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INCLUDING PATIENT CHOICE IN COST-EFFECTIVENESS DECISION RULES

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Abstract

There has been increasing discussion in the economic literature, about the appropriateness of using general population values within technology appraisal. This paper proposes an alternative approach to incorporating patient values into the cost-effectiveness decision rule that lies at the heart of funding decisions. Whilst the current decision rule is constructed around a technical question, namely, 'which treatment is the most cost-effective?', the key policy question is 'which treatments should be offered to the patient?'. A two-part decision rule is explored which gives the patient the choice of the most cost-effective treatment plus all cheaper options. Whilst the adoption of this patient-based cost-effectiveness rule may not alter many decisions compared to the current approach, it would represent a profound shift in the way that patient values and patient choice are incorporated into economic evaluation.

Background

Purchasers of health care across the world increasingly make decisions about which treatments can be used by patients using cost-effectiveness considerations. Within this framework, effectiveness is most frequently measured using quality-adjusted life years (QALYs). QALYs are calculated by summing morbidity weighted life-expectancy, with the weights typically based around mean values generated from a sample of the general population. The weights represent valuations of health-related well-being (or utility), and are anchored on 1 (representing full-health) and 0 (representing death or health states considered to be equivalent to death).

There has been increasing discussion in the economic literature, about the appropriateness of using these general population values within technology appraisal (Brazier *et al.*, 2005). It is argued, by some, that patient values are better estimates of health-related well-being as patients have first hand experience of the health state. Describing a particular health state, then asking a member of the general public to place a value on it, is potentially flawed by the limitations of the descriptive system used to describe the health state and the ability of a member of public to imagine what it is like to be in that health state. Given these problems, it is little wonder that large differences between patient and public values are observed.

Despite these problems, the use of general population values continue to dominate technology appraisals, although the reasons for this tend to focus on the potential disadvantages of using patient values. One group of influential economists argued that society should adopt a 'veil of ignorance' when choosing health state values to purposely avoid the influence of self interest (Gold *et al.*, 1996). Such self interest, it has been argued, can lead to strategic behaviour when collecting health values. Another problem avoided by the use of population values is that some aspects of patient values may want to be excluded from our valuation of health outcomes. Adaptation, it is argued can lead to patients being satisfied with their diminished functioning, thus leading to higher than expected values, and conversely, lower than expected gains from treatment.

The disquiet around the problems with general population values has led to calls for using better informed general population values. Such values are seen as having the benefits of maintaining a social perspective, with fewer of the

problems associated with their lack of experiential knowledge. Whilst there is an increasing amount of work around patient values, and their role within technology appraisal, the cost-effectiveness decision rule that lies at the heart of funding decisions has remained unquestioned.

The current cost-effectiveness rule

The cost-effectiveness rule used routinely in technology appraisal is that for any given monetary value placed on health (or a QALY), the recommended treatment for funding is identified as that with the highest incremental cost-effectiveness ratio (ICER) that falls beneath this threshold value[1].

This cost-effectiveness rule can be illustrated using Table 1. 'Do nothing' represents a situation where no active therapy is given to the patient, treatments 'A', 'B' and 'C' are new, more expensive therapies. Using the current decision rule, and a threshold value of £30 000 per QALY, we see that treatment A is deemed the most cost-effective, and is therefore recommended for patients with the condition.

Table 1: Cost-effectiveness of four hypothetical treatment choices

Treatment	Cost	QALYs	Incremental cost-effectiveness ratio relative to DN	Incremental cost-effectiveness ratio relative to next best option
C	100 000	5.5	39 600	100 000
A	50 000	5.0	24 500	20 000
B	20 000	3.5	38 000	38 000
Do nothing (DN)	1 000	3.0		

However, the current decision rule is constructed around a technical question, namely, 'which is the most cost-effective treatment?', when the question that should be asked is 'which treatments should be offered to the patient?'. Under the current rule, some patient choice remains as a patient can not be forced to accept the therapy deemed most-cost-effective; they actually have the choice of 'Do nothing' and 'A'. However, some patients may prefer treatment 'B' over treatment 'A', but are not allowed this under the decision rule, even though it is expected to cost less than A.[2]

This situation is produced because mean *ex post* general population values and *ex ante* individual patient values rank the treatments differently; population values suggest that treatment 'A' is preferred, whilst patient values suggest that treatment 'B' is preferred. It also produces an inconsistency with respect to the implied patient choice within the current decision rule; patients are able to choose one treatment which costs less but is deemed less effective based on mean general population values (i.e. 'Do nothing'), but not another (i.e. 'B').

[1] Although the decision rule should relate to the increment relative to the next best option, the rule is frequently operationalised with the increment relating to 'do nothing' or 'current treatment'.
[2] The position of treatment A is recognised by economists in terms of 'extended dominance' (Weinstein 1990), a concept that is used to rule out the treatment from further consideration as a potentially cost-effective treatment.

Whilst this is an interesting hypothetical example, is it likely to happen in the real world?

The case of osteoporosis

National Institute for Clinical Excellence (NICE) Technology Appraisal 87 (NICE 2005) sets out recommendations for the use of bisphosphonates, selective oestrogen receptor modulators and parathyroid hormone for the secondary prevention of osteoporotic fragility in postmenopausal women. The cost-effectiveness analysis for this appraisal is complex with alternative figures produced for alternative evidence bases, and patient populations described in terms of age and bone mineral density. A summary of the results are given in Table 2, which includes a single bisphosphonate (as opposed to the three which were assessed), raloxifene, oestrogen, and teriparatide.

In summary, the guidance recommended bisphosphonates as the preferred treatment, with the option for using raloxifene if bisphosphonates were contraindicated, produced an unsatisfactory response or if patients were physically unable to comply with the strict directions for taking bisphosphonate medications (NICE 2005). The use of bisphosphonates entails fasting and ingestion of medication at least 30 minutes before breakfast and remain standing for 30 minutes after taking the tablet.

Table 2: Cost-effectiveness of treatments for the secondary prevention of osteoporotic fragility in postmenopausal women at 70 years of age*

Treatment	Cost	QALYs	Incremental cost-effectiveness ratio relative to DN**	Incremental cost-effectiveness ratio relative to next best option***
Teriparatide	7 172	5.54	134 728	-1 257 781
Raloxifene	3 147	5.55	29 993	-24 371
Alendronate	2 818	5.56	16 934	8 934
Oestrogen	2 383	5.51	69 585	69 585
Do nothing	1 868	5.50		

* *Some treatments and analytic scenarios have been excluded from the full table presented by Stevenson and colleagues (2005) for simplicity.*

** *Figures taken from report. ICERs based on model estimates, whilst costs and QALYs are rounded.*

*** *Figures calculated from table, as they are not available from the report. ICER for Oestrogen kept the same as in previous column for consistency.*

The NICE guidance did not allow patients the choice of taking oestrogen, even though it is possible that they would consider the lifestyle restrictions associated with alendronate as being disruptive to the extent that oestrogen was considered preferable. In such a situation, the patient would be offered a treatment that they considered to be worse and more expensive (alendronate). Only if they were "physically unable" to follow the treatment directions would they be allowed to even consider another treatment choice (raloxifene), and then not oestrogen.

Whilst there are some added complexities with this technology appraisal, due to other uncertainties relating to oestrogen and raloxifene, the example serves to

highlight the potential for cheaper, patient-preferred treatment options to exist in real life and to be omitted from treatment options by reimbursement authorities.

A patient-based cost-effectiveness rule

Clearly, the current decision rule is capable of producing uncomfortable scenarios that are generated by differences between mean general population values and individual patient values. Some work has been undertaken to assess the feasibility of calculating patient specific ICERs (Sculpher 1998). However, this approach requires the elicitation of *ex ante* health states from all patients requiring treatment so as to calculate individual expected QALYs, which is a daunting prospect.

A partial resolution of the problem is to reformulate the current decision rule so that it better reflects the key question of 'which treatments should be offered to the patient?'. The proposal raised here, is to allow patients to have a choice over the most cost-effective therapy (as adjudged by mean general population values), those treatments that are less costly than the cost-effective therapy, and 'do nothing'. Patients would not be given the choice of therapies that are more expensive and more costly than the most cost-effective therapy (for example, 'C' in Table 1).

This reflects a two-part decision process; the identification of the most cost-effective therapy using mean general population values (i.e. the current rule), then relative to that, the identification of those treatments that are cheaper than the most cost-effective therapy.

Under this patient-based cost-effectiveness rule, if a patient prefers a treatment such as 'B' in Table 1 (or oestrogen in Table 2), they should be able to choose it. In effect they have adjudged that their well-being will be greater under 'B' than for 'A' and the mean costs are lower. In other words, when assessing the patient-preference ICER, 'B' dominates. Treatment 'C' would not be offered even if the patient chose it, as the increased well-being needs to be traded-off with increased costs.

One further issue is worth consideration. It is possible that a form of adverse selection could exist, whereby those who choose B are expected to have costs much greater than the mean population values (i.e. £20 000). This would result in higher patient well being but potentially very high costs. This can be guarded against by using sub-group analyses to see if they belong to a patient group who are expected to have higher costs that A.

The patient based cost-effectiveness rule then becomes: you offer the patient the choice of the treatment that has the highest ICER under the threshold, or a lower cost treatment *if and only if* they belong to a patient sub-group that has lower expected costs than the most cost-effective option.

Potential problems

Two issues are worth further consideration. Firstly, the nature of the cost savings produced by the proposed rule, as these are not certain. Secondly, the 'validity' of a rule which offers a pragmatic solution with no theoretical base.

Nature of the cost savings

The patient-based cost-effectiveness rule offers the possibility of patients choosing a cheaper treatment ('B') than that recommended using the current approach ('A'). However, it is possible that without this choice, some patients would have chosen 'do nothing'. In such circumstances, the offer of an alternative treatment ('B') raises the possibility of increased costs. Consequently, whether the proposed rule is cost-saving or cost-increasing at the population level, is an empirical question.

Theoretical validity of the rule

Whilst the proposed rule has been described in the context of cost-effectiveness decisions, and taps into notions of a patient-preference ICER, it does not have the theoretical base of cost-effectiveness analysis. It is a pragmatic solution, that mixes together societal and patient perspectives. It could also be argued that the wider choice it provides moves away from the notion of the public provision of health care, to a social insurance model where patients have a right to choose from a menu of approved treatments.

So, does the lack of a theoretical foundation and the mixing up different perspectives within a decision rule invalidate the rule? It is clear that the NHS does not operate a single all-encompassing evaluative framework. Whilst extra-welfarism is used by many health economists to justify the predominant evaluative framework used by themselves, the NHS uses a range of decision making criteria from a mixture of sources; theoretical, pragmatic and political.

These different views are recognised by Drummond and colleagues (Drummond et al, 2005) who contrast the pragmatic 'decision-making approach' with the theoretical approaches of welfarism and extra-welfarism. The rule forwarded in this paper is clearly pragmatic, and from a decision-making perspective, I would argue that its desirability can be evaluated. The decision maker needs to evaluate whether the benefits the rule confers in terms of greater choice and greater health benefits as evaluated by the patient, are worth the potential extra cost and reduced health benefits as evaluated by a population tariff.

Summary

Cost-effectiveness rules have developed to answer a technical question, without due regard for patient choice. Amending this rule allows greater choice for the patient without necessarily increasing the programme cost. However, using the metric of general population values this alternative decision rule reduces health gains. This loss of *ex post* society-valued health gain must be balanced against the increase in patient choice and *ex ante* patient-valued health gain. Whether society is willing to bear the potential extra cost for these gains becomes the central question.

It should be noted that when funding decisions are operationalised, health care professionals reinterpret them to allow patient choices of this nature. It is also

possible that such circumstances are rare, and made rarer still by the use of non-cost effectiveness information in the decision process to account for patient concerns. Consequently, the adoption of this patient-based cost-effectiveness rule may not alter many decisions compared to the current approach. Even if this were the case, it would represent a small but profound shift in the way in which evaluations are conceptualised, by recognising the central importance of patient values and patient choice.

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