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# NICE news in the annals

## NICE Health Technology Appraisal Programme

### The role of patient experts

#### Who are the patient experts?

The Technology Appraisals Committee (TAC) is interested in hearing first hand from both patients and clinicians with experience and knowledge of the condition for which the intervention being appraised is intended. The committee is also interested in patients' personal experience of the technology, or views on the outcomes they would like from the technology being appraised. For this reason, NICE invites patient experts, together with clinical experts, to attend the first meeting of the Committee. Physical presence means that the committee not only hears what the experts have to say but can also ask them to illustrate, explain, expand on or provide additional information.

'Patient experts' are lay people who can provide a patient or carer perspective on the patient issues relating to the technology being appraised. NICE tries to ensure that there are two nominated patient experts. One patient expert will usually be a patient (or carer of a patient) with the condition the technology treats and, if possible, experience of the technology. The other patient expert is usually someone who is able to bring in the views of a range of patients, for example someone who works for a patient organisation and is in touch with the issues facing a larger constituency of people with the condition.

#### Who chooses the patient experts?

Patient experts are nominated by patient/carer organisations who are consultees to the appraisal. Patient organisations are free to nominate whoever they want but NICE provides guidance to help them select people who are likely to have the necessary skills and experience to provide the TAC with the information it needs to gain a better understanding of the issues that patients consider important when assessing the effectiveness of a technology.

Patient organisations are the in the best position to identify the most effective people to represent the patient view. Where a particular technology is supported by a number of patient organisations, NICE encourages the separate organisations to work together when making their nominations.

#### The role of the patient experts

Patient experts attend the first meeting of the appraisal committee for a given technology to respond to questions put to them by the committee members or Chair. They will be asked to give their views on patient issues that have already been brought to the attention of the committee, explaining or expanding on certain themes, or providing information from their own personal experience. Patient experts are also encouraged to be pro-active, by raising their own points, asking their own questions, or commenting on issues raised by other experts or committee members.

#### The importance of the patient expert

Patient experts make an invaluable contribution by ensuring that committee members can explore in depth the issues that patients consider important in relation to a technology.

- What is it like to have the condition?
- What are the outcomes (from treatment) that matter most to patients?
- What difference does the technology make?
- What's it like to use the technology?

Although many patient experts may find the health economic analysis difficult to comment on directly, they are still able to inform the accuracy of the analysis by providing a vital human context to statistical and numerical measures. Frequently, this means that patient expert testimony significantly influences the interpretation of the cost per QALY estimates that the Committee are presented with.

#### Support for patient organisations and patient experts

NICE funds a dedicated project manager within its Patient and Public Involvement Programme (PPIP) to provide support both to patient organisations and patient experts. The project manager's role has developed over time, taking into account feedback from patient organisations, patient experts and committee members.

Consultation with patients and other stakeholders about what skills and experiences patient experts might need resulted in the production of written guidance on choosing patient experts, included in a handbook written specially for patient organisations to help them participate in all stages of the technology appraisal process.[1]

For patient experts themselves, both the prospect and actual experience of the committee meeting can be daunting. The

patient experts are. therefore. briefed by the project manager some weeks in advance of their attendance about what to expect at the committee meeting. In addition. they are sent information to help them to understand the appraisal process and prepare for their attendance at the committee meeting; a NICE representative also meets all patient experts on the day of the meeting, both before the meeting starts, to make them feel at ease and answer any last minute questions.

For many patient experts attending the appraisal committee meeting, this will be their first experience of such a meeting; it is a large and often fast-paced meeting with a lot of information to be taken in within a short time-frame. The meetings have a high technical content, with health economics being a significant feature of the committee debate posing a particular challenge for patient experts. Both NICE and its PPIP are continually assessing the process to identify ways of improving the experience of patient experts and the opportunities they are offered to participate in the debate.

### Reference

1. NICE. *A Guide for Patient/Care Groups – Contributing to a technology appraisal.* London: NICE, 2004.

## Appraisal of treatment for severe sepsis in intensive care units

In September 2004, NICE issued guidance on drotrecogin alfa (activated), recommending that it be used in 'adult patients who have severe sepsis that has resulted in multiple organ failure (that is, two or more major organs have failed) and who are being provided with optimum intensive care support'. Furthermore, NICE recommended that the use of drotrecogin alfa (activated) should only be 'initiated and supervised by a specialist consultant with intensive care skills and experience in the care of patients with sepsis'.

Drotrecogin alfa (activated) is licensed in the European Union for the treatment of adult patients with severe sepsis with multiple organ failure, when added to best standard care. It is understood to exert its action by modulating the coagulation cascade and inflammatory responses associated with severe sepsis. The recommended standard treatment regimen for drotrecogin alfa (activated) is to infuse 24 μg/kg body weight/h for 96 h. Therefore, the total acquisition cost of a full 96-h course for a 70-kg patient is estimated to be £4905 excluding VAT.

Licensing was based on a subgroup analysis of a single randomised controlled trial, PROWESS. When patients with two or more organ failures were analysed in that study, the relative risk of death was statistically significantly lower in those treated with drotrecogin alfa (activated) compared with placebo (mortality in these combined placebo and treatment groups was 33.9% and 26.5%, respectively). However, patients were recruited to this trial in centres across the world but not in the UK. The Committee was nevertheless persuaded by the current evidence and by that presented by experts for this appraisal that the survival advantages seen in PROWESS are robust and likely to be generalisable to the UK population.

In terms of the economics of treatment with drotrecogin alfa (activated), there was a substantial agreement in terms of results among the cost-effectiveness analyses considered by the Committee during this appraisal. The available UK analyses (including that provided by the Assessment Group) indicate a cost per QALY of less than £11,000 for patients with severe sepsis and multiple organ failure treated with drotrecogin alfa (activated). Other available studies are broadly consistent with these findings, although North American estimates of cost per QALY are higher.

An important aspect of the Committee's deliberations related to patient selection, especially with regards to the usefulness of the APACHE II and SOFA scoring systems. Both the experts for the appraisal and the Assessment Group advised against the use of the APACHE II or SOFA scoring systems. For example, it was argued that the APACHE II scoring system gives a high weighting to factors such as increased age and chronic ill health, and that it was not designed for individual prognostic use. In addition, it was noted that this tool was validated for use within the first 24 h of admission into the ICU, although in PROWESS, the APACHE II score was determined at the point of study entry. The Committee was also advised that the SOFA scoring system was not developed to predict patient outcomes. The Committee accepted that such tools would not be suitable aids for selecting patients for treatment with drotrecogin alfa (activated). However, the Committee considered that when selecting patients with sepsis and multiple organ failure for treatment with drotrecogin alfa (activated), the criteria used in defining organ failure should be based on those used in PROWESS and reflected in the Summary of Product Characteristics for drotrecogin alfa (activated). Overall, it was persuaded that the failure of two or more major organ systems (in particular, cardiovascular, respiratory and renal failure) was likely to indicate that drotrecogin alfa (activated) would be beneficial.

Recently published trial evidence has confirmed that drotrecogin alfa (activated) has no benefit in patients at low risk of death. Conversely, a recently published *post hoc* analysis indicates that patients with a high risk of death (as defined by an APACHE II $\geq 25$) treated with drotrecogin alfa (activated) have improved long-term survival compared to those on placebo. (No benefit was seen among those with lower scores.)

This evidence is thus supportive of the current guidance which will be considered for review in September 2007. NICE will take into account any changes that may have been made to the licensed indications for drotrecogin alfa at that time as well as any further evidence that becomes available.