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Sacramento, CA
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COMPARATIVE EFFECTIVENESS
AND THE LIMITS OF EVIDENCE
Why is Washington interested in comparative effectiveness research?
Medicare sources of non-interest income and expenditures as a percentage of Gross Domestic Product

Unfunded liability
$7600 per working age adult*

*In constant 2008 dollars

Source: Office of the Actuary, CMS; 2008 Medicare Trustees Report
Comparative Effectiveness Research May Reduce Expenditures

Lewin Associates: $368 billion 10-year savings from “Center for Medical Effectiveness”

*Lewin Associates calculations, in Bending the Curve, Commonwealth Fund Commission on a High Performance Health System, Dec. 2007*
What can we hope to achieve from the collection of better evidence...

...and how can we apply it?
Overriding questions about comparative effectiveness

Structure and funding of agency that is responsible for the research

Priorities - which questions should be studied first?

Data and methods - will new information be collected? What kind?

Implementation

Will comparative effectiveness ignore costs?
Priorities: How CMS Sets Evidentiary Priorities for Coverage with Evidence Development (CED)

Importance of question

Which diseases represent the greatest burden to Medicare beneficiaries?

Which diseases and their treatments are the costliest to the Medicare Program?
Value of incremental information

- Where are our greatest deficits in knowledge about the most important diseases and their treatments in the Medicare population as discussed in Questions 1 and 2?
Data and Methods: How Good Will the Evidence Be?
Options: Review existing data, or generate new information

- Randomized trials
- Registry
- Routinely collected clinical data
- Claims files and other administrative databases
Central methodological challenge: can you draw causal inferences about treatments and other health interventions from observational data?

*Observational data can be refined at a cost*
Common Criticisms of Observational Data

- Intervention may not be standardized
- No blinding
- Tests of statistical significance often misleading
Selection Effects

- Can they be eliminated?

*More detailed information about patient characteristics only partially mitigates*
Inference Techniques

- Exploit natural experiments
  - Geographic characteristics as instrumental variables
- Matched controls without randomization
  - Constructed: propensity scores
  - “Natural”: Intervention for disease whose outcome has little variability under alternative intervention
- Consider “randomization” by geography
- Declining costs of clinically detailed data will increase viability of observational approaches and decrease costs of RCTs
Should Comparative Effectiveness Research Ignore Costs?
### Impact Of Selected Medical Technologies On Spending And Life Years, 2015 And 2030

<table>
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<tr>
<th>Technology</th>
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<th>Increase in health care spending over status quo (%)</th>
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<td>8.7, 13.8</td>
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<td>0.1, 0.4</td>
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**SOURCE:** Simulations based on data from the Medicare Current Beneficiary Survey and the National Health Interview Study.

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Cost-Effectiveness Analysis

- A Complementary Tool
  - Compare incremental costs and incremental outcomes of using a treatment or diagnostic test in a well-defined population
  - Outcomes most frequently expressed as quality-adjusted life years (QALYs)
Gain in health benefit

Change in costs

- Spend less, get less
- Spend less, get more
- Spend more, get less
- Spend more, get more
Accounting for Value

Gain in health benefit

Change in costs

Negative

Positive

Spend less, get less

Spend less, get more

Spend more, get less

Spend more, get more

But is it worth it?
Accounting for Value

Change in costs

Less cost effective

Spend more, get more

More cost effective

Gain in health benefit
Accounting for Value

- Health benefit measured in QALYs
- Value accounted by incremental cost-effectiveness ratio (CER)

\[ \text{CER} = \frac{\text{Chg in $}}{\text{Chg in QALYs}} \]

- Greater CER means less cost effectiveness

Less cost effective

More cost effective

Gain in health benefit (Quality-Adjusted Life Years)
Applying Cost-Effectiveness Analysis:

COX-2 Inhibitors
COX-2 Inhibitors vs NSAIDS

Comparator: Naproxen

Gain in health benefit (QALYs) vs Change in costs

$12k
$6k
$0

$0
0.05
0.10

$100k per QALY

COX-2 Inhibitors vs NSAIDS

Comparator: Naproxen

Assumption: Excludes effects on heart

Change in cost: $11,600

Change in benefit: 0.04 QALYs

Incremental CER: $290,000/QALY

Gain in health benefit (QALYs)

**COX-2 Inhibitors vs NSAIDS**

**Comparator:** Naproxen

**Assumption:** INCLUDES effects on heart

**Change in cost:** $11,600

**Change in benefit:** 0.03 QALYs

**Incremental CER:** $395,000/QALY

COX-2 Inhibitors vs NSAIDS

Comparator: Naproxen

Assumption: High-risk patients

Change in cost: $4,720

Change in benefit: 0.08 QALYs

Incremental CER: $56,000/QALY

Should There Be a Role for Cost-Effectiveness?

- Modeling will almost always be needed to assess global effectiveness
- Comparative effectiveness = relevant comparisons
  - But doesn’t address costs
  - Effect on costs uncertain
Applying Evidence of Effectiveness - And Value

Agency conducting research need not make coverage decisions.
Information can be used to guide practice standards, identify high-performing hospitals and physicians, assist in benefit design.
May be used to negotiate prices.