Health policy

The uses of utilities – can an overemphasis on cost per QALY cloud decision making?

Cost utility analysis and health system decision making

Fixed budgets and the prevailing imperative to contain costs within healthcare systems around the world mean that active trade offs need to be made in the provision of pharmaceuticals and other healthcare interventions. An important feature in many evaluations is the impact on quality of life for the patient and, in some cases, the carer.

The unit of currency used by many, although by no means all, health technology assessment - agencies in evaluating new technologies is the quality adjusted life year (QALY). Proponents of the QALY argue that it offers the best, most transparent and most equitable measure available across multiple different diseases and indeed that is has proved its worth in practice by being widely accepted as the basis for a rational approach to decision making.

There is, however, a case against the QALY and the time could be right to revisit the way in which the QALY is used and might evolve. While the goal of enabling people to live longer lives of improved quality is uncontested, the way in which the QALY attempts to capture these two dimensions of health is open to challenge.

Methodology and mechanisms of measurement

Estimates of both survival and quality of life are of course beset with their own complications and methodological controversies. However, the main reservations about QALYs as a measure of health gain relate to the underlying utilities which have the task of reducing all the important features of health related quality of life to a single number on the 0-1 scale referenced to dead and perfect

health. The first point to note is that there are several valid and validated approaches to collecting data, notably:

- Direct methods using preference-based measures (time trade off, standard gamble) to obtain patients' valuation of their own health;
- Indirect methods using generic questionnaire led assessment according to an established descriptive system (EQ-5D, SF-6D), supported by a separate valuation set or 'tariff';
- Indirect methods using visual analogue scales;
- Indirect methods using disease specific questionnaires with validated mapping to general utility instruments.

The challenge here is that these methods rarely, if ever, achieve fully coherent results – with direct methods having a tendency to yield slightly higher overall utilities. Even in a well defined population with well characterised health states, utility estimation has a high level of implicit uncertainty.

Secondly, health state description is critical and often overlooked or given insufficient attention in utility estimation. In direct preference based estimation, elucidating meaningful patient preferences for a given health state critically depends on accurate and thorough description of complex health states in a way that can be understood and which is coherent with actual health states. In indirect methods employed within clinical trials, health states may not be fully documented or, despite all extrinsic factors being equivalent, may be inconsistent between patients.

Whose preferences?

A further source of uncertainty stems from participant bias and in some cases the use of surrogates for the patient to elucidate utilities. Clinical trial populations are perhaps the most relevant source of quality of life data which can be directly applied to a given population but are inherently skewed by the inclusion and exclusion criteria of the trials. Real world observation may therefore be preferred.

When valuing the health states defined by a system such as EQ-5D on a single scale, using a general population sample of individuals may confer democratic legitimacy on health state utilities used to allocate resources in collectively funded healthcare systems. However, true understanding of a described health state may be limited, leading to substantial over or under-valuation of quality of life. Similarly, the use of carer or physician estimates of utility is flawed given the empathy and often false perceptions held by these two groups, again giving rise to divergent utility estimates.

Complex patient populations

Where the patients being studied are considered the most appropriate population to obtain utilities, this may not be entirely straightforward. There are specific patient populations for whom elucidation of utilities is complex. This could be for cognitive or communication reasons (infant populations, those suffering from certain mental health conditions or in health states where communication is not feasible) or for other social or psychological reasons (in particular in populations with distrust of healthcare in general or the gathering of information). Furthermore, there are some patient populations where the assessment of utility may be open to question from ethical and practical perspectives (in particular infants and those with cognitive impairment).

Infants and those with severe cognitive impairments

While utility is interpreted as an expression of patient preference for one health state over another, for some patients there is a genuine question of ability to perceive differences or to adequately express a preference.

Ignoring for a moment the potential communication issues, consider a 1 day old child who has a severe but not life threatening disability – an adult may consider the disability to have a significant effect on quality of life but the child is unable to conceptualise the impact that the disability could have. Similarly, those with substantial cognitive dysfunction may have limited ability to make comparisons between health states.

If we now layer in the complexity that is brought by having complex questionnaires and the need to gather data requiring some form of shared communication, utility estimates for these groups can become highly uncertain.

It may be better in these cases to focus less on the short term impact of therapy on estimates of utilities than the potential for clinically meaningful change in the condition – a less universal but perhaps more apt metric.

Non-participative populations

Another group that presents challenges to the elucidation of utilities consists of those who, for various social, mental health or access issues, choose not to participate in research in healthcare in general. In some conditions, these groups may be over-represented compared with the general public and may have a very different interpretation of health states.

This group also contains some of the most disadvantaged in society and is as a result perhaps more deserving of careful attention and decision making around healthcare. At the same time, lack of inclusion in utility estimates may mean that resourcing decisions are made without their critically important opinions being taken into account.

Highly divergent populations and end of life care

One final set of populations to consider is those whose opinions of the importance of quality of life vary substantially, such as those in the later stages of terminal cancer. In these groups, utility estimates derived using indirect methodologies (for example, EQ-5D) can frequently be performed and, with a little care and a lot of planning, give estimates within a well-defined distribution and with a reasonable level of confidence.

The issue is one of contextualisation of the result. All may agree that the impact on quality of life is high but, when it comes to trade-offs between survival and quality of life, there are generally two clear camps. On the one hand, there are those who want to live at any cost to their quality of life – sometimes with the hope of making it to a specific threshold or the advent of perhaps more effective future therapies - and those who prioritise a shorter but higher quality end of life. By adopting an intermediate position informed by the views of the general population in the absence of treatment context, healthcare decision makers and reimbursement authorities are implicitly attempting to reconcile opposing views. Unfortunately, this is a state which reflects the preferences of neither group.

Alternatives and new ideas

Much has been written about the benefit and limitations of the QALY by both sides of the argument. The QALY may, in many applications, be appropriate and uncontroversial. However, any system of decision making will inevitably contain flaws. It is incumbent on decision makers to recognise these flaws and to be flexible to new approaches where the standard approach does not fit. It is also incumbent on companies to carefully plan and apply the standard rules and to document and explain when and why to break out beyond them.

Conclusion

Quality of life and utility measures are entrenched in value assessment and emphasise the importance of accounting for more than just survival. Utility estimation is, however, a source of uncertainty and an overreliance on cost utility analysis may cause value to be overlooked or incorrectly assigned in some populations.

A clear methodological framework is needed that allows the appropriate approach to utility estimation and, where needed, other approaches to be employed that:

- Establish the methodology of best fit for the patient population and the health states of interest;
- Consider the population (clinical trial, society, carers, patients, healthcare professionals) who will give the most useful estimates of utility;
- Consider multiple approaches to the same question to narrow uncertainty;
- Consider and document alternative approaches – going beyond utility – where utility estimation is highly uncertain or inappropriate.

Value based assessment is of clear advantage to societies and to all stakeholders involved in the process of deliberation on pharmaceutical pricing, reimbursement and access but careful application of complex and uncertain approaches is required to fully recognise the benefits of new interventions.

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