Consultation questions

1 Does proportional QALY shortfall appropriately reflect burden of illness?

It is unclear how burden of illness (BOI) as measured in this way can comfortably fit within the current NICE process. Since the QALY methodology is already a key part of the initial cost-effectiveness analysis – and the QALY is based on information about values and preferences provided by patients – there appears to be an issue of double counting. We do not see how this would add value to the existing process. The process will become less reflective of the value of new medicines, as well as more difficult for non-experts to understand.

Proportional QALY shortfall does appear to reflect the severity of an illness – while it is not particularly sensitive to age, it is sensitive to health-related quality of life (HRQoL). The more severe a disease, the higher the QALY loss and therefore the proportional QALY shortfall is generally higher.

There may be reason to believe that HRQoL is not always wholly captured within every appraisal – there could be further consideration of how well instruments such as the EQ-5D are able to standardise across different disease types, and how much patients taking part in trials understand how EQ-5D questionnaires will be used. Additionally, every patient is unique and their varied preferences will always represent a barrier to standardising across groups. If proceeding with this model, further consideration could be given to how much weight BOI is given earlier in the NICE technology appraisal process, as this is clearly a crucial factor in making QALY calculations and therefore determining cost-effectiveness. It should be more than an afterthought in decision-making.

We believe it is vital to ensure that the health effects of a treatment are properly measured and that the data captured for use in technology appraisals genuinely reflects both the severity of an illness and health improvements.
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<td>2 Does absolute QALY shortfall provide a reasonable proxy for wider societal impact of a condition?</td>
<td>No. While we accept that NICE has been asked to find a standard measure for wider societal impact (WSI), we cannot support the concept that an absolute loss of healthy life years – which is sensitive to age – could become a key factor in deciding whether some treatments are made routinely available within the NHS in England. NICE has a legal obligation to not discriminate against groups of patients based on protected characteristics such as age. While we believe that extremely severe conditions should be treated as highly important by NICE, it has not been clearly articulated how absolute QALY shortfall – which is sensitive to both severity and age – can comfortably align with NICE’s obligations. The consultation as it stands provides no guidance on how QALY shortfall calculations would be used in decision-making – if we do not know what ‘tolerance’ the Committees would have (e.g. a shortfall of 40 would be considered important, but a shortfall of 35 would be too low) and how this would be weighted it is very difficult to express an informed view. We note that independent committee members themselves have not yet received this guidance. Additionally, concerns have been expressed about the ability of the measure to act as a proxy for wider societal impact. As currently proposed, this appears to consider QALY loss only rather than broader factors such as self-care and reduction of morbidity or disability which can have a large bearing on the impact patients are able to have on wider society. Absolute QALY shortfall is a concept that is extremely difficult for many people, including those that are familiar with the NICE process, to accept as being equivalent to the value individuals are able to add to society. We would urge NICE to give further consideration to the inclusion of this modifier into the technology appraisal process, and to carry out further work to define wider societal impact.</td>
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<td>3 Does a maximum weight of 2.5 in circumstances when all modifiers apply function as a reasonable maximum?</td>
<td>See response to question 4.</td>
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<td>4 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</td>
<td>Flexibility within the NICE process is important to ensure that all relevant evidence can be considered which does not easily fit into the earlier stages of the process. The modifiers outside BOI and WSI are all broad in nature – for example, HRQoL inadequately captured could have a variety of implications, as could evidence around non-health objectives of the NHS. It would be extremely difficult to weight these to anticipate all possible eventualities, and would add unhelpful rigidity to the NICE process. No evidence has thus far been provided to suggest that specific modifiers should be weighted differently. Furthermore, there is a potential that some modifiers might be more relevant to certain appraisals and others to different appraisals. We would urge NICE to guard against this having the effect of drugs of certain classes, or for specific disease areas,</td>
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being favoured in the NICE process more than others.

Furthermore, it is crucial that NICE provides clear guidance, developed with reference to the views of the wider public, as well as clinical and patient communities, to the decision-making Committees.

5 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?

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<td>5</td>
<td>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?</td>
<td>No. As discussed, the modifiers are by definition used at the Committee’s discretion and the consultation document has not outlined how these would be employed in a technology appraisal. Some level of flexibility in the NICE process is welcome, but more information about how flexible decision making works in practice is needed to aid consistency, predictability and transparency. The QALY shortfall approach is likely to make the technology appraisal process less transparent. Absolute and proportional shortfall can both be given a clear numerical measure; however if arbitrary decisions are made about the level of tolerance Committees will accept then it will be more difficult for external organisations and individuals to understand the process. The current modifiers can sometimes involve levels of uncertainty – there may be debate around levels of innovation represented by a new technology, for example. But the new modifiers can be one of many numbers, so it is particularly important to understand how these number ranges impact on outcomes of NICE appraisals. This is extremely important to achieving reasonable levels of predictability in appraisals.</td>
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6 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?

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<td>6</td>
<td>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?</td>
<td>As discussed above, both measures of proportional and absolute QALY shortfall are problematic in that the additional benefits they would add to the technology appraisal process are not clear. Furthermore, we are concerned that the proposed measure for WSI would disproportionately take patient age into account. The lack of information about how the new modifiers will be used is extremely concerning. It is important to understand this to provide an informed view of the impact of the proposals in the consultation. The development by NICE of guidance around use of the new modifiers must involve patients, clinicians and the public. As discussed in our further comments below, an opportunity for a wider reconsideration of the policy framework around access to drugs has been missed.</td>
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7 Are there any other comments you wish to make?

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<td>7</td>
<td>Are there any other comments you wish to make?</td>
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Cancer Research UK is the world’s largest independent cancer charity dedicated to saving lives through research. We support research into all aspects of cancer through the work of over 4,000 scientists, doctors and nurses. In 2013/14 we spent £386 million on research. We are a leading funder of clinical research in the UK and have supported the development of over 140 novel anti-cancer agents.

We welcome the opportunity to respond to this consultation. We have consulted with both patients and clinicians, including the National Cancer Research Institute (NCRI) Clinical Studies Groups, the NCRI Consumer Liaison Group, and a small focus group of patients. Their comments were taken into account in formulating this response.

1. Cancer Research UK position

- Cancer Research UK believes that all cancer patients should be able to access the best, evidence-based treatments that are available for their condition.

There is overwhelming evidence of patient and clinician demand for more options to help treat people with cancer.

A number of oncology drugs have failed to receive a positive NICE recommendation since its establishment in 2000 – NICE’s calculus suggests that around 32% of appraisals for cancer drugs have produced a negative recommendation, and that this trend has increased in recent years. We believe that the quality of new treatment options is paramount, and that NICE must be focused on ensuring that the best treatments are made available ahead of simply increasing treatment options. Nevertheless, the knowledge that individual patient’s respond very differently to different treatment options means that increasing the range of options is also a desirable outcome. However, the methods used to judge the best treatments must be intellectually robust and defensible to clinicians, patients, the public and policy makers.

- NICE processes must be flexible enough to give appropriate consideration to complex diseases.

Treatments for complex diseases such as cancer are recognised as facing increased difficulties within NICE technology appraisals, often because data on clinical and cost-effectiveness may be less well developed or more uncertain than better-understood disease areas.

- The opportunity to move towards creating a long-term framework may have been missed.
The current Government committed to introducing value-based pricing and reforming NICE in 2010. The broader implication of this was that current structures for the pricing and setting of clinical access policy for high-cost treatments are not effectively supporting access to drugs that clinicians and patients believe are valuable - or that their ability to do this is in question. The current consultation may not fully address this debate.

- **NICE should work to better articulate the benefits of the value-based assessment proposals in their current form, as the measures chosen for Burden of Illness and Wider Societal Impact are difficult to recognise as proxies for these two concepts.**

The new Burden of Illness and Wider Societal Impact modifiers do not appear to be measured in a way which genuinely takes these concepts into account and the latter may be unfairly weighted towards average patient age. While both concepts are understandably difficult to capture through a quantitative approach, we are not confident that the QALY shortfall approach illustrated in the proposals act as appropriate proxies for these. The QALY methodology captures patient preferences, so the proposed calculus still does not capture much beyond the benefit to individual patients.

- **The added implication of ageism brought about by the Wider Societal Impact modifier – which is not a concern in the current NICE process – is unwelcome. NICE must provide further information on how the guidance it provides will ensure older groups of patients are not unfairly disadvantaged by the system.**

This could include further modelling, beyond the experimental data published alongside the consultation, once it has been determined how the Committees will use the modifier. There has been considerable public attention on this element of the proposals since they were published.

Increasing numbers of people in the UK are living longer than ever before, and making active contributions to society in later life. Many cancers occur primarily in later life, and we would want all patients to have appropriate access to treatments to help them to fight the disease. There is already evidence to suggest that elderly cancer patients have disproportionately lower cancer survival rates in the UK compared with other countries.

- **NICE should work to clarify the impact the proposed changes will have on access to treatments.**

Modifiers used by NICE have always allowed for discretionary flexibility to the basic process, so it is difficult to see how the addition of new modifiers (and removal of the end of life modifier) would have a practical impact. Clarity around this is essential to ensure that the NICE process is transparent – particularly to decision-making committees - and that the implications for conditions where the average patient tends to be older can be considered.

- **We believe that a long-term, robust framework is needed to ensure that patients can access the drugs that would benefit them.**

This includes proportionate mechanisms to consider the clinical and cost-effectiveness of all forms of treatment, as well
as better national mechanisms to monitor (1) what treatments patients are accessing in the NHS; and (2) what impact these treatments are having on outcomes, so that this understanding can support accepted guidance or trigger a review.

- The Government should initiate a wider discussion of improving policies around access to drugs, including individual funding requests, the prevalence of off-label prescribing, and the Cancer Drugs Fund.

There is also a clear need to better understand cost-effectiveness of care across pathways – in cancer this means reflecting the value of diagnostics, imaging, supportive medicines, and other curative treatments such as surgery and radiotherapy. Further priority should be given by the Government and NHS England to analysis of patient outcomes from use of the Cancer Drugs Fund.

A cohesive policy framework would be preferable to one that is fragmented through multiple groups, including local decision makers, NICE and NHS England through the CRG structures and Cancer Drugs Fund panel, determining clinical access policies around cancer drugs. The above discussion would involve these stakeholders as well as counterparts in Wales and Northern Ireland, where the public is also affected by NICE guidance.

The Cancer Drugs Fund (CDF) was established in 2011 in England in part to respond to this, and the broader issue that patients in the UK were shown to have worse access to cancer treatments than in comparable nations across the world. The CDF is guaranteed until the end of March 2016, and sits separately from NICE technology appraisals and the Individual Funding Request process. To date, there has been no published evaluation of the overall incremental clinical benefit provided by drugs funded through the CDF.

Please email this form to: 2014VBAmethods@nice.org.uk

Closing date: Friday 20 June 2014 5pm

PLEASE NOTE: NICE reserves the right to summarise and edit comments received during consultations, or not to publish them at all, where in the reasonable opinion of NICE, the comments are voluminous, publication would be unlawful or publication would be otherwise inappropriate.