

National institute for Health and Clinical Excellence

Appraising life-extending, end of life treatments

1. Summary

- 1.1 This document sets out supplementary advice to the Appraisal Committees, to be taken into account when appraising treatments which may be life-extending for patients with short life expectancy, and which are licensed for indications affecting small numbers of patients with incurable illnesses. The additional advice will apply when such treatments have an incremental cost effectiveness ratio (ICER) in excess of the upper end of the range normally approved by the Appraisal Committees, using the 'reference case' outlined in the Institute's *Guide to the Methods of Technology Appraisal*, and which may offer demonstrable survival benefits over current NHS practice.
- 1.2 The current appraisal methodology recognises that there will be circumstances in which it may be appropriate to recommend the use of treatments with high reference case incremental cost effectiveness ratios. It states (with reference to the Institute's standard appraisal criteria) that: *'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.'* The Appraisal Committee has, in the past, made recommendations above the normal threshold range when it has explicitly identified additional benefits not readily captured in the reference case. This has occurred when the treatment involved has been life-extending, licensed or otherwise indicated for small populations with incurable illnesses.
- 1.3 In developing this supplementary advice, the Institute has taken account the Appraisal Committees' previous decisions, together with the relevant principles in the guide to the use of Social Value Judgements. It has also had regard to the consideration given by the Citizens Council, at its meeting in November 2008, to the circumstances in which it might be appropriate to support the use of treatments outside the Institute's cost per quality adjusted life years (QALY) threshold range. In addition, the Institute has taken account of its responsibility to recognise the potential for long term benefits to the NHS of innovation. In this context, it considers it appropriate for its Appraisal Committees to have regard to the importance of supporting the development of innovative treatments that are anticipated to be licensed for small groups of patients who have an incurable illness.
- 1.4 The objective of this supplementary advice is to ensure that the Appraisal Committees fully consider all the benefits which it is appropriate to take into account in appraising treatments designed to extend life, at the end of life for small populations and in particular to

ensure that where benefits are not, or not adequately captured in the reference case, that the Appraisal Committees are provided with an appropriate supplementary analysis. For this supplementary advice to be applied, a treatment will need to have been through an appraisal by NICE where the most plausible reference case point estimate for the ICER exceeds the upper end (£30,000) of the range normally considered by the Appraisal Committees to represent a cost effective use of NHS resources. Each candidate treatment will also need to meet the criteria set out in section 2.

- 1.5 The Institute will normally recommend to the Department of Health that it should give consideration to a data collection exercise for treatments recommended for use on the basis of the criteria set out in section 2. The purpose of this will be to assess the extent to which the anticipated survival gains are evident when the treatments involved are used in routine practice. The outcome of this exercise will be evaluated when the guidance for that treatment is reviewed.

2 Criteria for appraisal of end of life treatments

- 2.1 This supplementary advice should be applied in the following circumstances and when all the criteria referred to below are satisfied:
 - 2.1.1 The treatment is indicated for patients with a short life expectancy, normally less than 24 months and;
 - 2.1.2 There is sufficient evidence to indicate that the treatment offers an extension to life, normally of at least an additional 3 months, compared to current NHS treatment, and;
 - 2.1.3 No alternative treatment with comparable benefits is available through the NHS, and;
 - 2.1.4 The treatment is licensed or otherwise indicated, for small patient populations.
- 2.2 When the conditions described in 2.1 are met, the Appraisal Committee will consider:
 - 2.2.1 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;
 - 2.2.2 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 current threshold range.

- 2.3 In addition, the Appraisal Committees will need to be satisfied that:
- 2.3.1 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 cross-over has occurred and been accounted for in the effectiveness review).
and;
 - 2.3.2 The assumptions used in the reference case economic modelling are plausible objective and robust.

3 Review of the resulting guidance

- 3.1 The guidance produced using these criteria will be subject to review in accordance with the Institute's current arrangements. The review will normally take place no later than 2 years after the guidance has been issued. The review can be either brought forward or delayed, depending on the outcome of any data collection exercise or the availability of other new evidence.
- 3.2 Treatments approved following the application of the supplementary advice will not necessarily be regarded or accepted as standard comparators for future appraisals of new treatments introduced for the same condition. Second and subsequent licences for the same product will be considered on their individual merits. The Appraisal Committee will take into account the cumulative population for each product in considering the strength of any case, for justifying decisions which employ, in whole or part, the supplementary criteria outlined above

4 Implementation and evaluation

- 4.1 This supplementary advice will be effective from 5 January 2009.
- 4.2 The Institute intends to ensure that this supplementary advice is robust for the long-term and that it achieves its intended purpose. It will therefore be subject to a methodological evaluation. The Institute will design and manage this evaluation, the results of which will be published and used to make modifications to the supplementary advice, if necessary.