Novel Approaches to Value Assessment, Within the Cost-Effectiveness Framework

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Extending the scope of patient reported outcomes and QALYs

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Novel approaches to value assessment within the cost effectiveness framework
Limitations of QALYs as a unit of outcome

- Health related quality of life (HRQoL)
  - What is ‘health related’, and what isn’t?
  - eg. mixed evidence on extent to which standard generic PROs capture wider impacts (productivity; income)
- By convention, analysis focuses on HRQoL and QALYs of patients
  - But family, carers and others also impacted by illness and treatment (negative and positive externalities)
  - Focus on health and health care misses impacts and outcomes from social care needs and care services.

Utility and income/productivity

- Do respondents consider lost income when valuing health states? (mixed evidence)
- How well does individual lost income reflect societal productivity? (probably not very well)
- But to the extent income is considered, potential for double counting. 2nd: Washington Panel: capture ‘pure’ effects on HRQoL.
Limitations of HRQoL utilities

- By convention, based on average stated preferences of the general public
  - Who is the general public?
  - Average value of people or value of the average person?

- Alternative normative arguments e.g. in favour of considering patients’ preferences (e.g. Sweden’s TLV)
  - But are ‘patients’ a homogeneous group?
  - Brouwer and Versteegh (2016): both perspectives are relevant.
  - Reconciling systematic differences?
  - Fundamental questions about the maximand; allocative efficiency; opportunity cost.

Example of research to address limitations

Extending the QALY

...developing a broad measure of quality of life for use in economic evaluations across health and social care...
Aim: to develop a new quality of life instrument

1. A questionnaire that picks up the impact of health treatments and social care interventions on
   • physical and mental health
   • and broader quality of life aspects as judged to be important by service users (and those who are impacted such as carers)

2. Amenable to
   • use in economic evaluation
   • being included in trials
   • (ideally) being translated to other languages and used internationally

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Extending the QALY
May 2017- Oct 2019

Project partners

- SchARR
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  - Julie Johnson (Project Co-ordinator)
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  - Clara Mukuria
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  - Bhash Naidoo
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- University Of Kent
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- Office of Health Economics (OHE)
  - Nancy Devlin

Governance groups

- Advisory Group – academics, wide range stakeholders
- Steering Group – key stakeholders & academics
- Public Involvement Group – members of the public
- NICE Citizen’s Council

Funders

- CLAHRC
- UK Medical Research Council (MRC)
- EuroQol Research Foundation
- Descriptive systems & valuation working groups

International teams

- China/Singapore
- Germany
- USA
- Argentina
- Australia
Stage I: Identify Domains / Themes (May - Feb 2017)

- Literature review of qualitative research on wellbeing and quality of life
- Psychometric analysis of existing patient data set (MIC)

Stage II: Generate potential questions to measure each theme (Feb / March 2018)

- Using other instruments & new questions based on the literature review
- Items required to meet specified criteria
- ~100 items identified

Feelings and emotions
- Sadness/Happiness
- Hopeless/hope
- Anger/frustration
- Anxious/calm
- Feeling safe

Activity
- Self-care
- Enjoyable or Meaningful activity/roles
- Hearing
- Communication/speech
- Mobility
- Vision

Self-identity
- Treated with dignity/respect
- Self-worth / self-respect / confidence

Autonomy
- Control
- Coping

Relationships
- Loneliness
- Feeling Supported
- Good relationships
- Stigma
- Belonging / connectedness

Physical sensation
- Pain
- Discomfort (e.g. itching, feeling sick, dizziness)
- Fatigue & Tiredness
- Sleep problems

Concentration & Memory
- Concentration & Thinking clearly
- Memory
- Confusion
Stage III: Face Validation Interviews (March - Aug 2018)

Aim:
• check interpretation of items
• explore alternative wording and response options
• identify embarrassing items

Interviews in England (n=45) (social care users, carers & patients).
Repeated in 5 other countries
• China, Germany, Australia, Argentina, USA

Select and refine questions for psychometric survey in England (~70 items)

Key face validity findings

- Instructions including the recall period were often ignored or forgotten
- Short items could be answered but respondents wanted more information on context
- Preferred response options varied (frequency, severity or difficulty)
- Respondents unable to say why they preferred one option over another
- Preference for simpler layouts
- Items can work well for one group e.g. carers but not another
- Work from different countries often complementary in findings
**Stage IV: Select questions for the instrument (Sept 2018 - Jan. 2019)**

Conduct survey in England (n=2000, patients/carers/social care users)
Include EQ-5D-5L, EQ-5D-3L, WEMWBS and ASCOT
Psychometric and IRT analysis – to test dimensionality and item performance

Select items for a profile measure and a classification system using all face validity and psychometric evidence

**Stage V: Valuation (Jan – Sept 2019)**

Face to face TTO interviews with general public in UK (n~600)
& ‘deliberative’ valuation exercise with NICE Citizen’s Council

**Stage VI: What difference would the new measure make? (July- October 2019)**

Compare to other instruments (EQ-5D, WEMWBS, ASCOT)
Potential mapping
Apply to existing cost effectiveness studies – what difference would using it make to resource decisions
Concluding remarks

- The QALY and existing measures of HRQoL can be extended in various ways to capture problems and outcomes not conventionally taken into account.
- There is also scope to incorporate, more systematically, patients’ preferences regarding HRQoL into HTA (although issues remain about how best to do that).
- If the E-QALY is measuring different things, that will also have implications for an (E-) QALY-based threshold.
- Ultimately, there is a limit to what can be included within the QALY - other considerations are already taken into account in HTA alongside QALYs. Not whether to consider other factors but how best to do that.

To find out more about the E-QALYs project:

See [https://scharr.dept.shef.ac.uk/e-qaly/](https://scharr.dept.shef.ac.uk/e-qaly/)

If you would like to join the E-QALY on-line Advisory Group please contact [Julie.Johnson@Sheffield.ac.uk](mailto:Julie.Johnson@Sheffield.ac.uk)
Limitations of PROs

- Ability of the individual to self-complete the instrument can be a limiting factor.
  - age; disability; cognitive impairment
  - Chronic diseases of unconsciousness; young children (eg < 8)
  - Proxy completion
- Can only capture ‘felt’ problems eg not high blood pressure
- PROs may not always adequately capture particular kinds of health problems (eg hearing/vision) or inadequately capture mild problems (‘ceiling effects’)

ISPOR Washington Summit: New approaches to value assessment
October 19th 2018