and Quality; prohibited from developing practice guidelines but can catalog guidelines developed by others

Source: BH Gray et al, Health Affairs, June 2003
Meanwhile, thinking evolves on the locus of evidence-based medicine...
+ New terminology evolves: “Comparative effectiveness”

- In one sense, all research is “comparative” to something
  - a placebo or standard therapy

- Most of cancer research has always been “comparative effectiveness” as new therapies are almost always compared to existing standard therapy, not placebo

- In 2000s, “comparative effectiveness research” becomes a buzz phrase meant to denote research comparing two or more active interventions.

  Source: Ashton and Wray, *Comparative Effectiveness Research*
Political trajectory since the 1990s

- 2002 – Then Representative Tom Allen (D-Maine) introduces legislation to require AHRQ to conduct comparative effectiveness studies on prescription drugs heavily used in Medicare and Medicaid

- CER provision introduced in debate over 2003 Medicare Modernization Act, which created the Part D drug benefit

- Controversial even then: Senator Mike Enzi (R-WY) says it will lead to “one size fits all medicine”

- Opposition from pharmaceutical manufacturers and device makers
Political trajectory since the 1990s

- Provision on CER that authorized $50 million for AHRQ to study “the clinical effectiveness and appropriateness of specified health services and treatments” incorporated into final version of Medicare Modernization Act

- Passed with bipartisan support

- Legislation forbade CMS from using research to withhold coverage for a particular prescription drug or to “mandate a national standard”
Political Trajectory in the 2000s

- Additional CER investments debated as part of CHIP reauthorization in 2007 – to be financed by tax on payers to form a Health Care Comparative Effectiveness Research Trust Fund

- Institute of Medicine (IOM) mounts two roundtables – one that proposes a public-private consortium for “effectiveness research”

- Opinion leaders including Gail Wilensky, former HCFA administrator, discuss prospect of a “multi-billion dollar agency” to carry out CER
Debate leading up to Affordable Care Act

- 2008: Competing visions develop in House and Senate, and among stakeholder groups, of how to move CER forward
- Senate Finance Committee wants it housed in a new nonprofit corporation; Senate HELP committee wants it lodged at AHRQ
- Pharmaceutical industry presses for representation of medical products sector on governing boards
- Coalition forms among PhRMA, BIO and AdvaMed to promote industry agenda on CER
American Recovery and Reinvestment Act, 2009 (Stimulus law)

- Created a Federal Coordinating Council for Comparative Effectiveness Research to coordinate research investment across agencies

- Allocated $1.1 billion as follows

  - $300 million to AHRQ
  - $400 million to NIH
  - $400 million to Office of the Secretary of HHS
Institute of Medicine Recommendations

- June 2009: Listed 100 questions on which there was urgency

- E.g., Compare the effectiveness of primary prevention methods, such as exercise and balance training, versus clinical treatments in preventing falls in older adults at varying degrees of risk.

- According to CDC, in 2000, falls in US health care system cost $30 billion in 2010 dollars
The ACA Debate and CER

- Concerns about restricting choices, rationing, death panels dog the debate

- Sen. Charles Grassley, R-Iowa: “When it’s used in a way of informing patients and providers about best practices, it’s a good thing to have. [But] “I’m also worried that this could be used as a tool for the government to ration care.”

- Could become “A program similar to the one in Great Britain and other foreign governments that decide which treatments you can and cannot have.”
PCORI is born

- Senate Finance committee version ultimately prevails in final law
- PCORI is created as “neither an agency nor establishment of the United States Government”
- Governmental oversight by the Comptroller General of the US and the Government Accountability Office
- Trust fund with triple revenue stream: appropriations from general fund, funds transferred from Medicare Trust Funds and fees imposed on health insurance and self-insured plans
- 19-member board of directors with representatives of key stakeholder groups and numbers specified to balance opposing interests
PCORI’s Definition of PCOR: Redefining the terms

“Patient-centered outcomes research helps people and their caregivers communicate and make informed health care decisions, allowing their voices to be heard in assessing the value of health care options. The research answers patient-centered questions such as:

1. Given my personal characteristics, conditions and preferences, what should I expect will happen to me?

2. What are my options and what are the potential benefits and harms of those options?

What can I do to improve the outcomes that are most important to me?

How can clinicians and the care delivery systems they work in help me make the best decisions about my health and health care?
PCORI: Redefining the terms

“To answer these questions, PCOR

Assesses the benefits and harms of preventive, diagnostic, therapeutic, palliative or health delivery system interventions to inform decision making, highlighting comparisons and outcomes that matter to people;

Is inclusive of an individuals preferences, autonomy and needs, focusing on outcomes that people notice and care about such as survival, function, symptoms, and health related quality of life;

Incorporates a wide variety of settings and diversity of participants to address individual differences and barriers to implementation and dissemination; and

Investigates (or may investigate) optimizing outcomes while addressing burden to individuals, availability of services, technology, and personnel, and other stakeholder perspectives.”
Where are we now? PCORI

- Slow to get going
- Board appointed in September 2010; as prescribed in statute, many different interests and objectives
- Set National Priorities for PCOR in 2012
- Has approved more than $303 million in awards since 2012
- Awarding grants to build a national data infrastructure to advance comparative effectiveness research
- Sunsets in 2019 unless reauthorized
Case in Point: Joint PCORI, National Institute on Aging Study on Falls Among Elderly

- Announced in June 2013
- Originally identified in IOM report in 2009
Friendly Critiques

“The Patient-Centered Outcomes Research Institute Should Focus On High-Impact Problems That Can Be Solved Quickly”

Harold Sox, MD

“Because its funding expires in 2019, the institute has little time in which to produce timely, practice-changing results that will build public support for comparative effectiveness research.”

Source: *Health Affairs*, October 2012
Building national data infrastructure for conducting CER
Will it Get Us To the Land of Effective, Patient-Centered Care?
KEEP CALM AND WATCH THIS SPACE
The End