What Values Should Count in HTA for New Medicines under Value Based Pricing in the UK?

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I. Introduction: Health, Values and Multi-Criteria Decision Analysis

The National Health Service is, once more, embarking on a programme of significant reform, particularly with regard to its response to finite budgets and scarce resources. These developments were foreshadowed by earlier interventions, including an Office of Fair Trading report on Value Based Pricing (OFT, 2007), and the continuing struggle by NICE to attempt to accommodate a concern for 'social values' (NICE, 2008, Rawlins, 2010, NICE Citizen's Council 2008).

If such issues as distributional concerns, or broader social benefits of health, are widely considered important by the public, yet do not explicitly figure in NICE assessments, then there is a danger that we are not doing the best – the ethical best – with our health budget and the "QALY is a QALY" approach of NICE must be revisited. Of course incorporating any value other than health gain means that probably health gain will not be maximized but where other considerations are taken to be more important than pure health gain this consequence has, in effect, been accepted in advance.

Hence there is an ethical and economic imperative for all interested parties to try to arrive at a decision process that reflects the social values that can legitimately be incorporated. The current discussion of Value Based Pricing (VBP) (Department of Health 2010) introduces the idea that pharmaceuticals should be priced to reflect their values. The consultation exercise makes clear that the Department of Health agrees that other values than health gain are to included in the future appraisal process. In moving beyond health gain as the sole currency of value in the NHS multi-criteria decision analysis (MCDA) will become an increasingly popular technique.

This paper does not attempt to address the technical questions of how to implement MCDA or to combine it with VBP. Rather it will focus exclusively on the preliminary ethical questions of what values should be included in a "long list" of values which might be employed in a health assessment MCDA (whether or not combined with VBP). Our methodology is to be as comprehensive as possible, starting with a wide 'long-list' of possible values, and then to consider a range of considerations for and against their inclusion. We will then make provisional suggestions about which values should go forward to the next stage.

In considering different values for inclusion it has become clear that certain disputes can sometimes be attributed to different views about the appropriate objectives of a health system. On one view the NHS has a single purpose: to improve population health to the maximum degree given a fixed budget. Another view is that the NHS must take other concerns into account, such as those of social justice or fairness between individuals, or aspects of individual well-being beyond health. A further view is that the aims of the NHS are not set in stone or a matter of decree, but, in a democratic society, should be responsive to what the people want them to be. This question of the overall objectives of a health system cannot be settled here. However we can, in some cases, diagnose disagreements as being explicable in terms of disagreement about objectives.

The list of candidate values we consider here is derived both by considering other attempts to provide an MCDA for HTA and our own reflection and analysis. The lists we consulted include: lists implied by the various reports of the NICE citizen's council; NICE guidance and the "social value judgements" (2008), or SVJ, document; SMC's 'Modifiers' list; the new AGNSS MCDA framework; lists implied from the "Social Value of a QALY project" (Baker, Bateman and Donaldson et al, 2008; Dolan et al, 2008); the ANNALISA list; Canada's EVIDEM framework; a list from Golan (2010) cited by Devlin and Sussex (2011); summaries of lists made available to us by Warren Cowell from briefing notes he has presented to Pfizer[1], and others. We do not claim that our list is exhaustive and the "true, best" list, only that it is representative of the concerns that are current in the literature.[2]

It is worth noting at the outset that although MCDA is often introduced as a way as incorporating values beyond health gain into the analysis, health itself is a complex notion with many elements, and decisions may need to be made about whether to sacrifice one component of health for the sake of another. Hence MCDA may be needed even if it is insisted that health is the only relevant value. Indeed, the QALY, especially if scored using EQ-5D methodology, is a way of combining separate elements of health. MCDA can help us understand and reflect upon the assumptions about combination of values implicit in the QALY model.

[1] "Multi Criteria Decision Analysis (MCDA) Approaches: Briefing for Pfizer working team" and "MCDA: Pfizer proposed criteria list/structure" both of August 2010.

[2] Naturally the lists often express slightly different ideas with the same language, or the same ideas in different language. We have had to reinterpret the lists so as to try to accommodate all distinct ideas in a non-overlapping fashion.

II. Populating a Long List

Our procedure is to set out the candidate values and assess which should be included in subsequent analysis. Our reasoning draws on several sources:

- *Ethics* (also known as 'normative' reasons): We rely on a loose definition of ethics as judgements that are informed by other-regarding concern. In principle we accept that ethical concerns can conflict with each other, as well as with other sources of judgement. Where possible we will refer to the health-related philosophical literature, and other sources, where such arguments are made.

- *Expert Opinion:* An expert opinion will most often be based on technical knowledge, especially from economics and psychology. We do not assume, however, that expert opinion is always correct, or that it should always override other considerations. Where possible we have provided the source in the literature for the expert opinions we have cited. Where no source is provided, the expert opinions are either our own or based on unpublished opinions we have heard from others.

- *Public Opinion:* We will refer explicitly to public opinion where there is clear evidence that members of the public have been consulted on an issue and have formed a view, whether positive, negative or indifferent. A difficulty, however, is that sources generally report the preferences of the members of the public without explaining their reasoning. This is important, as in some cases it may be that the public are swayed by considerations elsewhere on the list, and so there is a danger of double-counting. For example, in some studies the public wish to prioritise children. Is this because children will generally live for more years than adults, and so the health gain will be greater, or is it because there is an extra level of compassion for children? (Dolan et al. 2005, p. 205). The former 'double-counts' life extension, whereas the latter provides a separate reason. At this stage all we are able to do is to note the difficulties where they arise.

- *Legal:* Here we indicate those categories of value which otherwise have prima facie appeal, but which, it could be argued, should be prohibited because of concerns over legality. Once more where we are able to we have cited the source of such concerns.

- *Policy or Precedent:* There are a number of cases where values that could formally be included in MCDA have, in the past, been used in previous decision-making as a result of official guidance of various sorts. Here we note such cases as providing strong reasons for continuing to include such values, and in each case cite our source for the reason.

III. Value Summary

The values are grouped together under a number of headings. For each value, we will list:

- *Definition of the value*.
- *Reasons for inclusion*.
- *Reasons for exclusion*.
- *Summary judgement on inclusion*

In the summary we will state and explain our recommendation. There are three categories: near-certain inclusion (GREEN) possible inclusion (AMBER), and near-certain exclusion (RED).

Given the large number of values we consider, and the constraints of space, we can only be very brief in summarizing the different considerations. Where possible, however, we give references to further sources of discussion. Although this is an unconventional approach to an academic paper, because the debate over MCDA and what values should be included in the appraisal of health technologies is in its infancy, we believe that a more comprehensive and systematic review of possible values which could be included is necessary.

Before turning to the values we should mention the potential category 'cost', which in one sense is the most important value of all. Although it must figure in any resulting MCDA we have not included it in the discussion below as we assume that the situation in the future will continue to be one in which decision makers aim to make the most efficient use of a fixed sum of money. Our exercise is to consider what efficiency means when many sources of value are taken as legitimate goals to be included in decision-making. The fact that cost must be taken into account in effect goes without saying at this stage of the analysis.

IV. The List of Values

IV.A. Category I: Health Related Quality of Life and Well-Being Gains

Here we refer to any kind of gain to health and survival (defined using whatever scale) but also to the impact on quality of life that an intervention may provide. We also include patients, carers and dependents.

IV.A.1. Degree of life extension to patient (QALY 1)[3]

Definition: Some amount of time is necessarily associated with any health gain, otherwise there would be no actual "vessel" for the health gain.

Reasons for inclusion:

i) As noted in the definition, the degree of life extension is a conceptual necessity for considering health gain at all. (Expert opinion)

ii) Studies have been interpreted to suggest that members of the public give value to the mere fact of living alone, over and above any quality of life improvement. (Mason et al 2005) (Public/Expert opinion).

Reasons for exclusion:

i) Some studies indicate that a significant portion of the public do not think that life extension is valuable in itself unless the life is in some way worth living. (Public Opinion, Brock and McKibben, 2011).

Summary judgement on inclusion: **Green** - The reasons for exclusion provide grounds for considering how quantity and value of life extension interact (see Tilling, Devlin, Tsuchiya and Buckingham, 2009.) but do not justify exclusion.

IV.A.2. Degree of health gain to patient (QALY 2)

[3] Within the QALY we disaggregate the "quality" from the "life years" component because health' and 'life extension' can be considered separate sources of value and within the context of an MCDA it is important that no assumptions are inadvertently made about trade-offs between values.

Definition: For current purposes we assume that most of the important elements of health states can be captured by a generic health related quality of life measure such as the EQ-5D scale, or similar (e.g., HUI3, SF-6D).

Reasons for inclusion:

i) Given the nature of the exercise it would be conceptually impossible to leave out health gain. (Expert opinion)

Reasons for exclusion:

i) Some health gains are very trivial; others though significant could be unaffordable. (Expert opinion)

ii) It may not be possible to define health in a 'value-neutral' fashion. (~~SOURCE~~ Expert opinion)

Summary judgement on inclusion: **Green -**The difficulties identified show that health is not the only concern, and that some pragmatic decisions have to be made, rather than justify exclusion.

IV.A.3.Impact on patient's well-being

Definition: The process of treatment itself could have well-being effects, independently of the final health outcomes.

Reasons for inclusion:

i) An unpopular form of treatment is less likely to be complied with. This is directly important for the efficacy of treatment. (~~SOURCE~~ Expert opinion)

ii) Well-being and dignity effects can be valuable independently of health gain. (~~SOURCE~~ Ethics)

iii) The quality of the 'process of care', independently of effects on health, is included as a desirable goal in the NHS Outcomes Framework. (Department of Health 2010; ~~)~~ ~~(~~Precedent)

Reasons for exclusion:

i) Ensuring compliance should be the responsibility of the GPs or social services. (~~SOURCE~~ Expert opinion)

ii) Issues of compliance should already have been taken into account in trials, and therefore this would involve double counting. (~~SOURCE~~ Expert opinion)

iii) Only health effects should be included. (Expert opinion)

Summary judgement on inclusion: **Green** - The health gains will already have been included in the analysis. However, the non-health gains to wellbeing are important to patients and should be included.

IV.A.4. Impact on health of carer

Definition: Some conditions put burdens on carers. This category considers health effects.

Reasons for inclusion:

i) Detriments to the physical and mental health of a carer are undesirable and may lead to future calls on the NHS. Taking measures to reduce such impact is an enlightened application of preventative medicine (~~SOURCE~~ Expert opinion)

Reasons for exclusion:

i) It will be rare that such effects can be predicted. (~~SOURCE~~ Expert opinion)

ii) Each patient should be considered on his or her own merits and not on their effects on others. (~~SOURCE~~ Expert opinion)

Summary judgement on inclusion: **Green –**Some chronic conditions, such as Alzheimer's, clearly can have health effects on carers. Even if the exclusive focus of the NHS is on health, it is reasonable to include the health of carers.

IV.A.5.Impact on carers' well-being

Definition: This category considers non-health effects on carers, in their role as carer, and not as a family member or dependent, which we address below.

Reasons for inclusion:

i) This has a straightforward justification in that it improves the welfare of a citizen. (Ethics, expert opinion)

Reasons for exclusion:

i) It will be rare that such effects can be predicted. (Expert Opinion)

ii) Only health effects should be considered. (Expert Opinion)

Summary judgement on inclusion: **Green** – Once more, there are some clear cases. We do not accept the restriction to health gains only.

IV.A.6. Impact on health gain of family or dependents

Definition: Here we are concerned with family members or dependents as such, and not in their possible roles as carers.

Reasons for inclusion:

i) Living with and/or being dependent upon a person with an illness can be detrimental to physical and mental health, which may also lead to future calls on the NHS. Taking account of the effect of a patient's health on their family and dependent's health is an enlightened application of preventative medicine (Expert opinion).

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Reasons for exclusion:

i) It will be rare that such effects can be predicted. (Expert opinion)

ii) Each patient should be considered on his or her own merits and not on their effects on others. (Expert opinion)

iii) NICE's Social Values guidance rules out discrimination on the basis of family membership. (NICE 2008) (Legal)

Summary judgement on inclusion: **Red –** It will be very hard to sort between cases of illness that have a tendency for significant effects on the physical or mental health of family and dependents and those that do not. Even in the case of infectious disease, the reason for treatment includes the possibility of transmission to anyone, whether family, dependent, or not. In the case of maternal health, however, there does there seem to be a direct connection between a mother's health and that of the fetus, but this would already be included in a QALY assessment.

IV.A.7. Impact on well-being of family and dependents

Definition: Many cases will involve patients who have financially and emotionally dependent children, or other family members.

Reasons for inclusion:

i) 'Knock-on' effects should be taken into account, and the greater the level of need, the greater the weight applied. (Ethics)

ii) The NICE Citizen's Council report on *Departing from the Threshold* (2008) considered the category "the intervention will have a major impact on the patient's family" as one of the most important consideration to take into account. (Popular opinion)

Reasons for exclusion:

i) The NICE Social Value Judgments document rules out discrimination on the basis of family status, which is unlawful. (Legal)

ii) It is not possible generally to classify treatments into those that will address the health needs of people with dependents or family and those of people without. (Expert judgement)

Summary judgement on inclusion: **Red** - both reasons for exclusion seem decisive.

IV.A.8. Unavailability of alternative treatment

Definition: In some cases a patient group is already receiving a certain treatment, and NICE faces the question of whether to licence a treatment that offers extra benefit at extra expense. In other cases a new treatment offers a first chance of addressing a condition.

Reasons for inclusion:

i) It is commonly thought that pharmaceutical companies put too much research effort into 'me too' drugs, to provide an improvement on existing treatments, when there is a much more urgent need for treatment for neglected conditions (OFT 2007). (Policy, expert opinion)

ii) Targeting untreated condition gives preference to those in greatest need and was considered important in the NICE Citizen's Council *Departing from the Threshold* report (2008). (Public opinion)

iii) The NHS offers its services to all. Those who have a condition for which no treatment is offered have a reason to think that they are being unfairly excluded. (Ethics)

Reasons for exclusion:

i) Severity of condition and innovation are considered under different categories. Hence there is a serious danger of double counting. (Expert opinion)

ii) It is not true that those for whom no pharmaceutical is available are excluded by the NHS. Other forms of treatment may be available, and the patient will have access to medical advice in the same way as any other patient. (Expert opinion, ethics)

Summary – **Amber** - To some degree this is accounted for under severity and innovation. However it would be worth exploring whether there is public support for this factor independently.

IV.A.9. Leading to other definitive treatments

Definition: Some treatments provide relatively little health gain, but are necessary but not sufficient for some other health gain to be obtained from another treatment. When taken in isolation such treatments are not cost-effective but as part of a broader treatment package with a very low £/QALY the summed £/QALY of the two-part intervention might well be under the threshold.

Reasons for inclusion:

i) Obviously when considered as part of a package, this can be represented as a straightforward health gain. (Expert opinion)

Reasons for exclusion:

i) In principle it opens up a Pandora's box where any intervention that might produce some health benefit in concert with another intervention could be funded, which might complicate matters beyond reasonable limits. (Policy constraints)
ii) An accurate evaluation of such technology will already have included the health gain available when used in combination. Hence to add this as an extra factor will be double-counting. (Expert opinion)

Summary judgement on inclusion: **Red -** The double counting considerations seem to be decisive.

IV.B. Category II: Prioritized Subgroups

We here refer to sub-groups that could be given priority in treatment.

IV.B.1.Severity of illness

Definition: Disease severity concerns those conditions that have the lowest score in terms of the EQ-5D index, such as those conditions that leave people permanently bed-ridden with severe health problems.[4]

Reasons for inclusion

i) The normative pull of equality and prioritizing the worst off is well established in political philosophy (Parfit 1991, Ethics) and giving priority to the most severe conditions was given high weight in the NICE Citizen's Council *Departing from the Threshold* report (NICE Citizen's Council 2008), and other studies (Dolan 2008). (Public opinion)

[4] There is often overlap between the categories of least health population (severity of condition), pre-existing health state, life-saving and end of life treatments. However, we separate them for conceptual reasons, and we have noted that with all categories we must be wary of the possibility of double counting.

Reasons for exclusion:

i) Some studies suggest that the public do not give priority to treating those in severe conditions if the improvement to their health is not substantial. (Baker, Bateman, Donaldson et al 2008) (Public opinion)

ii) Those with severe health conditions may be very difficult to treat and it may be may be impossible to provide any significant health gain. (Expert opinion)

Summary judgement on inclusion: Our conclusion is **Green**, in cases where a non-marginal health gain is possible.

IV.B.2. Pre-existing health state

Definition: This differs from IV.B.1 above in that it refers not just to the current condition of an individual but also their long-standing health state. The treatment may or may not be exclusive to people in their condition (e.g. a disabled person who needs both special therapy for the disability and a heart operation that is not specific to people with their particular disability).

Reasons for inclusion:

i) It has been a long-standing criticism of the QALY approach that it 'discriminates against the disabled' in that those of permanent low health are capable of fewer QALYs than others, and so would be a lower priority for treatment. (SOURCE Ethics)

Reasons for exclusion:

i) The standard response to the above is that NICE licences treatments and does not judge how much benefit particular individuals will derive from the treatment. Therefore there is no discrimination. (Expert opinion, ethics)

ii) Those with severe, long-standing, health conditions may be very difficult to treat in a way that provides more than a very small health gain. (Expert opinion)

Summary judgement on inclusion: **Green** - It may be that a treatment specially designed for a patient group could improve their health status to a significant degree proportionately to their previous state, yet not be cost effective in QALY terms because of long-standing disability. There seems,

therefore, good reason to include this as a special factor, provided that the health gain available is of a reasonable dimension.

IV.B.3. Life saving treatments

Definition: Where a person is facing an urgent and immediate threat to life, and an intervention could return them to a reasonable degree of health, perhaps even to their previous health state. These are distinct from the normal case of life extension, in that the threat of death is immediate, perhaps in the next few days or weeks, and a good recovery is possible. An example may be emergency surgery after an accident, or the provision of a powerful anti-biotic in the case of severe infectious disease.

Reasons for inclusion:

i) This was given the highest rating by NICE Citizen's Council *Departing from the Threshold* Report. (Public opinion)

ii) There has been a certain level of support in the philosophical literature for the so-called 'rule of rescue' in such cases. (~~SOURCE~~ Ethics)

iii) It seems very difficult to justify policies where society declines to save the life of an identified person (who is capable of a good standard of future health) on the basis of cost-effectiveness, especially in the light of media scrutiny. (Policy)

Reasons for exclusion:

i) While the rule of rescue has great intuitive plausibility in cases such as miners trapped underground, where a special weight is given to saving their lives, and cost-effectiveness analysis is not normally applied, it is possible to defend these as special cases as they are rare, and have low budgetary impact. In a medical setting situations where a life can be saved are very common, and thus cannot be treated as special cases. (Policy)

ii) Saving a life and returning someone to full health where they might live for many more years generates many QALYS. Those who advocate special attention to life-saving may not have realised that life-saving will already get very high priority under standard methods. Hence there is a possibility of double-counting. (Expert opinion)

Summary judgement on inclusion: **Green -** The policy considerations seem very powerful, especially when combined with public opinion and the ethical arguments. Double-counting is a concern, but it does not seem that the reasons in favour of life-saving are exhausted by issues of health gain.

IV.B.4. Life extension for those near the end of life

Definition: Those who are facing imminent death who will be able to survive for a longer period, though not restored to full health, if given a particular treatment. An example may be a treatment that allows a cancer patient a few more months of life, though in a poor health state.

Reasons for inclusion:

i) Compassion decrees that we should extend lives when we can. (Ethics)

ii) NICE has issued guidance that allows for exactly this situation under certain circumstances. (NICE 2009) (Precedent)

Reasons for exclusion:

i) Support for ‘life extension’ was much lower than for other criteria, such as ‘life saving’ in the NICE Citizen’s Council *Departing from the Threshold* report. (NICE Citizen’s Council 2008) (Public opinion)

ii) Often those in the situation so described will have lived a full life and, in Alan Williams’s famous expression, have had a ‘fair innings’. (~~SOURCE~~ Ethics)

Summary judgement on inclusion: **Green** - While we feel that the arguments in favour of this consideration are less strong than arguments concerning severity, it would be very difficult in the current context to reverse the guidance that NICE has provided. Further, while some may see justification for age-weighting, the ‘fair innings’ argument is arguably illegal on grounds of discrimination.

IV.B.5.Type of illness and “dread”: Cancer

Definition: Some conditions are particularly feared.

Reasons for inclusion

i) If a condition is feared then there may be a higher desire among patients for treatments than for other conditions of comparable health severity. There is some reason to believe that the public regard cancer in this way. (Public opinion)

ii) Although the Health and Safety Executive does not use the QALY (using the VPF instead) nevertheless in some of its guidance it 'takes the view' that cancer should be given double weight. (Health and Safety Executive 2001) (Precedent)

iii) The government have recently made extra funds available for cancer treatment. (Policy)

Reasons for exclusion:

i) No studies have been conducted that show how this factor should be weighted. (Expert opinion). In particular, no studies have shown that if the severity of the health state is constant across cancer and some other condition and this is made clear to respondents that the respondents will place a greater weight on avoiding cancer.

ii) Such dread is irrational. (Expert opinion)

Summary judgement on inclusion: **Amber** -It may well be true that the public dread cancer beyond its morbidity and mortality effects, yet the empirical base for this claim is lacking at the moment. Making judgements about rationality is problematic.

IV.B.6.Children and adolescents

Definition: Those under a particular age.

Reasons for inclusion:

i) This was a highly weighted category in the NICE Citizen's Council *Departing from the Threshold* Report. (NICE Citizen's Council 2008(Public opinion)

Reasons for exclusion:

i) Treatment of children is likely to have long-tem beneficial health effects and so treating children will generally provide many more QALYs than the same treatment for adults. To include this as a further consideration would be to double-count benefits. (Expert opinion).

ii) Some studies suggest that the public give priority to adults in the 20-40 year age range over children and older adults. (Baker, Bateman, Donaldson et al, 2008) (Public Opinion)

Summary judgement on inclusion: **Amber** -Further consideration is needed to see if the public's position can be clarified.

IV.B.7.Socially disadvantaged population

Definition: The socially disadvantaged can for practical purposes be defined in terms of their place in the net income scale.

Reasons for inclusion

i) For several decades it has been noted that, on average, health and life expectancy correlates with social class. Hence those of lower social and economic status have, on average, greater health need. (Marmot 2004, Expert opinion)

ii) Independently of the issue of the social gradient of health there is reason for giving priority in the allocation of public services to those who are worst off. (Parfit , 1991)(Ethics)

iii) The NHS currently adjusts the health allocation formula to take account of the extra health needs of the socially disadvantaged in an attempt to reduce inequalities in health. (Precedent)

Reasons for exclusion:

i) The NICE SVJ guidance explicitly rule out using considerations of social class (see above). (Legal)

ii) Opinion surveys do not provide clear support for giving priority according to social class (Dolan et al, 2008). (Public opinion)

iii) It may be hard to classify treatments on their appropriateness to different social classes. (Expert opinion) (However historically this has not always been the case (rickets) and this may also be currently the case for some conditions (TB)).

Summary judgement on inclusion:

We do not think that SVJ is an insuperable obstacle. It is well established in UK social policy that policies that attempt to improve the position of the disadvantaged are not discriminatory in the prohibited sense. NICE also says: “NICE can recommend that use of an intervention is restricted to a particular group of people within the population (for example, people under or over a certain age, or women only), but only in certain circumstances. There must be clear evidence about the increased effectiveness of the intervention in this subgroup, or other reasons relating to fairness for society as a whole, or a legal requirement to act in this way” (p. 25). **Green**, in cases where it is possible to aim at the target group.

IV.B.8. Individual responsibility

Definition: Some individuals suffer from ill-health as a result of their own actions (smoking, drinking, over-eating). Where this is so, should their treatment be a lower priority for the NHS than that of other patients who have not been at all responsible for their own ill-health?

Reasons for inclusion

i) The moral logic of reciprocity and the “principle of fair play” naturally endorse a criterion of responsibility. (~~SOURCE~~ Ethics, public opinion)

Reasons for exclusion:

i) The ‘solidaristic’ foundation of the NHS provides reasons against holding people responsible for their own illness. (Ethics)

ii) Surveys are very mixed in their results and there is no clear majority in favour of including responsibility in this way. (Dolan et al 2005) (Public opinion)

iii) It can be very difficult to untangle genuine individual responsibility, due to complexities of causality and in theories of responsibility, freedom, etc. (Expert opinion)

Summary judgement on inclusion: **Red** - Note, however, given "media amplification" on responsibility, sentiment may change on this in the future.

IV.B.9. NHS responsibility

Definition: Some negative health outcomes can be traced to causes within the health system itself, such as hospital-acquired infections, medical negligence or misdiagnosis.

Reasons for inclusion:

i) The NICE Citizens council report *Departing From The Threshold* gives this close to highest priority (NICE Citizen's Council 2008) (Public opinion)

ii) This consideration is very close to a notion of 'compensation for harm caused'. (Ethics)

Reasons for exclusion:

i) The cause of health loss should not matter. (Ethics)

Summary judgement on inclusion: **Green -** The argument that 'if the health system has caused your health problem, then it has a special responsibility to help' seems compelling, even though the cost will ultimately fall on other patients.[5]

IV.B.10.Immigration status

Definition: Different beneficiaries of the NHS might have different degrees of citizenship and longevity of stay, and hence have "paid into" the system to different degrees.

Reasons for inclusion:

[5] We are aware that this judgement appears to conflict with the judgement given on individual responsibility above, where it was said that the cause of a condition should not affect whether one receives treatment. However in this case the NHS, which exists in order to take care of our health, has a special level of responsibility, and if it fails in that duty so badly that it causes illness in patients, then an exception to the general principle that causes should not matter seems justified

i) It is a basic notion of fairness that those who have paid into a system should receive priority over those who have not. (Ethics)

ii) It is predicted that opinion surveys would support this criterion. (Predicted public opinion)

Reasons for exclusion:

i) It is not part of the NHS ethos that those who contribute most (for example in taxes) should get preferential treatment. (Expert judgement)

ii) This would be ruled out by anti-discrimination provisions. (Legal)

iii) It would be impossible to target immigration status through HTA. (Expert opinion)

Summary judgement on inclusion: **Red** - the reasons against appear very strong.

IV.C. Category III: Industrial

Industrial reasons will generally fall into two categories: first, those that provide beneficial incentives to the pharmaceutical industry, especially in terms of research and development; and second, those that provide beneficial effects for the UK economy as a whole. This has been a controversial area recently (Ferner, Hughes and Aronson, 2010; Goldman et al, 2010) in response to the Kennedy Report (Kennedy, 2009).

IV.C.1.Innovation 1: Dynamic efficiency

Definition: It is sometimes suggested that treatments emerging from a research programme at an early stage should be licenced at a cost above the threshold to encourage a branch of research that may yield future beneficial and cost-effective treatments.

Reasons for inclusion

i) Encouraging research may lead to greater cost-effectiveness in the longer term, including, ultimately, the creation of generic drugs. (Expert opinion)

Reasons for exclusion:

i) It is not the business of the health allocation mechanism to pay for innovation, rather than by means of dedicated research funding. (Policy)

ii) To divert funding in this way will be to favour future patients to the detriment current patients. (Expert opinion)

iii) In all areas of a modern market economies, risk taking and innovation is encouraged through the profit motive. To pay an "innovation premium" now and pay for the health value gained from that innovation at a later stage is to double count or pay twice for the same innovation. (Expert opinion)

iv) In those cases where no profit is ever obtained from a particular innovation, it is not clear that this is worth any kind of rectification by the public purse when similar policies are not pursued in other areas of the economy (Claxton et al, 2009, Kanavos et al, 2010)). (Expert opinion)

Summary judgement on inclusion: **Amber** - It is a question of policy for the government whether the health research budget should be partly consolidated into the health budget directly, or through a health related "innovation fund" or kept entirely separate.

IV.C.2. Innovation 2: Generic markets

Definition: A 'generic market' is understood to be one where currently the best treatment for a condition is a cheap generic drug.

Reasons for inclusion:

i) Where the current best treatment is a generic it may be very difficult for a pharmaceutical company to show that a new treatment offers additional incremental benefits at a cost-effective price, even if the health benefits are very significant. The low price of the comparator makes the existing regime very cost-effective even if its effectiveness is low. Hence it may be that companies will be unwilling to take the financial risk of research and development in generic markets and therefore particular conditions for which generics are available will not gain the benefits of innovation. This is unfair to future patient groups. (Expert opinion)

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Reasons for exclusion:

i) If a condition is severe, and a generic provides only a small health benefit, then the resulting situation is equivalent to one where there is no existing treatment for a moderately severe condition. In such a case the existence of the generic is not a significant barrier for innovation. If, on the other hand, the cheap generic provides a reasonable health gain it is unclear why aiming for further health gain should be a priority. (Expert opinion)

Summary judgement on inclusion: **Red** – the arguments against seem convincing.

IV.C.3. Promoting domestic industry and economic growth

Definition: Industry can relocate in response to changing demand for goods, and the NHS decision-making, can, in principle, have wider effects on the economy.

Reasons for inclusion:

i) There are sound economic reasons for taking macro-effects of NHS funding into account. (Precedent)

Reasons for Exclusion:

i) It is not the business of the health allocation mechanism to stimulate the economy: if this is to be done it should be by means of dedicated investment funding. (Expert opinion)

ii) In practice it is very unlikely that the health allocation mechanism will have an effect on economic growth. (Expert opinion)

iii) Some practices of preference to domestic industry may violate international trade agreements. (Legal)

Summary judgement on inclusion: **Amber -** There is good reason to include this element only if a decision is made to fund economic development through the health allocation mechanism, that good evidence can be presented that such consequences are likely to follow, and doing so is not illegal.

IV.C.4.Patient Productivity

Definition: The illness of some patients will have greater consequential effect for the economy than others, especially those in their working years.

Reasons for Inclusion:

i) This consideration is already taken into account in calculating the global burden of disease using the DALY, (Precedent) and is consistent with at least one study of public opinion (Baker, Bateman, Donaldson et al 2008) (Public Opinion).

ii) In some cases, such as the treatment of health workers in a health emergency, such policies will be of general public benefit. (Expert Opinion, Ethics)

Reasons for Exclusion:

i) Applied generally it is contrary to NICE SVJ anti-discrimination policy. (Legal)

ii) It violates the general solidaristic norm of equal treatment for equal need. (Ethics)

iii) Although there are good reasons for treating health workers first in cases of emergency, this can be justified in terms of the likely aggregate health gain, given that if they fall ill others will go untreated. Hence there is an element of double-counting. (Expert Opinion)

Summary judgement on inclusion: **Amber** - Applied generally this would be highly problematic, although restricted to health, and other essential, workers in times of emergency it appears defensible, provided considerations of double-counting are kept in mind.

IV.C.5. Orphan drugs

Definition: An orphan drug is defined in the EU as a drug to treat a condition with a prevalence of less than 5 per 10,000 of the population. Ultra-orphan conditions have a prevalence of less than 1 in 50,000 of the UK population (NICE, 2006).

Reasons for inclusion:

i) Unless extra incentives are given to produce orphan drugs the industry will not invest in such areas as it will not be profitable for them to do so, and rare conditions will be neglected. (Expert opinion)

ii) This consideration received a high degree of support from the NICE Citizen's Council *Departing from the Threshold* report, probably driven by great compassion for those suffering unusual threats to health. (Public opinion).

iii) The total budget impact of special provision for rare conditions is likely to be relatively low. (Expert opinion)

Reasons for exclusion:

i) There is no moral difference between a treatment that is expensive because research and development costs have to be recouped from a small patient population, and a treatment that is expensive for other reasons. Both, though matters of undeserved bad luck, should be treated the same way, and if a cost-effectiveness threshold is applied in one case, the same threshold should be applied in the other. (Ethics)

ii) Including the economic argument is a form of double-counting as this will be covered under the 'industrial heading'. Similar considerations apply to budgetary impact, and severity. (Expert opinion)

Summary judgement on inclusion: **Green –** There is an important difference between orphan drugs and other expensive treatments. As research and development costs need to be recouped then if there is no special treatment for orphan drugs rare conditions will never be researched. In other cases research may well be conducted and lead to cost-effective treatments, over time. Hence a principle of equal concern provides a strong justification for special consideration for orphan conditions.

IV.D. Category IV: Political Drivers

Government policy can override the normal health allocation mechanism. In some cases entrenched policy will need to be taken into account into future decisions. Here we indicate some of the major areas.

IV.D.1.National Priority Area

Definition: For policy reasons the government or other agency may declare a condition a national priority.

Reasons for inclusion:

i) Where an NPA has been defined it will be politically very difficult to refuse extra funds in such an area. (Policy)

Reasons for exclusion:

i) If there are good reasons for NPAs they will have already been covered by other considerations. (Expert judgement)

Summary judgement on inclusion: **Amber –** it would be politically very difficult not to follow such instructions.

IV.D.2.International Comparison

Definition: Certain treatments may be available in some countries that are considered relevant comparators to the UK health system.

Reasons for inclusion

i) It may be very difficult to resist pressure to keep up with what is made available in other similar economies. (Policy)

Reasons for exclusion:

i) If there are good reasons to refund the treatment they will have already been covered by other considerations, and the fact that another country might have reason for refund a treatment doesn't constitute a good reason for the UK. (Expert opinion)

Summary judgement on inclusion: **Amber -** There is good reason to make international comparisons as a type of 'check' on UK decision making, but generally these comparisons should be regarded as informative rather than precedents.

IV.D.3.Inter-Departmental Comparison

Definition: Government departments use conflicting approaches to the valuation of life and health, which sometimes leads to differing valuations.

Reasons for inclusion:

i) It seems that for reasons of fairness and consistency valuations should be the same throughout all government departments. (Ethics, policy)

Reasons for exclusion:

i) There is no reason for the NHS to follow valuations from other departments that may have been derived for other purposes using other methodologies, and may either be wrong or not appropriate for the context of the NHS. (Wolff and Orr, 2009)(Expert opinion).

Summary judgement on inclusion: **Amber -** Consistency between government departments is not an overriding goal where there are good reasons for differences. Nevertheless, inconsistency in valuation of the same injury between departments, e.g., the DH valuing a minor injury at a different level than the DfT, seems problematic and indicates inefficiency.

IV.D.4. Budget Impact

Definition: Funding some treatments which meet the standard threshold may have a very significant effect on the overall budget and thereby crowd out other treatments.

Reasons for inclusion:

i) The system cannot cope with enormous drains of resources to supply very many people with a (collectively) expensive treatment especially when the benefits to any particular individual are relatively small. (Policy)

Reasons for exclusion:

i) If this situation occurs it can only show that the method of valuation is faulty (for example that severe conditions have been under-valued), for otherwise it should be acceptable that a greater value gain crowds out a lesser gain. (Expert judgement)

ii) To consider the size of the benefit is to make the judgement that the conditions in question are not severe. Hence this consideration will double-count severity. (Expert judgement)

Summary judgement on inclusion: **Amber** – Although having to take into account budget impact appears to show a failure of methodology, nevertheless it cannot be ignored.

IV.D.5. Uncertainty of outcome

Definition: The evidence base for some treatments is poor, and there can be uncertainty in outcome.

Reasons for inclusion:

i) Caution in the face of uncertainty can lead to great gains being forgone. (Expert opinion)

Reasons for exclusion:

i) This should already have been taken into account in arriving at estimates of expected benefit. (Expert opinion)

ii) Uncertainty cuts in both directions and it is unclear how it could be taken into account. (Expert opinion)

Summary judgement on inclusion: **Red –** Uncertainty is not a value to be weighted, but should be considered at the level of risk analysis.

IV.D.6. Stakeholder Persuasion

Definition: The force of public opinion on a particular treatment.

Reasons for inclusion

i) The NHS should be responsive to its stakeholders. (Ethics);

Reasons for exclusion:

i) Strength of preference, where based on good reasons, is already picked up by other measures, so there is a possibility of double-counting. (Expert opinion)

ii) Such a consideration opens up the possibility of external interference into the political process through lobbying by the pharmaceutical industry. (Expert judgement)

iii) This was universally rejected by the NICE Citizen's Council in their *Departing from the Threshold* report. (Public opinion)

Summary judgement on inclusion: **Red** – The ability of a group to "shout loudest" is not a normative reason for weighting**.**

IV.D.7.Cross-Department Effects

Definition: It is possible that an intervention could have effects elsewhere within governments. For example, a treatment regime may require lengthy absence from work, affecting welfare benefits. Or greater population health might significantly affect the number of people able to work.

Reasons for Inclusion:

(i) It would seem to be an aspect of 'joined-up' government to consider the wider consequences of any action. (Expert opinion, public opinion)

(ii) It would be arbitrary to exclude this consideration while including 'patient productivity' which is a non-health benefit of health for individuals. (Expert opinion)

Reasons for exclusion:

(i) Such effects are very hard to calculate. (Expert opinion)

(ii) Presently, the NHS only considers costs to the NHS and to the DSS. This is ultimately a matter of constitutional choice. (Expert opinion)

Summary judgement. **Amber** - Further consideration is necessary to see what this would come to in practice.

V. Summary

The following are coded GREEN for inclusion

- Degree of life extension (QALY 1)
- Degree of life health gain (QALY 2)

- Impact on patient's well-being
- Impact on health of carer
- Impact on carers' well-being
- Severity of illness
- Pre-existing health state
- Life saving treatments
- Life extension near end of life
- The socially disadvantaged
- NHS responsibility
- Orphan Drugs

The following are coded AMBER for further consideration

- Unavailability of alternative treatment
- Type of illness and "dread": Cancer
- Children and adolescents
- Dynamic efficiency
- Promoting Domestic Industry and Economic Growth
- Patient Productivity
- National Priority Area
- International Comparison
- Inter-Departmental Comparison
- Budget Impact
- Cross-Departmental Effects

The following are coded RED for exclusion

- Impact on health gain of family and dependents
- Impact on well-being of family and dependents
- Leading to other definitive treatments
- Individual responsibility
- Immigration status
- Generic markets
- Uncertainty of outcome

- Stakeholder persuasion

It should be emphasized that we regard this classification as provisional, rather than definitive.

It is worth commenting briefly on how following the recommendations here would alter existing practice. Despite its central concern with the QALY, NICE takes pains to emphasise that its decisions are also influenced by social value judgements and the deliberations of the Citizens' Council. On examination, we note that the values labeled Green here are consistent with this approach. NICE evaluations have taken (or could take) into account: life extension; health gain; impact on carers' well-being; convenience and acceptability of treatment; severity of condition; socially disadvantaged groups; life-saving; life-extension; pre-existing health state; NHS responsibility and orphan drugs.

Indeed it is no surprise that there is this degree of convergence with NICE's actual practice, as NICE has made strenuous efforts to respond to expert and public opinion as well as the general legal framework. The remaining challenge is to lay out a methodology in which all of these factors can be included in some sort of systematical fashion.

The amber categories on the current list require further deliberation or evidence sources. Further evidence would be very useful on the question of whether the public wish to give priority to children beyond the extra health gain that would result. It would also be very useful to know if there is empirical support for privileging the 'dreaded' condition of cancer, and those with rare conditions. In other cases, especially the 'industrial' amber categories, inclusion depends on a government decision of whether an innovation fund should be established (or whether the health budget should already be considered to have a research or industrial element). Other amber categories such as patient productivity may turn out, under the conditions specified, to have a very minor importance. But once more, many of these considerations seem broadly consistent with NICE's direction of travel.

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