

HTA Policies and Principles Week 6: Outcomes

Part 2



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Lecture Outline

Off-the shelf quality of life instruments

Condition-specific quality of life instruments

Recent developments in outcome measurements

Case study

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Lecture Outline

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Part 1:

- Off-the shelf quality of life instruments

Part 2:

- Generic quality of life instruments
 - EQ5D
 - SF-36
 - SF-6D

Part 3:

- Condition-specific quality of life instruments
- Patient Reported Outcome Measures (PROMs)
- Obtaining utilities from Clinical trials

Part 4:

- Recent developments in measuring and valuing health
- Measuring and valuing health in children and adolescent health utilities
 - CHU9D
- Capability measures
 - Preference based capability measure for the elderly (ICECAP-O)

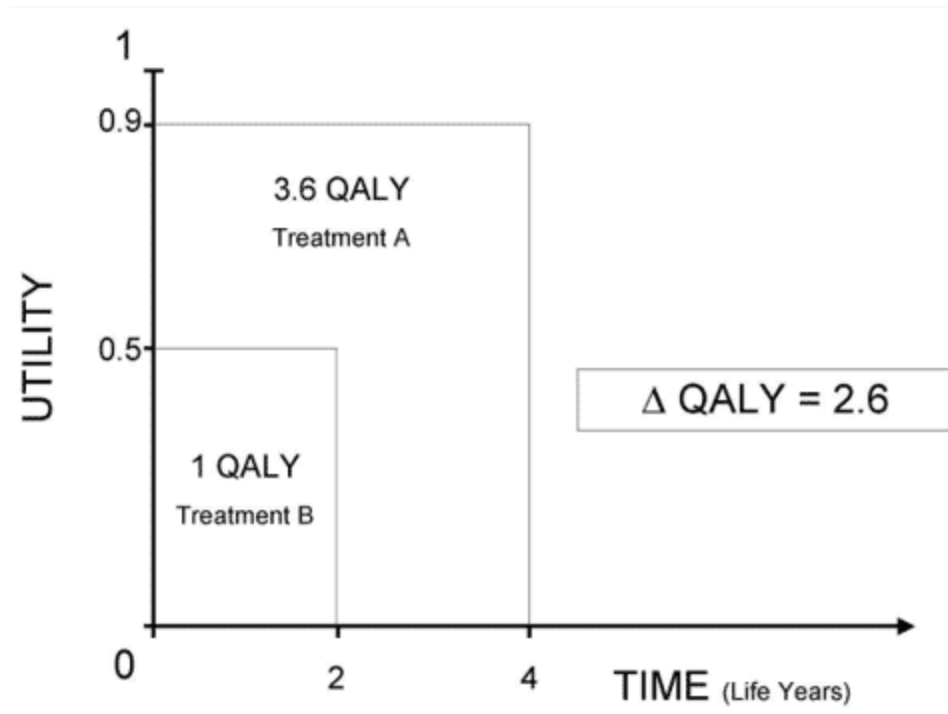
Part 5:

- Case study: Measuring outcomes in Parkinson's disease



Off-the shelf quality of life instruments

Recap



- In economic evaluation, we use utility as a type of effectiveness index
- We use utility to weight survival to produce or Quality-adjusted life year (QALY) metric
- In week 5 we looked at direct elicitation methods to measure preferences and calculate utilities

Direct elicitation methods comparison

But...there is some variation in the utility values that we get using these different measures:

Health State	Method	Utility (Median)
Minor stroke	Rating scale	0.71
	Time trade-off	0.88
	Standard gamble	0.91
Major stroke	Rating scale	0.31
	Time trade-off	0.51
	Standard gamble	0.61

Source: Hallan et al. (1999) Journal of Internal Medicine; 246: 309-316

We can standardise the measurement of utilities by using the same instrument for each study, and this is where multi-attribute utility measurement instruments come in.

Multi-attribute utility measurement instruments

- These are sometimes also referred to as 'off the shelf' instruments
- They help to standardise utility measurement
- Using a standardised instrument also averts a substantial amount of the time and cost required to undergo a preference-measurement exercise

Quality of life (QoL) measurement instruments

	Generic	Condition-specific
Preference based	EQ5D SF-6D HUI CHU9D ICECAP	EXACT-U (For use in COPD)
Non-preference based	SF-36 SF-12 Sickness Impact Profile	Asthma quality of life questionnaire Parkinson’s Disease Questionnaire

In addition to the multi-attribute utility instruments shown in the top left corner of the table, there are also 'off the shelf' instruments that are generic and non-preference based (such as SF-36), and standardised instruments that are condition-specific.

This table is not an exhaustive list of QoL measurement instruments – but it is useful to give you an idea of what instruments are available and how they would be classified.

In this lecture we will go into detail for a few of these instruments (highlighted in yellow), but keep in mind there are many others out there.

EuroQol 5D (EQ5D)

	Generic	Condition-specific
Preference based	EQ5D SF-6D HUI CHU9D ICECAP	EXACT-U (For use in COPD)
Non-preference based	SF-36 SF-12	Asthma quality of life questionnaire Parkinson's Disease Questionnaire

- The EQ5D is a standardised, off the shelf multi-attribute utility instrument
- EQ-5D is primarily designed for self-completion, which is well-suited for administering in clinical trials
- It is cognitively simple, taking only a few minutes to complete
- Country-specific valuation sets available
- Available on www.euroqol.org

EQ-5D-3L structure

- Self-description of current health-related QoL on 5 domains:
 - Mobility
 - Self care
 - Usual activities
 - Pain/discomfort
 - Anxiety/depression
- Three levels in each domain:
 - No problems
 - Some, or moderate problems
 - Unable, or extreme problems
- 245 different possible health states, including 'unconscious' and 'dead'

From EQ5D to utility values

- Preferences were measured for a sample of the 245 possible health states in a representative sample of approximately 3000 UK adults
- Time Trade Off approach was used for valuation of the health states
 - Dolan, Paul DPhil. Modeling Valuations for EuroQoL Health States. Medical Care: November 1997 - Volume 35 - Issue 11 - p 1095-1108
- Responses from this study were used to create a EQ-5D scoring formula (UK value set)
- A score for a patient from the EQ5D can be directly translated into utility values using this formula

EQ5D Scoring System



Mobility

- 1 No problems walking
- 2 Some problems walking about
- 3 Confined to bed

Self-care

- 1 No problems with self-care
- 2 Some problems washing or dressing self
- 3 Unable to wash or dress self

Usual activities

- 1 No problems with performing usual activities (e.g. work, study, housework, family or leisure activities)
- 2 Some problems with performing usual activities
- 3 Unable to perform usual activities

Pain/discomfort

- 1 No pain or discomfort
- 2 Moderate pain or discomfort
- 3 Extreme pain or discomfort

Anxiety/depression

- 1 Not anxious or depressed
- 2 Moderately anxious or depressed
- 3 Extremely anxious or depressed

Consider the state 11223

- Mobility: 1
- Self-care: 1
- Usual activities: 2
- Pain/discomfort: 2
- Anxiety/depression: 3

Coefficients for TTO tariffs	
Dimension	Coefficient
Constant	0.081
Mobility	
Level 2	0.069
Level 3	0.314
Self-care	
Level 2	0.104
Level 3	0.241
Usual activity	
Level 2	0.036
Level 3	0.094
Pain/discomfort	
Level 2	0.123
Level 3	0.386
Anxiety/depression	
Level 2	0.071
Level 3	0.236
N3	0.269

Calculation of utility for the 11223 health state:

Full health	=1.000
Constant	-0.081
Mobility (level 1)	-0.000
Self-care (level 1)	-0.000
Usual activity (level 2)	-0.036
Pain/discomfort (level 2)	-0.123
Anxiety/depression (level 3)	-0.236
N3	-0.269
Estimated utility for 11223	=0.255

CONTINUE

EQ-5D-3L to EQ-5D-5L

- EQ-5D-5L was launched in 2009
 - 5 levels for each dimension instead of 3
 - Increased sensitivity of the instrument
- Value sets have been developed to translate scores into utilities for multiple countries
- Valuation of the health states is still ongoing for the UK

- Currently, we're using an algorithm to translate EQ-5D-5L to EQ-5D-3L utilities while waiting for UK value set

Under each heading, please tick the ONE box that best describes your health TODAY.

MOBILITY

- I have no problems in walking about ☐
- I have slight problems in walking about ☐
- I have moderate problems in walking about ☐
- I have severe problems in walking about ☐
- I am unable to walk about ☐

SELF-CARE

- I have no problems washing or dressing myself ☐
- I have slight problems washing or dressing myself ☐
- I have moderate problems washing or dressing myself ☐
- I have severe problems washing or dressing myself ☐
- I am unable to wash or dress myself ☐

USUAL ACTIVITIES (e.g. work, study, housework, family or leisure activities)

- I have no problems doing my usual activities ☐
- I have slight problems doing my usual activities ☐
- I have moderate problems doing my usual activities ☐
- I have severe problems doing my usual activities ☐
- I am unable to do my usual activities ☐

PAIN / DISCOMFORT

- I have no pain or discomfort ☐
- I have slight pain or discomfort ☐
- I have moderate pain or discomfort ☐
- I have severe pain or discomfort ☐
- I have extreme pain or discomfort ☐

ANXIETY / DEPRESSION

- I am not anxious or depressed ☐
- I am slightly anxious or depressed ☐
- I am moderately anxious or depressed ☐
- I am severely anxious or depressed ☐
- I am extremely anxious or depressed ☐

Short Form 36 (SF-36)

	Generic	Condition-specific
Preference based	EQ5D SF-6D HUI CHU9D ICECAP	EXACT-U (For use in COPD)
Non-preference based	SF-36 SF-12	Asthma quality of life questionnaire Parkinson's Disease Questionnaire

- The Short Form 36 instrument was briefly introduced in Week 5 under the 'Non-preference based measures' section
- It is widely used as a measure of general health
- Consists of 36 questions, grouped into 8 dimensions
- Responses are scored within each dimension and transformed onto a 0-100 scale
- It is possible to combine responses into two summary scores of physical and mental health
- A shorter version (SF12) has also been developed

SF-36 dimensions

Dimension	Number of items	Summary of content
Physical functioning	10	Extent to which health limits physical activities such as bending, lifting, and moderate and vigorous exercise

Dimension	Number of items	Summary of content
Role limitations: physical	4	Extent to which physical health interferes with work, accomplishing less than wanted, limitations in the activities
Bodily pain	2	Intensity of pain and effect of pain on normal work
General health	5	Personal evaluation of health, including current health
Vitality	4	Feeling energetic and full of life versus feeling tired
Social functioning	2	Extent to which physical health or emotional problems interfere with social functioning
Role limitations: emotional	3	Extent to which emotional problems interfere with work, decreased time spent on activities, accomplishing less than wanted
Mental health	5	General mental health, including depression, anxiety, positive affect

Source: Gray AM, Clarke PM, Wolstenholme JL, Wordsworth S. Applied methods of cost-effectiveness analysis in healthcare. OUP Oxford; 2010 Oct 14.

SF-36 limitations

- While the SF-36 is useful for its clinical relevance and descriptive properties, it is limited in its usefulness for economic evaluations
 - Dimensions are not linked to underlying valuation of health states: cannot obtain utility values
 - Difficulty in trading off across dimensions: if a change in health state leads to improved scores in one dimension but lower scores in another
- To address these limitations, the SF-6D was developed

SF-36 --> SF-6D

	Generic	Condition-specific
Preference based	EQ5D SF-6D HUI CHU9D ICECAP	EXACT-U (For use in COPD)
Non-preference based	SF-36 SF-12	Asthma quality of life questionnaire Parkinson's Disease Questionnaire

Short Form 6D (SF-6D) is a utility instrument that allows researchers to convert SF-36 responses into utilities

To use the SF-6D system:

Use the SF-36
questionnaire plus 3
additional questions



Responses are
classified in the SF-6D
system



Use scoring tables to
compute utilities



Condition-specific quality of life instruments

There are many examples of condition-specific quality of life instruments, and new ones are being developed all the time

They are useful as a broad measure of effectiveness of interventions

Wider picture of health status than clinical measures such as blood pressure

If there are no value sets attached, however, these will not give you utilities

There has been a lot of work to translate non-preference based measures into preference-based measures, but some conditions may not yet have a condition-specific preference-based measure

Condition-specific preference-based measures

- These instruments are often more sensitive to changes in health-related quality of life, compared to generic measures (such as EQ5D)
 - This makes them useful in clinical trials, when studies may not be powered to detect change in EQ5D-derived utilities
- However, there is a loss of comparability between utilities obtained from condition-specific measures and those obtained from generic measures
- Thus, based on the research question, researchers must decide the most appropriate approach

- Further reading, if interested: Rowen, D., Brazier, J., Ara, R. et al. The Role of Condition-Specific Preference-Based Measures in Health Technology Assessment. *Pharmacoeconomics* 35 (Suppl 1), 33–41 (2017). <https://doi.org/10.1007/s40273-017-0546-9>
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Patient reported outcome measures (PROMs)

“A Patient Reported Outcome (PRO) is a measurement of any aspect of a patient’s health status that comes directly from the patient”

- FDA Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims, 2009

- PROQUOLID:
 - A database of Patient Reported Outcomes and QoL instruments
 - Facilitates the choice of an appropriate PRO instrument
 - Focuses on the original Clinical Outcome Assessment developed for a condition
 - <https://eprovide.mapi-trust.org/about/about-proqolid>
- Patient Reported Outcome Measures (PROM):
 - continuation of the PRO concept
 - UK initiative to assess the quality of care delivered to NHS patients, from the patient perspective

Obtaining utilities from clinical trials

- Methods for obtaining utilities from randomised clinical trials typically involve the use of a generic 'off the shelf' preference-based instrument such as the EQ-5D , SF6D, CHU9D or HUI
- Within-trial utilities are required to enable researchers to calculate QALYs typically using an 'area under the curve' approach adjusting for baseline utility
- Such instruments are typically embedded within trial data collection questionnaires with participants often asked to self-complete them
- The same utility instrument is administered at baseline and all subsequent follow-up periods within the trial
- In some cases, a condition-specific non-preference based measure may be required, as a generic preference-based measure is not sensitive enough to detect changes between groups
 - In many cases, mapping algorithms exist to derive EQ5D based utilities from the scores obtained using the condition-specific measure



Recent developments in outcome measurements

In this next section of the lecture we're going to go through some of the recent developments in the field of outcome measurement for HTA, focusing on:

- Measuring and valuing health in children and adolescents
 - **Child Health Utility 9D**
- Outcome measures which capture a broader definition of 'outcomes' such as social care outcomes and capability wellbeing
- Preference based capability measure for the elderly
 - **ICECAP-O**

Child health utilities

- Childhood is a period of significant transition in terms of biological and psychosocial development
 - Physical ability as well as social role is constantly changing
 - Individuals become increasingly responsible for their own health and healthcare
- Childhood also presents opportunities for significant improvement in both short- and long-term outcomes through timely and appropriate prevention and treatment interventions
- Majority of preference based outcome measures are focused in adults – child and adolescent health often marginalised

Child Health Utility 9D (CHU9D)

	Generic	Condition-specific
Preference based	EQ5D SF-6D HUI CHU9D ICECAP	EXACT-U (For use in COPD)
Non-preference based	SF-36 SF-12	Asthma quality of life questionnaire Parkinson's Disease Questionnaire

- Instrument developed for measurement and valuation of health in young people for use in economic evaluations
- Developed using in-depth qualitative interviews with young people aged 7-11
- Generic multi-attribute utility instrument with 9 dimensions, each with 5 levels
- Targeted for use in those aged 7-17
- Can be self-completed or proxy-completed for younger children
- Requires a license to use (and to obtain formula to translate scores to utilities)

CHU9D dimensions and levels

Dimensions

Levels in the 'Worry' dimension:

1. Worry

0. I don't feel worried today

2. Sad

3. Pain	1. I feel a little bit worried today
4. Tired	
5. Annoyed	2. I feel a bit worried today
6. School work/Homework	
7. Sleep	3. I feel quite worried today
8. Daily routine	
9. Able to join in activities	4. I feel very worried today
Similar levels for each of the other dimensions	

Copyright: CHU9D at www.chu9d.org. Contact CHU9D group for application details and registration for use.

This gives you an idea of what the CHU9D instrument looks like. There are 9 dimensions, each with five increasing levels of health impairment attached to it, ranging from mild to the most severe impairment. Here are the levels associated with the Worry dimension, and each of the other dimensions have their levels presented similarly. The child or adolescent is asked to place a tick or mark next to the level corresponding to their health that day. The scoring algorithm to obtain utility values from the responses is currently based on preferences from the UK adult general population (using the standard gamble method), but there is work ongoing to develop a scoring algorithm based on preferences of an adolescent population, using discrete choice experiments (this has already been done in Australia).

DCEs to obtain adolescent preferences

CHU9D: Traditional DCE Example

Life A	Life B
I feel quite worried today	I don't feel worried today
I don't feel sad today	I feel quite sad today
I have a bit of pain today	I don't have any pain today
I feel quite tired today	I feel very tired today
I don't feel annoyed today	I don't feel annoyed today
I have a few problems with my schoolwork today	I have some problems with my schoolwork today
Last night I had some problems sleeping	Last night I couldn't sleep at all
I have a few problems with my daily routine today	I have many problems with my daily routine today
I can join in with a few activities today	I can join in with some activities today
Live for 4 years	Live for 10 years
Which life do you prefer?	

Reminder from last week: in DCEs, we present choices to respondents in such a way that we are able to weight their preference for attributes

Here, the dimensions of the CHU9D are used as the attributes, with the levels from the instrument used as the levels in the attributes

Choices made between the given options in the DCE choice sets allows us to analyse the value placed on CHU9D dimensions and levels and obtain utility values

Capability measures

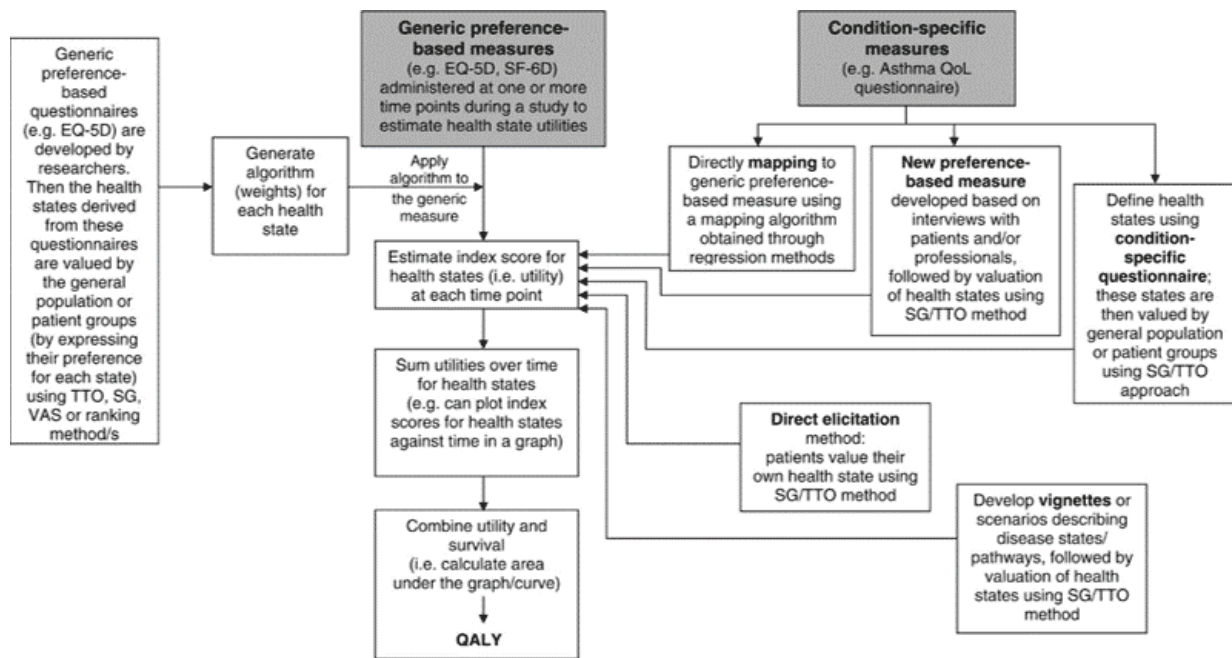
	Generic	Condition-specific
Preference based	EQ5D SF-6D HUI CHU9D ICECAP	EXACT-U (For use in COPD)
Non-preference based	SF-36 SF-12	Asthma quality of life questionnaire Parkinson's Disease Questionnaire

- There has recently been movement to go beyond health when measuring the outcomes of an intervention
- Broader measures of quality of life may be able to more adequately capture the benefits of interventions
 - Particularly those with a focus on rehabilitation, reablement or care of the elderly
- Capability: defines wellbeing in terms of an individual's ability to 'do' and 'be' the things that are important in life
- ICECAP-A: Measure of capability for the adult population
 - Dimensions: Security, love, independence, achievement and enjoyment
- ICECAP-O: Measure of capability for the elderly
 - Dimensions: attachment, security, enjoyment, role and control
- The dimensions included in the ICECAP measures show how capability measures differ from the health-related quality of life measures

ICECAP-O

- Similar to the scale of 0-1 utility, the ICECAP-O has a scale of 0-1 capability
- A set of index measures have been estimated using a **best-worse scaling** study of older people in England
 - This can be used to attach capability scores to responses and thus be used similarly to utility values obtained from the EQ5D
 - There is debate about the extent to which the ICECAP-O measure may be used in a cost-utility analysis, since it measures capabilities rather than utilities
- A study was conducted to empirically compare the ICECAP-O and EQ5D instruments, if you are interested in how scores compare between the two, you can read further in: Milte, R., Crotty, M., Miller, M.D. et al. Quality of life in older adults following a hip fracture: an empirical comparison of the ICECAP-O and the EQ-5D-3 L instruments. Health Qual Life Outcomes 16, 173 (2018). <https://doi.org/10.1186/s12955-018-1005-9>

Summary of common methods used to obtain health state values



Whitehead SJ, Ali S. Health outcomes in economic evaluation: the QALY and utilities. Br Med Bull. 2010;96:5-21. doi: 10.1093/bmb/ldq033. Epub 2010 Oct 29. PMID: 21037243.

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This figure shows the various methods used to obtain utility values for health states. It brings together a lot of the concepts covered over the course of the last 2 weeks, and hopefully helps you understand how the different methods fit together, and bring us to our ultimate aim: measuring health outcomes for the purpose of economic evaluation.



Case study

Case study: Parkinson's disease

- Parkinson's disease is a progressive, neurological condition with a wide range of physical and mental health symptoms, which can have a substantial impact on quality of life
- Approximately 145,000 people in the UK are living with Parkinson's ([Parkinson's UK](#))
- Incidence increases rapidly with age, most patients develop initial symptoms between 50-70 years of age
- Symptoms vary widely, can affect a broad range of dimensions of well-being
- Due to the wide variety of presentations of this illness, it is useful to consider the application of various outcome measures for people with Parkinson's

Parkinson's disease symptoms

- Physical symptoms include:
 - Motor symptoms: tremor, slowness of movement, stiffness, falls, postural disturbance, etc.
 - Non-motor physical symptoms: bladder and bowel problems, sleep difficulties, saliva control difficulties, speech, swallowing problems, etc.
- Mental symptoms include: depression, hallucinations and delusions, anxiety, memory problems, dementia

Clinical measures for Parkinson's

- Unified Parkinson's disease rating scale (UPDRS) and Movement Disorder Society (MDS)-UPDRS
- Instruments targeting specific symptoms include:
 - Hoehn and Yahr scale (H&Y): motor symptoms
 - Non-motor symptoms scale (NMSS): non-motor symptoms
 - Freezing of Gait Questionnaire (FOG-Q): freezing;
 - Modified Dyskinesia Rating Scale (MDRS): dyskinesia;
 - Mini-Mental State Examination (MMSE): cognitive function;
 - Beck Anxiety Inventory (BAI): anxiety;
 - Beck Depression Inventory (BDI): depression;

Condition-specific QoL measures for Parkinson's

	Generic	Condition-specific
Preference based	EQ5D SF-6D HUI CHU9D ICECAP	None
Non-preference based	SF-36 SF-12	PDQ-39 PDQL PDQUALIF

- **Parkinson's Disease Questionnaire-39 (PDQ-39)**
 - Self-administered questionnaire
 - Includes both motor and non-motor symptoms
 - 39 questions, relating to 8 key areas of health and daily activities
 - The 8 areas form 8 discrete scales, so you obtain a score for each dimension
 - You'll get a chance to use the PDQ-39 in this week's exercise, so it is available in the exercise materials
- **Other condition-specific QoL measures for Parkinson's include:**
 - Parkinson's Impact scale
 - Parkinson's disease quality of life questionnaire (PDQL)
 - Parkinson's quality of life questionnaire

Which measure?

- So how do we decide which outcome measure to use?
 - Clinical measure
 - Condition-specific quality of life measure
 - Generic quality of life measure (e.g. SF-36)
 - Generic multi-attribute utility instrument (e.g. EQ5D)



Think about the different measures and what kind of research questions they may be able to answer – post your answers in the discussion forum

CONTINUE

To help you think about the answers you have written down, here are two examples of Parkinson’s disease research. Look at the outcome measures used in each one, and think about how they relate to the title of the paper. Also think about why researchers might want to use more than one measure. Write your insights in the discussion forum.

Levodopa vs levodopa-sparing		Dopamine agonist vs MAOI	
Estimate† (95% CI)	p value	Estimate† (95% CI)	p value
1.8 (0.5 to 3.0)	0.005	1.4 (0.0 to 2.9)	0.05
1.9 (0.7 to 3.0)	0.002	0.3 (-1.1 to 1.7)	0.7
-0.2 (-1.1 to 0.7)	0.7	0.3 (-0.8 to 1.4)	0.6
1.3 (0.2 to 2.3)	0.02	1.3 (0.0 to 2.5)	0.01
0.1 (-0.6 to 0.8)	0.8	0.8 (-0.1 to 1.7)	0.01
1.0 (0.0 to 2.0)	0.05	1.7 (0.5 to 2.9)	0.01
0.9 (0.0 to 1.8)	0.05	0.5 (-0.6 to 1.5)	0.4
1.4 (0.3 to 2.4)	0.01	0.7 (-0.6 to 2.0)	0.3
1.0 (0.3 to 1.7)	0.008	0.8 (0.0 to 1.7)	0.01
0.03 (0.01 to 0.05)	0.0002	0.004 (-0.01 to 0.02)	0.6

†Average differences between levodopa and levodopa-sparing groups, and MAOI, in the different PDQ-39 subscales and in EQ-5D utility scores.

Analysis of Deep Brain Stimulation Surgery Versus Best Medical Therapy in Patients: Economic Evaluation Alongside the PD

1, PhD,^{1*} Alastair Gray, PhD,² Jane Daniels, PhD,³ Steven Gill, FRCS,⁴ Nati, DPhil,⁵ Rosalind Mitchell, FRCS,⁶ Hardev Pall, MD,⁶ Smita Patel, MSc,^{2,7} John Wheatley, DPhil,⁸ and Adrian Williams, MD,⁹ on behalf of The PD SURF

TABLE 2. EuroQol EQ-5D utility values at baseline and 12 months, and QALYs gained

	DBS	BM	Mean (95% CI) Difference (DBS)
	(n = 177)	(n = 178)	
	0.49 (0.26)	0.49 (0.25)	0.01 (-0.04, 0.06)
	(n = 152)	(n = 153)	
	0.56 (0.27)	0.50 (0.26)	0.06 (0.003, 0.12)
	(n = 177)	(n = 178)	
	0.54 (0.27)	0.49 (0.26)	0.05 (-0.01, 0.11)
	(n = 159)	(n = 151)	
	0.0296 (0.167)	0.0088 (0.144)	0.020 (-0.015, 0.055)
	(n = 177)	(n = 178)	
	0.0222 (0.17)	0.0077 (0.15)	0.020 (-0.13, 0.17)

Source: Lancet 2014; 384: 1196–205

Source: Movement Disorder. 2016;31:1173-82.

CONTINUE

Summary of preference vs non-preference based measures

Feature	Preference-based	Non-preference based
Construction		
Descriptive system of attributes and levels	✓	✓
Value set attached to each health state	✓	✗
Generic or condition-specific	Mostly generic (Some specific instruments do exist)	Both generic and specific
Score range	0 (death) – 1 (full health) Sometimes negative values are available (states worse than death)	Any e.g. PDQ-39: 0-100 PDQL: 37 (worst) – 185 (best)
Use in cost-utility analysis	✓	✗ (Unless a mapping algorithm exists)

This table summarises some of the main features of preference-based measures compared to non-preference based measures.

In terms of their components, both of them have a descriptive system of attributes and levels, but only the preference-based measures have value tariff attached to each health state, which incorporate people's preference. Most of the preference-based measures are generic, allowing their use across conditions and can be compared within and between conditions. There are other advantages to the generic instruments. They allow the assessment of overall quality of life affected by multiple conditions

and are useful when the intervention has both treatment effect and adverse effect on the patients. Generic measures allow the assessment of the overall quality of life and are not tied to any specific condition or attribute.

However, there are also limitations to generic measures. They cover broad areas of health which may not be sensitive to changes in specific symptoms. Sometimes the domains in the measure may not be related to specific symptoms. For example, the lack of specific focus on mental health may make a generic measure inappropriate for use with mental health conditions.

The non-preference based measures can be generic, such as SF-36, or specific such as PDQ-39. Specific measures have the advantage of being more sensitive to small changes in the symptoms related to a condition. On the other hand, they cannot be compared between conditions. Further, because each of them has different score range and descriptive system, specific measures (even from the same condition) cannot be compared with each other.

In terms of whether they can be used in economic evaluation, obviously preference-based measures are designed for the purpose, with incorporated public's preferences. The non-preference based measures typically assume all questions or domains are equally important and the differences of impact on quality of life between the domains are equal. In contrast, as we have seen in the scoring algorithm of the EQ-5D, the decrement between the levels are varied, and the decrements from different domains are also varied. This is because the preferences for them are different.

Despite their wide use in economic evaluations, there are criticisms around the use of generic preference-based measures (multi-attribute utility instruments).

They are criticized for lack of relevance and sensitivity to specific conditions, such as mental health and cognitive performance.

Secondly, there are several preference-based measures available and they are all anchored at 0 (death) and 1 (full health) to be able to be used as weight to be combined with length of life, to calculate a QALY. However, due to their different domains and scoring system, their scores cannot be used interchangeably and it is difficult to compare across them.

Another criticism is related to the question - whose value matters? The values attached to the health states are generated from the general public (as recommended by NICE) rather than the specific population in the health states. It is argued that the general public does not have the same experience of the disease as patients and thus cannot reveal the true preference of the specific population being evaluated.

One of the main questions to be asked is: can we make use of the non-preference based measures in economic evaluations? The answer is usually we can't. They can only be used when a mapping algorithm exists from a specific measure to utility values. For instance, if we need to derive utility values from another study, where only a non-preference based measure was used, we can use a mapping algorithm. This usually translates the dimension scores from the non-preference based measure to EQ5D health states, which we can then use to calculate utilities. However, care must be taken to ensure that the patient population in which the mapping algorithm was created is generalizable to the patient population where it is being applied.

For example, in rheumatoid arthritis patients, there is a mapping algorithm to convert HAQ (Health assessment questionnaire) scores to EQ-5D. If this were to be applied in a patient population with psoriatic arthritis patients, then this may be biased because differences between the patient population might influence the mapping.

Exercise

Now that we have introduced you to the concepts of various instruments, this week's exercise will give you the opportunity to apply them

The exercise instructions, along with an Appendix containing the necessary instruments are available in Moodle





References

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