Introduction to Economic Evaluation of Healthcare

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Pharmacoeconomics
April 12, 2012
www.uphs.upenn.edu/dgimhsr/fda2012.htm

Evaluation of Medical Care (I)

TRADITIONAL ISSUES

Safety

Efficacy

Effectiveness

Side effects acceptable?

Can it work?

Does it work?

Evaluation of Medical Care (II)

ECONOMIC ISSUES

Efficiency

Are we getting the best outcome for the expenditure
Principles of Economic Assessment

- Rules exist for assessing costs and benefits
- Assumptions are made explicit
- As a result:
  - There is consistency of approach
  - It is clear what is included and excluded from calculations

Resources

- Resources are limited
- Choices must be made
- When a resource is used, the opportunity to use it for something else is lost
- The value of a resource in its best alternative use is its "opportunity cost"

Health Economic Analysis

Types of Analysis
- Identification
- Effectiveness
- Benefits

Types of Costs and Benefits
- Intangible
- Indirect
- Direct

Point of View
- Society
- Patient
- Payor
- Provider

Bombardier and Eisenberg, 1984
Types of Analysis

- Types of analysis generally distinguished by the outcomes included (e.g., costs only vs costs and effects) and how they are quantified (e.g., all in terms of money or in terms of health and money)

Cost Identification

- Also referred to as cost minimization and cost-cost analysis
- Estimates costs of an intervention, but not benefits
- Appropriate only when two options of equal efficacy are compared

original article


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Background: Economic evaluation alongside a randomized phase III study to assess docetaxel–gemcitabine (DG) relative to vinorelbine–cisplatin (VC) combination as front-line treatment of patients with advanced/metastatic non-small-cell lung cancer.

Methods: No differences were found in efficacy, thus a cost-minimization analysis was carried out. Treatment cost accounts for the administration of first- and second-line chemotherapy, for concomitant medications, for laboratory and biochemical examinations, and for hospitalizations due to adverse events. Unit prices used reflect 2008 and are common among National Health Service hospitals in Greece.

Cost-Minimization, Non-Small-Cell Lung Cancer *

<table>
<thead>
<tr>
<th></th>
<th>DG</th>
<th>VC</th>
<th>Diff</th>
<th>95% CI</th>
</tr>
</thead>
<tbody>
<tr>
<td>Chemotherapy</td>
<td>7805</td>
<td>1035</td>
<td>6770</td>
<td>5899 to 7622</td>
</tr>
<tr>
<td>2nd line drugs</td>
<td>933</td>
<td>1836</td>
<td>-903</td>
<td>-1695 to 111</td>
</tr>
<tr>
<td>G-CSF</td>
<td>3074</td>
<td>3016</td>
<td>58</td>
<td>-195 to 83</td>
</tr>
<tr>
<td>Hospitalization</td>
<td>724</td>
<td>711</td>
<td>13</td>
<td>-84 to 112</td>
</tr>
<tr>
<td>Diagnostics</td>
<td>621</td>
<td>613</td>
<td>8</td>
<td>-56 to 72</td>
</tr>
<tr>
<td>Laboratory</td>
<td>719</td>
<td>706</td>
<td>14</td>
<td>-83 to 111</td>
</tr>
<tr>
<td>Toxicity</td>
<td>169</td>
<td>227</td>
<td>-58</td>
<td>-195 to 83</td>
</tr>
<tr>
<td>Total</td>
<td>14,045</td>
<td>8143</td>
<td>5902</td>
<td>4237 to 7528</td>
</tr>
</tbody>
</table>

* Euros


Should This Be a Cost-Minimization Analysis?

- Clinical trials “indicate that the two alternative treatment options considered have similar efficacy in terms of survival”
- “DG option has a more favorable toxicity profile”
- “DG regimen represents a more costly approach in the management of patients”
  - “VC combination could be recognized as the preferred treatment regimen and the significantly higher cost of the DG regimen is an issue that should be taken into account for the final therapeutic decision”
Maniadakis Cost / YOLS

<table>
<thead>
<tr>
<th></th>
<th>DG</th>
<th>VC</th>
<th>Diff</th>
<th>95% CI</th>
</tr>
</thead>
<tbody>
<tr>
<td>Years of life</td>
<td>13.00</td>
<td>12.22</td>
<td>0.78</td>
<td>-1.77 to 3.33</td>
</tr>
</tbody>
</table>

- Cost per YOLS
  - Point Estimate, DC costs more and yields more life years (PE, 7,567 / YOLS) (~69% acceptable at 50k)
  - 95% confident that EITHER
    - DG as or more effective than VC, costs more than VC, and its cost / YOLS ratio falls between ~1700 / YOLS (good value) and ∞ (bad value)
  OR
  - VC is more effective than DG and saves money (i.e., VC dominates DG)

Cost-Effectiveness Analysis (I)

- Estimates costs and outcomes of intervention
- Costs and outcomes are measured in different units
- Results meaningful in comparison with other interventions or a predetermined standard
  - (e.g., $50,000 per quality-adjusted year of life saved)

Cost-Effectiveness Analysis (II)

- Incremental cost-effectiveness ratio:

\[
\frac{\text{Costs}_1 - \text{Costs}_2}{\text{Effects}_1 - \text{Effects}_2}
\]
Cost-Utility Analysis

- Outcomes expressed in units of utility (e.g., QALYs)
- Referred to either as a fourth type of analysis or as a subset of cost-effectiveness analysis

Choosing Among Alternative Interventions

<table>
<thead>
<tr>
<th>Costs</th>
<th>A &lt; B</th>
<th>A &gt; B</th>
</tr>
</thead>
<tbody>
<tr>
<td>A &gt; B</td>
<td>B Dominant</td>
<td>Incremental Cost-Effectiveness Analysis</td>
</tr>
<tr>
<td>A &lt; B</td>
<td>Incremental Cost-Effectiveness Analysis</td>
<td>A Dominant</td>
</tr>
</tbody>
</table>

Cost-effectiveness of extended buprenorphine-naloxone treatment for opioid-dependent youth: data from a randomized trial

Daniel Polley\textsuperscript{1}, Henry A. Glick\textsuperscript{1}, Jiaying Yang\textsuperscript{2}, Geetha A. Subramanian\textsuperscript{2}, Sabrina A. Poole\textsuperscript{2} & George E. Woody\textsuperscript{2}
Buprenorphine/Nalaxone: Opioid Addicted Youth

- The data *

<table>
<thead>
<tr>
<th></th>
<th>Cost</th>
<th>Opioid Free Year</th>
</tr>
</thead>
<tbody>
<tr>
<td>Usual Care</td>
<td>9210</td>
<td>0.319</td>
</tr>
<tr>
<td>Bup/Nal</td>
<td>9293</td>
<td>0.589</td>
</tr>
</tbody>
</table>

- Cost-effectiveness ratio

\[
\frac{9293 - 9210}{0.589 - 0.319} = \frac{83}{0.27} = 307
\]

* 1-year results Polsky et al., Cost-effectiveness of extended buprenorphine-nalaxone... Addiction. 2010;105:1616-24

League Table Cost per QALY

<table>
<thead>
<tr>
<th>Intervention</th>
<th>Ratio (US $*)</th>
</tr>
</thead>
<tbody>
<tr>
<td>CABG for Left Main CAD</td>
<td>4,200</td>
</tr>
<tr>
<td>Neonatal Intensive Care (Birthweight 1-1.499 kg)</td>
<td>4,500</td>
</tr>
<tr>
<td>Neonatal Intensive Care (Birthweight 0.500-0.999 kg)</td>
<td>31,800</td>
</tr>
<tr>
<td>CABG for Single Vessel Disease</td>
<td>36,300</td>
</tr>
<tr>
<td>School TB Testing Program</td>
<td>43,700</td>
</tr>
</tbody>
</table>

* 1983 value Source: Torrance, 1986

Alternatives to QALYS

- Years of life gained
- Lives saved
- Successful treatments
- Cases of illness avoided
- Intermediate outcomes gained

BUT TO BE INFORMATIVE, NEED TO UNDERSTAND WILLINGNESS TO PAY FOR SUCH OUTCOMES
Cost-Benefit Analysis (I)

- Estimate costs and benefits in the same (usually monetary) units
- While analysis still is based on the difference in costs and the difference in benefits, don’t need to include result in a league table or compare to a maximum willingness to pay

Cost-Benefit Analysis (II)

- Net benefits
  Benefit – Cost

- Cost-Benefit Ratio
  Cost / Benefit

Long-term cost-minimization analysis comparing laparoscopic with open (Lichtenstein) inguinal hernia repair

A. El환2, F. Carlson2, A. Banixed1, A. Montgomery3, L. Berglin4 and C. Radduff2 for the Swedish Multicenter Trial of Inguinal Hernia Repair by Laparoscopy (SMUR) study group

1Department of Surgery, Oulu University Hospital, Oulu, Finland, 2Department of Surgery, Theodor Kasenbergs Hospital, Halmstad, Sweden, 3Department of Surgery, University Hospital of Linköping, Linköping, Sweden, 4Department of Surgery, Malmo University Hospital, Malmo, Sweden.

Correspondence to Dr. A. Elna, Department of Surgery, Oulu University Hospital, 52900 Oulu, Finland (e-mail: a.elna@oulu.fi)
Cost-Benefit Analysis, Hernia Repair

Net benefit: (2662.7 + 107.9 + 1767.0) - (1952.1 + 23.4 + 2270.1) = 292 (p=.02)

- Composite measure (part cost-effectiveness, part cost benefit analysis), usually expressed in dollar terms, that is derived by rearranging the cost-effectiveness decision rule:
  \[ W^* > \frac{(Costs_1 - Costs_2)}{(Outcomes_1 - Outcomes_2)} \]
  where \( W^* \) = maximum acceptable cost-effectiveness ratio (e.g., 50,000 per QALY)
- NMB routinely (but not necessarily) expressed on the cost scale, known as net monetary benefits (NMB)
  \[ W \times [Outcomes_1 - Outcomes_2] - (Costs_1 - Costs_2) \]
- Particularly important for statistical evaluation of cost-effectiveness analysis (e.g., sample size; direct statistical testing by use of patient-level data; etc.)

Net Monetary Benefit (2)

- Principal difference between CBA and NMB relates to the source of \( W \)
  - When \( W \) directly measured from "society", CBA
  - When \( W \) administratively determined (e.g., 50,000 or 100,000 / QALY), NMB
Review

- Investigators compared 2 treatments, “LessCost” and “MoreCure”
- They found that “LessCost” was less expensive and recommended its adoption by physicians
  - 100,000 vs 300,000
- What type of economic analysis are the investigators carrying out?
- Do you agree with their conclusion?

Example 2

- Investigators compared 2 treatments, “LessCost” and “MoreCure.” They observed the following:

<table>
<thead>
<tr>
<th></th>
<th>LessCost</th>
<th>MoreCure</th>
<th>Difference</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cost</td>
<td>100,000</td>
<td>300,000</td>
<td>-200,000</td>
</tr>
<tr>
<td>Benefit</td>
<td>100,000</td>
<td>500,000</td>
<td>400,000</td>
</tr>
</tbody>
</table>

- The authors concluded that MoreCure is net beneficial.
- What type of economic analysis are the investigators carrying out?
- Do you agree with their conclusion?

Example 3

- Investigators compared 2 treatments, “LessCost” and “MoreCure.” They observed that MoreCure cost 200,000 more than LessCost and provided 8 additional QALYs
- The authors recommended that MoreCure was good value for the cost
- What type of economic analysis are the investigators carrying out?
- Do you agree with their conclusion?
Types of Costs and Effects Included

Point of View
- Society
- Patient
- Payor
- Provider

Types of Costs and Benefits Included
- Intangible
- Indirect
- Direct

Types of Analysis
- Identification
- Effectiveness
- Benefit

Types of Costs and Benefits Included

Direct Cost

Variable Costs
Fixed Costs

Marginal Cost (I)
- Costs incurred in providing an extra unit of service, or savings realized by providing one less unit
- Calculation unaffected by fixed costs
Marginal Versus Average Cost

- Suppose that:
  - Total drug costs = $50
  - Total doses = 10
  - Average cost / dose = $5

- Suppose, however, that:
  - 9 doses = $49
  - 10 doses = 50
  - Marginal cost of 10th dose = $1

Cost Estimation

- Standard economic assumption
  - Purchase price = cost
- Health care (particularly U.S.)
  - Purchase price ≠ cost or there is no price to observe
- Difference relates to
  - Regulation; free care; cross-subsidization
  - High levels of insurance
  - Health care consumers not having adequate information
Indirect Cost (I)

- Human capital approach
  - Advantages
    - Easy to measure
    - Assess actual gains / losses in productivity
  - Disadvantages
    - Not theoretically correct measure
    - Poor proxy for "Willingness to Pay" (although in some common situations may be a lower bound)
    - "Undervalues" anyone not earning a wage

Indirect Cost (II)

- Willingness to pay approach
  - Advantages
    - Theoretically correct measure
  - Disadvantages
    - Function of ability to pay
    - May be difficult to measure in practice

Utility Assessment

- Methods of utility assessment
  - Direct Assessment
    - With Risk
      - Standard gambles
    - Without Risk
      - Time – tradeoff
      - Category scaling
      - Difference method
  - Indirect Assessment
    - e.g., EQ-5D, HUI, SF-6D
  - Whose preferences
    - General public vs patients
Point of View

Types of Analysis

Identification
Effectiveness
Benefit

Society
Patient
Payor
Provider

Intangible
Indirect
Direct

Types of Costs and Benefits Included

Sensitivity Analysis

- Demonstrates dependence/independence of a result on a particular assumption
- Identifies critical values of variables
- Identifies uncertainties requiring further research

Discounting

- Costs and benefits incurred now are greater than those with a similar nominal value incurred later
- Future costs and benefits must be expressed in terms of present value

\[ PDV = \sum_{t=0}^{N} \frac{C_t}{(1+r)^t} \]
Discounting: an Example

- Assume that a program costs $1,000 this year and for the next 2 years

\[
PDV = \frac{1000}{1.03^1} + \frac{1000}{1.03^2} + \frac{1000}{1.03^3}
\]

i.e., \(PDV = 1,000 + 970.87 + 942.60\)

Hence, \(PDV = 2,913.47\)

Issues in Discounting

- What is the appropriate discount rate for costs?
- Should the monetary costs and non-monetary outcomes be discounted at the same rate?

Distributional Issues

<table>
<thead>
<tr>
<th></th>
<th>Program 1</th>
<th>Program 2</th>
</tr>
</thead>
<tbody>
<tr>
<td>Net Cost</td>
<td>250,000</td>
<td>250,000</td>
</tr>
<tr>
<td>Net Effect</td>
<td>10 Years</td>
<td>10 Years</td>
</tr>
<tr>
<td>C/E Ratios</td>
<td>25,000</td>
<td>25,000</td>
</tr>
<tr>
<td># of Patients</td>
<td>1</td>
<td>5</td>
</tr>
<tr>
<td>who Benefit</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
Features in Health Economic Analysis

- Consistent application of rules
- Marginal costs
- QALYS (and other measures of preference)
- Perspective
- Discounting

Objectives of Health Economic Assessments

- Economic assessments of health care aim at demonstrating the most efficient use of available resources, not at cutting expenditures