

How the DoH intends to improve access to medicines

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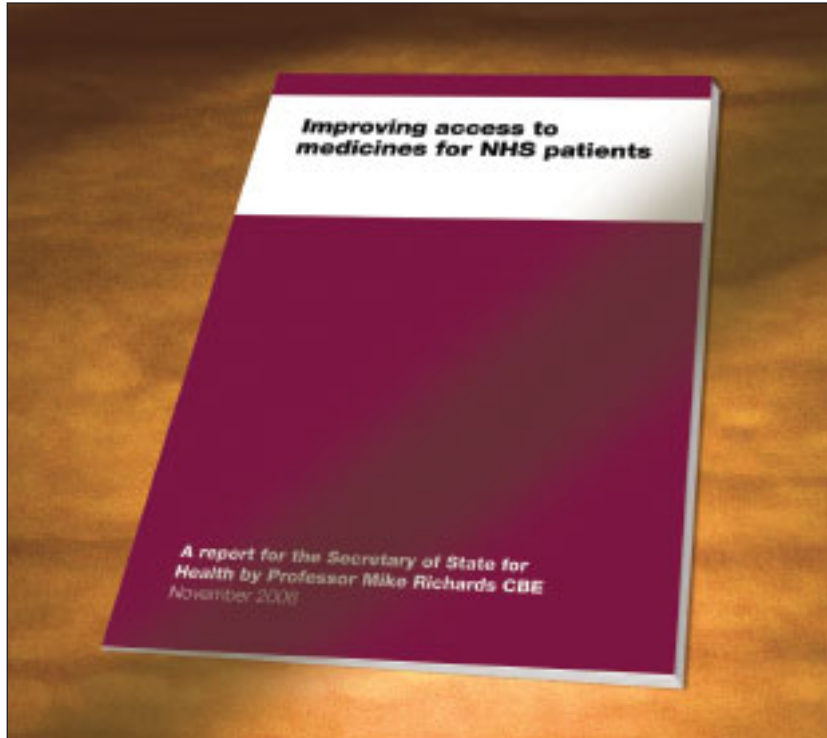


Figure 1. The DoH has acted quickly on the report's recommendations

Steve Chaplin discusses the range of measures introduced by the DoH to improve access to medicines within the NHS.

One purpose of the National Institute for Health and Clinical Excellence (NICE) is to ensure that NHS patients have fair access to cost-effective medicines. Paradoxically, its evidence-based decisions have proved to be a lightning conductor for complaints from patients, health professionals and the pharmaceutical industry that treatment with potentially valuable drugs is being denied.

The problem has been particularly acute for expensive new treatments for small groups of patients that offer seemingly modest gains in relation to their cost. A recent example is the use of ranibizumab (Lucentis) for age-related macular

degeneration, which NICE judged not cost effective until a compromise risk-sharing scheme was agreed with the manufacturer. Others include treatments for rarer forms of cancer that might realistically prolong life and for multiple sclerosis.

In 2008, the Government acted. It commissioned a review by Mike Richards, the National Cancer Director, on access to medicines not funded by the NHS.¹ It incorporated his recommendations into the new Pharmaceutical Price Regulatory Scheme (PPRS),² issued guidance to facilitate access to top-up treatment for NHS patients,³ and requested NICE to

consult on appraising life-extending treatments for people with terminal illness.⁴

What is the scale of the problem?

As part of the Richards review, the DoH conducted a survey of 80 PCTs to estimate demand for unfunded medicines, as measured by formal requests for exceptional funding for treatment – that is, funding for medicines rejected or not yet considered by NICE, or for off-licence uses.¹

There were approximately 15 000 applications for exceptional funding of drug treatments in England in 2008 – an average of

about 100 requests per PCT annually. Of these, about one-quarter were for cancer treatments. Thirteen per cent of PCTs said they had funded treatments rejected by NICE and 43 per cent said they funded treatments not yet considered. Applications involved about 50 drugs, the largest category being products that NICE has yet to consider. Overall, 64 per cent of requests for cancer treatments and 74 per cent of requests for non-cancer treatments were approved.

This method offers a useful overview in the absence of other data but it is probably inaccurate

because the level of funding requests is sensitive to local procedures for providing exceptional treatment. This probably explains why PCT approval rates ranged from zero to 100 per cent. Further, it does not include patients who did not pursue a funding request when they could have benefited from treatment.

The Richards report cited evidence that many patients with cancer are prepared to pay for treatment that offers relatively little benefit, and a survey by the Joint Collegiate Council for Oncology that found that over

half of patients denied funding by the PCT subsequently paid for their treatment (though it is unknown what proportion was paid by insurance).¹

Changing procedures

The DoH promised that the NICE appraisal process would be speeded up to reduce delay in access to approved medicines.³ Unusually, it included its proposals in the new PPRS (formerly an agreement solely concerned with prices and profits) and proposed prescribing incentive schemes to encourage the uptake of innovative products.²

In particular, it promised to 'refresh and extend good practice guidance in England so that it is clear that absence of NICE guidance is not a reason for refusing funding' and to facilitate discussions between industry and NICE on issues of concern. The role of patient access schemes such as risk-sharing was also formally recognised as one strategy to overcome the obstacle presented by high-priced medicines.

Treatments that may extend life

The Richards report noted that NICE procedures were being 'chal-

lenged' by increasing numbers of drugs developed to treat, for a short period, patients nearing the end of their lives.¹ In December 2008, after a five-week consultation, NICE adopted new guidance for its appraisal committees on assessing treatments for terminally ill patients.

NICE's standard appraisal criteria state that, as the cost per quality-adjusted life-year (QALY) increases above £30 000, there must be 'an increasingly stronger case for supporting the technology as an effective use of NHS resources'.⁴ NICE says it has in the

past used this criterion to recommend life-extending treatment for small numbers of patients with incurable illness, but adds that appraisal committees will now fully consider all the appropriate benefits and ensure that the supporting evidence and modelling is robust.

The criteria for considering treatments under the new guidance are listed in Table 1. When estimating QALYs, quality of life during the period of life gained from treatment will be considered to be equivalent to that of a healthy individual of the same age.

incremental cost-effectiveness ratio (cost per QALY) above £30 000
 indicated for patients with life expectancy normally less than 24 months
 sufficient evidence that the treatment will increase life expectancy by at least 3 months beyond current NHS treatments
 no alternative with comparable benefits is currently available in the NHS
 the treatment is licensed or indicated for small populations of patients

Table 1. Criteria for appraisal under supplementary advice on life-extending treatments⁴

Appraisal committees will also consider by how much quality of life would have to improve to bring the cost per QALY estimate below the £30 000 threshold. Treatment will be monitored to determine whether actual benefits match those predicted by the evidence and economic modelling.

In restricting its criteria to a small patient population, NICE initially defined this as fewer than 7000 patients. After consultation it accepted this figure was arbitrary and difficult to calculate.⁵ However, it did not alter its definition of short life expectancy (24 months) or clarify how this should be measured (from diagnosis, last treatment, with or without treatment²), allowing its committees to use their judgement.⁵

The new guidance will be widely welcomed but its application will not be universal. It would not have changed some of NICE's more controversial decisions, such as rejecting new drugs for macular degeneration and multiple sclerosis because they do not prolong life and the conditions are not terminal. By contrast, metastatic renal cancer affects around 6000 patients annually with a five-year survival of only 10 per cent; innovative drugs such as bevacizumab (Avastin) and sunitinib (Sutent) offer a significant

increase in progression-free survival over current therapy but, in draft appraisals, were not approved because the costs per QALY exceeded £70 000.⁶

The guidance came into force on 5 January. It will not be retrospective because of the resource implications but it will be applied to reviews scheduled in the normal way and these can be requested on the basis of evidence submitted to NICE.⁵ NICE will review the methodology in its supplementary guidance, though it offers no timescale.

Summary

The DoH has responded quickly to the Richards report by implementing a range of measures to widen and speed up access to innovative drugs and treatments for patients with terminal illness. If new pricing schemes for expensive drugs are successful, there should be little need for patients to top-up their NHS care. Some high-profile treatments are outside the scope of the new NICE

Key points

- Richards review conducted in response to complaints about access to medicines
- DoH has implemented range of measures that came into force 5 January
- NICE appraisal process to be speeded up to improve access and new guidance adopted on assessing treatments for the terminally ill
- prescribing incentive schemes proposed to encourage uptake of innovative medicines
- role of patient access schemes such as risk-sharing formally recognised
- guidance is not retrospective but will be applied to reviews

guidance and many patient groups will have to wait for NICE to review its guidance, or the introduction of another new medicine, before their case for special consideration is heard.

Bearing in mind that NICE appraisals are compulsory, there will need to be additional funding for PCTs to meet their new obligations.

References

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