Introduction to Effectiveness, Patient Preferences, and Utilities

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HERC Cost Effectiveness Analysis Course
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Overview

- Outcomes measurement in CEA
- Concept of QALYs for a CEA
- Estimating QALYs
- Guidelines on selecting measures
The ICER

- CEA compares the outcomes and costs of two (or more) interventions

\[
\frac{\text{Cost}_{\text{treatment}} - \text{Cost}_{\text{control}}}{\text{Outcomes}_{\text{treatment}} - \text{Outcomes}_{\text{control}}}\]
CEA/CUA review

Compare outcomes and costs across interventions

- Outcome defined by the health benefit achieved with the intervention.

- Outcome(s) quantified in a single scale
Which outcome to use?

1) Mortality/life years gained

- Primary objective is to extend life (e.g. cancer therapies)
- Generic outcome across life-saving interventions
- Does not capture QoL or patient preferences
Which outcome to use?

2) Morbidity/disease specific outcomes

- Choosing among therapies for same condition

  ✓ More practical in clinical trials

  - Limits comparisons between other types of interventions
Which outcome to use?

3) Quality adjusted life year (QALY)

- Combines both quantity and quality of life in one generic measure
- Takes into account patient preferences
- Most guidelines recommend using QALYs
What is a QALY?

- Measure of a person’s length of life weighted by a valuation of their HRQoL

Length of life

\[ \times \]

Quality of life valuations (health utilities)
How to Interpret QALYs

- 1 year in full health = 1 QALY
- 1 year in health state 0.5 = 0.5 QALYs
- Death = 0 QALYs
- Negative values possible
### QALY Example #1

- **Prophylactic antibiotic Rx vs. standard of care**

<table>
<thead>
<tr>
<th></th>
<th>3 mo.</th>
<th>3 mo.</th>
<th>3 mo.</th>
<th>3 mo.</th>
<th>Total QALYs</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>New Txt.</strong></td>
<td>.50</td>
<td>.60</td>
<td>.80</td>
<td>.80</td>
<td>?</td>
</tr>
<tr>
<td><strong>UC</strong></td>
<td>.50</td>
<td>.35</td>
<td>.50</td>
<td>.80</td>
<td>?</td>
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</table>
QALY Example #1

- Prophylactic antibiotic Rx vs. standard of care

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</thead>
<tbody>
<tr>
<td>New T xt.</td>
<td>.50</td>
<td>.60</td>
<td>.80</td>
<td>.80</td>
<td>(.125+.15+.20+.20)</td>
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<td></td>
<td>(.50 x .25)</td>
<td>(.60 x .25)</td>
<td>(.80 x .25)</td>
<td>(.80 x .25)</td>
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<tr>
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<td>(.125+.0875+.125+.20)</td>
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<tr>
<td>UC</td>
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<td>.35</td>
<td>.50</td>
<td>.80</td>
<td>(.125+.0875+.125+.20)</td>
</tr>
<tr>
<td></td>
<td>(.50 x .25)</td>
<td>(.35 x .25)</td>
<td>(.50 x .25)</td>
<td>(.80 x .25)</td>
<td>= .5375</td>
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</table>
Calculating cost/QALY

- ICER – New Rx vs. standard care
  - (hypothetical all other costs are equal)

\[
\frac{($10,000 - 0)}{(.675 - .5375)} = \frac{$10,000}{.1375} = $72,727/QALY
\]
QALY Example #2a

QALY Example # 2b

Source: Phillips, 2009
QALY Example #3

<table>
<thead>
<tr>
<th></th>
<th>1 year</th>
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<th>1 year</th>
<th>Total QALYs</th>
</tr>
</thead>
<tbody>
<tr>
<td>A</td>
<td>.50</td>
<td>.50</td>
<td>.75</td>
<td>.75</td>
<td>?</td>
</tr>
<tr>
<td>B</td>
<td>.50</td>
<td>.50</td>
<td>.50</td>
<td>.50</td>
<td>?</td>
</tr>
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</table>
Poll

What are the additional QALYs generated by Treatment A?

a) 1 QALY
b) 2 QALYs
c) 0.5 QALYs
d) 0.25 QALYs
## QALY Example #3

<table>
<thead>
<tr>
<th></th>
<th>1 year</th>
<th>1 year</th>
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<th>1 year</th>
<th>Total QALYs</th>
</tr>
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<tbody>
<tr>
<td><strong>A</strong></td>
<td>.50</td>
<td>.50</td>
<td>.75</td>
<td>.75</td>
<td>.50+.50+.75+.75 = 2.5</td>
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<tr>
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<td>(.50*1)</td>
<td>(.50*1)</td>
<td>(.75*1)</td>
<td>(.75*1)</td>
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</tr>
<tr>
<td></td>
<td>.50</td>
<td>.50</td>
<td>.75</td>
<td>.75</td>
<td></td>
</tr>
<tr>
<td><strong>B</strong></td>
<td>.50</td>
<td>.50</td>
<td>.50</td>
<td>.50</td>
<td>.50+.50+.50+.50= 2.0</td>
</tr>
<tr>
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<td>(.50*1)</td>
<td>(.50*1)</td>
<td>(.50*1)</td>
<td>(.50*1)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>.50</td>
<td>.50</td>
<td>.50</td>
<td>.50</td>
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Deriving Preferences or Utilities

Basic methodology:

- Individuals provide a personal reflection on the relative value (preference weight) of different health states experienced or described.
Deriving preferences or utilities

Three methods to derive preferences:

- Direct
- Indirect
- Off-the-shelf
Direct Methods

- Individuals asked to choose (declare *preferences*) between their current health state and alternative health status scenarios
Direct: Valuation Method

- Standard Gamble
- Time trade-off
- Rating scale (visual analogue scale)
Direct: Standard Gamble

Source: Sinnott et al., 2007
Direct: Standard Gamble

- Rest of life in current health state; or
- “take a pill (with risks) to be restored to perfect health”
- Scale represents risk of death respondent is willing to bear in order to be restored to full health.

Health Economics Resource Center
Standard Gamble Scenario

- You are able to see, hear and speak normally.
- You require the help of another person and a cane to walk or get around.
- You are occasionally angry, irritable, anxious and depressed.
- You are able to learn and remember normally.
- You are able to eat, bathe, dress and use the toilet normally.
- You are free of pain and discomfort.
Standard Gamble Scenario

- Treatment A: allows you to live 10 years in this health state

- Treatment B: Gives a $p\%$ chance of returning to full health and $(100-p\%)$ chance of death
  - Successful = 10 years of full health
  - Unsuccessful = immediate death
Standard Gamble Scenario

- Your doctor tells you that the chance the second treatment will succeed is not known

- Please indicate the minimum chance of success (i.e. p%) that you would require to accept the second treatment
Direct: Time Trade-off

Value

Years of life

0

\( t_1 \)

\( t_2 \)

Current health

Value
Considering the health state described

- How many years of life in your current state would you be willing to give up to live out your life in perfect health?
  - 5 years
  - 10 years
  - No years
Direct: Rating Scale (VAS)

- Place health state on line

- Anchors:
  - Best possible health state
  - Worst possible health state

- Generates values, not utilities
Poll

With which valuation method would a respondent’s utility be affected by their willingness to take on risk?

a) Standard gamble
b) Time trade-off
c) Visual analogue scale
Direct Methods

- SG measures preferences under conditions of uncertainty
- TTO choices are made under conditions of certainty
- VAS involves neither choice nor uncertainty
Direct Methods

- May be necessary if effects of intervention are complex:
  - Multiple domains
  - Effects not captured in indirect or disease-specific instruments
Direct: Whose preferences?

- Patient
  - Experience disease and treatment
  - Recruitment challenges
  - Higher valuations of health states

- General public/“community preference”
  - Society’s resources
Indirect Methods

- Study subjects complete surveys
- Multiple domains of health
- Composite describes the health status
- Composite state is linked to community results (or “weights”)
How are you today? (EQ-5D)

Which statements best describe you today?

- Mobility:
  - No problems, some problems, extreme problems
- Self-care
- Usual Activities
- Pain/Discomfort
- Anxiety/Depression
Indirect Measures

- Health Utility Index (HUI)
- EuroQol (EQ-5D)
- Quality of Well-Being Scale (QWB)
- SF-6D
Indirect Measures

- Vary with respect to:
  - Dimensions or attributes included;
  - Population used to establish the weights;
  - Health states defined by the survey; and
  - Method of valuation
Indirect measures

- Standard surveys that are widely used

- Describe generic health states

- May lack sensitivity in specific contexts
EuroQol EQ-5D

- 5 questions in 5 domains of health
  - Mobility, self-care, usual activity, pain/discomfort, or anxiety/depression
  - 245 health states.

- Basis of domain weights:
  - Past studies based on British community sample
  - New US weights recently published
Health Utility Index (HUI)

- 41 questions

- 8 domains of health and 972,000 health states
  - vision, hearing, speech, ambulation, dexterity, emotion, cognition, and pain

- Basis of domain weights:
  - Canadian community sample rated hypothetical health states
  - Utility theory
SF-6D*

- Converts SF-36 or SF-12 scores to utilities

- 6 health domains
  - physical functioning, role limitations, social functioning, pain, mental health, and vitality
  - Defines 18,000 health states

- Basis of domain weights
  - British community sample
Indirect: Disease-specific surveys

- Key methods issues:
  - Difficult to describe health state to community respondent
  - Difficult to establish values when there are a large number of possible health states
- Expensive, but potentially sensitive to variations in quality of life for this disease
- Often used in addition to generic measure
Off-the-shelf values

- Use preference weight determined in another study for health state of interest

  - Not all health states have been characterized

- Useful in decision modeling
Which method to use?

- Trade-off between sensitivity and burden

- Start with a literature search re:
  - The condition of interest
  - In the population of interest
  - For the outcomes of interest
Ease of Use

- Off-the-shelf utility values
- Indirect Measures (HUI, EQ-5D, QWB, SF-6D)
- Disease-specific survey during trial and transform later to preferences
- Direct measures (SG, TTO)
Issues surrounding QALYs

- Lack of sensitivity
- Inadequate weight attached to emotional/mental health problems
- Lack of consideration for non-health outcomes
- A QALY is a QALY?
Example


- Collect EQ-5D data at baseline and follow up
- Generate EQ-5D index scores
- Calculate QALY gains for intervention and control groups
Important Resources

- Tufts Center for Evaluation of Value and Risk in Health

- National Institute for Health Research, UK
  http://www.nets.nihr.ac.uk/
Important Resources

  
  [http://www.nets.nihr.ac.uk/projects/hta/934708](http://www.nets.nihr.ac.uk/projects/hta/934708)

- Brazier et al. Developing and testing methods for deriving preference-based measures of health from condition-specific measures (and other patient-based measures of outcome).
  
  [http://www.nets.nihr.ac.uk/projects/hta/069704](http://www.nets.nihr.ac.uk/projects/hta/069704)
Important Resources

- Tufts Cost-Effectiveness Analysis Registry

- PROMIS
  http://www.healthmeasures.net/explore-measurement-systems/promis

Upcoming HERC Seminars

Estimating Transition Probabilities for a Model
– Risha Gidwani-Marszowski
– 02/28/2018

Medical Decision Making and Decision Analysis
– Jeremy Goldhaber-Fiebert
– 03/07/2018
QUESTIONS and COMMENTS
References

Jodar-Sanchez et al. (2015). Cost-Utility Analysis of a Medication Review with Follow-Up Service for Older Adults with Polypharmacy in Community Pharmacies in Spain: The conSIGUE Program. Pharmacoeconomics 33(6), 599-610
