Designing Economic Evaluation Alongside Clinical Studies

Health Economics Short Course

For more information and course dates, please visit our website:
Or email us: health-economics@unimelb.edu.au

Module 2 – Measuring health-related quality of life and use of clinical outcomes

Centre for Health Policy
Melbourne School of Population and Global Health

Overview

• Identifying Outcomes for an Economic Evaluation
• Cost-Effectiveness Analysis alongside a Study
• Cost-Utility Analysis and the Quality-Adjusted Life Year (QALY)
• Measuring and Valuing Health States
• Practical Recommendations
• Exercises

Cost-effectiveness: where do outcomes fit in?

Costs

Outcomes

Costs

Outcomes

Examples of incremental cost-effectiveness ratios:

$1,200/case of hospitalisation averted

$50/case of malaria averted

$4,500/QALY gained

Relationship between health outcome measurements

Attitude change

Recognise need to improve eating habits

Knowledge gain

Understand that 2 serves of fruit and 5 serves of vegetables is daily goal

Change in behaviour

Increase in fruit and vegetable intake

Risk factor improvement

Cholesterol reduction

Disease reduction

Less heart disease, diabetes

Survival gain

Life expectancy increase

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Examples of health outcome for an economic evaluation

- Surrogate/Intermediate Outcome
  - Viral load (HIV)
  - Glucose control (HbA1c)
  - Diastolic blood pressure
  - Vaccine uptake, attack rate
  - Schizophrenia relapse
  - Adverse event averted
  - Disease/cases averted or detected
  - Symptom-free days
  - Episode-free days

- Final Outcome (mortality and morbidity)
  - Survival (change in life expectancy) expressed as life years (LYs) gained
  - Disability days avoided
  - Disability adjusted life years (DALYs) avoided
  - Quality adjusted life years (QALY) gained

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Cost-Effectiveness Analysis alongside a study – example 1

- Cost-effectiveness of a long-term internet-delivered worksite health promotion programme on physical activity and nutrition: a cluster randomised controlled trial

Cost-Effectiveness Analysis alongside a study – example 2

- Specialized rheumatology nurse substitutes for rheumatologists in the diagnostic process of fibromyalgia: a cost-consequence analysis and a randomized controlled trial

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Cost Utility Analysis (CUA)

- CUA uses a preference-adjusted unit of consequence (e.g. QALY or DALY) as the outcome measure
- Preferences for disparate outcomes are captured via utility weights (quality of life weights)
- It combines more than one attribute of health (e.g. include both physical health and mental health)
- CUA can involve a comparison of health care for different disease types (e.g. a comparison of care for cancer vs care for diabetes)
What is a QALY?

• An economic outcome that combines preferences for length of survival and quality during survival into a single measure

• The QALY is a measure of preference-adjusted (QoL) survival time

• QALYs have an explicit time dimension:
  – QALY = QoL weight × duration

Health utility (or health-related QoL) weights

• Values (generally) range between 0 (death) and 1 (perfect health)
  – Ex: A value of 0.8 indicates that a year lived in that state is worth 0.8 of a year in perfect health
  – NB: It is possible to have states perceived as worse than death (<0)

• Utility weights have equal intervals properties
  – A given change in absolute value means the same regardless of where you are on the scale
  – Ex: 0.2 to 0.3 same as 0.7 to 0.8

• There is a difference in how we use QoL weights in economic evaluation compared to psychology

Overview

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Health State Measurement and Valuation

• Measuring health status: Non-preference based measures
  – Disease specific measures (e.g. Minnesota living with heart failure questionnaire)
  – Generic measures (e.g. Short Form 36 (SF-36); Peds QL)
  – www.qolid.org

• Measuring & valuing health status: Preference based measures
  – Direct elicitation (e.g. Time-trade-off, Standard Gamble, Visual Analogue Scale)
  – Multi-Attribute Utility Instruments (MAUIs) (e.g. EuroQol-5D (EQ-5D), Health Utilities Index (HUI), AQoL)

Disease specific measures

• Example of the Minnesota living with heart failure questionnaire
• 21 Questions representative of the ways heart failure and treatments can affect quality of life dimensions:
  – Physical
  – Emotional
  – Social
  – Mental

Ex: “Did your heart failure prevent you from living as you wanted during the past month by [making your walking about or climbing stairs difficult]?”

Generic measures

• Example of the Short Form 36 (SF-36) domains

- Physical
  - Role - physical
  - Bodily pain
  - General health/vitality

- Mental
  - Social functioning
  - Role emotional
  - Mental health
  - General health/vitality
• Measuring health status: Non-preference based measures
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  – Generic measures (e.g. Short Form 36 (SF-36); Peds QL)
    - [Website Link]

• Measuring & valuing health status: Preference based measures
  – Direct elicitation (e.g. Time-trade-off, Standard Gamble, Visual Analogue Scale)
  – Multi-Attribute Utility Instruments (MAUIs) (e.g. EuroQol-5D (EQ-5D), Health Utilities Index (HUI), AQoL)

Multi-Attribute Utility Instruments

Examples of Multi-Attribute Utility Instruments (MAUIs):
  – EQ-5D (Europe/UK)
  – HUI II/III (Canada)
  – AQoL (Australia)
  – 15-D (Finland)
  – Rosser-Kind Index (UK)
  – Others: CHU-9D-child instrument

http://www.sheffield.ac.uk/scharr/sections/heds/mvh

Comparing instruments

<table>
<thead>
<tr>
<th>Number of Questions</th>
<th>Answer level (Sensitivity)</th>
<th>Dimensions Captured</th>
<th>Children/ Youth?</th>
<th>Costs</th>
</tr>
</thead>
<tbody>
<tr>
<td>EQ-5D</td>
<td>5</td>
<td>3-level (5-level scale, valuations under development)</td>
<td>Physical: Diverse (psychosocial limited)</td>
<td>Available (Youth)</td>
</tr>
<tr>
<td>Aqol</td>
<td>25-45 (45-50)</td>
<td>4- to 7-level for adolescents version</td>
<td>Physical &amp; Psychosocial Diverse (not sexed)</td>
<td>Available (Adolescent)</td>
</tr>
<tr>
<td>PedsQL</td>
<td>20</td>
<td>5-level</td>
<td>Physical &amp; Psychosocial Diverse</td>
<td>Available (Age 6-12)</td>
</tr>
<tr>
<td>HU II</td>
<td>8 (HUI III)</td>
<td>3- to 6 level (varies)</td>
<td>Physical: Diverse (incl senses)</td>
<td>Available (HUI II)</td>
</tr>
<tr>
<td>HUI III</td>
<td>8 (HUI III)</td>
<td>3- to 6 level (varies)</td>
<td>Physical: Diverse (incl senses)</td>
<td>Available (HUI III)</td>
</tr>
<tr>
<td>SF-12</td>
<td>12 (SF-12); SF-36 (SF-36)</td>
<td>3- to 6-level</td>
<td>Physical &amp; Psychosocial Diverse</td>
<td>Not available</td>
</tr>
<tr>
<td>CHU 9D</td>
<td>9</td>
<td>5-level</td>
<td>Physical &amp; Psychosocial Diverse</td>
<td>Only for Children</td>
</tr>
</tbody>
</table>

How do we obtain utility scores from MAUIs? Example of the EQ-5D

A person has some problems with moving around, no problems with self-care and usual activities, has moderate pain and is extremely anxious/depressed

- Mobility: I have some problems with walking about
- Self-care: I have no problems with self-care
- Usual activities: I have some problems with usual activities
- Pain/discomfort: I have moderate pain
- Anxiety/depression: I am extremely anxious/depressed

EQ-5D health state = 21123
How do we obtain utility scores from MAUIs?

**Questionnaire**

**Scoring algorithm & formula** (ex: additive / multiplicative)

**Utility scores**

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Developing scoring algorithm for MAUIs

**Example of the EQ-5D:**

A sample of the 243 EQ-5D health states valued using TTO from 3,337 British adults (similar in Australia, n = 417)

- From this, obtain a scoring algorithm (tariff) for all the health states
- Using an (additive) formula, produce an overall single value for health status (utility)

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EQ-5D algorithms for calculating utility scores

**Table:**

<table>
<thead>
<tr>
<th>Dimension</th>
<th>Level</th>
<th>UK Coefficient</th>
<th>Australia Coefficient</th>
</tr>
</thead>
<tbody>
<tr>
<td>Constant</td>
<td>any downward move</td>
<td>-0.081</td>
<td>-0.105</td>
</tr>
<tr>
<td>Mobility</td>
<td>2: some problems</td>
<td>-0.069</td>
<td>-0.088</td>
</tr>
<tr>
<td></td>
<td>3: confined to bed</td>
<td>-0.314</td>
<td>-0.374</td>
</tr>
<tr>
<td>Self Care</td>
<td>2: some problems</td>
<td>-0.104</td>
<td>-0.087</td>
</tr>
<tr>
<td></td>
<td>3: unable to</td>
<td>-0.214</td>
<td>-0.267</td>
</tr>
<tr>
<td>Usual activities</td>
<td>2: some problems</td>
<td>-0.036</td>
<td>-0.053</td>
</tr>
<tr>
<td></td>
<td>3: unable to</td>
<td>-0.094</td>
<td>-0.139</td>
</tr>
<tr>
<td>Pain/discomfort</td>
<td>2: moderate</td>
<td>-0.123</td>
<td>-0.068</td>
</tr>
<tr>
<td></td>
<td>3: extreme</td>
<td>-0.386</td>
<td>-0.449</td>
</tr>
<tr>
<td>Anxiety/depression</td>
<td>2: moderate</td>
<td>-0.071</td>
<td>-0.097</td>
</tr>
<tr>
<td></td>
<td>3: extreme</td>
<td>-0.236</td>
<td>-0.397</td>
</tr>
<tr>
<td>Other coefficients/terms</td>
<td>Level 3 constant</td>
<td>Interaction terms</td>
<td></td>
</tr>
</tbody>
</table>

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The time trade-off method

Imagine you are in the following health state:

You have some problems with moving around, no problems with self-care and usual activities, moderate pain and are extremely anxious/depressed

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EQ-5D algorithms for calculating utility scores

**Graph:**

Health State Measurement and Valuation: Summary

**Measuring health status:**

Non-preference based approach

- Disease specific measures
- Generic measures (e.g. SF36)

**Measuring and valuing health status:**

Preference based approach

- Direct elicitation
- Multi-Attribute Utility Instruments

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**Map to utility score**

**Utility scores**

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References:


Mapping algorithms

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Choice of instrument or scale

- Choosing between instruments should be based upon their suitability for and sensitivity (responsiveness) to the characteristics of the particular population and intervention → Domains / levels
- Special population group (e.g. children)
- Length and mode of questionnaire (time costs, drop-outs, incomplete questionnaires)
- Some have costs and require registering

Response variation

- Considerable regional variation when responding to quality of life questions
- Asian populations consistently report less problems
- Cannot be explained by demographic or clinical differences

Outcomes for CEA/CUA: Conclusions

- Selecting appropriate (intermediate and final) outcomes
- CEA or CUA?
- To collect (or not to collect) quality of life data
  - NB: In many cases it will be necessary to attach utility weights to health states that are not observed within a study, perhaps because they are due to events that occur outside the study timeframe
- Economic evaluation typically tries to align to the policy/real world decision context

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Extra Slides – Further Information on Generic Health Status Instruments and MAUIs

EQ-5D Further Information

- **Website:** [http://www.euroqol.org/](http://www.euroqol.org/)

EQ-5D

- EQ-5D available, officially, in more than 100 languages
- Several country-specific valuation sets (tariffs) are also available
  - 10 EU countries: Belgium, Finland, Germany, Greece, Hungary, Netherlands, Slovenia, Spain, Sweden, UK
  - Others: Armenia, Canada, Japan, NZ, Australia and Zimbabwe
- If planning to use in your study, need to register first
- Licensing fees depend on type of study, funding source, sample size and number of requested languages.

EQ-5D Demo – Web version demo available:

EQ-5D-5L (5 Levels)

- Launched in 2009
- Identifies new levels previously omitted by EQ-5D which were found to be important to patients (5 levels of response: no problem, slight, moderate, severe, extreme)
- Wording has changed
- Currently 43 official language versions
- A valuation set (tariff) is being developed for a number of countries including the UK
- Cross walk values are available (relationship between EQ-5D-3 and EQ-5D-5 scores)
Health Utilities Index (HUI)

- Website: [http://www.healthutilities.com/](http://www.healthutilities.com/)

HUI II / III independent and complementary

**Domains:**

<table>
<thead>
<tr>
<th>HUI2</th>
<th>HUI3</th>
</tr>
</thead>
<tbody>
<tr>
<td>Sensation: 4-level</td>
<td>Vision: 6-level</td>
</tr>
<tr>
<td>Mobility: 5-level</td>
<td>Hearing: 6-level</td>
</tr>
<tr>
<td>Emotion: 5-level</td>
<td>Speech: 5-level</td>
</tr>
<tr>
<td>Cognition: 4-level</td>
<td>Ambulation: 6-level</td>
</tr>
<tr>
<td>Self-care: 4-level</td>
<td>Dexterity: 6-level</td>
</tr>
<tr>
<td>Pain: 5-level</td>
<td>Emotion: 5-level</td>
</tr>
<tr>
<td>Fertility: 3-level</td>
<td>Pain: 5-level</td>
</tr>
</tbody>
</table>

- Licensing fees apply

Assessment of Quality of Life (AQoL) (Australian)


 AQoL

- Choice of AQoL Instrument:

<table>
<thead>
<tr>
<th>Instrument</th>
<th>Items</th>
<th>Completion time</th>
<th>Dimensions included</th>
</tr>
</thead>
<tbody>
<tr>
<td>AQoL-6D</td>
<td>25</td>
<td>3-6 minutes</td>
<td>Independent Living, Happiness, Mental Health, Coping, Relationships, Self Worth, Pain, Senses</td>
</tr>
<tr>
<td>AQoL-7D</td>
<td>26</td>
<td>3-4 minutes</td>
<td>Independent Living, Mental Health, Coping, Relationships, Pain, Senses</td>
</tr>
<tr>
<td>AQoL-6D</td>
<td>20</td>
<td>2-3 minutes</td>
<td>Independent Living, Mental Health, Coping, Relationships, Pain, Senses</td>
</tr>
<tr>
<td>AQoL-6D</td>
<td>12</td>
<td>1-2 minutes</td>
<td>Independent Living, Mental Health, Relationships, Senses</td>
</tr>
</tbody>
</table>

- AQoL-6D Adolescent Instrument
  - Scoring algorithms available for Australia, New Zealand, Fiji, Tonga

- Questionnaires

- Licensing fee
  - No fees associated with registration or use of the AQoL

Pediatric Quality of Life Inventory (PedsQL)

- Website: [http://www.pedsqol.org/](http://www.pedsqol.org/)
**PedsQL**

- **Target group**
  - Children and adolescents (age 2-18)
- **Two surveys available**
  1) Child-Self Report
  2) Parent-Proxy Report
- **Multidimensional domains**
  - 8 Q’s on Health and Activities
  - 5 Q’s on Feelings
  - 5 Q’s on Relationships with others
  - 5 Q’s on School Life

**Questionnaire samples**
- Child-Self Report
  [http://www.pedsql.org/PedsQL4-0Ch.doc](http://www.pedsql.org/PedsQL4-0Ch.doc)
- Parent-Proxy Report
  [http://www.pedsql.org/PedsQL4-0PC.doc](http://www.pedsql.org/PedsQL4-0PC.doc)

**Licensing fee**
[http://www.pedsql.org/PedsQL-CostStructure.pdf](http://www.pedsql.org/PedsQL-CostStructure.pdf)

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**SF Health Surveys**

- **Domains for SF-36, SF-12, SF-8**
  - All measure the same eight health domains
    - Physical Functioning (PF)
    - Role-Physical (RP)
    - Bodily Pain (BP)
    - General Health (GH)
    - Vitality (VT)
    - Social Functioning (SF)
    - Role-Emotional (RE)
    - Mental Health (MH)
- **Each survey provides psychometrically-based physical component summary (PCS) and mental component summary (MCS) scores.**

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**SF Health Surveys**

- **Target group**
  - Adults of 18 years and older
  - Not recommended for children/adolescents

**Licensing fee**
- Information available upon request
- Need to complete and submit the License Application Form online

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**The Child Health Utility 9D (CHU 9D)**

- **Website**: [https://www.shef.ac.uk/scharr/sections/heds/mvh/paediatric/about-chu9d](https://www.shef.ac.uk/scharr/sections/heds/mvh/paediatric/about-chu9d)
• Paediatric generic preference based measure of health related quality of life
• Number of dimensions (questions): 9
• Number of levels per dimension: 5
• Age range: 7-17 years
• Mode of completion: self completion (proxy completion also available for younger children)
• Recall period: today/last night

• Questions cover the following dimensions (areas/feelings):
  - Worried
  - Sad
  - Pain
  - Tired
  - Annoyed
  - School Work/Homework
  - Sleep
  - Daily routine (getting dressed, etc)
  - Ability to join in activities (sports, etc)

• Sample questionnaire
  https://www.shef.ac.uk/polopoly_fs/1.44111!/file/Health-Questionnaire-final-watermarked.pdf

• Licensing fee
  - Non-commercial applications: free of charge
    • E.g. work funded by research councils, Government agencies and charities
  - Commercial applications: charges apply
    • Prices to be negotiated upon application