NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Report to the Methods Review Working Party

Key issues arising from workshop on patient evidence

This report is written by members of the Institute's team of analysts. It is intended to highlight key issues arising from discussions at the workshop on patient evidence. It is not intended to provide a detailed account of all comments expressed at the workshop. The report has been written independently of the people who attended the workshop.

The report is 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/technologyappraisalprocessguides/GuideToMethodsTA201112.jsp.

1 Summary

- In the current methods guide, there is no clear definition of patient evidence and the myriad ways it might be obtained and used (patient attendance at Committee meetings, written statements, patient organisation submissions, qualitative research, synthesis of qualitative research, patient involvement in consultations). Importantly, there is blurring and confusion between patient involvement, and qualitative research in the methods guide.

- Some of the discussion on maximising the potential for identifying and incorporating patient evidence in technology appraisals focused on the NICE processes rather than methodology, particularly in relation to earlier involvement. There was discussion of the need for further

research looking at previous submissions so that the qualities of a good submission could be identified more clearly than is currently the case.

- There was much discussion of the integration of patient evidence into the decision making of the Committee. Many felt that evidence from patients had a low prominence in technology appraisals because it could not easily be integrated into the economic analysis that usually forms the basis of the decision. There was some discussion of how the economic modelling could incorporate patient evidence.

- The role of patient experts as critics of the assumptions made in economic analyses and of the extent to which both models and patient reported outcome measures capture outcomes that are of importance to patients was discussed. The methods guide currently mentions this role, but could perhaps give more guidance to patient experts and patient organisations on how they might best fulfil it.

- Some delegates felt that the technical language in the methods guide made it inaccessible to patient experts.

- The current methods guide expresses a preference for *"a synthesis of information […] rather than as a series of individual testimonials"*. This implies a preference for evidence derived from qualitative research. Workshop delegates were not supportive of this implied preference and generally felt that too much analysis could result in a loss of the richness of the language in the direct testimony of patients. There was little support for subjecting the written patient statements received in the current processes to formal analysis.

- Some, but by no means all, patient organisations have the capacity to conduct primary qualitative research in support of a submission and the timeframes of a NICE appraisal do not facilitate this. Appraisal Committee members among delegates generally agreed that Committee didn't necessarily prefer this type of submission to the more informal reporting of patient experiences, meaning pertinent

submissions need not be out of reach of organisations that do not have this capacity.

- There was agreement that review and synthesis of existing qualitative research could usefully contribute to technology appraisals but unless this becomes a requirement of the NICE methods, it was unclear whether it would be useful to provide guidance on how this should be done in the methods guide.

- There was agreement that patient evidence is an important form of evidence alongside clinical and economic evidence but that it is complex and needs to be teased out into its component parts. There needs clearer definition of what we mean by patient involvement and patient evidence.

2 Questions posed to the workshop participants

1. *What more should NICE do to maximise the potential for identifying and incorporating evidence from patients and carers in technology appraisals, within current processes? What is unique about the contribution that patient evidence makes within the context of technology appraisals?*

2. *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? Is it clear what level of involvement is required at different stages in the process? How could the guidance on nature and types of evidence and knowledge be improved?*

3. *What role could patient experts and patient organisations have in evaluating the adequacy of patient-reported outcome measures (PROMs) data, in relation to content validity? What guidance could be included in the methods guide to help patient experts and patient organisations contribute in this way?*

4. *Should the submissions NICE currently receives be treated as qualitative evidence which needs to be formally analysed? If so, by whom? Using what methods? What guidance should be given about the nature and quality of submissions?*

5. *To what extent should the submission of new qualitative research evidence be encouraged in the Technology Appraisals methods guide? From whom and with whom should such research be undertaken? How would the scope and methods of enquiry be determined? What guidance should be given to optimise the methodological quality of qualitative research evidence used in the Technology Appraisals programme?*

6. *Should the Technology Appraisals methods guide give guidance on the use of syntheses of qualitative research? By whom should these be undertaken? How could these syntheses be incorporated into the overall clinical and cost effectiveness evidence base?*

3 Summary of the workshop discussions

This workshop involved two presentations, each of which focused on different aspects of evidence from carers and how the technology appraisals programme might make best use of it. The first presentation and questions 1–3 above focused on the nature of patient evidence, its contribution to NICE decision making and the role of patient experts and patient organisations in supplying this evidence. The second presentation and questions 4–6 focused on clarifying what patient evidence is, and ensuring that patient involvement and qualitative research are used to their best advantage in the NICE process.

In addition to considering how the methods guide might be improved, the participants also considered how NICE might maximise the usefulness of patient submissions by providing further support and education for patient organisations submitting to the NICE technology appraisals programme. There was also consideration given to the value of research reviewing

previous submissions in order to learn from successful strategies. Workshop attendees were generally supportive of such research.

Similarly, issues around the technology appraisals process were raised relating to the stages at which patients were involved. There was an emphasis on earlier involvement if there was to be a move from a consultative approach to a more collaborative approach. The possibility of improving patient submissions by supplying an enhanced template was also considered. At the end of the process, some delegates considered that a post-appraisal debriefing for patient organisations could be useful (as is currently offered to manufacturer consultees).

Comments relating to these issues have been noted but will not be covered in detail in this paper, which will focus on the methods guide.

3.1 The contribution of patient evidence in technology appraisals

This issue was discussed in the context of the current reference case in which EQ-5D is the preferred measure of health-related quality of life in adults. The Delegates felt that direct patient involvement in the form of patient testimony and written submissions offers valuable insight into the impact of conditions and interventions on individuals' daily lives which cannot be captured in a health-related quality of life measure such as EQ-5D.

Delegates noted that concepts surrounding personal and social acceptability of interventions are best captured using directly reported patient testimony. An example was given of an intervention which was taken orally when the existing comparator intervention was given intravenously. Although the benefits of the two interventions in terms of QALYs generated might be similar, patients would favour the new oral treatment if it improved their everyday experience. The possible consequences of these preferences on adherence may also be assessed from patient evidence, particularly from accounts from patients with experience of a particular condition, rather than from data that relies on theoretical assumptions.

One delegate expressed the view that the unique contributions from patients involved in the NICE process may be divided into information that should be

(but is not) captured by the QALY [through the imperfections of the methodology], and information which cannot or should not be captured by the QALY.

3.2 Increasing the prominence of patient evidence

A view expressed by some delegates, including lay contributors to previous submissions, was that evidence contributed through patient involvement was given low prominence and visibility in technology appraisals in comparison to health economic data and modelling results. It was felt that if patient organisations were more confident that patient involvement is an integral part of the decision process they would be more enthusiastic about contributing and submitting. Other delegates suggested that some patients and carers may feel that their contribution is in some way less valuable than that from clinical and economic experts.

It was suggested that having improved written qualitative and patient evidence in the topic's evidence-base would reduce the pressure that patient experts might feel to adequately represent all those issues. However, by and large the solutions to these problems discussed by the delegates were related to improvements in the process by which patients are involved in appraisals and improvements in the support offered by NICE rather than methodological issues that could be covered by the methods guide.

3.3 The clarity of the current methods guide

The majority of delegates felt that the methods guide does not give clear guidance on the nature and type of evidence expected from patient experts and patient organisations.

There was repeated comment that technical language in the current guide may be inaccessible for patients and patient organisations. This might be related to the lack of clarity about the different types and sources of patient evidence that are potentially available and useable. While some delegates felt that more information on the types of evidence required should be included in the methods guide, others raised concerns that favouring one type of evidence over another may discourage organisations with an 'unfavoured'

evidence type from submitting at all. This was linked with concern from some patients that smaller, less financially secure patients and patient organisations could be disadvantaged if a demand for more robust evidence was explicitly favoured.

Delegates from patient organisations were clear in their desire for more guidance about what type of evidence is useful to NICE. There was repeated suggestion that examples of cases where patient evidence has had an impact on a decision in the past would be useful [this relates to the non-methods guide issues mentioned above].

3.4 The level of involvement of patient organisations

Issues relating to the level of involvement were discussed in the context of the briefing paper and presentation outlining three levels of involvement: patient-led, collaborative and consultative. The majority of delegates felt that it is not clear from the methods guide what level of involvement is required at each stage of the process. There was consensus that getting patients and patient organisations involved in the early stages of the appraisal process (that is, during scoping) is important. There was repeated suggestion across tables that the process would benefit from patient participation in the development of the economic analysis in some way to ensure that the model reflected the experience of patients, for example in terms of the health states included. This perhaps indicates a desire for more involvement; a more collaborative rather than consultative approach.

3.5 The role of patient experts and organisations in evaluating the adequacy of PROMs data

Again, this question was discussed in the context of the current methods reference case that indicated a preference for the EQ-5D. Consultees discussed the adequacy of EQ-5D in relation to capturing the impact of health technologies on patients. They also discussed the role of patient organisations and patient experts in evaluating the adequacy of EQ-5D for their particular patient populations.

It was acknowledged that current methods do not necessarily capture all the aspect of quality of life that are important, and that patient experts and patient organisations have a role in identifying those missing parts and bringing them to the attention of the Committee. There seemed to be a general consensus that EQ-5D had a number of weaknesses; for example certain domains might be missing like vision and hearing, it might include non-responsive dimensions and levels and it might be insufficiently sensitive to measure some important changes in quality of life.

One group noted that EQ-5D or any other PROMs tools are only attempting to capture health-related quality of life and will not capture experiences and impacts due to the processes involved in health care delivery. These can sometimes be more important in determining the most appropriate treatment.

One of the groups also raised the issue of ability and capability of patient organisations, in terms of resources and skill mix, in evaluating PROMs instruments and in conducting appropriate research to inform NICE decision making. They felt it was more for researchers to develop tools and measures that are as comprehensive as possible rather than to rely on directly reported patient evidence to fill the gaps.

Some delegates raised the issue of how the additional information from patients could be incorporated into decision making; would it be considered robust enough for cost-effectiveness analysis? There were concerns about how much weighting would be given to this additional information, what would be the recognised way of presenting it. It was unclear how non-preference-based PROMs are dealt with in the decision making process.

It was generally agreed that the methods guide should clarify what is expected from patient experts and patient organisations because it takes a lot of time and effort on their part to undertake this activity. There were suggestions that the methods guide should emphasise EQ-5D’s common deficiencies as well as specific deficiencies for particular patient populations.

Some delegates emphasised the use of lay and user friendly language within the methods guide for ease of understanding.

3.6 What guidance should be given to patient organisation about the nature and quality of submission?

Delegates were concerned that not all organisations have the capacity and resources to conduct extensive research in preparing their submissions. Also small organisations would have less capacity to produce submissions of as high quality as those from larger patient organisations. This could mean that their 'voice would not be as loud' as that of better funded organisations.

Appraisal Committee members among delegates generally agreed that Committee didn't necessarily want patient organisations to produce large amounts of material, meaning pertinent submissions need not be out of reach of smaller organisations. Appropriate guidance could benefit both patient organisations (who write the submissions) and committee members (who read them).

Appraisal Committee delegates identified broadly two important purposes for patient expert and patient organisation submissions and statements:

1) To provide the experiential context of the clinical decision;

2) To highlight aspects of the experience of either having the condition or taking the treatment for the condition that may not be clear or appropriately represented within the quantitative clinical or economic metrics (for example alopecia as an adverse effect of certain cancer therapy might be ignored in health economic modelling but it could be very important to some patients).

The experiential context broadly means getting a better sense of what it feels like to have the condition, and what it feels like to have the treatment for the condition. Even though this information wouldn't necessarily lead to the Committee making different recommendations, many Committee members considered this important to have as it helped to humanise the decision-making process and make the implications and importance of their decisions clearer. The second purpose was also considered important, especially where the quantitative evidence was lacking or ambiguous and the decision was near the margins.

Some participants suggested producing guidance for patient organisation for the submission like ‘Hints and Tips for Patient Experts’ produced by NICE for patient experts participating in Appraisal Committee meetings.

Some participants cautioned about the risk of being too prescriptive. This could suppress individual patients concerns from being highlighted. Getting the balance right between informing people about how to produce a submission that has the right sort of information from Committee’s perspective, without being so prescriptive that it discourages engagement was considered very important.

3.7 Submissions as qualitative evidence for analysis

At the moment, main themes are identified in patients’ submission and presented at the Committee meeting, normally by the lead team (including the lay lead) in their presentations. In addition committee members are supplied with and expected to read the original submissions. Some noted that the current method by which patient evidence submissions are presented to the committee with the specific input of a lay lead could be seen as analogous to the content analysis methodology of qualitative research although this might be done in very variable ways as it cannot be assumed that the lay leads have experience of qualitative analysis. Since no structured tools are used for the data collection, a framework analysis of patient submission is not possible. The participants agreed that quotations from patients in submissions and written statements add life to the discussion and humanise the complex clinical and statistical data presented at the meeting.

The participants agreed that main purpose of patients submission is to bring insight into the condition and were concerned that an over formal analysis of the patient submission will take the life experience out of the discussion and potentially ‘dehumanises’ the evidence, getting further away from the patient experience (although a good qualitative analysis should retain the patient voice). The other factors which can potentially discourage a formal content analysis of patient submissions were the associated time and resource cost, particularly in the light of the tight timelines of a technology appraisal. It might be the case that sometimes there would be negligible added value, especially

when the cost-effectiveness evidence strongly indicates that a technology is cost-effective (unless of course there is a view that the technology is not acceptable to patients).

3.8 The submission of new and existing qualitative research evidence

There was a lack of agreement over the extent to which the methods guide should encourage the submission of new qualitative research. Some delegates believed that such evidence would add little, because the main driver of the decision is the cost-effectiveness evidence. Unless the qualitative research evidence informs this analysis then it may not be useful. Others believed that qualitative research evidence could be useful in assessing the acceptability, appropriateness, effectiveness and utility of a technology from the patient perspective and would provide vital context for considering cost-effectiveness data.

Undertaking primary research was seen as unrealistic most of the time, but it was important that there was the opportunity to present existing qualitative research. There was a range of views as to whether it was feasible to undertake some level of (rapid) review of existing evidence during the appraisal process. However it was not established where the burden of finding this evidence and should fall. Some felt that any level of additional work would not be possible if the burden was placed on patient groups. The possibility of evidence review groups (ERGS) or Assessment Groups a rapid review was also considered, but tight deadlines and variation in the level of expertise available would place constraints on this option too (see also section 3.9 below).

There was general agreement that if qualitative research evidence was required, then any guidance regarding this evidence should not be overly prescriptive. Delegates felt that a certain minimum standard for reporting qualitative research would be required to ensure the evidence is useful to the committee. It was commented by some that poor evidence may actually harm the case being presented.

3.9 Systematic review and synthesis of qualitative research

Most tables agreed that syntheses of existing qualitative research could usefully contribute to technology appraisals. If there is useful qualitative evidence already 'out there' then there ought to be a means by which it can be incorporated into the appraisal process. One table queried whether NICE would require syntheses of qualitative research evidence for all appraisals. If so, they felt that guidance would be useful. Otherwise, it was unclear whether it would be useful to provide guidance in the methods guide on the use of syntheses of qualitative research.

One commentator expressed the opinion that relying on syntheses of qualitative research would be yet another step removed from the patient experience. As has been noted previously, there is some power in the language that patients use to describe their conditions, and because qualitative research evidence is already one step removed from the individual patient view, further synthesis would lose the context within which the information was obtained.

Again the question of who is best placed to provide this evidence arose. Most tables agreed that there were four main options: the manufacturer, patient organisations, the ERG in single technology appraisals (STAs) and the assessment group in multiple technology appraisals (MTAs) and other independent academic organisations. The delegates identified potential difficulties with each of these groups. For example, there would be an inherent assumed bias in manufacturer provided the synthesis of qualitative research. For patient groups, the problem would be one of funding and resources, as well as a lack of early enough involvement in the appraisal process. STAs present a challenge with respect to time, the involvement of ERGs is limited to eight weeks, during which it would be difficult to undertake this additional work. It might, be feasible for Assessment Groups to conduct this additional research if they were properly resourced to do so. Some of the panellists commented that only a few of the assessment groups would have the capacity and expertise necessary to undertake these syntheses.

Finally, independent academic organisations were suggested as an alternative. It was also suggested that these could potentially be commissioned by the manufacturer. One delegate expressed a concern that if the syntheses were provided or funded by the manufacturer, then they may be regarded more sceptically than if they were provided by a patient group or other source without a commercial interest.

3.10 Incorporating qualitative evidence into the overall clinical and cost effectiveness evidence base?

One table suggested that most of the additional information that could be derived from qualitative research and syntheses of qualitative research should already be captured in the QALY and the only reason that it wasn't is because EQ-5D is deficient. If EQ-5D could be improved, then it would remove the need for formally incorporating qualitative evidence.

Another suggestion was to give each element of an appraisal a fixed weight of importance. For example, cost effectiveness 40%, clinical effectiveness 40% and patient evidence 20%. It was acknowledged that this method would lend itself to being unscientifically applied and may lead to inconsistent results.

Another table suggested that qualitative research could be used alongside utilities used in the economic models, acknowledging that general population values and those from patients are usually different. It was also suggested that qualitative research could be used to assist committees in deciding what the range of acceptable incremental cost effectiveness ratio might be for a given topic. Other delegates indicated that the committee already performs this function adequately without this additional input.

4 Key issues for consideration by Working party

1. Is the current information in the methods guide on the purpose of patient evidence adequate and complete?

2. Could more helpful guidance be given on the role of patients in critiquing the clinical and economic evidence?

3. As highlighted in briefing paper 2, the current methods guide does not clearly distinguish between qualitative evidence, qualitative research evidence and syntheses of qualitative research evidence. Discussions at the workshop suggest that Appraisal Committee members do not necessarily value formal research more highly than directly reported patient testimony as they fulfil different roles. Given this:

    a) To what extent should the methods guide require or encourage the submission of primary qualitative research?

    b) Does the methods guide need to expand on the methods of qualitative research, such as the methods for identifying, sampling and recruiting participants?

4. Qualitative evidence that already exists in the literature is frequently overlooked in current appraisals. To what extent should the methods guide encourage systematic review and synthesis of existing evidence?

5 Authors

Prepared by Janet Robertson on the basis of workshop feedback from Lizzie Amis, Richard Diaz, Anwar Jilani, Bindweep Kaur, Anju Keetharuth, Heidi Livingstone, Jon Minton, Paul Richards, Donna Rowen, Will Sullivan, Jon Tosh and Helen Tucker whose contributions are gratefully acknowledged.

February 2012