NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Briefing paper for methods review workshop on patient evidence 1: making the most of patient-based evidence and patient and public involvement

The briefing paper is written by Dr Sophie Staniszewska in collaboration with members of the Institute's Technology Appraisals team. It is intended to provide a brief summary of the issues that are proposed for discussion at a workshop to inform an update to the Institute's Guide to Methods of Technology Appraisal. It is not intended to reflect a comprehensive or systematic review of the literature. The views presented in this paper are those of the authors and do not reflect the views of the Institute.

The briefing paper is circulated to people attending that workshop. It will also be circulated to the members of the Method's Review Working Party, the group responsible for updating the guide.

For further details regarding the update of the Guide to the Methods of Technology Appraisal please visit the NICE website at http://www.nice.org.uk/aboutnice/howwework/devnicetech/technologyappraisallprocessguides/GuideToMethodsTA201112.jsp

1 Review of the 'Guide to Methods of Technology Appraisal'

The Institute is reviewing the 'Guide to the methods of technology appraisal', which underpins the technology appraisal programme.

The original Methods Guide was published in February 2001, and a revised version was published in 2007. The Methods Guide provides an overview of the principles and methods used by the Institute in assessing health technologies. It is a guide for all organisations considering submitting

evidence to the technology appraisal programme and describes appraisal methodology.

The current 'Guide to methods of technology appraisal' is available from the NICE website at http://www.nice.org.uk/aboutnice/howwework/devnicetech/technologyappraisalprocessguides/guidetothemethodsoftechnologyappraisal.jsp

The review of the Methods Guide will take place between October 2011 and April 2012. As part of the process, a number of workshops will be held to help identify those parts of the Guide that require updating. These workshops will involve a range of stakeholders, including methods experts, patient representatives, industry representatives, NHS staff and NICE technology appraisal committee members.

A summary of the discussion at the workshop will be provided to the Methods Review Working Party, the group responsible for preparing the draft update of the Methods Guide. Further details of the process and timelines of the review process are available from the NICE website.

The revised draft of the Methods Guide will be available for a 3-month public consultation, expected to begin in May 2011. We encourage all interested parties to take part in this consultation.

2 Background

The current 'methods guide' states the following (see section 4.3):

> *"Submissions are invited from all patient/carer groups involved in the appraisal. Patient evidence can include the views, assessments and evaluations of: individual patients, individual carers, groups (such as groups of patients, carers or voluntary organisations that represent patients). Patient evidence refers to any information originating from patients and/or carers that may inform the appraisal of a technology. […]*

There are two principal reasons for presenting patient evidence. Patients and carers are a unique source of expert information about the personal impact of a disease and its treatment, which can help set the correct scope for the assessment of the evidence and enable the realistic interpretation of the clinical and economic data as the appraisal progresses. Patient evidence can identify limitations in the published research literature; in particular, the failure to capture the true concerns of individual patients related to HRQL over and above measurements using standardised instruments (such as questionnaires) developed using psychometric techniques.

For the purpose of informing its technology appraisals, the Institute is looking for a concise and balanced overview that reflects the range of patient and carer perspectives. Two groups of experts – clinical specialists and patient experts – are selected by the Committee Chair from nominations provided by (non-manufacturer) consultees and commentators. Clinical specialists and patient experts provide written evidence and attend the Committee meeting to help in the discussion of the technology being appraised."

Section 4.5 gives further guidance to people attending committee meetings as experts:

"The experts attending the Committee meeting are asked to submit, in advance, a brief written personal view of the current management of the condition, the (expected) role of the technology and its use in the NHS, as well as to provide oral commentary during the meeting. The purpose of the oral commentary provided by the experts is to explore the evidence that is provided in the written submissions from consultees. During the open part of the meeting, clinical specialists and patient experts are encouraged to interact fully in the debate with the Committee, including responding to and posing questions. The clinical specialists and patient experts are asked to withdraw from the meeting before the Committee discusses the content of the guidance.

Views expressed orally by the experts at the Committee meeting can usefully inform the debate in a variety of ways, including the following.
- Identifying important variations in clinical practice in both the management of the condition in general and specifically in the current use of the technology. […] - Giving personal perspectives on the use of the technology and the difficulties encountered, including the important benefits to patients and the range and significance of adverse effects as perceived by patients. - Providing views on the nature of any rules, informal or formal, for starting and stopping use of the technology. This might include the requirement for additional analysis: to identify appropriate subgroups of patients for treatment with the technology, to assess response to treatment and the potential for discontinuation.
- Responding to queries that arise from: the lead team presentation (the lead team being two Committee members who make a brief presentation to introduce the topic of the appraisal), issues raised by the Chair and other Committee members, issues raised by other experts.

A lead team, selected from the Committee members at the start of each STA, helps the NICE technical lead prepare a summary of the evidence, known as the premeeting briefing. One of the lay representatives on the Committee is also selected to advise the lead team when developing the premeeting briefing. At the Appraisal Committee meeting, the lead team makes a brief presentation, based on the premeeting briefing, to introduce the STA topic.

The ‘lay lead’ role was designed to further develop the role of the 12 lay members on the Technology Appraisals Committees. When starting this lay lead process, two main areas of potential impact were proposed: increasing lay member involvement with the work of the committee, and increased visibility of patient/carer evidence. The three lay members per committee take it in turns to be the lay lead, with one of them being assigned to every topic. They advise the lead team about the key patient, carer and public issues and evidence within the committee topic documentation. This helps ensure that these issues and evidence are explicitly referred to in the presentations given at the start of the committee meeting.

NICE Patient Experience Guidance

The NICE Patient Experiences Guidance will be published in 2011. A scoping study of patient experiences was carried out as part of this work, to identify key generic dimension of patient experience that apply to all patients (Staniszewska et al 2011, in review). This scoping study, which was included in an appendix in the NICE Consultation on this Guidance, may provide a helpful context for discussions about the dimensions of experience that can be considered in technology appraisal.

3 Proposed issues for discussion

From the description in the current methods guidance it is clear that NICE Technology Appraisal Committees consider a variety of patient-based evidence.

This workshop will focus on exploring whether current processes of technology appraisal are maximising the potential for using patient-based evidence and the potential for patient and public involvement in the identification, synthesis and interpretation of patient-based evidence. This paper provides some context for this discussion and considers the concept of patient-based evidence and the levels of patient and public involvement.

3.1 The concept of patient-based evidence

The conceptual framework drawn on to inform this discussion includes clinical evidence, economic evidence and patient-based evidence (Staniszewska et al 2010, Rycroft-Malone 2004, Doll 1974). Patient-based evidence includes qualitative and quantitative forms of evidence, such as studies that have used qualitative methods to explore patient experiences, surveys that have attempted to measure different dimensions of patient experiences. Patient-based evidence can also include patient-reported outcomes (PROs) with measures patients' assessments of their health status and well-being (Staniszewska 2010).

Compared to clinical and economic forms of evidence, patient-based evidence is less well defined conceptually and methodologically. This makes it more difficult to integrate automatically with the clinical and economic forms of data, as there are few agreed frameworks to facilitate this process, although some research has started to examine the possibilities (McInnes et al 2011). In the absence of ready-made frameworks and methods, the role and contribution of patient-based evidence needs to be carefully considered within Technology Appraisal to ensure the benefits of this form of evidence are maximised.

The synthesis of qualitative data will be considered more fully in Ruth Garside's presentation. There are also issues around the synthesis of experiences data with quantitative experiences data, or other forms of patient-based evidence, such as patient-reported outcome measures. In addition, the syntheses of qualitative data with data from quantitative systematic reviews that identify interventions to enhance some aspect of patient experience also needs to be considered.

3.2 Patient and public involvement

Patient experts can be nominated by a range of organisation which has been identified as having a close interest in the technology under appraisal. As well as nominating one or more experts to attend the committee meeting, patient organisations are also invited to make written statements or submissions. The patient expert who attends the meeting presents their own opinion, which may differ from the views presented by the nominating organisation.

Patient experts provide evidence, sometimes through a formal presentation, that contributes to discussions about the appropriateness, relevance and acceptability of a particular technology. The patient experts may have different philosophical underpinnings and may vary in the forms of knowledge and evidence they contribute to the process. Some initial unravelling of philosophical perspective and nature of evidence that patient experts may provide is given below to stimulate discussion about the key questions:

- **Philosophical underpinning**: The philosophical underpinnings that guide a patient expert in relation to level of involvement may influence

the way in which they provide evidence and their expectations of the process. For example, consultative forms of involvement might involve patient experts being asked for a view, but they may expect less or no involvement in the discussion or synthesis of evidence or the decision on a recommendation. Some patient experts may favour more collaborative roles where they are inherently involved in contributing to the synthesis of different forms of evidence and in the formation of a recommendation. Some patient experts may also be familiar with the concept of user-led research, where users of service or user-researchers lead a project. The way patient experts from this background may provide evidence may differ from those more used to collaborative or consultative forms of involvement.

- **Experiential knowledge or perspective**: The patient expert may be someone who has experiential knowledge based on their own experiences. In this way they offer a perspective, which can generate valuable discussion. This issue of representation in this context is really a red herring as the focus should be on their perspective, as with other experts. Alternatively experiential knowledge may be drawn from the experiences of a broader constituency of people who have come together in some form, for example, as a patient organisation and may represent the range of views.

- **Research-based knowledge:** The patient expert may be someone with a broader knowledge and evidence base about experiences with a particular technology. Their analysis and synthesis of research-based knowledge may be undertaken with a different 'lens,' appraising aspects of experience according to different criteria in the context of a technology. The knowledge or evidence they are aware of may come from research, such as a meta-ethnography or may be more diverse and can include grey literature.

- Research-based knowledge can include **methods critiques**, for example, whether assumptions made in economic modelling have validity. For example, that people can make a choice between

interventions when they have not experienced a condition. Another example is patient-reported outcomes measures (PROMS) where concerns have emerged about the extent to which PROMS capture outcomes of importance to patients (Haywood et al, 2011 Staniszewska et al 2011).

4 Questions for discussion

1. How can we maximise the potential for identifying and incorporating evidence from patients and carers in technology appraisals, within current processes?

2. Is the methods guide clear on the level and nature of involvement we expect from patient experts in technology appraisal?

3. Does the methods guide give clear guidance on the nature and type of evidence and knowledge we expect patient experts and patient organisations to contribute?

4. How could the guidance on nature and types of evidence and knowledge be improved?

5. What role could patient experts and patient organisations have in evaluating the adequacy of PROMS data, in relation to content validity?

5 References

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