NICE appraisals should be everyone's business

When Newbury and Community Primary Care Trust appealed against NICE's decision on Herceptin, it was the first to do so. **Jane Wells** and **Claire Cheong-Leen** explain the process and why other trusts should make their voices heard in appraisals of new treatments

Publicly funded health services should aim to provide the best possible health care within the available budget. With finite resources and demand for health care growing both in quantity and cost, they are faced with increasingly difficult decisions about the services they should provide. They must balance their responsibilities to the whole population and to individual patients; consider the need for preventive, therapeutic, and long term care; weigh the merits of new against established treatments; and deliver the services they wish to provide as well as those that are mandatory. In England, primary care trusts are mainly responsible for these decisions. Following the latest NHS reorganisation there are now 152 primary care trusts, each of which commissions health services for a population of up to about 600 000.

NICE and the NHS

NHS provision is fundamentally influenced by the National Institute for Health and Clinical Excellence (NICE), which produces national guidance on health technologies, public health, and clinical practice.¹ NHS organisations in England and Wales are required to implement NICE guidance on new technologies within three months of issue.

A large proportion of the treatments that NICE appraises are drugs, many of them expensive, and most appraisals result in a recommendation that they should be used. NHS organisations must then give funding of these new treatments precedence over other interventions, which can affect their ability to provide other services.

NICE appraisals consider the cost effectiveness of each intervention, with a threshold of about £30 000 (£44 000; \$60 000) per quality adjusted life year (QALY). However, the appraisals do not explicitly take into account the affordability of the technologies within the NHS, and NICE does not advise on what interventions should be stopped in order to implement the new guidance.² This is crucial given the overriding requirement of primary care trusts to keep within budget.

Despite the potential effect of NICE guidance, NHS organisations have made little use of the opportunities to participate in NICE appraisals and consultations. In contrast, there has often been stronger representation from groups such as drug companies, advocacy or special interest groups (which may themselves be funded by the drug industry), and professional organisations.

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Box 1: The trastuzumab story

March 2002: NICE approved trastuzumab for metastatic breast cancer

May 2005: Abstracts of preliminary results from trials of trastuzumab in early breast cancer presented at American Society for Clinical Oncology conference

October 2005: A few women receive NHS funded treatment for early breast cancer as "exceptional" cases, some after high profile court actions³

February 2006: Roche applies for a European licence for the drug to be used "for the treatment of patients with HER2 positive early breast cancer following surgery, chemotherapy . . . and radiotherapy (if applicable)"⁴

June 2006: NICE publishes draft guidance on use in early stage breast cancer after a single technology appraisal⁵

August 2006: Final guidance published requiring NHS implementation within 3 months.⁶ In a primary care trust serving a population of 400 000, about 77 women will require treatment annually at a cost of around £30 000 each, totalling about £2.3m a year (Berkshire Priorities Committee, paper 18a/2006)

Trastuzumab and beyond

In July 2006, we submitted an appeal to NICE on behalf of Newbury and Community Primary Care Trust after the publication of draft guidance on the use of trastuzumab (Herceptin) in HER2 positive early stage breast cancer (box 1). This was the first time an NHS organisation had appealed against NICE guidance. Trastuzumab was also the first intervention to be appraised under the (then draft) single technology appraisal process, and our trust was one of two invited to participate as a stakeholder.

Single technology appraisal was established "for the appraisal of single products, with single indications . . . normally . . . close to their introduction into the UK market."⁷ It differs from standard appraisal in that the evidence comes from the manufacturer rather than an independent review by an academic unit. Although the evidence is reviewed by an independent evidence review group, the manufacturer is not required to include evidence that it does not consider appropriate. Another key difference is that stakeholders can comment at several stages during a standard appraisal whereas in the single technology appraisal we were not given the opportunity to comment during the appraisal process, only a right of appeal before the final guidance was issued.

When the draft guidance was published we had serious concerns about whether it would allow trastuzumab to be used to achieve the greatest benefit for

Box 2: The primary care trust's appeal

Newbury and Community Primary Care Trust was invited to participate in the single technology appraisal of trastuzumab as a stakeholder organisation, one of two primary care trusts selected apparently at random. As a stakeholder the trust had the right to appeal against the guidance once it was published in draft.

The process gave stakeholders 15 days to respond to the draft guidance, setting out the grounds for appeal within the conditions set by NICE. This required them to show that the guidance was “perverse in the light of the evidence submitted.”

The appeal did not aim to prevent the use of trastuzumab, but sought clarification on several points:

- Which HER2 positive women should receive trastuzumab—this was not specified, except in relation to cardiac function
- When treatment should start—women in the published trials⁸⁻¹⁰ started trastuzumab shortly after completing their initial treatment. Under the guidance women whose initial treatment had been completed years previously could receive trastuzumab, despite there being no evidence of benefit
- How long treatment should last—the guidance stated that treatment should be for one year despite evidence that the clinical rationale for this was open to question and evidence from a trial excluded from the submission by Roche that 3 months' treatment might be as effective¹⁰
- The effect on NHS resources and on patients whose treatment might be displaced by the treatment and monitoring of patients receiving trastuzumab
- The uncertainty about the long term risks and benefits of trastuzumab the main study on which assumptions about effectiveness were based had a median follow-up of only a year

A team of five from the trust presented the appeal to a public hearing of the NICE appeal committee. NICE dismissed the appeal, but the final guidance contained clarification of several of the above points.

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In contrast with patient advocacy and special interest groups, NHS organisations have made little use of the opportunities to participate in NICE appraisals and consultations

patients, and whether the requirement to implement the guidance would undermine primary care trusts' ability to provide health care for the whole population. We received comments supporting this view, along with concerns about the appraisal process, from individuals and NHS organisations, and the trust's board and executive agreed to support an appeal (box 2).

Although NICE dismissed the appeal, it responded to several of the points on which we had appealed, clarifying questions about when treatment should start and which women should receive it. We had also shown that it was possible for NHS organisations to have a greater role in the NICE appraisal process. The single technology appraisal process has since been reviewed to allow earlier involvement by primary care trusts.

Participation by primary care trusts in technology appraisals is important because they have responsibility for the whole population, rather than representing a particular professional, commercial, or special interest. Our trust was the only stakeholder organisation in the trastuzumab appeal with this perspective; the other stakeholders were three patient groups, Cancer Research UK, and nine professional groups including

pharmaceutical and oncology associations and royal colleges.

Other countries have systems that help ensure a wider perspective. In New Zealand the body that makes decisions about the implementation of new drugs, PHARMAC, works within a capped budget and considers wider health sector costs. It currently does not recommend funding for trastuzumab in early breast cancer because of insufficient evidence of long term effectiveness.¹¹ In Australia, the Pharmaceutical Benefits Advisory Committee considers the effectiveness and costs of proposed benefits compared with alternative treatments. It recommended government funding of trastuzumab, but the assessment included total cost and had to be approved by the Australian Federal Cabinet. It also placed several conditions on the manufacturer before final approval.¹²

Greater participation

One of the professed aims of the recent NHS reorganisation was to strengthen commissioning.¹³ Primary care trusts and commissioners are ultimately responsible for making the best use of public funds to improve the health of the population. They need to

make explicit decisions about healthcare priorities, considering all patient groups and including prevention and care as well as treatment. Robust and transparent systems for decision making and priority setting are therefore essential.

The lack of involvement of NHS organisations in NICE appraisals probably reflects their limited capacity for assessing effectiveness and prioritisation. Our trust's ability to respond to the NICE draft guidance within the required 15 days was helped by support from the Thames Valley Priority Setting Unit, which had already considered the published evidence on trastuzumab. However, many areas do not have a similar resource to draw on. The new larger primary care trusts should have more scope to include such capacity or to share their skills across a wider area, but this needs to be recognised in their plans and resourced adequately.

We were also helped by the information, comments, and support we received from a large number of individuals and NHS organisations. Good informal networks and a readiness to share the outcomes of work and offer contributions to others' work would help organisations make best use of their limited capacity. Forums such as email discussion groups offer excellent opportunities to do this. NHS organisations selected as stakeholders in NICE appraisals may then be able to contribute more by drawing on work done elsewhere.

NHS staff and organisations should take every opportunity to participate in consultation and debate about the services the NHS should provide. NICE has now identified 18 more topics that it will consider under the single technology appraisal process,¹⁴ in addition to the ongoing programme of health technology appraisals. NHS organisations are likely to be invited to participate as stakeholders and should contribute.

For example, NICE is currently appraising the use of ranibizumab and pegaptanib in age related macular degeneration, a common cause of blindness in older people. These drugs could benefit large numbers of patients but are extremely expensive. Pegaptanib could cost the NHS over £0.5bn annually, and ranibizumab even more than this.¹⁵ Studies suggest that bevacizumab, which is currently unlicensed for this indication, could be as effective at a fraction of the cost. Input from NHS organisations could ensure that NICE's decision considers not only the effectiveness of individual drugs but also how effective treatment can be provided for the maximum number of patients with age related macular degeneration without imposing unaffordable costs.

NHS organisations should also continue to engage with NICE about how its appraisal systems work. NICE has changed its single technology appraisal process to allow NHS organisations to comment earlier in the appraisal.⁷ However, NHS organisations might have more confidence in appraisal decisions if consultation was to be the rule rather than the exception, and if other evidence could be considered apart

SUMMARY POINTS

NICE does not provide guidance on affordability when assessing new treatments

As stakeholders, NHS organisations have an opportunity to present the wider perspective

Short time frames and limited resources make participation difficult

Greater cooperation between organisations and more flexible processes are needed

from that submitted by the manufacturer. The NHS's ability to implement new technologies would also be helped by clarity on how the appraisal process has considered the effect of implementation on other NHS services.

NICE could make it easier for NHS organisations to participate in appraisals through some relatively simple steps such as flexibility over meeting dates, realistic timescales (bearing in mind that most such organisations have limited capacity), and clarity over what is required. NICE has committed to keep the single appraisal process under review and NHS organisations should have the opportunity to contribute to this.

Conclusion

National policy decisions about treatment must consider affordability in the context of all healthcare provision, as well as the clinical and cost effectiveness of individual interventions. Without this, local providers with a fixed budget may be required to give higher priority to new treatments than to established services that are more cost effective.

A two way link is essential between the makers of national policy and those required to implement it. To achieve this, NHS organisations must build their skills and capacity to participate in policy development and NICE needs to make it easier for stakeholders to submit their views.

Competing interests: JW and CCL are both employed by NHS organisations that are required to implement NICE guidance.

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