Comparative Effectiveness Research in the United States

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Comparative Effectiveness and Improved Patient Health Outcomes

- Comparative Effectiveness as a Public Good
- Turning Evidence Into Action
- Right care to the right patient at the right time
Comparative effectiveness should be a public good that:

- Gives health care decision makers – patients, clinicians, purchasers and policy makers – access to the latest open and unbiased evidence-based information about treatment options
- Informs choices and, where possible, is closely aligned with the sequence of decisions patients and clinicians face

The Right Treatment for the Right Patient at the Right Time
Sometimes we can’t see the picture clearly.
Current Challenges

- Growing concerns about health spending – about $2.3 trillion per year in the U.S. and the causes of increased spending
- Large variations in clinical care
- A lot of uncertainty about best practices involving treatments, procedures, and technologies
- Pervasive problems with the quality of care that people receive
Sometimes we can’t see the picture clearly
If we answer the wrong question, we can’t see the whole picture
What Healthcare Decision Makers Need to Know

- **Can it work?**
- **Will it work?**
  - For this patient?
  - In this setting?
- **Is it worth it?**
  - Do benefits outweigh harms?
  - Do benefits justify costs?
  - Does it offer important advantages over existing alternatives?

Adapted from Brian Haynes
ACP Journal Club
Questions for Setting Clinical Policy: A Systematic Process

1. What is the outcome I care most about?
2. How good is the evidence that the interventions can improve those outcomes?
3. How sure am I that it will work in “real world”?
4. How do the potential benefits compare to possible harms and costs?
5. What constitutes “good enough” evidence?
6. What other considerations are relevant?
Sometimes we can’t see the picture clearly

If we answer the wrong question, we can’t see the whole picture

Success means breaking away from traditional beliefs
What Evidence-Based Medicine Is

“Evidence-based medicine is the integration of best research evidence with clinical expertise and patient values”

Sackett, et al 2001
Misperceptions About Evidence-Based Medicine

- Restricted to Randomized Controlled Trials
  - sets unattainable standard for evidence
- Excludes role of clinical judgment
- Does not take into account the individual patient
- Aims only to limit health services
- Ignores realities of practice
  - reimbursement, liability concerns, patient expectations
- Useless when evidence is uncertain
Comparative Effectiveness Research Cannot

- Solve controversies due to values, costs, etc.
- Solve barriers due to misaligned incentives, patient factors, and system failures
- Ensure appropriate application to policy
But Can...

- Reduce the chance of getting it wrong
- Help make decisions more consistent, transparent and rational
- Clarify nature of disputes over practice and policy
- Persuade skeptical parties(?)
Comparative effectiveness research addresses issues including:

- Relevancy
- Timeliness
- Impact on priority populations
- Disproportionate impact on subpopulations
- The ability to enhance treatment decisions
AHRQ’s Role in Comparative Effectiveness

- Lead USA national agency for comparative effectiveness research
- HTA at the request of CMS
- Analyze data/options for CED and post CED data collection
- Provide translation of comparative effectiveness findings
- Promote and fund comparative effectiveness methods research
- Fund training grants focused on comparative effectiveness
Comparative Effectiveness Research at AHRQ

- Created in 2005, authorized by Section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act (MMA) of 2003

- Purpose is to compare the effectiveness of different health care interventions

- Goal: to provide patients, clinicians and policy makers with reliable, evidence-based healthcare information
$1.1 B for comparative effectiveness research:

- AHRQ: $300M
- NIH: $400M (appropriated to AHRQ and transferred to NIH)
- Office of the Secretary: $400M (allocated at the Secretary’s discretion)
- Funds are available through September 30, 2010
AHRQ’s CER Role under ARRA

- AHRQ to continue its CER work under the auspices of section 1013 of MMA

- AHRQ will continue to have a collaborative, open, and transparent process for comparative effectiveness that allows for input from all perspectives.

- AHRQ will continue to involve all stakeholders in the research process.

- To determine what priorities and projects will be funded under this new authority, we are undertaking a process to determine what will be funded, working with the Office of the Secretary, NIH, and IOM. We also will be seeking external input as AHRQ has done in the past.
Effective Health Care Home

One of the greatest challenges in making health care decisions is finding reliable and practical data that can inform these decisions. The Effective Health Care Program is dedicated to facilitating decision making by providing findings from high-quality research in formats for different audiences. Learn more.

Spotlight

6/19/2008  Pastillas para la diabetes tipo 2: Guía para adultos
Spanish language version of Pills for Type 2 Diabetes: A Guide for Adults Consumer Summary Guide now available.

Consumer Summary Guide Published

6/11/2008  Fracture Prevention Treatments for Postmenopausal Women with Osteoporosis
Clinician Summary Guide published

5/30/2008  A Case Control Study to Assess Association of Variations in OCT Genes with Effectiveness of Metformin in Diabetic Patients
New DECIDE abstract published

5/23/2008  Methods for Studying Dementia Treatment and Outcomes in Observational Databases
New DECIDE final report now available

5/23/2008  Comparative Effects of Classes of Antidepressants on the Risk of Aspiration Pneumonia in the Aged
New DECIDE final report now available

Get Involved

These are several ways you can get involved in Effective Health Care.

- Suggest Research
- Read Suggestions
- Provide Comments

http://effectivehealthcare.ahrq.gov
Conceptual Framework

- Horizon Scanning
- Evidence Need Identification
- Evidence Generation
- Dissemination & Translation
- Evidence Synthesis
- Research Training
- Career Development
- Stakeholder Input & Involvement
Overall Goals of Topic Selection

- Identify research topics that address important decisional dilemmas for stakeholders
- Ensure that the research agenda is stakeholder-driven
- Use priority health conditions to help guide research
- Define and implement specific criteria for topic selection
- Achieve transparency
Selection Criteria

Appropriateness
- 3 criteria

Importance
- 7 criteria

Duplication
- 1 criterion

Feasibility
- 1 criterion

Potential Impact
- 6 criteria
Selection Criteria

- Appropriateness
- Importance
- Duplication
- Feasibility
- Potential Impact

- A healthcare drug, intervention, device, or technology available in the US
- Relevant to 1013 enrollees
- Priority conditions
Selection Criteria

- Appropriateness
  - Significant disease burden
  - High public interest
  - Nominated/strongly supported by one or more stakeholder groups
  - Area of uncertainty
  - Variation in care

- Importance
- Duplication
- Feasibility
- Potential Impact
Selection Criteria

- Appropriateness
- Importance
- Duplication
- Feasibility
- Potential Impact

- Would not be redundant (i.e. the proposed topic is not already covered by available or soon to be available research)
Selection Criteria

- Appropriateness

- Importance

- Duplication

- Feasibility

- Potential Impact

Utilizes existing research and knowledge by considering adequacy & volume of research
Selection Criteria

- Appropriateness
  - Potential for significant health impact
- Importance
  - Potential risk from inaction
- Duplication
  - Potential for change
- Feasibility
  - Addresses inequities, vulnerable populations
- Potential Impact
Emerging Methods in Comparative Effectiveness & Safety

- Variation in methods among systematic reviews undercuts transparency
- Poorly done new research can be misleading
- Methods must continue to evolve and not remain stagnant
- AHRQ has and will continue to make investments in improving methods, esp. in understanding clinical heterogeneity.
The Future of CER

- Public-private funding and participation likely a necessity
- More effort to get more relevant and responsive research
- Methods, methods, methods
- Need to tackle important issues
  - Ethical
  - When to know when the evidence is sufficient
  - Transparency
  - Setting priorities and for whom
  - Patient-centered, what is good for the public may not be good for the individual
Future Challenges

- Downstream effects of policy applications
  - Diffusion of technology
  - Effects on innovation
  - Unintended consequences
  - International collaboration
  - Understanding the role of costs in the U.S.
  - Determining where comparative effectiveness should reside
To achieve widespread use and influence, trust must be established among:

- Patients, who will benefit from informed decisions and understanding the importance of participating in studies
- Industry, so they understand they will not be inappropriately dealt with
- Physicians, who need comparative effectiveness information to treat patients
- Providers/plans and policymakers, must make tough decisions
According to Yogi Berra

- If you don't know where you are going, you might wind up someplace else.