Whose perspective? Implications on cost-effectiveness modelling of differences between country value sets (a case study)

17th April 2018 - Basel Biometrics Society (BBS) meeting

Susan Edwards, PhD
Health Economics and Evidence Synthesis
MORSE - Health Technology Assessment Group
Global Access
Pharmaceuticals Division
F. Hoffmann-La Roche Ltd
Basel, CH
susan.edwards.se1@roche.com
How do decision makers decide which healthcare interventions to fund?

- Incremental cost-effectiveness ratio (ICER) =

\[
\frac{\Delta \text{cost}}{\Delta \text{effectiveness}} = \frac{(\text{cost}_{\text{treatment}} - \text{cost}_{\text{control}})}{(\text{effectiveness}_{\text{treatment}} - \text{effectiveness}_{\text{control}})}
\]

- A means of standardising health benefits
  - across endpoints
  - across therapy areas

- Often measured in Quality-Adjusted Life Years (QALYs)
To calculate QALYs, we multiply the **length of life** expected to be gained by the new treatment or invention by the **quality of life** a patient can expect to have.

Quality of life is measured on a scale of 0 to 1. This score or ‘weight’ represents the **value** of different levels of health.

**How do we find the ‘Q’ in QALY?**
1. We need to describe the health state that is going to be valued
2. We need a way to value the health state that we have described
3. We need a group of people to provide the values

Measuring and Valuing Health (University of Sheffield), online course, [https://www.futurelearn.com/courses/valuing-health/6/](https://www.futurelearn.com/courses/valuing-health/6/)
1. How to describe the health states?

- Objective vs. subjective measures
- Condition specific (AQL-5D for asthma) vs. generic measures (SF-36, EQ-5D)
- Clinician (or proxy) vs. patient reported measures
- Health related quality of life vs. overall well-being measures
Example: EQ-5D-3L

- A generic PROM which has 5 domains.
- Each domain has a single question which has 3 severity levels.
- A health state is formed from the responses to each question.
- Total of 243 possible health states
2. Method to value the health benefit?

- There are different techniques that can be used. The most common ones are
  - time trade-off, which asks individuals **how much time** they would be willing to give up in order to avoid poor health states
  - standard gamble, which asks individuals **how much risk** they are willing to take in order to avoid poor health states.

[https://www.futurelearn.com/courses/valuing-health/6/steps/283811](https://www.futurelearn.com/courses/valuing-health/6/steps/283811)
Example of Time trade-off (TTO) method:

Value of health state = 6 / 10

Choice A (6 years)

- Full Health

Choice B (10 years)

- I have no problems in walking about
- I have no problems with self-care
- I have some problems with performing my usual activities
- I have extreme pain or discomfort
- I am moderately anxious or depressed

=
Time trade-off (TTO) method

• To find out the value an individual places on this health state,
  – vary the time lived in the full health state until the respondent is indifferent
  – divide the years in perfect health by the years in the health state

• **We all value health states differently.**

• The different preferences that we have mean that we place different values on health states. This can be due to our own experiences of ill health or simply our views on what we think matters in health.
3. Who should value health?

- General population
- Patients
- Clinicians
Case study

Multiple sclerosis

**RRMS**
Relapsing–remitting MS

**SPMS**
Secondary progressive MS

**PPMS**
Primary progressive MS
Expanded Disability Status Scale (EDSS)
Methods

• ORATORIO, a double-blind, placebo-controlled trial, evaluated the efficacy and safety of ocrelizumab patients with PPMS

• Disease activity was assessed using the EDSS, the 9HPT and the MFIS at baseline, and at 48 weeks and 120 weeks after treatment initiation

• Patients also completed the 3-level EQ-5D (EQ-5D-3L) at the same time points to determine the impact of PPMS on HRQoL by EDSS score-derived health states
  – EQ-5D-3L value sets from 10 countries were used to derive country-specific health utilities for each of the EDSS score derived health states

• A repeated-measures linear model was used to regress health utilities on EDSS score-derived health states, clinically significant upper limb dysfunction, clinically significant fatigue, sex, age and region of the world using the UK value set

• The value sets from each of the 9 other countries were applied to this model
Results
Implications for decision making?
Implications for decision making?

<table>
<thead>
<tr>
<th>Country</th>
<th>Incremental QALYs (as a proportion of ref case)</th>
</tr>
</thead>
<tbody>
<tr>
<td>UK (reference)</td>
<td>1</td>
</tr>
<tr>
<td>Portugal</td>
<td>0.87</td>
</tr>
<tr>
<td>Portugal (extrapolated)</td>
<td>0.60</td>
</tr>
<tr>
<td>Sweden</td>
<td>0.97</td>
</tr>
<tr>
<td>Sweden (extrapolated)</td>
<td>0.42</td>
</tr>
</tbody>
</table>

Orange arrows = show the direction in which patients may move along the EDSS scale.

Grey boxes in ‘PPMS treated’ = these indicate the stages at which treatment is discontinued.
Implications for statistics?

• Role of statistics in extrapolating patient preferences?
  – missing states (EDSS 0, EDSS 9, EDSS 10)
  – missing countries
  – complex models required
In sum

- Relationships exist between health utilities derived from the EQ-5D-3L and EDSS score-derived health states in PPMS; varying across countries

- Data collection required, as predicting preferences is challenging

- Complicated modelling methods to be run, but also communicated to non-analysts

- There may be different decisions coming out of different countries that otherwise share a same decision framework due to differences in valuation of different levels of health
Acknowledgments

- Joshua Ray
- Monica Daigl
- Federico Felizzi
- Gurleen Jhuti
- Iain Bennett
Thank you for your attention!

• What questions do you have?
Doing now what patients need next