NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

CENTRE FOR HEALTH TECHNOLOGY EVALUATION

Consultation Paper

Value Based Assessment of Health Technologies

Introduction

1 The *Guide to Methods of Technology Appraisal*, describes the key methodological principles that consultees should consider when submitting evidence to the Technology Appraisal Programme. It also describes the concepts and principles that underpin the Appraisal Committees' assessment of evidence and how they reach their decisions. The Guide applies to appraisals in both the single technology appraisal (STA) and multiple technology appraisal (MTA) processes. The current version of the Guide was published in April 2013.

2 In March 2013, the Government announced, in its response to the Health Select Committee report into NICE, what it described as the 'central role NICE will take in assessing the value of new medicines for value based pricing'[1].

3 In July 2013, Ministers referred the terms of reference for what is now referred to as 'value based assessment' (see Appendix 1). The Institute has

[1] The Department of Health commissioned its Policy Research Unit in Economic Evaluation of Health and Care Interventions (EEPRU), a collaboration between the School of Health and Related Research, University of Sheffield and the Centre for Health Economics, University of York, to research and develop the scientific basis for the three attributes to be included in what was referred to as 'value-based pricing'. These three attributes are 'burden of illness', 'therapeutic improvement and innovation', and 'wider societal benefits'. Towards the end of 2012, the Department of Health held two workshops with stakeholders to present this academic work and to set it in the context of other research published on the three topics. These workshops were followed by an engagement session with patient representatives and a workshop on equalities considerations for value-based pricing.

not been asked to make any other changes to its methods. In particular, the Government has agreed with industry[2] that the baseline cost effectiveness threshold should be kept at a level consistent with the current range (£20,000 per QALY up to £30,000 per QALY subject to the application, in individual cases, of a number of modifying factors) for the duration of the 2014 Pharmaceutical Price Regulation Scheme.

This consultation

4 We are consulting on changes to the way we make recommendations on the use in the NHS of health technologies (new medicines, medical devices and other technologies) for use in the NHS, to take account of the new terms of reference for value based assessment.

5 The methods we use are carefully developed and open to scrutiny by the public (including patients and patients' groups) clinicians, economists and academics, industry, and other interested groups, both to ensure they are fit for purpose and to engender trust in the way NICE works. We keep them up to date through regular reviews and when, as in this case, significant modifications become necessary.

6 This consultation sets out proposals to amend our *Guide to Methods of Technology Appraisal 2013* and contains our response to the new terms of

[2] The 2014 Pharmaceutical Price Regulation Scheme (the voluntary agreement to control the price of branded medicines, made between the Department of Health and the Association of British Pharmaceutical Industry) states that NICE will not negotiate, publicly set or publicly indicate prices. In addition, it is now clear that the Department of Health does not intend use value based assessment as a means of settingan acceptable price or, after a NICE appraisal, formally indicating that a particular price has been accepted.

The 2014 PPRS also states that the basic cost-effectiveness threshold will be retained at a level consistent with the current range and not changed for the duration of the agreement. The Government has made clear that this is possible largely because of the financial protection offered by the limit in the growth in NHS spending on branded medicines agreed as part of the scheme. The 2014 PPRS also confirms that the Terms of Reference for value based assessment will not prevent NICE Appraisal Committees from applying deliberation in the assessment process as they do now. The product of a NICE technology appraisal and the form in which it will appear will therefore be largely unchanged.

reference for value based assessment. The Guide is used by the independent committees of experts who appraise medicines and other technologies on behalf of NICE, for use in the NHS.

Current arrangements

7 Our Appraisal Committees begin their consideration of a new technology on the basis that the quality and length of life that might be restored by a new treatment (measured in QALYs) have the same value, regardless of the nature of the disease or condition it is designed to treat. The Appraisal Committees are able to take account of particular features of both the illness and the treatment.

8 The current Methods Guide (section 6.3.3) provides discretion for the Appraisal Committees to exercise judgement about the relevance and significance of modifiers in individual appraisals. Section 6.3.3 indicates that the judgements of the Appraisal Committees are influenced, in summary, by:

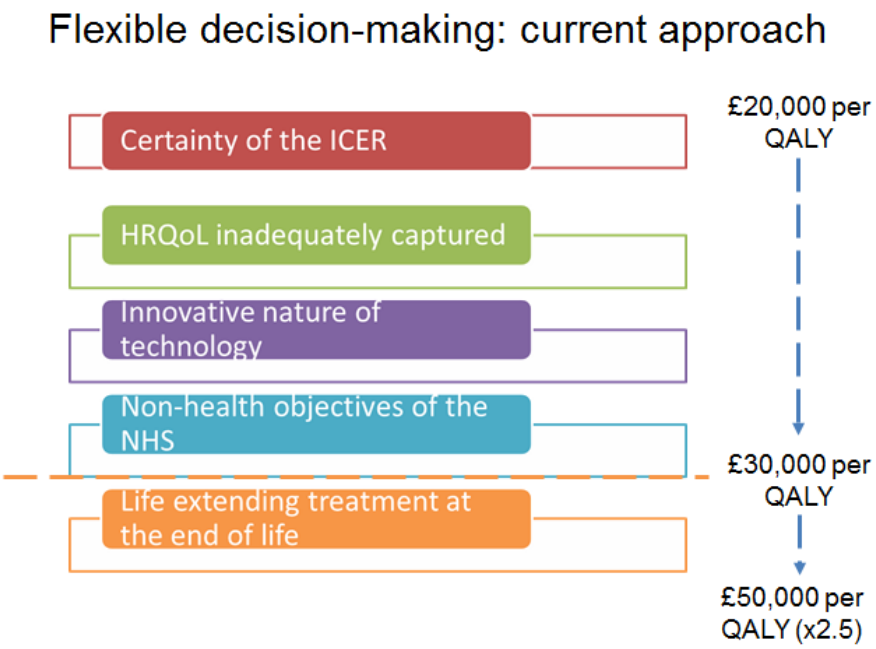
- the degree of certainty around the incremental cost effectiveness ratio;
- whether the assessment of change in health-related quality of life has been captured appropriately;
- the innovative nature of the technology;
- whether the technology meets the 'end of life criteria'; and
- aspects that relate to non-health objectives of the NHS (such as making better use of NHS resources).

9 The Appraisal Committees have discretion to consider whether the NHS should accept a higher opportunity cost (threshold) than they would normally recommend, for example when something new might offer the same overall health gain than it will displace, but has other elements of value not captured in the QALY. Appraisal Committees also have discretion not to recommend a

technology that has an incremental cost effectiveness ration lower than the threshold, for example when the benefit of something may not extend to all people who may receive it in routine practice. When they exercise this discretion, the Appraisal Committees are asked to consider the principles outlined in our Social Value Judgements guide (see Appendix 2).

10 In 2009, we issued advice to our Appraisal Committees, to be taken into account when considering life-extending treatments, used at the end of life (the end of life treatments protocol). This advice indicated, for the first time, a set of specific circumstances in which Appraisal Committees could attach a different weight to the health benefits achieved by technologies. No specific instruction was given to the Appraisal Committees with regard to the magnitude of this additional weight they should consider reasonable, but over time, practice has led to the application of a maximum weight of 2.5 from a starting point of £20,000 per QALY.

11 The way that our Appraisal Committees work through the elements of value that they take into account in making their recommendations is illustrated in the diagram below.



Our proposals

12 In order to respond to the terms of reference from the Department of Health, we intend to take the concept of 'burden of illness' into account more explicitly and systematically. Burden of illness is the loss (or shortfall) in quality and length of life, measured in QALYs (quality adjusted life years[3]), which occurs as a consequence of having a disease or condition, when compared with the QALYs that people would expect to have over the rest of their lives without the condition. The burden of illness is measured from the point at which the new treatment is to be used. The shortfall in QALYs will be considered *relative* to what people could expect without the condition at the time of treatment and is therefore called the 'proportional QALY shortfall'.

[3] NICE measures the clinical and cost effectiveness of a technology by using quality adjusted life years or QALYs. This is a useful way of comparing the costs and benefits of sometimes very different drugs in different conditions by looking at the gains to quality of life and life expectancy. This way of comparing drugs allows our independent Appraisal Committeesto understand the impact that introducing a new treatment will have on the ability of the NHS to maintain the services it already provides.

13 Our proposal for incorporating burden of illness replaces the current approach in which our Appraisal Committees consider life extending treatments used at the end of life (the ‘end of life treatments protocol’).

14 In addition, we intend to consider the wider impact of a disease on people's ability to be part of society. We refer to this as the ‘wider societal impact’ and define it as the loss (or shortfall) in a person’s capacity to engage with society as a result of living with the disease or condition, compared with their capacity to engage with society without the condition. We propose calculating wider societal impact by measuring the absolute shortfall in QALYs. Absolute shortfall is measured by subtracting the total QALYs expected as a consequence of having the condition from the total QALYs expected for people with the same age and gender distribution without the condition.

15 These two new 'value elements' - burden of illness and wider societal impact - will be added to the existing set that an Appraisal Committees is able to take into account when considering a new technology. In the *Guide to the Methods of Technology Appraisal,* these value elements are described as ‘modifiers’. NICE Appraisal Committees use these modifiers to help decide whether to recommend a technology with an incremental cost effectiveness ratio above £20,000 per QALY. One of these modifiers, the end of life treatments protocol, is applied by ‘weighting’ the additional QALYs a new treatment provides. This will be replaced by the new burden of illness modifier.

16 To ensure consistency in the way the Appraisal Committees applies both the existing and the new modifiers, we propose setting a maximum cumulative weight of 2.5 in circumstances where all modifiers apply. This is the maximum that has been considered acceptable by the Appraisal Committees during the fifteen years the Technology Appraisal Programme has been running.

17 A list of documents that have been considered as part of the development of these proposals can be found in Appendix 2.

Calculating QALY shortfall

18 The two ways that QALY loss or shortfall can be calculated are described below.

19 First, **absolute shortfall**, which is calculated by subtracting the reduced number of QALYs that people would be expected to have when living with an illness, from the total QALYs that healthy people with the same age and gender distribution would be expected to have. In this approach, larger absolute losses of quality of life are more significant than smaller losses. Longer durations of disease are more significant than shorter durations (for example, a chronic condition compared with a treatable acute illness). Diseases which cause very premature death are more significant than those in which death is delayed (such as childhood cancer compared with chronic heart failure).

20 Second, **proportional shortfall** in which the total QALYs for a person with a disease or condition is measured relative to those expected for a person of the same age and gender without the condition. This approach recognises the position of those patients who stand to lose the greatest proportion of their remaining health expectancy (such as diseases where a person is facing imminent death).

Using QALY shortfall to measure burden of illness

21 Burden of illness[4] is defined as the total amount of future health lost for people with a condition, measured in QALYs. The start point from which the loss is calculated is the moment of intervention with the technology being appraised. Research undertaken for the Department of Health showed support for the notion that when a choice needs to be made, members of the

[4] Work commissioned by the Department of Health to their Policy Research Unit in Economic Evaluation of Health and Care Interventions (EEPRU).

public felt that providing treatment for people with a larger burden of illness was a higher priority than treatment for those with a lower burden of illness[5].

22 The concept of proportional shortfall is broadly consistent with NICE's current 'end of life' approach. At the moment, the Institute's Appraisal Committees are asked to consider applying a weight to QALYs gained for people at the end of their life (defined as less that 24 months to live with current treatment) and who are likely to benefit from treatment (normally more than 3 months overall survival). This 'end of life' consideration, like proportional shortfall, is not particularly sensitive to the age at which people are diagnosed.

23 The Department of Health has calculated the burden of illness (QALY shortfall) for different conditions using a set of data developed from work undertaken by the University of York. This 'reference dataset' presents the burden of illness by the three digit ICD-10 code, aggregated across 16 age/gender categories. A web link to a more detailed description of the reference dataset can be found in Appendix 2, which lists the supporting documentation for this consultation.

24 This data is the starting point for establishing the burden of illness for a condition. However, the use of this data leads to a very broad level of aggregation by ICD-code (for example, all of breast cancer). Within diseases, there will be different levels of burden and it is important to capture this. In an actual technology appraisal, it will be necessary to focus on the burden of illness for the specific patient population for the drug or other product being appraised. Examples of the shortfall for a selection of conditions that have been subject to NICE technology appraisals are included in supporting documentation to this consultation document (see Appendix 2).

[5] Research survey performed by EEPRU, in which more than 3000 participants were asked to choose between treating two hypothetical patients ('discrete choice experiment'), in a number of scenarios.

Using QALY shortfall to measure wider societal impact

25 When making judgements on the value of a new technology, the Appraisal Committees currently have flexibility to consider the impact of a disease beyond its effect on someone's health. In practice, the Committees' ability to do this is limited. To do so routinely and consistently, the Committees need to measure the impact of an illness on a person's ability to interact with, and contribute to wider society.

26 The principal reason for incorporating wider societal impact into a technology appraisal is to provide a mechanism for the Appraisal Committees to consider a wider range of potential benefits of new technologies and, in doing so, to give preference to technologies developed for conditions that have the potential to restore the ability of individuals to contribute to society.

27 The effect of an illness on a person's ability to interact with society - the wider societal impact of a condition – can be captured through the concept of 'societal shortfall'. This shortfall is the loss in an individual's capacity to engage with society as a result of living with a disease or condition, compared with their capacity to engage with society without the illness.

28 Quality and length of life (measured in QALYs) can be used as indirect measures of societal shortfall. This can be achieved by using absolute QALY shortfall calculated over the remaining lifetime of the person with the condition. The higher the shortfall, the larger the effect.

Using 'wider societal benefit' as an alternative to wider societal impact

29 Wider societal benefit considers the impacts of a treatment on the rest of society. The concept of wider societal benefit has been explored by developing an analytical framework[6] that translates the health benefit of an

[6] The method used to calculate net impact on society employs existing data sources and commissioned studies to estimate the effect of treatments on the patient population's contribution and use of resources using information on patients': age, gender, ICD-code, and quality of life. The existing data sources used are, amongst others: the Annual Survey of Hours and Earnings (2011); the Time Use Survey (2000) - a government survey which reports data on how, on

intervention to the people being treated into an estimate of the net impact on society - which could be added to the value of the health benefits of the treatment when evaluating the overall value of the treatment. Impact in this context means the balance (or net effect) of the treatment on patients' contribution to society including, amongst other things, working in paid or unpaid employment, or taking care of someone (a child or a relative) and receiving something paid for or provided by society, such as informal care, social care or universal credit.

30 In this framework, wider societal benefits are measured by considering the difference between the amount of resources a person contributes to society as a consequence of their condition (production) and the amount they use (consumption), both without and with the new treatment.

31 For example, if a treatment enables a patient to return to work, they may pay more tax which benefits others in society, or they may be able to contribute more to family finances. A treatment can also benefit the rest of society even if the patients who receive it do not work. They might be able to contribute more to childcare or other domestic activities that benefit their family or others. A treatment could also mean patients need less care outside the NHS; for instance in residential care homes, or by family members. Reducing the need for care may free up resources that can be used to help others, or relieve the burden on family members.

32 It is also possible that treatments, which extend lives, while providing the benefit of extra life to patients (though without accompanying benefits in terms of improved quality of life), increase the burden on other members of

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average, people spend their time; the Survey of Carers in Households (2009); and the Health Outcomes Data Repository (reporting date not known). While this gives the best available estimate of wider societal benefit given existing information, it is also possible to use this approach with data on an individual technology's effects to calculate a more accurate estimate for the product in question. It also allows estimation of the net impact on society of the treatments that are displaced elsewhere in the NHS if funds are re-allocated to a new technology. See Appendix 2 for documentation explaining this approach in more detail.

society. For example, if a patient in residential care lives longer as a result of a treatment but their care need is not reduced, this may impose additional costs on social care budgets, which means that resources will not be available for others who may need them.

Which approach should be used?

33 The conceptual framework for wider societal benefit cannot comfortably be integrated into the methodology of a NICE technology appraisal without mitigations for some effects that are considered to be unacceptable to society. On average, people are at their most productive when they are young to middle aged and conventional measures of productivity are sensitive to gender. This is true even when taking account of caring and other unpaid roles. A possible mitigation for this would be to use average productivity measures across the genders. In addition, as wider societal benefit is a consequence of the difference between consumption and production, some patient characteristics will result in negative absolute wider societal benefits for patients whose condition means that they have to receive more from society than they are able to give back (notably where patients experience very poor quality of life, even with best available treatments). Where this is the case it is, of course, possible that new treatments could have either a positive or negative impact on wider societal benefits, as it is the net change in a patientgroup's wider societal benefits that would influence the treatment's valuation under this approach.

34 We consider that using the societal shortfall approach, where wider societal impact is considered by identifying the health lost by people as a result of their condition, fits more easily into the current methods of technology appraisal than using the wider societal benefit framework. Since loss of good health affects a person's ability to engage in society, societal shortfall can be assessed by measuring the absolute QALY loss. Using societal shortfall, the wider benefit for patients of the technology itself would be measured, as now, through its impact on changes in health status.

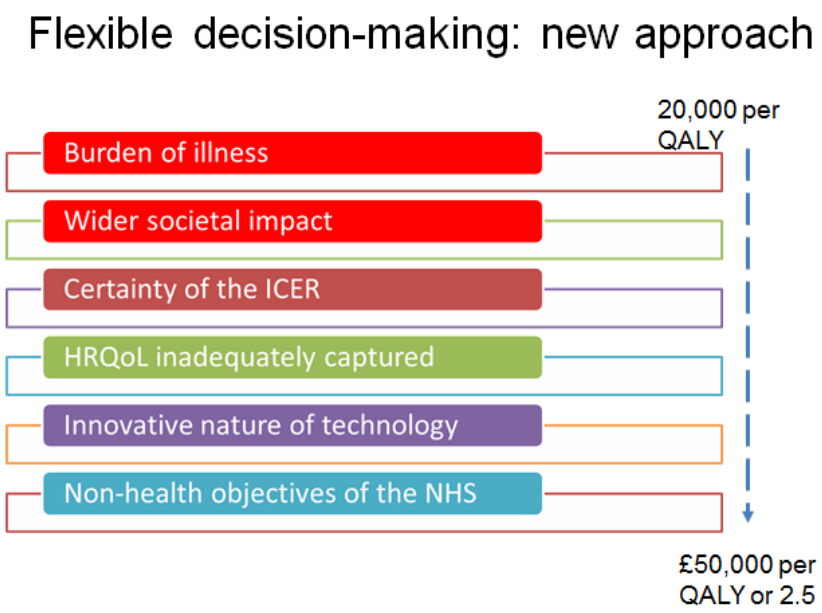
35 It is important to note that any approach that aims to capture wider societal benefit or impact will inevitably take age into account to some degree. However broadly the concept is drawn, our age has an impact on what we are able to contribute and what we take from society.

36 However, we want to make it clear in this consultation that our Appraisal Committees will not use the age of people with particular conditions as the basis for deciding whether or not the NHS should offer new treatments. Similarly, they will not use gender or any of the other 'protected' characteristics under equalities legislation. This is consistent with the Government's position which implemented a ban on age discrimination in October 2012[7]. Regardless of the way the proposals in this paper are incorporated into the appraisal process, we will ensure that age or any of the other protected characteristic will not tip the balance of a recommendation against the use of a treatment.

How we will apply the proposed changes

37 Subject to the outcome of consultation, we propose that proportional and absolute QALY loss values will be calculated as part of a technology appraisal and that they are used as the basis for assessing burden of illness and wider societal impact respectively. The Appraisal Committees will be asked to adopt a more favourable approach when considering treatments for people whose conditions have a progressively higher burden of illness and wider societal impact. These factors will be considered, alongside other modifiers referred to in paragraph 8, meaning that ICERs above £20,000 per QALY gained would be acceptable in circumstances where these factors are considered relevant and where absolute and proportional QALY shortfall is high. The way in which this will work is shown in the following illustration.

[7] "Implementing a ban on age discrimination in the NHS - making effective decisions" https://www.gov.uk/government/publications/implementing-a-ban-on-age-discrimination-in-the-nhs-making-effective-appropriate-decisions.



38 We need to place an upper limit on acceptability of an ICER, because valuable care elsewhere in the NHS might be displaced without at least an equally valuable gain being achieved. This holds true even when taking into account burden of illness and wider societal impact

39 The maximum weighting applied by the Appraisal Committees using their current flexibilities is 2.5 and this is normally only applied to end of life treatments. We believe this represents the maximum weighting that the Appraisal Committees should consider when taking into account the cumulative impact of all the modifiers, including burden of illness and wider societal impact. While this would not extend the upper limit of an acceptable ICER, it will broaden the circumstances in which an ICER above the £20,000 baseline might be acceptable.

40 The proposed changes to the current Guide to the Methods of Technology Appraisal which would need to be made as a consequence of the preferred approached to burden of illness and wider societal impact are set out in Appendix 4.

41 These proposals have been developed taking into account the need to ensure consistency, predictability and transparency in the value-judgements applied by the independent Appraisal Committees when they consider the clinical and cost effectiveness of health technologies.

Your views

42 We want to hear from anyone interested in how we appraise health technologies including patients, carers, patient groups, clinicians, academics, economists, industry and members of the public to help ensure that NICE's processes are robust and fair. The questions set out below might help to guide your response.

- **Does proportional QALY shortfall appropriately reflect burden of illness?**
- **Does absolute QALY shortfall provide a reasonable proxy for wider societal impact of a condition?**
- **Does a maximum weight of 2.5 in circumstances when all modifiers apply function as a reasonable maximum?**
- **Should we allocate specific 'weights' to each of the 'modifiers' so that they add up to a maximum of 2.5? If so, do you have a view on what weight should be added in each case?**
- **Will the approach outlined in this document achieve the proposed objectives of improving consistency, predictability and transparency in the judgements made by our independent Appraisal Committees when they consider the clinical and cost effectiveness of health technologies?**
- **Are there any risks which might arise as a result of adopting the value-based assessment approach as outlined above? If so, how might we try to reduce them?**

Appendix 1

Terms of Reference to NICE from the Department of Health

The methods for value assessment of branded medicines under VBP should:

- Be applied to medicines within the scope of the VBP system, and incorporated into the methods for other categories of guidance at NICE's discretion
- Adopt the same benefit perspective for all technologies falling within the scope of VBP, and for displaced treatments (1)
- Be as transparent and predictable as possible
- Be informed by the best available evidence
- Include a simple system of weighting for burden of illness that appropriately reflects the differential value of treatments for the most serious conditions (2)
- Encompass the differential valuation of 'End of Life' treatments in the current approach within the system of Burden of Illness weights
- Include a proportionate system for taking account of Wider Societal Benefits
- Not include a further weighting for Therapeutic Innovation and Improvement
- Produce guidance for patients and the NHS which describes the clinical and cost effectiveness of the technology and its position in clinical practice

(1) That is, the value of a new treatment is considered net of the value of what is displaced, and the valuation methodology is applied consistently across treatments, including where the net value impact in respect of an element of VBP may be negative
(2) For example, using a simple percentage weighting that is proportionate to the QALY loss suffered by patients with the condition
(3) The perspective adopted for measuring WIDER SOCIETAL BENEFITs should, in principle, be as set out in the HMT Green Book for Appraisal and Evaluation in Central Government - which specifies the cross-Government approach for evaluating costs and benefits of spending decisions. However in practice it will be important to reflect uncertainties in the evidence for the magnitude of WIDER SOCIETAL BENEFITs, the novelty of the approach, and the degree of consensus among stakeholders. Options may in practice include constraining the weight given to different elements of WIDER SOCIETAL BENEFITs in the valuation of treatments, or initially taking a selective approach to the types of benefit included in the assessment framework, in order to support incremental broadening of the value perspective. It will be important to ensure that the approach to incorporating WIDER SOCIETAL BENEFIT is applied systematically and consistently.
(4) To ensure that innovation is rewarded only when the technology's use brings extra value.

Appendix 2 List of supporting documents

- Department of Health – Briefing document for NICE Working Party 1 (available on www.nice.org.uk)

- Department of Health – Methodology for estimating 'Wider Societal Benefits' as the net production impact of treatments – briefing document for NICE Decision Support Unit (available on www.nice.org.uk)

- Department of Health - Using a Reference Dataset to support Value Assessment in VBP – briefing document for NICE Decision Support Unit (available on www.nice.org.uk)

- Illustrative list of Technology Appraisals and QALY shortfall - (summary pdf available on www.nice.org.uk / excel spreadsheet on request from Lynn.Woodward@nice.org.uk)

- Research Unit in Economic Evaluation of Health and Care Interventions (EEPRU) - Eliciting societal preferences for burden of illness, therapeutic improvement and end of life for value based pricing: a report of the main survey. EEPRU Research Paper 01/13 (available from: http://www.eepru.org.uk/Publications(2353189).htm)

- Research Unit in Economic Evaluation of Health and Care Interventions (EEPRU) - Review of reviews on social value of a QALY (Available on request from www.eepru.org.uk)

- NICE Decision Support Unit – Department of Health proposals for including Burden of Illness into Value Based Pricing: A Description and Critique - Briefing paper for NICE TA Methods Working Party (available on www.nice.org.uk), including specification.

- NICE Decision Support Unit – Department of Health proposals for including Wider Societal Benefits into Value Based Pricing: A Description and Critique - Briefing paper for NICE TA Methods Working Party (available on www.nice.org.uk), including specification.

- NICE Decision Support Unit - Incorporating wider societal benefits into estimates of cost per QALY: implications of Value Based Pricing for NICE (available from: http://www.nicedsu.org.uk/)

- NICE Decision Support Unit - Incorporating wider societal benefits into estimates of cost per QALY: case studies (available from: http://www.nicedsu.org.uk/)

- Office of Health Economics – Note on proportional versus absolute shortfall – Briefing Paper for NICE Working Party (available on www.nice.org.uk)

- NICE - Guide to the Methods of Technology Appraisal 2013 (available on www.nice.org.uk)

- NICE - Social Value Judgements; principles for the development of NICE guidance. Second edition. (available on www.nice.org.uk)

Appendix 3

NICE Methods Working Party

Professor Andrew Stevens (Chair)
Chair, Appraisal Committee and Professor of Public Health, University of Birmingham

Dr Amanda Adler
Chair, Appraisal Committee and Consultant Physician, Addenbrooke's Hospital

Lizzie Amis
Senior Public Involvement Adviser, Public Involvement Programme, NICE

Meindert Boysen
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Nick Bruce
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Professor Martin Buxton
Professor of Health Economics, Brunel University

Dr Paul Catchpole
Value & Access Director, Association of the British Pharmaceutical Industry

Ben Cavanagh
Senior Policy Officer, Prostate Cancer UK

Claire Cheong-Leen
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Dr Sarah Garner
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Sally Greenbrook
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Dr Pall Jonsson
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Helen Knight
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Professor Carole Longson
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Honorary Professor of Clinical Decision Science, University of Sheffield

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Dr Kay Nolan
Associate Director, Centre for Public Health, NICE

Professor Stephen Palmer
Professor of Health Economics, University of York

David Pearce
Director, Health Outcomes Policy, GSK

Seren Phillips
Associate Director, Scientific Advice, NICE

Simon Reeve
Head of Clinical and Cost-effectiveness Medicines, Pharmacy and Industry Group Strategy, Finance and NHS Directorate, Department of Health

Karen Samuels
Head of HTA and Medicines Management, Programme Director, All Wales Therapeutics and Toxicology Centre

Professor Ken Stein
Professor of Public Health, Peninsula College of Medicine and Dentistry, University of Exeter

Professor Matt Stevenson
Reader in Health Technology Assessment, University of Sheffield

Dr Paul Tappenden
Reader in Health Economic Modelling, University of Sheffield

Paul Trueman
VP Market Access, Healthcare Systems, Smith & Nephew

David Wonderling
Head of Health Economics, Royal College of Physicians

The following attended 1 or more meeting on behalf of a working party member:

Dr Robert Bracchi
Chairman, New Medicines Group, All Wales Therapeutics and Toxicology Centre

Ailsa Brown
Principal Health Economist, Scottish Medicines Consortium

Warren Cowell
HTA Policy, Pfizer

Lee Marriott Dowding
Team Leader, Patient Support, James Whale Fund for Kidney Cancer

Gordon Lundie
Director of Market Access and Government Affairs, UCB

Judith Mellis
Senior Manager, UK Market Affairs, Association of British Healthcare Industries

Chris O'Regan
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Danny Palnoch
Head of Medicines Analysis, Medicines Pharmacy and Industry, Department of Health

Dr Catherine Swann
Associate Director, Centre for Public Health, NICE

Specialist advisers to the working party

Professor John Brazier
Professor of Health Economics, University of Sheffield

Professor John Cairns
Professor of Health Economics Public Health and Policy, London School of Hygiene and Tropical Medicine

Professor Karl Claxton
Professor of Health Economics, University of York

Dr Gavin Roberts
Economic Adviser, Medicines Pharmacy and Industry, Department of Health

Marta Soares
Research Fellow, University of York

Dr Aki Tsuchiya
Faculty of Social Sciences Advanced Research Fellow, University of Sheffield

Professor Adrian Towse
Director, Office of Health Economics

Professor Allan Wailoo
Professor of Health Economics, University of Sheffield

Appendix 4 Proposed changes to the Guide to the Methods of Technology Appraisal

43 The sections that have been amended to incorporate the aspects of Value Based Assessment are described below.

Relevant current text of the Guide to Methods of Technology Appraisal 2013, with amendments underlined, or deleted, where appropriate.

5.4 Equity considerations in cost-effectiveness analysis
5.4.1 In the reference case, an additional QALY should receive the same weight regardless of any other characteristics of the people receiving the health benefit.
5.4.2 The estimation of QALYs, as defined in the reference case, implies a particular position regarding the comparison of health gained between individuals. Therefore, in the reference case, an additional QALY is of equal value regardless of other characteristics of the individuals, such as their socio-demographic characteristics, their age, or their level of health. The Committee has discretion to consider a different equity position, and may do so in certain circumstances and when instructed by the NICE Board (see section 6).

Relevant current text of the Guide to Methods of Technology Appraisal 2013, with amendments underlined, or deleted, where appropriate.

5.1.7 For the reference case, the perspective on outcomes should be all direct health effects, whether for patients or other people. The perspective adopted on costs should be that of the NHS and personal and social services.
5.1.8 The reference-case perspective on outcomes aims to maximise health gain from available healthcare resources. Some features of healthcare delivery often referred to as 'process characteristics' may ultimately have health consequences, for example, mode of treatment delivery through its impact on adherence. If characteristics of healthcare technologies have a value to people independent of any direct effect on health, the nature of these characteristics should be clearly explained and if possible the value of the additional benefit should be quantified. These characteristics may include convenience and the level of information available for patients.
5.1.9 The Institute does not set the budget for the NHS. The appropriate objective of the Institute's technology appraisal programme is to offer guidance that represents an efficient use of available NHS and personal social services resources. For these reasons, the reference-case perspective on costs is that of the NHS and personal social services.
5.1.10 Some health technologies may have substantial benefits to other government bodies (for example, treatments to reduce drug misuse may have the effect of reducing crime). These issues should be identified during the scoping stage of an appraisal. ~~Appraisals that consider benefits to the government incurred outside of the NHS and personal social services will be agreed with the Department of Health (and other relevant government bodies as appropriate) and detailed in the remit from the Department of Health and the final scope.~~ For these non-reference-case analyses the benefits and costs (or cost savings) should be presented separately from the reference-case analysis. Productivity costs are not included in ~~either~~ the reference-case ~~or non-reference-case analyses~~.

Relevant current text of the Guide to Methods of Technology Appraisal 2013, with amendments underlined, or deleted, where appropriate.

6.2.9 In the reference case, the Committee will regard all QALYs as being of equal weight. However, when considering the overall ~~health~~ benefits, the Appraisal Committee ~~can accept analysis~~ should consider analyses ~~analyse~~ that explore~~s~~ a QALY weighting that is different from that of the reference case when ~~a technology appraisal concerns a 'life-extending treatment at the end of life'~~ the 'burden of illness' and 'wider societal impact' of the condition to which the (proposed) licensed indication refers is greater than that of the average displaced treatments in the NHS, or in other circumstances detailed below and when instructed by the NICE board.

~~6.2.10 In the case of a 'life-extending treatment at the end of life', the Appraisal Committee will satisfy itself that all of the following criteria have been met:~~

~~□ the treatment is indicated for patients with a short life expectancy, normally less than 24 months and~~

~~□ there is sufficient evidence to indicate that the treatment offers an extension to life, normally of at least an additional 3 months, compared with current NHS treatment and~~

~~□ the technology is licensed or otherwise indicated, for small patient populations normally not exceeding a cumulative total of 7000 for all licensed indications in England.~~

~~In addition, the Appraisal Committees will need to be satisfied that:~~

~~□ the estimates of the extension to life are robust and can be shown or reasonably inferred from either progression-free survival or overall survival (taking account of trials in which crossover has occurred and been accounted for in the effectiveness review) and~~

~~□ the assumptions used in the reference case economic modelling are plausible, objective and robust.~~

~~□ the impact of giving greater weight to QALYs achieved in the later stages of terminal diseases, using the assumption that the extended survival period is experienced at the full quality of life anticipated for a healthy individual of the same age and~~

~~□ the magnitude of the additional weight that would need to be assigned to the QALY benefits in this patient group for the cost effectiveness of the technology to fall within the normal range of maximum acceptable ICERs.~~

~~6.2.12 Treatments approved following the application of the 'end-of-life' criteria listed in section 6.2.10 will not necessarily be regarded or accepted as standard comparators for future appraisals of new treatments introduced for the same condition. Second and subsequent extensions to the marketing authorisations for the same product will be considered on their individual merits.~~

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6.2.10 ‘Burden of illness’ is defined as the amount of future health lost (shortfall) for people living with the condition that is subject to the proposed licensed indication, or the (sub)population for which NICE guidance is sought, measured in QALYs lost, in comparison with expected future QALYs if they did not have the condition, calculated from the moment of intervention with the technology of interest.
6.2.11 ‘Wider societal impact’ is defined as the loss (shortfall) in an individual’s capacity to engage with society as a result of living with the condition that is subject to the proposed licensed indication, or the (sub) population for which NICE guidance is sought, compared with the capacity to engage with society without the condition, calculated from the moment of intervention with the technology of interest.
6.2.12 In considering burden of illness and wider societal impact, the Appraisal Committee will review the ‘absolute’ QALY shortfall and the relative, or ‘proportional’, QALY shortfall compared with the expected future QALYs without the condition.

Relevant current text of the Guide to Methods of Technology Appraisal 2013, with amendments underlined, or deleted, where appropriate.

Structured decision-making: non-health factors

6.2.20 In general the Committee uses the most plausible ICER as the primary consideration when making judgements about the acceptability of technologies as a cost-effective use of NHS resources. However, its overall conclusions are also affected by the following additional considerations:

- Whether or how its judgements have a bearing on broader social considerations to the extent that these are covered by NICE's principles on social value judgements
- Whether a substantial proportion of the costs (savings) or benefits are incurred outside of the NHS and personal and social services, or are associated with significant benefits other than health~~, only when requested specifically by the Department of Health as part of the remit~~.

6.2.21 The concept that underlies the Committee decision-making is that of the opportunity cost of programmes that could be displaced by the introduction of new technologies. This way, NICE seeks to maximise the health benefit gained from a fixed NHS budget. This principle is correct if the sole purpose of the health service is to improve health. While this may be the primary purpose of the NHS, it is acknowledged that care delivered by the NHS could have other benefits that are considered socially valuable but are not directly related to health and are not easily captured in a cost per QALY analysis. Techniques exist to consider the trade-off between health benefits and non-health benefits quantitatively. These techniques require that all relevant criteria are identified in advance, quantified and then weighted to reflect aspects of social value in a way that can be regarded as legitimate by all stakeholders. At present the introduction of such techniques into the Committee's decision-making is considered unsuitable. ~~Therefore the Committee will take non-health objectives of the NHS into account by considering the extent to which society may be prepared to forego health gain in order to achieve other benefits that are not health related.~~ As indicated above, the Committee will take into account the shortfall in the capacity to engage with society resulting from the condition that is subject to the proposed licensed indication, or the (sub) population for which NICE guidance is sought.

Relevant current text of the Guide to Methods of Technology Appraisal 2013, with amendments underlined, or deleted, where appropriate.

6.3.1 The Appraisal Committee does not use a precise maximum acceptable ICER above which a technology would automatically be defined as not cost effective or below which it would. Given the fixed budget of the NHS, the appropriate maximum acceptable ICER to be considered is that of the opportunity cost of programmes displaced by new, more costly technologies. ~~NICE does not have complete information about the costs and QALYs from all competing healthcare programmes in order to define a precise maximum acceptable ICER. However, NICE considers that it is most appropriate to use a range as described in sections 6.3.2 to 6.3.5. Furthermore,~~ Consideration of the cost effectiveness of a technology is a necessary, but is not the sole, basis for decision-making. Consequently, the Institute considers the value of technologies in relation to ~~this range of maximum acceptable~~ the most plausible ICER~~s, such that the influence of~~ and other factors ~~upon~~, described below which influence the decision to recommend a technology ~~is greater~~ when the ICER is higher than £20,000 per QALY gained ~~closer to the top of the range~~.

6.3.2 Below a most plausible ICER of £20,000 per QALY gained, the decision to recommend the use of a technology is normally based on the cost-effectiveness estimate and the acceptability of a technology as an effective use of NHS resources. When the estimated ICERs presented are less than £20,000 per QALY gained and the Committee judges that particular interventions should not be provided by the NHS, the recommendations will make specific reference to the Committee's view on the plausibility of the inputs to the economic modelling and/or the certainty around the estimated ICER. This might be affected, for example, by sensitivity analysis or limitations to the generalisability of findings regarding effectiveness.

6.3.3 Above a most plausible ICER of £20,000 per QALY gained, judgements about the acceptability of the technology as an effective use of NHS resources will specifically take account of the following factors:

- ~~The technology meets the criteria for special consideration as a 'life-extending treatment at the end of life'~~ The degree to which the 'burden of illness' of the condition and 'wider societal impact' of the condition, in terms of both proportional and absolute shortfall, to which the (proposed) licensed indication refers is greater than that of the displaced treatments in the NHS (see section 6.2.10).
- The degree of certainty around the ICER. In particular, the Committee will be more cautious about recommending a technology for routine use when they are very uncertain ~~less certain~~ about the ICERs presented.
- Whether there are strong reasons to indicate that the assessment of the change in health-related quality of life has been inadequately captured, and may therefore misrepresent the health utility to be gained.
- The innovative nature of the technology, specifically if the innovation adds demonstrable and distinctive benefits of a substantial nature which represent a step change in the management of the condition and may not have been or cannot be adequately captured in the reference case QALY measure.
- Aspects that relate to non-health objectives of the NHS (see sections 6.2.20 and 6.2.21)

6.3.4 As the ICER of an intervention increases in the range of £20,000 to £30,000 per QALY gained, the Committee's judgement about the acceptability of the technology as an effective use of NHS resources will make explicit reference to the relevant factors listed in section 6.3.3.
6.3.5 Above a most plausible ICER of £30,000 per QALY gained, the Committee will need to identify an increasingly stronger case for supporting the technology as an effective use of NHS resources, with regard to all of the factors in section 6.3.3.
6.3.6 The QALY weighting to be applied when considering the strength of the factors listed in 6.3.3 is no more than 2.5.