WE HAVE TWO OPTIONS. EITHER AN EVIDENCE-BASED TREATMENT OR AN EXCITING, RISKY ALTERNATIVE.
Comparative Effectiveness and Improved Health Outcomes

- Comparative Effectiveness as a Public Good
- Turning Evidence Into Action
- Right care to the right patient at the right time
Shared Perspectives on Comparative Effectiveness

- 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 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
“In the varied topography of professional practice, there is a high, hard ground, where practitioners can make effective use of research-based theory and techniques, and there is a swampy lowland where the situations are confusing ‘messes’ incapable of technical solution. The difficulty is that the problems of the high ground, however, great their technical interest, are often relatively unimportant to clients or the larger society while in the swamp are the problems of greatest human concern.”
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

73 yo with DM, CHF, and HTN
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
- 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(?)
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

- New federal initiative to compare the effectiveness of different health care interventions

- Goal: to provide patients, clinicians and policy makers with reliable, evidence-based healthcare information
Comparative Effectiveness: AHRQ Effective Health Care Program

- Uses current, unbiased evidence in making comparisons to show which health interventions:
  - Add Value
  - Offer minimal benefit above current choices
  - Fail to reach their potential
  - Work for some patients, but not others

Goal: to develop and disseminate better evidence about benefits and risks of alternative choices
New Priority Conditions for the Effective Health Care Program

- Arthritis and non-traumatic joint disorders
- Cancer
- Cardiovascular disease, including stroke and hypertension
- Dementia, including Alzheimer Disease
- Depression and other mental health disorders
- Developmental delays, attention-deficit hyperactivity disorder and autism
- Diabetes Mellitus
- Functional limitations and disability
- Infectious diseases including HIV/AIDS
- Obesity
- Peptic ulcer disease and dyspepsia
- Pregnancy including pre-term birth
- Pulmonary disease/Asthma
- Substance abuse
Approaches to research

- Review and synthesize published and unpublished scientific evidence on a topic
- Generate new scientific evidence to address knowledge gaps
- Translate research into concise reports for practical use by clinicians, patients and policy makers
Comparative effectiveness research addresses issues including:
- Relevancy
- Timeliness
- Impact on priority populations
- Disproportionate impact on subpopulations
- The ability to enhance treatment decisions
The US Landscape for CER

- Well intentioned
- Ad hoc except for AHRQ’s mandate
- Limited capacity to do the research and translate the research into meaningful and useable applications
Available products

- Research Reviews: comparative effectiveness reviews and synthesis of existing scientific research
- New Research: reports covering new evidence
- Technical Briefs: brief reports synthesizing research on new medical technologies
- Summary (translation) Guides: guides explaining research results for practical use by health care decision-makers.
Emerging Methods in Comparative Effectiveness & Safety

- Variation in methods among systematic reviews undercuts transparency
- Methods reduce the likelihood of scientific impartiality
- Methods help minimize misclassification of data
- Methods must continue to evolve and not remain stagnant
- AHRQ has and will continue to make investments in improving methods
The Future

- Public-private funding and participation likely a necessity
- More effort to get better conditional reimbursement study designs/protocols
- Need to tackle important issues
  - Ethical
  - When to know when the evidence is sufficient
  - Transparency
  - Setting priorities
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
According to Yogi Berra

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