Comparative Effectiveness in Health Care Reform

Carrie Hoverman Colla, Ph.D.
Dartmouth Medical School
Norris Cotton Cancer Center
Doctors and cancer patients often face uncertainty about which treatment is best.

Source: Dartmouth Atlas.
Use of “effective” care does not vary across regions.
Problem

- Hard evidence is often unavailable about:
  - which treatments work best for which patients
  - whether the added benefits of more effective but more expensive treatments are sufficient to warrant their additional cost

  ➢ Variation in treatments may be greatest when evidence about relative effectiveness is lacking
What is Comparative Effectiveness Research?

• **IOM Definition:** The generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition, or to improve the delivery of care.

• **The hope:** Generation of evidence to help doctors and patients make better decisions.
Characteristics of CER

• Direct, head-to-head comparisons

• Broad range of topics:
  ◦ tests, treatments/procedures, strategies for prevention, care delivery and monitoring

• Broad range of beneficiaries:
  ◦ patients, clinicians, purchasers, and policy makers

• Study populations representative of clinical practice

• Focus on patient-centered decision-making
  ◦ tailor the test or treatment to the specific characteristics of the patient (determine treatment response heterogeneity)
CER Methods and Drawbacks

- Randomized Controlled Trials
  - Expensive, long duration
- Observational Studies (e.g. claims analyses or registries)
  - Confounding
- Systematic Reviews
  - Contribution is constrained by the extent and the quality of the underlying evidence
Should costs be a part of CER?

- CER should generate data to support clinical decision making
  - Includes utilization, patient preferences, and outcomes
  - Clinical prediction rules for estimating probabilities
Recent Focus on CER

- **2009: Stimulus package**
  - $1.1B divided between AHRQ, NIH, and the Secretary DHHS
  - IOM report on national priorities for CER

- **2010: Affordable Care Act establishes the Patient-Centered Outcomes Research Institute (PCORI)**
  - $500M annual funding, not subject to annual appropriation
  - Government and private insurance will be taxed a fee per insured life
  - ACA limits role of PCORI-funded research in coverage decisions
PCORI

• Is a public-private partnership
• Is required to release findings to the public and seek comment during deliberations
• Will set the CER research agenda by identifying priorities and funding and facilitating new studies
IOM prioritized 11 studies in Oncology and Hematology
Example IOM Priority Areas

Compare the effectiveness of...

• management strategies for localized prostate cancer (e.g., active surveillance, radical prostatectomy, and radiotherapy) on survival, recurrence, side effects, quality of life, and costs.

• film-screen or digital mammography alone and mammography plus MRI in community practice-based screening for breast cancer in high-risk women of different ages, risk factors, and race or ethnicity.

• new screening technologies (such as FIT and CT colonography) and usual care (FOBT and colonoscopy) in preventing colorectal cancer.

• imaging technologies in diagnosing, staging, and monitoring patients with cancer including PET, MRI, CT.

• genetic and biomarker testing and usual care in preventing and treating breast, colorectal, prostate, lung, and ovarian cancer, and possibly other clinical conditions for which promising biomarkers exist.
Center for Comparative Effectiveness Research in Cancer Imaging

• Supported by a GO grant from NCI
• Provides a national infrastructure for CER initiatives for advanced imaging in cancer
• 5 Institutions: Dartmouth, Brown, VCU, Wash U & Tufts (PI: Anna Tosteson)
• Aims to: advance methodologies that extend CER capacity and undertake CER projects specific to PET
Awards From The National Institutes Of Health For Evidence Development And Synthesis, By Institute Of Medicine Priority Area, August 2010.

Source: Benner J S et al. Health Aff 2010;29:1768-1776
CER and the American Public

National Survey of Attitudes toward CER (N=2,017, May 2010)

The public supports the use of CER findings to provide information to health consumers, but there is less support for the use of study results to restrict treatment options.

<table>
<thead>
<tr>
<th></th>
<th>Mean Support Score (X/100)</th>
</tr>
</thead>
<tbody>
<tr>
<td>All respondents</td>
<td>62</td>
</tr>
<tr>
<td>Party affiliation</td>
<td></td>
</tr>
<tr>
<td>Democrat</td>
<td>71</td>
</tr>
<tr>
<td>Republican</td>
<td>51</td>
</tr>
<tr>
<td>Independent</td>
<td>60</td>
</tr>
</tbody>
</table>

Source: Gerber A S et al. Health Aff 2010;29:1872-1881
Americans’ Support For Uses Of Comparative Effectiveness Research, 2010

To what extent would you support or oppose using comparative effectiveness research to:

Provide information about whether a given treatment works better than alternative ways of treating patients with the same condition

Create warning labels for treatments that are not supported by strong scientific evidence

Determine whether Medicare and private insurers will cover new treatments that have just become available

Determine whether Medicare and private insurers will cover old treatments that doctors have used for some time

Provide information to Congress, doctors, and patients about whether an expensive treatment is worth its cost

Determine what groups of patients should be protected from budget cuts in Medicare and other government health programs

Charge a patient more to get a treatment that research has not shown to be effective, even if the patient’s own doctor recommends it

Source: Gerber A S et al. Health Aff 2010;29:1872-1881
CER in Cancer Care: An Example

Medications To Reduce the Risk of Primary Breast Cancer: tamoxifen and raloxifene.

Tamoxifen and raloxifene are both effective at reducing the risk of primary invasive breast cancer in women age 35–70.
Level of Confidence: ●●●

Raloxifene and tamoxifen reduce the likelihood of a woman developing breast cancer by a similar amount.
Level of Confidence: ●●○

Neither tamoxifen nor raloxifene reduce all-cause mortality.
Level of Confidence: ●●●

Raloxifene and tamoxifen both increase the risk of thromboembolic events (deep vein thrombosis and pulmonary embolism).
Level of Confidence: ●●●

Tamoxifen increases the risk of endometrial cancer.
Level of Confidence: ●●●

<table>
<thead>
<tr>
<th>Drug Name</th>
<th>Brand Name</th>
<th>Dose¹</th>
<th>Route</th>
<th>Price Per Month² Generic</th>
<th>Price Per Month² Brand</th>
</tr>
</thead>
<tbody>
<tr>
<td>Raloxifene</td>
<td>Evista*</td>
<td>60 mg once a day³</td>
<td>Oral</td>
<td>NA</td>
<td>$115</td>
</tr>
<tr>
<td>Tamoxifen</td>
<td>Nolvadex*</td>
<td>20 mg daily for 5 years</td>
<td>Oral</td>
<td>$115</td>
<td>$245</td>
</tr>
</tbody>
</table>

For women who have never had breast cancer, both tamoxifen and raloxifene reduce invasive breast cancer risk by about 50 percent.

For example, this chart shows the overall risk for women in the United States age 50 to 59. Their risk of getting invasive breast cancer in the next 10 years is about 2 in 100. This means that out of every 100 women, about 2 will get breast cancer. If all 100 of these women take medicine to reduce the risk of breast cancer, about half as many (1 woman) will get breast cancer.

Ḫ = the number of women who will get invasive breast cancer without taking medicine (2 out of 100).

Ḫ = the number of women who will get invasive breast cancer when all the women take medicine (1 out of 100).

Raloxifene does not lower the risk of non-invasive breast cancers (LCIS and DCIS). Research can’t tell us yet about tamoxifen and non-invasive breast cancers.
AHRQ has also supported CER studies on:

- Radiotherapy treatments for head and neck cancers
- Diagnostic technologies for breast cancer screening
- Therapies for localized prostate cancer
- Red blood cell-stimulating agents for managing anemia in cancer
Questions

• Who should pay for treatments that are part of CER studies?
• Will CER lead to more personalized medicine?
• Will doctors and patients use the results of CER?
• Will insurance design change based on CER results?
• With better evidence, will high-intensity health care regions reduce their use of resources?

