Resource allocation

NICE work

The National Institute for Health and Clinical Excellence is charged with getting the best from NHS resources. James Raftery looks at how it makes its decisions and what they have been while Robin Ferner and Sarah McDowell examine the influences it has come under.

Review of NICE’s recommendations, 1999-2005

James Raftery

The creation of the National Institute for Clinical Excellence (NICE) in 1999 put the English NHS in a leading role in setting healthcare priorities. Although Australia, New Zealand, and Canada have systems that judge the cost effectiveness of drugs, they do not assess other health technologies. Bodies similar to NICE are reportedly being established in other countries, notably Germany and France. By April 2005, NICE had published 86 guidances on the use of health technologies and 39 guidelines on the treatment of diseases. It has received several generally favourable reviews from independent agencies including the House of Commons Health Committee, the World Health Organization, and independent academics.

NICE recommendations

NICE appraises the clinical and cost effectiveness of health technologies referred to it by the Department of Health. This is different from a scientific assessment and synthesis of the evidence, which is subcontracted to independent academic groups. The two NICE committees appraise this often incomplete evidence. The committees rely on the judgments of their members, who comprise clinicians, health scientists, managers, and patient representatives.

NICE’s recommendations are issued in the form of mandatory guidance to the NHS. It started controversially by recommending against the use of zanamavir, an antiviral drug for flu. However, reviews of the guidances issued in the first years show that few recommendations can be classified as simply yes or no.

Despite suggestions to the contrary, NICE has repeatedly stated that it does not have a threshold at which cost effectiveness becomes unacceptable. However, it has clarified that when the cost per quality adjusted life year (QALY) is above £20 000 (€29 000; $37 000), “judgements about the acceptability of the technology as an acceptable use of NHS resources are more likely to make more explicit reference to factors including the range of uncertainty surrounding the calculation, the innovative nature of the technology, the particular features of the condition and population receiving the technology, and where appropriate the wider societal costs and benefits. Above an incremental cost effectiveness ratio of £30 000/ QALY, the case for supporting the technology on these factors has to be increasingly strong.”

NICE is unique internationally in having a formal appeals process. Stakeholders, including companies and clinical organisations, can appeal against its findings on the grounds of process (due process), perversity (given the evidence), or powers (exceeding its powers). Appeals are heard by a panel composed largely of non-executive members of NICE and industry and patient representatives.
Review of guidance

I reviewed the guidance issued by NICE to the NHS between 1999 and 2005. Some appraisals included more than one technology, and some technologies could be used in different groups of patients. For each guidance, I identified one or more patient technology topics and classified the recommendation for each topic as yes, yes with major restrictions, yes with minor restrictions, and no (box). Restrictions for drugs were relative to licence, and for non-pharmaceuticals, relative to the size of the potential patient group. The classification of each guidance was validated by nine experts (see acknowledgments). I also noted the cost effectiveness (incremental cost per QALY) associated with each recommendation and the results of any appeals.

The 86 guidances published up to April 2005 covered 117 technology or patient topics (see bmj.com). Recommendations were fairly evenly distributed across the four categories, with NICE deciding no for 22 (19%), yes for 27 (23%), yes with major restrictions for 38 (32%) and yes with minor restrictions for 30 (26%).

Of the negative recommendations, almost two thirds were on the grounds of insufficient evidence, the rest were because of unacceptable cost effectiveness. The recommendations for use with major restrictions generally imposed restrictions to improve cost effectiveness. The recommendations with minor restrictions usually specified good clinical practice (monitoring required, use by specialist) but sometimes also recommended use of the lowest cost equivalent technology.

Acceptable cost effectiveness

The highest cost per QALY that NICE has accepted is an estimated £39 000 (range £35 000-£43 000) for riluzole to treat motor neurone disease. The guidance noted "the values which patients place on the extension of tracheostomy free survival time." I also noted the cost effectiveness (incremental cost per QALY) associated with each recommendation and the results of any appeals.

Classification of NICE recommendations

Yes
- Should be used routinely
- Can be considered as an option

Yes with major restrictions
- Use only as second or subsequent line treatment
- Use only if intolerant to other treatment
- Must show response within specified time
- Restricted to sub-groups within licensed indications

Yes with minor restrictions
- Use the least costly option
- Monitoring required
- Use by specialist only

No
- Insufficient evidence for use
- Do not use because of poor cost effectiveness

pessimistic. With imatinib for chronic myeloid leukaemia, NICE initially accepted a cost per QALY from £22 000 to £56 000 but later reduced this to £26 000, which it calculated using a different comparator.

Interferon beta (and glatiramer acetate) for multiple sclerosis were deemed not cost effective at an incremental cost per QALY of £35 000-£104 000 (estimated mean £70 000). The government then intervened with a risk sharing scheme with a cost effectiveness threshold "set, for the purpose of this scheme only, at £36 000." Under the scheme, eligible patients who consent have their clinical progress monitored against that required to meet the target cost effectiveness. Drug prices would be reduced for patients whose progress fell below the target. The scheme, established in 2002, had recruited more than 5000 patients by 2005. No reports of its progress have been published, but it will be interesting to see how well the scheme succeeds in achieving its target level of cost effectiveness.

Although NICE does not officially prioritise interventions that save lives over those that improve quality of life, its treatment of some topics suggests the rule of rescue, or prioritising life saving therapies, may play a part. With cancer drugs such as imatinib and trastuzumab, which extend life expectancy, NICE accepted relatively poor cost effectiveness. However, the acceptance of riluzole was based on considerations of quality of life rather than on mortality.

Consistency

Some topics have been appraised several times, partly because of appeals. Antiviral drugs for flu have had three appraisals as well as a rapid review in 1999. Each time NICE reiterated its recommendation against use of these drug by healthy people but in favour of their use in vulnerable groups. Three obesity treatments (two medical and one surgical) have been appraised. NICE concluded for each that they should be used only in people with proved determination and appropriate progress on treatment.

Appeals

NICE's 86 guidances have been subject to 25 appeals (29%). Fifteen were dismissed. Of the 10 appeals that
were upheld, five resulted in relatively minor changes in the wording of the guidance. But five decisions (interferon beta in multiple sclerosis, drugs for colorectal cancer, flu antivirals, growth hormone in adults, and renal immunosupression in adults) were referred back to the appraisal committee for further appraisal. The appeals process has required NICE to show that it has been comprehensive in its examination of the evidence and consistent in its treatment of each topic.

Discussion

At its current rate of appraisal—around 20 a year—NICE can cover only a minority of new and existing treatments. This led to announcements in late 2005 of a more rapid review process. However, a more rapid process is likely to be considerably less intensive. The appraisal of drug treatments for multiple sclerosis, for example, took much of NICE’s first two years, with 338 documents listed on its website. It eventually recommended against use of interferon beta and glatiramer acetate because of their high cost per QALY. Despite considerable effort, including additional research, NICE was unable to identify a subgroup of patients in whom these drugs might have a more acceptable level of cost effectiveness. The fact that the government then intervened with a special purchase scheme based on a cost per QALY gained of £36 00010 indicated that the government thought this was an acceptable level, at least for these drugs.

Overall NICE must be judged to have succeeded in surviving some controversial decisions. Its appeal system has imposed consistency and has so far prevented appellants proceeding to legal challenge. Although clinicians have understandably feared blanket restrictions, these have been fairly rare. NICE continues to be best characterised not by saying no, but by saying yes but . . .

I thank Amanda Burls, Andy Clegg, Rumona Dickson, Ruairidh Milne, Alex Miners, Mark Sculptor, Ken Stein, Rod Taylor, and Tom Valley for help with classification of NICE guidance and Carey Hendron for administrative help.

How NICE may be outflanked

R E Ferner, Sarah E McDowell

We argued a decade ago that the NHS should not have to pay for new drugs unless they are at least as good as older ones, nor for expensive drugs whose benefits are uncertain. Since then, the National Institute for Health and Clinical Excellence (NICE) has been created. NICE appraises technologies that are available to the NHS and recommends whether they should be used unrereservedly, with restrictions, or not at all. Part of its remit is to ensure equity, but equity is not in everyone’s interests. Here, we consider how individuals or groups with specific interests may seek to outflank NICE.

Individual benefit or common good?

When many people share common resources, it is rational for each individual to increase personal use of the resources. But if all individuals do this, the resources are overexploited and eventually everyone will be ruined. This is termed the tragedy of commons. The NHS is a common resource. A patient acts rationally in seeking an expensive treatment that produces a benefit (even if small), because the cost falls almost entirely on others. But the NHS cannot support overexploitation indefinitely. It already spends £10.3bn (£15bn; $19bn) a year on drugs, and costs are rising rapidly. One way to avoid overexploitation is to appoint a guardian to administer the commons. NICE plays this role but faces many challenges.

References w1-w36 are on bmj.com

23 National Institute for Clinical Excellence. NICE to issue faster drugs guidance to NHS London: NICE, 2005. (Accepted 23 March 2006)
same background and same personality. In my opinion, he will be back. And what he needs is an understanding and supportive general practitioner who will try to nurse him through the next medicalised crisis, avert hospital admission whenever possible, and try to protect him from unnecessary investigations. Forget the much abused “insight” and other terms that negatively imply that it is all in the patient’s mind. It may have a psychological basis, but his pain and suffering are real.

He is clearly a high achiever, with good grades and peer recognition at school, and someone who is already showing leadership skills. He is likely to be successful in his chosen profession. But no one has every-thing. You can give it a nice medical title if you wish, but it is just the way he is. As he gets older, it is possible that he will, at different times, be anxious about various lumps and bumps, have different types of chest pain, worry about his bowels, and have many investigations. If that happens, a good general practitioner will be there to listen. What is most frightening about patients with this sort of recurrent presentation is that some day they will have something serious and, in all the medical clutter, it might be missed.

Competing interests: None declared.


Commentary: A clinical challenge

Robert Logan

Several learning points arise from Mr Neville’s case.1 The most important relates to the diagnostic approach when we are challenged with pieces of a clinical puzzle that do not neatly fit together.

Gastro-oesophageal reflux disease usually poses few diagnostic challenges, especially when there is a good symptomatic response to empirical anti-secretory therapy. However, in this case, Mr Neville’s poor response to treatment and persistent symptoms led to further investigation and several protracted stays in hospital. The normal oesophageal manometry and lower oesophageal sphincter pressures were an appropriate trigger for further investigations to eliminate underlying organic disease, especially distal obstrucing lesions or intermittent torsion of a hiatus hernia (although symptoms are more typically episodic with a hernia).

As with difficult to diagnose diarrhoea, admission to hospital provided the main clue to the final correct functional diagnosis. The importance of the careful clinical observations made while Mr Neville was eating cannot be emphasised enough. It is difficult to judge to what extent the possible underlying psychological components confounded the diagnosis, but seeking a second opinion from a fresh perspective is often very helpful when faced with a diagnostic challenge. More generally, patients and their doctors often mistakenly refer to regurgitation as vomiting, without recognising the importance of differentiating between the effortless nature of the former in contrast to most causes of vomiting.

Managing patients with psychological disorders

The other important learning point illustrated by this case is how to deal with patients who have potentially insoluble problems. One approach is to ask patients about their concerns or thoughts about the diagnosis. Another, not possible in this case, is to offer to review and repeat tests at some future time. Although it is essential always to be sympathetic and understanding, adopting the most appropriate approach to patients in whom underlying psychological problems may be contributing to their symptoms is fraught with difficulties and is a real challenge for the clinician.

A useful approach is to mention the importance of the “brain-gut axis” in functional gastrointestinal disorders at the outset. In this case, sharing the results of the impedance measurements together with getting Mr Neville to place his hand on his abdomen was a neat method of providing the patient with an opportunity to gain immediate insight into his problems. I suspect that the patient’s sense of relief as his symptoms resolved and his weight increased was similar to that of his doctors.

Competing interests: None declared.


Corrections and clarifications

Minerva

Several readers spotted Minerva’s assertion, in her second item in the 17 June issue, that lithium is a divalent ion (BMJ 2006;332:1460). It is in fact a monovalent ion (unlike calcium, which, as she said, is divalent).

Rational prescribing for children

An authors’ oversight in this editorial by Alastair G Sutcliffe and Ian Chi Kei Wong (BMJ 2006;332:1464-5, 24 Jun) led to Dr Sutcliffe having the wrong email address: this should have been a.sutcliffe@medsch.ucl.ac.uk.

Review of NICE’s recommendations, 1999-2005

Our editing of the author’s comments on assessment of non-drug technologies made them more definitive than intended and not strictly true (BMJ 2006;332:1266-8; 27 May). The published article, by James Raftery, said that Australia did not have a body that assessed health technologies. In fact, the Medical Services Advisory Committee in Australia and the New Zealand Health Technology Assessment Unit both have some form of assessment of non-drug technologies.

Obituary

We managed to make a nonsense of the date of death for Helen Ann Adami (BMJ 2006;332:1456, 17 Jun). She did die on 26 December, but in 2005, not in 2006.

Editor’s choice

In her column in the BMJ issue of 24 June, the journal’s editor, Fiona Godlee, slipped up in her assertion that a recent BMJ study showed that “One in 10 [patients aged over 45 who developed new rectal bleeding] had colon cancer.” In fact, 1 in 10 of the patients developed bowel neoplasia, and about half of these neoplasms were cancer. The study was by Jennifer du Toit and colleagues (doi:10.1136/bmj.38846.6848902F).