Conducting comparative effectiveness research: the view of a practicing epidemiologist from the other Washington

Bruce M. Psaty, MD, PhD

Cardiovascular Health Research Unit, Seattle, WA

Introduction

• Patient-centered outcomes research in context

• A short historical narrative about comparative effectiveness research

• Epidemiological observations and recommendations

EBM versus OBM

• No one boasts doing opinion-based medicine (OBM)

• Evidence is generally clear, but not its interpretation or application

• Universal appeal to evidence, reason and science

PCOR Institute

• CER has succeeded evidence-based medicine (EBM) as the current fashion

• In EBM, comparators were not banned, and a major focus was choice of outcomes important to and for patients

• Now CER has legislative mandate

The evidence

• Stroke and CHD incidence
  – Low-dose diuretics: 1.87 events / 100 py
  – Placebo: 2.71 events / 100 py

• NNT: 120 persons for one year

• What is the effect of the SHEP results on the hypertension drug treatment patterns in older adults in the US?

SHEP: JAMA 1991; 265: 3255-64.
Treatment trends in older adults

Comparative effectiveness and safety

β-Blockers and Primary Prevention of Coronary Heart Disease in Patients With High Blood Pressure

The Relative Risk of Incident Coronary Heart Disease Associated With Recently Stopping the Use of β-Blockers

Next generation studies

Academic response

The Risk of Myocardial Infarction Associated With Antihypertensive Drug Therapies

Sounding Board

THE MESSENGER UNDER ATTACK — INTIMIDATION OF RESEARCHERS BY SPECIAL-INTEREST GROUPS

Selected consequences

First recommendation

The Need for Large-Scale Randomized Evidence Without Undue Emphasis on Small Trials, Meta-analyses, or Subgroup Analyses
Marciniak’s review of outcomes

<table>
<thead>
<tr>
<th>Outcome</th>
<th>RSG</th>
<th>Control</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number reviewed</td>
<td>278</td>
<td>271</td>
<td>549</td>
</tr>
<tr>
<td>With problems</td>
<td>45</td>
<td>25</td>
<td>70</td>
</tr>
<tr>
<td>Favors rosiglitazone</td>
<td>44</td>
<td>13</td>
<td>57</td>
</tr>
<tr>
<td>Favors control</td>
<td>1</td>
<td>12</td>
<td>13</td>
</tr>
</tbody>
</table>

Psaty BM. JAMA 2010; 304: 793-4

AHA policy statement on CER

“Comparative effectiveness research should ideally build on data provided by randomized clinical trials by evaluating medical interventions in more diverse populations and in broader clinical contexts.”


Limitations of CER

“… often the inevitable conclusion of a thoughtful attack [on the study design] may be that there are no reliable answers available from the existing data sets…it is better, for eventual progress, to know that we cannot yet reliably answer questions than to pretend that we know the answers based on bogus assumptions and hopelessly inadequate data.”


Concluding observations

- Health of the public
  - Prevention paradox
  - Treatment paradox
- Use best methods and use advocacy
- Predict and expect incremental improvements