



House of Commons  
Health Committee

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# National Institute for Health and Clinical Excellence

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***Volume I***

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## The Health Committee

The Health Committee is appointed by the House of Commons to examine the expenditure, administration, and policy of the Department of Health and its associated bodies.

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### Committee staff

The current staff of the Committee are Dr David Harrison (Clerk), Adrian Jenner (Second Clerk), Christine Kirkpatrick (Committee Specialist), Ralph Coulbeck (Committee Specialist), Frances Allingham (Committee Assistant), Julie Storey (Secretary) and Jim Hudson (Senior Office Clerk).

### Contacts

All correspondence should be addressed to the Clerk of the Health Committee, House of Commons, 7 Millbank, London SW1P 3JA. The telephone number for general enquiries is 020 7219 6182. The Committee's email address is [healthcom@parliament.uk](mailto:healthcom@parliament.uk).

### Footnotes

In the footnotes of this Report, references to oral evidence are indicated by 'Q' followed by the question number, and these can be found in HC 27-II. Written evidence is cited by reference in the form 'Ev' followed by the page number; Ev x for evidence published in HC 503-II, Session 2006-07, on 17 May 2007, and NICE x for evidence to be published in HC 27-II.

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## Summary

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Healthcare systems throughout the world have suffered from a lack of clear, authoritative advice about clinical and cost-effectiveness. They have been confronted by multiple, often conflicting, guidelines on the use of medical technologies and variations in patients' access to care depending on where they live. NICE was established in 1999 to address these problems in England.

In the eight years since it was established, in response to internal and external review, NICE has shown itself ready to change. Initially, it appraised technologies (mainly medicines) and produced clinical guidelines. Its remit was soon expanded to cover interventional procedures. Subsequently, technology appraisals were made mandatory and the single technology appraisal was established to speed up the evaluation process. Since 2004, it has also examined public health issues.

The environment in which NICE operates has continued to change. Sir David Cooksey's report on the single health research budget reiterated this Committee's recommendation in its 2002 report that technology appraisals of drugs should be carried out more quickly, and nearer to the time of launch. There has also been increasing criticism of NICE's decisions, mainly from patients, patient organisations and pharmaceutical companies. Eisai and the Alzheimer's Society obtained the right to a judicial review of a NICE decision. There has also been pressure from Ministers to make some drugs more readily available.

During the inquiry, we received much evidence that NICE is carrying out many of its functions effectively. Reviews have shown its evaluation processes to be generally robust. However, NICE also has its critics. Patient and professional groups as well as the pharmaceutical industry have highlighted the failings of NICE, such as the slow release of guidance and perceived unfairness in NICE's recommendations. The decisions of NICE are often appealed, and these appeals are widely reported. We examined three main areas of concern: the evaluation process, the affordability of guidance and its implementation. In addition, following the Office of Fair Trading's (OFT's) report on the Pharmaceutical Price Regulation Scheme (PPRS), we decided to look at the potential role of NICE in such a new system.

We identified several problems with the evaluation process, including:

- Topic selection. Only a few selected medical technologies are chosen as suitable for assessment as technology appraisals. There is also far too little emphasis on disinvestment. Here we found NICE's responses to our questions disingenuous. While few older treatments may do no good at all, many will not be cost-effective;
- The wider benefits of treatment to society, for example to carers, are not included in NICE's economic evaluations;
- NICE often does not have all the information it needs to make a full assessment. It does not have access to all the information the Medicines and Healthcare products Regulatory Agency (MHRA) uses and clinical trials are usually designed without

NICE's work on cost-effectiveness in mind;

- Experts are not sufficiently well used; and
- Publication of guidance is slow; licensed medicines are often not prescribed while PCTs and clinicians wait for NICE to make a decision.

We make a number of recommendations to address these problems. Key among them is the need for a system whereby all medicines are assessed at launch. A shorter, less in-depth evaluation should be made between the time of licensing authorisation and marketing, so that clinicians can prescribe useful and cost-effective drugs as soon as they are launched. A lower cost-per-quality adjusted life year (QALY) threshold should be employed during this early assessment to ensure that only the products that are most cost- and clinically effective are available straightaway. Subsequently there should be a full appraisal; the threshold could then be increased if warranted.

In order to ensure that NICE has the information it needs, NICE should have access to the same material used by the licensing body, clinical trials should be registered and there should be closer working between NICE and the pharmaceutical industry. This will be particularly important for the effective assessment of drugs in time for launch.

We recommend that more be done to encourage disinvestment. No evaluation of older, possibly cost ineffective therapies has taken place to date; two are currently underway. Our predecessor committee made the same recommendation; it is not acceptable that NICE continues to ignore this recommendation.

Our last report on NICE also recommended that the legislation be changed to accommodate the need to ensure that assessments of products take account of the wider benefits to society; we make the same recommendation here.

The affordability of NICE guidance and the threshold it uses to decide whether a treatment is cost-effective is of serious concern. The threshold is not based on empirical research and is not directly related to the NHS budget. It seems to be higher than the threshold used by PCTs for treatments not assessed by NICE. Some witnesses, including patient organisations and pharmaceutical companies, thought NICE should be more generous in the cost per QALY threshold it uses, and should approve more products. On the other hand, some PCTs struggle to implement NICE guidance at the current threshold and other witnesses argued that a lower threshold should be used. We recommend that the threshold used by NICE in its full assessments be reviewed; further research comparing thresholds used by PCTs and those used by NICE should be undertaken. An independent body should determine the threshold used when making judgements of the value of technologies to the NHS.

The implementation of non-mandatory guidance is variable. This is due to a variety of causes, including the threshold used by NICE for determining cost-effectiveness, lack of clarity about the status of guidance, lack of involvement of PCTs in the development of guidance and clinicians' disagreement about the worth of some NICE guidelines.

To improve the implementation of NICE guidance we recommend:

- More help for PCTs to implement guidance;
- Better assessment of the level of uptake;
- PCTs should play a larger role in the development of guidance;
- Better use of experts in the development of guidance; and
- A change in the terminology used by NICE, to clarify to patients what they can and cannot expect by right from their local NHS organisation.

We also recommend that elements of clinical guidelines be made mandatory. A suitable example would be risk assessment for all patients at risk of developing venous thromboembolism.

Some of the problems we heard in relation to the affordability and implementation of NICE guidance relate to the price of medicines. The OFT recently recommended that a new system of value-based medicines pricing replace the PPRS. We found support for such a system, but there were concerns about how it would work in practice. Discussions between the Government and the pharmaceutical industry are underway, so we make no large-scale recommendations regarding the new scheme. We agree with the Government, however, that better mechanisms are needed to ensure that the NHS pays a fair and affordable price for medicines. We recommend that NICE should be involved in any new system and that any change to its remit should be adequately resourced.

The OFT report indicated that more use should be made of schemes whereby the financial risk associated with some new medicines (where the cost : benefit ratio is uncertain) is shared between the NHS and the manufacturer. We recommend that risk-sharing schemes, such as the recently approved case of bortezomib (Velcade) and the older example of beta interferon and glatiramer acetate, should be used with caution. They should not be used as a catch-all in cases of uncertainty over a drug's benefit. Uncertainty would be better addressed by the careful design and performance of a publicly funded randomised controlled clinical trial. Better use should be made of NICE's 'only in research' recommendation in this regard.

We conclude that NICE does a vital job in difficult circumstances. The development of more and more health technologies and procedures, alongside rising patient expectations and the ageing population, is going to make it even more difficult in the future. Healthcare budgets in England, as in other countries, are limited. Patients cannot expect to receive every possible treatment. Demand outstrips resources and priorities have to be determined. In other words rationing is essential, and NICE has a key role to play. In the past NICE has changed in response to new challenges, and we are sure it can do so again. Given the difficult environment, NICE requires the backing of the Government. Ministers must support NICE, not seek to undermine it. NICE must not be left to fight a lone battle to support cost- and clinical effectiveness in the NHS.



# 1 Introduction

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1. In December 1999, the Rt Hon Alan Milburn MP, Secretary of State for Health, stated:

The NHS, just like every other healthcare system in the world—public or private—has to set priorities and make choices. The issue is not whether there are choices to be made, but how those choices are made. There is not a service in the world, defence, education or health, where this is not the case.<sup>1</sup>

These comments were made to the first conference of the National Institute for Clinical Excellence<sup>2</sup> which had been established that year as an independent organisation to help the NHS set priorities and make choices. The organisation was expected to contribute to a more effective system of allocating resources by analysis of the clinical and cost-effectiveness of treatments.<sup>3</sup> It was to examine the treatments and other technologies and approaches that patients receive from the NHS. In particular, NICE was to address a number of problems, including the presence of multiple, often conflicting, guidelines on the use of medical technologies and variations in local commissioning which meant that patients' access to certain types of care depended on where they lived. It was envisaged that NICE would provide a "single source of advice" to the health service, making clinicians' jobs easier and clarifying what patients could and could not expect from the NHS.

2. The Health Committee has taken a close interest in NICE's work over the last eight years. In 2001/02 the Committee undertook its first inquiry into the organisation.<sup>4</sup> It found an institution which was carrying out its vital task competently but the Committee also saw a number of ways the organisation could be improved. Many of the Committee's recommendations were accepted and implemented and NICE's remit has changed.<sup>5</sup> The Institute now takes much more seriously the need to ensure its guidance is implemented. Disappointingly, a number of the Health Committee's recommendations, for example that NICE should conduct technology appraisals at the time of launch, were not implemented.

3. Since 2002, the environment in which NICE operates has continued to change. Its work seems to have become ever more contentious. It has been increasingly subject to criticism. Those patient organisations, drug companies and clinicians which believe that NICE has come to the wrong decision have been vociferous in their protests. Recently Eisai, supported by the Alzheimer's Society, was given the right to a judicial review of a NICE decision. Ministers too have intervened following public outcries. For instance, in November 2005 the Rt Hon Patricia Hewitt MP publicly announced her concern about the refusal of a PCT to prescribe trastuzumab (Herceptin) to a patient with breast cancer for a then unlicensed indication before it had been assessed by NICE.<sup>6</sup> This made it almost impossible for NICE not to approve the drug, once licensed, regardless of cost.

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1 Rt Hon Alan Milburn MP, Speech to Clinical Excellence 1999, 8 December 1999

2 Since 2005 the National Institute for Health and Clinical Excellence

3 The proposals for NICE were set out in the Consultation Paper: *A first class service: quality in the new NHS*

4 Health Committee, Second Report of Session 2001–02, *National Institute for Clinical Excellence*, HC 515–1

5 The functions of the Institute, as described in its terms of reference, are listed in the Annex.

6 *Times*, 8 November 2005, <http://www.timesonline.co.uk/tol/news/uk/article588013.ece>, *BMJ*, 2005;331:1162

4. We were surprised by the vehemence of the criticisms and keen to find out how valid they were or, alternatively, whether NICE was subject to unfair and unjustified pressure. Accordingly, five years after our first inquiry we decided to hold another inquiry into NICE. Our terms of reference were as follows:

- Why NICE's decisions are increasingly being challenged;
- Whether public confidence in the Institute is waning, and if so why;
- NICE's evaluation process, and whether any particular groups are disadvantaged by the process;
- The speed of publishing guidance;
- The appeal system;
- Comparison with the work of the Scottish Intercollegiate Guidelines Network (SIGN); and
- The implementation of NICE guidance, both technology appraisals and clinical guidelines (which guidance is acted on, which is not and the reasons for this).

5. We received 124 memoranda and took oral evidence from 31 witnesses, including pharmaceutical companies, patient and professional organisations, PCTs, clinicians and health economists as well the Chairman and Chief executive of NICE and the responsible Minister. We visited similar organisations to NICE in Scotland, France and Canada. We were very fortunate to have the assistance of our Specialist Advisers Professor Joe Collier, Professor Alan Maynard and Dr Hilary Pickles.

6. The evidence we received contained praise for NICE's work but also, as might be expected, criticisms. Some criticisms had already been widely reported in the press: there were concerns about the time taken to produce guidance, topic selection and the lack of emphasis on disinvestment. In contrast, other criticisms had had less publicity, in particular the fear that PCTs were unable to afford NICE guidance, notably its technology appraisals of new drugs. As a consequence, PCTs were unable to provide other possibly more cost- and clinically-effective treatments, which had not been assessed by NICE. Thus, while some witnesses thought NICE was rejecting too many treatments, especially new drugs, others thought it was probably not rejecting enough.

7. During the inquiry the OFT published its report on value-based medicines pricing, in which it recommended that medicines should be priced according to their clinical value to the NHS. Such a move would mean a major new role for NICE in helping to set the price of drugs. The pharmaceutical companies opposed the OFT report and in December the Government had still not made a definitive response. Given the role envisaged for NICE in the OFT report we decided to expand the scope of our inquiry to examine this issue.

8. In this report we look first at what NICE does and how it works, changes made since its establishment and the new challenges it faces. In the following three chapters we cover the criticisms made of NICE: first of the evaluation process, then of concerns about affordability and in Chapter 5 we look at implementation. In Chapter 6, we consider drug

pricing in the light of the OFT report. Finally, we summarise our main conclusions and recommendations.

## 2 Background

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9. When NICE was first established its role was:

to appraise the clinical benefits and the costs of those interventions notified by the Secretary of State and the National Assembly for Wales and to make recommendations.<sup>7</sup>

NICE initially appraised medical technologies (mainly medicines) and developed clinical guidelines. Shortly after its establishment, its remit was expanded to include interventional procedures. Since 2004, NICE has also examined public health approaches. In 2005, it changed its name from the National Institute for Clinical Excellence to the National Institute for Health and Clinical Excellence to reflect its new remit.

10. In this chapter we look at:

- What NICE does and how it works;
- International comparisons;
- Developments since 1999; and
- Successes, criticisms and future challenges.<sup>8</sup>

### The remit of NICE and how it works

11. NICE produces several types of guidance:

- Health technology appraisals (covering medicines and medical or diagnostic interventions). Technology appraisals of medicines are mandatory;
- Clinical guidelines (covering broader clinical practice). These are advisory and not mandatory; and
- Public health guidance. These are advisory and not mandatory.

As of December 2007, NICE had published 133 technology appraisals, 248 interventional procedure appraisals, 65 clinical guidelines and seven pieces of public health guidance.

These different types of guidance, and the processes involved in their development, are described below.

### *Health technology appraisals*

12. Health technology appraisals include assessments of medicines, devices (such as pacemakers and inhalers for asthma), surgical procedures and other interventions. The initial selection of health technologies for consideration by NICE involves both the

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<sup>7</sup> National Institute for Clinical Excellence Framework Document, June 2000

<sup>8</sup> The selection of topics and disinvestment are discussed in more detail in the next chapter

Institute and the Department of Health. Prior to September 2006, the Department referred topics directly to NICE; now possible topics are forwarded by the National Horizon Scanning Centre at the University of Birmingham to the Institute's seven new Consideration Panels. These panels, which are divided by therapeutic area, perform an early 'sifting' of the topics. The sifted topics are then referred to the Department of Health for final selection.<sup>9</sup>

13. NICE has mainly examined new interventions to date. However, as the Committee recommended in its first report on NICE, the Institute has now started to evaluate older treatments in order to encourage disinvestment by primary care trusts (PCTs) in treatments that are likely to be ineffective.<sup>10</sup>

### *Technology appraisals (of medicines)*

14. The technology appraisal process is described in the box below.

<b>NICE technology appraisal process</b>
1. Consultees and commentators are identified. These may include national organisations, such as professional and research bodies, manufacturers, patient groups and those representing carers;
2. A scoping document is prepared, setting out what the appraisal will cover. Consultees and commentators may comment at this point;
3. NICE commissions an independent academic centre to review the available evidence on the technology in question and prepare an assessment report. Consultees and commentators may comment at this point.
4. An evaluation report is prepared. The assessment report and comments on it are brought together in the evaluation report;
5. An independent Appraisal Committee <sup>11</sup> considers the evaluation report and hears evidence from nominated groups, including the manufacturer. It then makes its first recommendations in the appraisal consultation document (ACD). Consultees and commentators have four weeks to comment on the ACD. The ACD is also made available online and is open to public comment;
6. The Appraisal Committee considers the comments on the ACD, then makes its final recommendations in the final appraisal determination (FAD). The FAD is submitted to NICE for approval. Consultees can appeal against the recommendations.
7. Guidance is issued. If there are no appeals, or an appeal is not upheld, the final recommendations are issued as NICE guidance.
Note: NICE's technology appraisal methodology is being reviewed. A draft 'Guide to the methods of Technology Appraisal' is now open for public consultation.

#### **Box 1.**

9 More 'sifting' occurs later, and the topics referred at this point may not necessarily end up as the subject of NICE guidance.

10 The first two topics to be examined specifically with a view to disinvestment are: *The surgical management of children with otitis media with effusion (OME) for children*, to be published February 2008. <http://www.nice.org.uk/guidance/index.jsp?action=byID&o=11659>; and *The prescribing of antibiotics for self-limiting respiratory tract infections in adults and children in primary care*, to be published June 2008: <http://www.nice.org.uk/guidance/index.jsp?action=byID&o=11816>

11 NICE's Technology Appraisal Committee is made up of individuals drawn from the NHS, patient and carer organisations, academia and the pharmaceutical and medical devices industries. Members stay on the committee for 3 years. The committee is divided into three branches, A, B and C. See [http://www.nice.org.uk/aboutnice/howwework/devnicetech/technologyappraisalcommittee/technology\\_appraisal\\_committee.jsp](http://www.nice.org.uk/aboutnice/howwework/devnicetech/technologyappraisalcommittee/technology_appraisal_committee.jsp) for details.

### Single technology appraisals and multiple technology appraisals

15. Technology appraisals are divided into single technology appraisals (STAs) and multiple technology appraisals (MTAs).

16. The STA process was introduced in September 2005. This procedure is used to assess a single product for a particular condition. An STA should be completed within 9-12 months. According to NICE, if the technology is a new medicine and the process starts around the time the manufacturer requests marketing authorisation, and there is no appeal, it should issue guidance on use of the treatment within the NHS within three months of the drug obtaining a licence.

17. An MTA compares several different types of treatment for the same condition, and so the guidance is longer and more detailed. An MTA should be completed within 24 months.

18. The funding of products recommended by technology appraisals is mandatory; if the treatment covered by a health technology appraisal is requested by a clinician, the PCT or trust must be in a position to fund it within three months of the guidance being published.

### NICE's recommendations

19. NICE technology appraisals to date have been fairly evenly spread between wholly negative, wholly positive and positive with major or minor restrictions. Its decisions between 1999 and 2005 were:

- negative (a 'no' decision) in 19% of cases;
- positive ('yes') in 23% of appraisals;
- 'yes with major restrictions' in 32% of cases; and
- 'yes with minor restrictions' 26% of cases.<sup>12</sup>

20. NICE may recommend that a technology is used 'only in research', which means that the treatment is available from the NHS but only as part of a controlled clinical trial. This option is rarely used, and some believe that a recommendation of 'only in research' is equivalent to a rejection of the product or approach.<sup>13</sup> However, there are occasions when a recommendation that a technology be used only in research has been followed up with effective testing and led to eventual approval of the technology by NICE. For example, liquid cytology for cervical cancer screening was recommended only in research by NICE in 2000. Pilot studies and subsequent review led to a positive recommendation of the technology by the Institute in 2003.<sup>14</sup>

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12 Ev 230

13 Chalkidou K et al, *Journal of the Royal Society of Medicine* 2007, 100: 453-460

14 *Ibid*

### **Interventional procedures**

21. NICE guidance on interventional procedures may include diagnostic tests or procedures that involve accessing the inside of the body. Examples of interventional procedure guidance issued by NICE include *Deep brain stimulation for Parkinson's disease* and *Customised titanium implants for orofacial reconstruction*.

22. The steps involved in the appraisal of interventional procedures are similar to those included in appraisal of medicines, in that consultation takes place before guidance is issued, and stakeholders may challenge draft guidance. There are differences in the means of topic selection, however, in that procedures suitable for evaluation are suggested by the public (usually by a clinician). The full process is described in the Annex.

### **Clinical guidelines**

23. Clinical guidelines advise on the appropriate treatment and care of patients with specific conditions. Their implementation is not mandatory. NICE often works with external professional bodies and academic centres, via its National Collaborating Centres (NCCs), to compile this type of guidance. The process of clinical guideline development is described in the Annex.

24. Clinical guidelines take around 24 months to produce. These guidelines are long and detailed and changes are underway to produce a greater number of shorter guidelines on narrower areas. Such guidelines address areas where there is a particular clinical question, but not the need for a full-scale guideline. A short guideline is produced in 9–11 months.

### **Public health guidance**

25. Public health guidance provides indications of how good health can be promoted and ill health avoided. It is aimed at those working in the NHS, local authorities and the voluntary sector as well as the general public.

26. NICE produces two types of guidance on public health. The first, public health intervention guidance, focuses on specific interventions that encourage a healthy lifestyle. Published intervention guidance includes *Smoking cessation* and *Preventing sexually transmitted infections and reducing under-18 conceptions*.

27. The second type of public health guidance developed by NICE is public health programme guidance. This provides broader advice on promoting health and avoiding ill health. It may focus on a population, activity or setting. Published topics of public health programme guidance include *Behaviour change*, which set out the planning, tools and types of skill needed in order to change health-related behaviour. Programme guidance currently being developed includes *Alcohol use disorders in adults and adolescents*, which covers issues relating to prevention and early identification of alcohol use disorders, and *Community engagement*, which is an assessment of development approaches to improving health and reducing health inequalities, such as the use of citizens panels and juries, and community champions. The process of public health guidance development is described in the Annex.

## NICE evaluation

28. All the processes described above require the evaluation of the information available on the drug, intervention, procedure, approach or condition in question. This information may be limited or flawed. Therefore the Institute must make “social and scientific value judgements” in relation to the evidence.<sup>15</sup> Social value judgements concern the principles involved in the care and treatment of patients by the NHS; scientific value judgements concern how the available evidence is interpreted.

29. NICE, in collaboration with its Citizens Council<sup>16</sup>, defined a series of social value judgements that should be considered during the development of guidance. Key principles include:

- The need for consistency in the guidance development process;
- NICE should not recommend interventions when the evidence base is weak;
- The need to look beyond cost per quality-adjusted life year (QALY, see Chapter 3) when considering cost-effectiveness;
- Discrimination between different groups of patients on the basis of age or any other factor should be avoided, unless there is clinical evidence of a difference in the effect of treatment in such groups (eg. a treatment may only be effective in patients in a certain age range, or with a specific gene);
- NICE should respond to comments from stakeholders and consultees and amend guidance where necessary.<sup>17</sup>

Departures from these principles should be explained.

30. Scientific value judgements require an assessment of the type and quality of evidence presented as well as the results of the various analyses. These factors contribute to decisions relating to the cost effectiveness of a treatment or intervention. NICE makes great use of QALYs when judging cost effectiveness. The technical processes involved in NICE evaluations are discussed in the next chapter.

## Appeals

31. NICE decisions may be appealed by a range of stakeholders, including manufacturers and patient or professional organisations. By November 2007, NICE had published 130 technology appraisals, 47 of which had been appealed. When appeals are upheld, often only small changes to the wording are required. See the table below for the numbers of appeals that were upheld or rejected in every year since the establishment of NICE. We describe the appeals process in more detail in Chapter 3.

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15 Ev 5

16 A committee of 30 lay people. The membership changes regularly.

17 Ev 10

Year	Technology appraisals published	Appeals submitted	Appeals allowed (withdrawn or dismissed without hearing)	Appeals upheld after hearing	Appeals dismissed after hearing
2000	17	8	8(0)	2	6
2001	14	5	4(1)	1	3
2002	24	11	10(1)	7	3
2003	19	4	4(0)	1	3
2004	13	5	2(3)	1	1
2005	6	3	3(0)	0	3
2006	20	6	6(0)	3	3
2007	17	5	5(0)	4	1
<b>Total</b>	<b>130</b>	<b>47</b>	<b>42(5)</b>	<b>19</b>	<b>23</b>

Table 1.

## International comparisons

32. Many organisations concerned with the evaluation of medicines and other health interventions exist elsewhere. The Committee visited Scotland, France and Canada to learn about the arrangements in place in those countries.

### Scotland

33. NICE guidance applies in England, and much of its guidance also applies in Wales and Northern Ireland. Although some NICE guidance applies in Scotland (such as that relating to interventional procedures), the Scottish Executive has established its own arrangements for the assessment of medicines (equivalent to NICE technology appraisals) through the Scottish Medicines Consortium (SMC). Advice on clinical practice (equivalent to clinical guidelines) is produced by the Scottish Intercollegiate Guidelines Network (SIGN).

#### Scottish Medicines Consortium

34. The SMC assesses and makes recommendations on all new drugs. That is, it provides advice to NHS Boards and their Area Drug and Therapeutics Committees across Scotland about:

- all newly licensed medicines;
- all new formulations of existing medicines; and
- all new indications (ie. conditions) that existing medicines are licensed to treat.

35. Unlike NICE health technology appraisals, SMC recommendations are not mandatory. The following classification system is used:

- Unique: all NHS Boards should make these drugs available within three months;
- An advance on alternatives/the same as alternatives: Area Drugs and Therapeutics Committees within each Board will decide whether to allow the use of the drug in their area;

- Worse than alternatives: Boards are advised not to use the medicine.<sup>18</sup>

36. In comparison with NICE technology appraisals, less in-depth analysis informs SMC decisions. There are no published processes and methods to which SMC must adhere. It has no scoping phase and public consultation is limited. The whole process is swifter, lasting around 4 months.<sup>19</sup>

37. The pharmaceutical industry is represented on the committee that evaluates the drug. There is more dialogue between the Consortium and the manufacturer throughout the SMC process compared to that of NICE. Perhaps for this reason, appeals are rare. When they do occur, SMC will review a decision on the basis of process or scientific issues.

#### Scottish Intercollegiate Guidelines Network

38. SIGN develops national guidelines for use by clinicians working within NHS Scotland. Like NICE clinical guidelines, they are not mandatory. Guidelines take between 24 and 30 months to compile.

39. Topics are selected on the basis of clinical uncertainty, strength of the evidence of treatment efficacy, risk, priority for NHS Scotland and perceived need. Anyone can propose topics for consideration by SIGN. Topics are sent to one of a number of speciality subgroups, overseen by the Guideline Programme Advisory Group. SIGN Council, which comprises representatives from medicine, nursing, healthcare management, research, social care and patient groups, makes the final topic selection.

40. SIGN guidelines are then developed by multidisciplinary working groups, which include healthcare professionals, NHS managers, researchers and patients. The guideline development groups are selected in consultation with the member organisations of SIGN.

41. There is no formalised relationship between SMC and SIGN.

#### *France*

42. In France, the French Haute Autorité de la Santé (HAS) is an independent body that provides medical and scientific advice to the French Government. Much of its remit mirrors that of NICE including:

- The production of health technology assessments;
- The production and promotion of clinical guidelines;
- The provision of public health guidance.

43. HAS assesses more technologies than NICE and performs its assessments more quickly; as a result, its assessments tend to be based on less evidence than those of NICE. See the box below for details of the HAS appraisal process.

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18 Office of Fair Trading, February 2007. Annexe B: Review of NICE, SMC and AWMSG:  
[http://oft.gov.uk/shared\\_oft/reports/comp\\_policy/oft885b.pdf](http://oft.gov.uk/shared_oft/reports/comp_policy/oft885b.pdf)

19 The SMC aims to provide advice to the NHS in Scotland within 3 months of product launch.

**HAS technology appraisal process**

HAS has two types of health technology assessments: a full-scale assessment and a rapid assessment. Topics covered by the full-scale assessment are broader than those covered by NICE technology appraisals. In addition to evaluating pharmaceutical products and medical devices, it assesses areas such as screening programmes, diagnostic procedures, the distribution and replacement of medical equipment, medical research and changes in the legislative framework.

The rapid assessment programme evaluates the benefit of medicines, devices, diagnostic techniques and therapeutic procedures and advises on whether they should be reimbursed by the French healthcare system. This work is done by the Committee for the Assessment of Medical Devices (CEPP) for devices and the National Agency for Accreditation and Evaluation in Healthcare (CEAP) for diagnostics and therapeutic procedures. The Transparency Commission provides this service for medicines.

The Transparency Commission assesses all new medicines, once licensed, that are subject to applications for reimbursement by the state. Its opinion is used to assess the benefit provided by a new drug and the improvement of a medical service subsequent to its use. External experts assist the Commission in its work. HAS assesses the stand-alone effectiveness of each technology, as well as its effectiveness in comparison with other available technologies (ie. the absolute and comparative clinical effectiveness of the drug). Unlike NICE the Transparency Commission assesses a medicine before its price has been set.

Recommendations from all three groups are passed on to the Economic Committee for Health Products (CEPS), which negotiates with industry to fix the price of drugs and devices. A further body, the Association of National Health Insurance Funds, which fixes the reimbursement rate for medicines and sets tariffs for procedures, also receives advice from the Transparency Commission, CEPP and CEAP.

The Ministry of Health makes the final decision regarding the drugs and devices that will be reimbursed by the state. A product cannot be launched in France until it has gone through the Transparency Commission and a price is agreed by CEPP.

In 2006, HAS carried out nine full-scale health technology appraisals, 1,192 rapid appraisals of medicines, 134 of medical devices, and 130 of diagnostic and therapeutic procedures.

**Box 2.**

44. In addition to technology appraisals, HAS is involved in the development of disease management programmes for chronic conditions, the promotion of medical information and products, such as prescription software, and ensuring the quality of information provided by, for example, medical sales representatives. HAS also advises drug companies on the protocols used for post-marketing clinical trials, and validates these protocols.

45. HAS is responsible for accrediting both healthcare organisations and individual clinicians and continuing professional development. As a result, it is able to monitor the uptake of its recommendations.

**Canada**

46. The Canadian equivalent of NICE is the Canadian Agency for Drugs and Technologies in Health (CADTH). CADTH advice applies in all the provinces and territories of Canada except Quebec.

47. CADTH has three core programmes:

- the Common Drug Review (CDR), which assesses new drugs;<sup>20</sup>
- the Health Technology Assessment, which conducts wider assessments of drugs and other health technologies;
- the Canadian Optimal Medication Prescribing and Utilization Service (COMPUS), which identifies and promotes best practices in drug prescribing and use.

48. The CDR was established in 2003 by federal, provincial and territorial Health Ministers to avoid duplication of drug reviews by public drug plans, improve the quality and consistency of the review processes; and address the differences in drug coverage among the publicly funded drug plans.

49. The CADTH Health Technology Assessment programme takes a broader look than the CDR at areas of healthcare, or drug classes, and is more closely comparable to NICE's multiple technology appraisals or its clinical guidelines. The programme aims to provide information about the clinical effectiveness, cost-effectiveness, and wider impact of drugs, medical technologies, and health systems. Recent topics examined include *Technologies for Aiding Reduction of Medication Errors in Hospitals*, *Telemedicine for Acute Stroke Management (Telestroke)*, and *Procedural Sedation in the Emergency Department*.

50. The COMPUS programme was created in 2004. It identifies and promotes evidence-based, clinical and cost-effectiveness information on optimal drug prescribing and use. For each priority topic, it produces a series of optimal therapy reports, aimed at policy-makers, provincial drug plans, prescribers and patients. It also provides strategies, tools, and services to encourage the use of evidence-based clinical and cost-effectiveness information in decision-making among healthcare providers and consumers.

51. The CDR and Health Technology Assessment relate most closely to NICE's health technology appraisal processes. See the box below for more details. COMPUS is not directly comparable to any of the programmes run by NICE, although similar types of information are included in NICE's evaluation of treatments and in its guidance.

#### **CADTH technology appraisal processes**

##### *CDR*

Once Health Canada has approved a drug for sale in Canada, the drug must be submitted to the CDR if the manufacturer wishes to obtain coverage under any of Canada's public drug plans (with the exception of Quebec). The CDR process is as follows:

1. A drug is submitted by CADTH's Advisory Committee on Pharmaceuticals, a drug plan or manufacturer;
2. A review team is established;
3. The evidence (that submitted and extra independently sourced material) is systematically reviewed;
4. The review is edited and assessed for quality before it is sent to the manufacturer for comment;
5. A dossier including the review, manufacturer's comments and a response to those comments is

<sup>20</sup> For potential coverage by participating publicly funded federal, provincial and territorial drug benefit plans

sent to the Canadian Expert Drug Advisory Committee (CEDAC);

6. CEDAC considers the submission and makes recommendations, which are sent to drug plans and the manufacturer in confidence;

7. An embargo period ensues, during which time clarification or appeals may be sought;

8. The final recommendations are made, sent to the drug plan and manufacturer and then released to the public.

#### *Health Technology Assessment*

This examines four questions:

- How will this health technology affect the health of Canadians?
- How does it compare with alternatives?
- Does it provide value for the investment?
- Are there other health service implications to consider?

There are several steps involved in the technology assessment process:

1. Topic selection. Topics can be suggested by anyone, including members of the public, medical directors or medical societies, or may come from CADTH's own Horizon Scanning Program;

2. Definition of the question the assessment will address. The Advisory Committee, the organisation or individual that suggested the topic and other groups are consulted at this point;

3. Formation of a project team and protocol development;

4. Assembly and review of the evidence. Published and unpublished evidence is brought together for analysis;

5. Writing the report;

6. Reviewing the report. External experts and members of CADTH's Scientific Advisory Board peer-review the report;

7. Dissemination of the final report.

#### **Box 3.**

52. CADTH's guidance is not mandatory. However, the Agency encourages uptake of its guidelines through the use of individual Liaison Officers, who are located in most provinces and work with local users and stakeholders.

53. To summarise, there are a number of differences between NICE and the organisations in Scotland, France and Canada. In Scotland, the SMC carries out a shorter, less in-depth evaluation of all new medicines and indications at the time of launch. It does not examine anything else. SIGN produces clinical guidelines along similar lines to those of NICE, but does not look at any other areas. HAS and CADTH examine similar areas to NICE but there are differences in their processes. In France, medicines are examined at the time of launch; they are not available to patients until they have been assessed by the Transparency Commission. Uniquely, as far as the bodies mentioned here go, pricing negotiations take place after evaluation. In Canada, new medicines are not available on publicly funded health plans until they have been evaluated by the relevant body.

## **Developments since 1999**

54. The way in which NICE operates has changed since its establishment. Here we discuss a number of important factors that have affected the work of the Institute:

- This Committee's first report on NICE;
- The mandatory funding of technology appraisals;
- Sir David Cooksey's review of the health research budget;
- Political interference in NICE's work; and
- The judicial review of Alzheimer's medicines.

### **First Health Committee report on NICE**

55. The remit of NICE has changed and expanded since 1999. Our predecessor Committee played a major role in influencing these changes. In 2001/02, the Committee undertook an inquiry to determine whether NICE was meeting the requirements laid upon it.<sup>21</sup>

56. The report emphasised that a body such as NICE was necessary to provide evidence-based clinical advice. The Committee highlighted the achievements of the Institute over the first few years of its existence, particularly in developing a robust appraisal process, but also saw room for improvement. It stressed the need for better information, an improved appeals system, better implementation and more emphasis on producing clinical guidelines. Other recommendations included the need for:

- Greater involvement of the NHS, patient groups and other stakeholders in the development of NICE technology appraisals, and the use of more information relating to patient and carer experiences and quality of life in NICE guidance;
- Closer working with the then Medicines Control Agency (now the Medicines and Healthcare products Regulatory Agency, MHRA) to improve the access of NICE to relevant information;
- Reform of the appeals process (including removal of the Chair of NICE from the proceedings);
- The need to ensure that no conflict, or risk to NICE's credibility, arose as a result of actions or recommendations issued by the Department of Health. This concern was raised in relation to policy on medicines for multiple sclerosis (MS);
- Publication of technology appraisals at the time of drug launch, or of 'interim' appraisals when a full-scale appraisal is not possible; and
- Improved monitoring of the implementation of NICE guidance, and research into systems within trusts that could improve levels of implementation; and
- A review of NICE by an independent body.

57. The Government accepted that NICE should produce more clinical guidelines rather than focus on technology appraisals alone. It also agreed that measures were needed to ensure that companies submitted all relevant information to NICE. The Government

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21 As set out in *A first class service: quality in the new NHS*

conceded that more information on quality of life and wider societal cost : benefit analysis would improve NICE guidance and that improvements in the monitoring and implementation of guidance were needed.

58. NICE itself told us that it had made many changes in response to the Committee's first set of recommendations. These included:

- The extension of the involvement of interested stakeholders in the guidance development process and increased opportunities for stakeholders such as PCTs, to contribute;
- Closer working with the MHRA, including the sharing of information;
- Greater transparency regarding what information NICE uses when making its decisions;
- Removal of the Chair from the Appeals Committee;
- Faster reviews of drugs (with the aim of producing guidance on new technologies at launch);
- Improvements to implementation, through the establishment of the Implementations Systems Directorate.

On the other hand, a number of our recommendations have not been fully implemented, notably in relation to disinvestment in less effective therapies and NICE's access to all unpublished information.

### **Funding of technology appraisals**

59. In the years following the establishment of NICE, it became clear that not all technology appraisals were being uniformly implemented.<sup>22</sup>

60. To improve consistency in patients' access to treatment, the Secretary of State, Dr John Reid MP, made it a legal requirement that funding for all positive advice arising from technology appraisals should be made available within three months of publication.<sup>23</sup> This requirement only applied to technology appraisals.

### **Cooksey report**

61. In December 2006 Sir David Cooksey published a report on healthcare research and development.<sup>24</sup> It highlighted the world-class health research which takes place in the UK, but stated there were weaknesses both in the way that new research was 'translated' into the development of treatments and products to treat disease and in the way such products, once developed, were introduced to the health system.

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22 Eg. Civitas 2003. *Nice or nasty: has Nice eliminated the 'postcode lottery' in the NHS?*

23 Department of Health, July 2003. *Directions to PCTs and NHS trusts in England concerning arrangements for the funding of technology appraisal guidance from the National Institute of Clinical Excellence*

24 HM Treasury, 6 December 2006. *A review of UK health research funding*. [http://www.hm-treasury.gov.uk/media/4/A/pbr06\\_cooksey\\_final\\_report\\_636.pdf](http://www.hm-treasury.gov.uk/media/4/A/pbr06_cooksey_final_report_636.pdf)

62. The Cooksey report made recommendations to promote health research in the UK, and maximise the health and economic benefits of the new single research budget.<sup>25</sup> Those recommendations which most concerned NICE related to the development of new, and particularly innovative, technologies. In particular, the report stressed that NICE should evaluate drugs more quickly. The recommendations included:

- A greater focus on the development of therapies for areas of unmet medical need, with evidence-based input from the Department of Health and devolved administrations on their priorities for healthcare;
- Advantages for therapies addressing areas of unmet need, such as expedited approval of clinical trials and faster consideration of new technologies by NICE;
- Fostering more support for health research from the NHS, with a more systematic approach to the uptake of new technologies by the health service;
- Pilot schemes to test the benefits of NICE involvement at an early stage in drug development should take place, with NICE and the pharmaceutical industry working together to identify emerging technologies suitable for inclusion;
- Clearer processes for following up recommendations provided by NICE to manufacturers should be developed.

63. If implemented, the recommendations would mean that new therapies addressing areas of unmet need would be brought to market quicker than at present. A new “drug development pathway”, including the “conditional licensing” of particularly promising treatments, would support this endeavour. According to the Cooksey report, these changes would benefit patients primarily but also the healthcare industry, Government and the wider economy.

64. NICE is currently consulting about the Cooksey report’s recommendations.

### **Political interference**

65. During the inquiry witnesses expressed concern that health ministers had undermined the work of NICE.<sup>26</sup> We were given two examples. The first involved the risk-sharing agreement established to allow NHS patients access to treatments for MS. The second concerned the breast cancer drug trastuzumab (Herceptin).

66. NICE evaluated beta interferon and glatiramer acetate for MS in 2001. In 2002, it did not recommend the treatments as neither was judged to be cost-effective. A public outcry followed. Witnesses believed that as a result of this outcry the Department of Health sought a way to make the drug available despite the NICE recommendation.<sup>27</sup> The Department came to an agreement with manufacturers of the drugs whereby the drug could be used in the NHS as part of a 10-year trial. It was agreed that if the drug was less effective than

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25 A single, ring-fenced budget was set by the Treasury in 2006 for the purpose of health research and development

26 Q 322. Dr Tim Crayford

27 From *Testing Treatments*, Evans et al. Cited in Ev 248, NICE 117

£36,000 per QALY, industry would recompense the NHS. However, there have been problems with the scheme; in particular it has proved impossible to assess how cost-effective the drug has proved in practice (see box on page 89). We asked to see the analysis produced by the University of Sheffield, including appendices, which the Department of Health has had for over 12 months, but at the time the Committee agreed the report, the Department had not made a decision about our request.

67. Witnesses were also concerned about the former Secretary of State's role in the case of trastuzumab. In November 2005, North Stoke PCT reversed its earlier decision to refuse the drug to a patient with early stage breast cancer<sup>28</sup> after the Secretary of State announced that she was "very concerned", called a meeting with PCT officials and asked PCTs to consider assessing the receptor status of their patients on the assumption that if the drug became available it could be licensed quickly.<sup>29</sup> Trastuzumab had not been assessed by NICE, or licensed for use in early stage breast cancer, at this point. However, evidence of the drug's efficacy for this type of cancer had been published on the manufacturer's investor website. The patient had threatened to take the PCT to the High Court to contest its original decision not to allow her access to the drug. Shortly after, the Department asked NICE to examine the drug early. Professor Mike Richards described this as "a political decision".<sup>30</sup> Professor Rawlins stated:

The scientific basis for Herceptin being effective and safe in early cancer had not been demonstrated ... Added to which, at that stage the company had not even made an approach to the regulatory authorities, it had not made an application, so it was a surprising remark from a Secretary of State.<sup>31</sup>

68. We questioned the Minister of State, the Rt Hon Dawn Primarolo MP, about political interference in NICE's work. We were told:

I would absolutely stress that it is not the role for ministers to contradict, override or directly seek to influence a process where NICE are already engaged in consideration.<sup>32</sup>

She added that "NICE's final guidance will be final".<sup>33</sup>

**We note that it is not the role for Ministers to directly or indirectly seek to influence the NICE decision-making process.**

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28 *British Medical Journal*, 2005; 331:1162

29 *Times*, 8 November 2005, <http://www.timesonline.co.uk/tol/news/uk/article588013.ece>

30 Q 70

31 Q 649

32 Q 720

33 Q 728. The Minister was referring to NICE guidance on drinking alcohol during pregnancy. The Department of Health recommended in May 2007 that pregnant women should avoid alcohol completely. In October 2007, NICE draft guidance stated that moderate amounts of alcohol were not harmful. Final guidance from NICE has not yet been published.

## Judicial review

69. In March 2007, a judicial review was brought against NICE by the drug company Eisai, supported by the Alzheimer's Society and other patient groups. Eisai and Pfizer jointly market the medicine donepezil (Aricept) for the treatment of Alzheimer's disease. The case concerned guidance issued by NICE stating that NHS patients with newly diagnosed, mild Alzheimer's disease should not be prescribed the drugs donepezil, rivastigmine and galantamine, as treatment was not cost-effective. Rather, only those with moderate disease should receive the drugs. The case was brought on three grounds:

- Procedural. NICE refused to disclose a fully executable version of the cost-effectiveness modelling tool used by its Collaborating Centre, Southampton University, to evaluate the treatments for mild Alzheimer's disease. Instead a 'read-only' version was released to the manufacturers. The claimant stated that the process leading to the Final Appraisal Determination (FAD) and the resulting treatment guidance breached the principles of procedural fairness.
- Irrationality. Some of the assumptions made or conclusions drawn in the FAD were irrational or could not be supported.
- Discrimination. It was claimed that the use of Mini Mental State Examination scores as a rigid diagnostic tool discriminated against certain patient groups.

70. Only the last point was upheld. In August 2007, the judge ruled that the guidance did indeed discriminate against some patient groups and ordered NICE to change its guidance to reflect this finding.<sup>34</sup>

71. In September 2007, Eisai applied to the Court of Appeal for leave to appeal the finding in respect of access to the Southampton University model. The company has been granted leave to appeal.<sup>35</sup>

## Successes and criticisms

### Successes

72. Many witnesses stressed that the processes used by NICE were well-established and robust.<sup>36</sup> The different procedures for the evaluation of medicines and interventional procedures, and the development of clinical guidelines and public health guidance have been carefully considered.<sup>37</sup> They provide a good example of evidence-based working.

73. NICE has clearly attempted to incorporate effective consultation into its topic selection and guidance development processes. It consults widely and a variety of different groups,

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34 *Pharmaceutical Marketing*, 28 September 2007. <http://legalnewsarchive.blogspot.com/2007/08/alzheimer-judgment-in-full.html>

35 The Court of Appeal has agreed to hear arguments from Eisai relating to the process used when NICE decided not to recommend the drugs for patients with newly-diagnosed mild Alzheimer's disease. <http://www.pharmafocus.com/cda/focusH>

36 Ev 2, 231, 70, 192

37 Ev 202

ranging from patient and carer organisations to professional bodies and the healthcare industry, have the opportunity to contribute to the draft guidance.<sup>38</sup>

74. NICE is well regarded internationally. An external review of NICE by the World Health Organization commended the appraisal processes used by the Institute.<sup>39</sup> Many countries look to NICE decisions when determining the cost-effectiveness of treatments for their own populations. Some of its clinical guidelines are regarded as the international gold standard of medical practice.<sup>40</sup>

75. Most importantly, NICE has encouraged the NHS and industry to put more focus on cost-effectiveness. It has also encouraged medicines manufacturers to produce evidence of the cost-effectiveness of their products, albeit with limited success to date.<sup>41</sup>

### Criticisms

76. Over the course of the inquiry, however, we became increasingly aware that the job of NICE is more difficult than was first imagined. It faces difficulties related to prioritisation of topics for evaluation and the strength of the evidence on which it must base its decisions. There has been concern about the phenomenon known as ‘NICE blight’ and the implementation of guidance. Inevitably NICE makes controversial decisions which attract criticism. As a result there is a danger that public confidence in its work is affected.

77. Witnesses referred to three main areas of criticism:

- The evaluation process, including the speed of guidance and selection of topics;
- The affordability of guidance; and
- Implementation.

These criticisms are discussed in more detail in later chapters.

### Future challenges

78. Rationing occurs in all healthcare systems; not every treatment can be provided to all patients.<sup>42</sup> Decisions about what is affordable are likely to become ever more contentious. Public expectations of the NHS are rising. At the same time, more expensive new drugs are coming to market and the rate of growth in funding in the NHS is declining. As a result, the role of NICE in ensuring that the most cost-effective treatments are available to the patients that need them is becoming ever more important and ever more difficult.

**79. It is clear that the environment in which NICE operates has changed considerably since the Institute was established in 1999. It is also clear that there is a vital role for NICE in the rationing of healthcare and in encouraging best clinical practice. In the**

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38 Ev 62, 229

39 Ev 248

40 Eg. schizophrenia. Chidgey et al, *Journal of the Royal Society of Medicine* 2007, 100: 448–452

41 See paragraphs 121–134

42 Ev 77

**future the role of NICE will be ever more important and demanding with new expensive drugs and a slower rate of growth in NHS expenditure. There remains, however, concern about aspects of how NICE does its job.** How it should change to address these concerns is considered below.

## 3 The evaluation process

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80. The procedures used by NICE to evaluate new and existing therapies and interventions for use in the NHS have been developed over time. Some have changed as a result of both internal and external reviews.<sup>43</sup> However, problems remain. The evaluation process was at the heart of many of the criticisms raised over the course of this inquiry. This is discussed below.

81. Witnesses highlighted a range of issues relating to the evaluation process, including:

- The quality and scope of the process;
- The appeals system;
- The speed of guidance.

82. Technology appraisals were the main focus of criticism with some criticisms also applying to clinical guidelines. We received little evidence about the appraisal of interventional procedures or the development of public health guidance, but some of the points made were also relevant to these types of guidance.

### The quality and scope of the evaluation process

83. NICE has responded to the changing environment in which it operates by introducing changes of its own, such as its fast-track system for the appraisal of specific medicines. The ongoing development of NICE's methods of working and its open discussion of them were praised by the Department:

It [is] right that NICE's process and methodology is the subject of continued development and debate and [the Department] welcomes the open and consultative approach NICE takes to the development of its work.<sup>44</sup>

Many witnesses also praised the robust nature of the processes used by the Institute to evaluate interventions and treatments. For instance, the British Psychological Society stated:

The methods chosen by NICE are rigorous, transparent and stand scrutiny by any international standards.<sup>45</sup>

84. Nevertheless, concerns about how medicines and other treatments are assessed by NICE were raised by many witnesses. In this section we discuss:

- Topic selection;
- Problems with the use of QALYs, including the range of costs taken into account;

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43 For example, the Committee's report in 2001 and the review by the WHO in 2003

44 Ev 2

45 Ev 192

- The evidence on which NICE bases its recommendations;
- Expert involvement in the development of guidance; and
- Consultation.

### *Topic selection*

85. As we described earlier,<sup>46</sup> NICE, in collaboration with the Department of Health, uses information provided by the Horizon Scanning Centre at the University of Birmingham to select the topics to be examined by the Institute as health technologies, clinical guidelines and public health guidance. Most of our evidence related to topics selected for health technology appraisal.

86. NICE does not assess every new medicine which enters the market, or every new indication for that medicine.<sup>47</sup> Instead, the NICE technology appraisal programme is limited to a small number of new, often expensive, products chosen to address “the questions that those who are delivering services in the UK healthcare system need answering”.<sup>48</sup> This means that most medicines and other technologies which are prescribed in England have not been assessed by NICE. PCTs told us that NICE-evaluated treatments constituted only a “fairly small percentage of [their] work”.<sup>49</sup>

### *Skew towards secondary care*

87. Witnesses argued that the selection of only a small proportion of interventions, and more specifically new drugs, skewed NHS spending towards new and expensive medicines for acute illness. We were told that this was likely to continue, with many more cancer medicines, for example, in the pipeline.<sup>50</sup> Some witnesses warned that the narrow selection of topics for technology appraisal caused greater focus on secondary care as opposed to primary care. The Association of the Directors of Public Health (ADPH) stated:

Since these are almost invariably more expensive than what went before and in the acute sector, the net effect is a diversion of resources away from lower tech and more affordable models of care, and from primary and community care into the acute sector...<sup>51</sup>

88. According to Dame Gill Morgan from the NHS Confederation, “There will always be a bias towards curative care”.<sup>52</sup> This is in contrast to recent Government policy, which has encouraged a move towards the provision of procedures and consultations in local settings that would previously have taken place in hospital.

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46 See Chapter 2

47 Unlike the Scottish Medicines Consortium (SMC); see above

48 Q 75. Andrew Dillon

49 Q 291

50 Q 31

51 NICE 111

52 Q 316

## Disinvestment

89. In keeping with the criticism outlined above, we heard that more should be done to encourage disinvestment from approaches and technologies that offer poor value for money.<sup>53</sup> This is an area that NICE has pledged to make a larger component of its work<sup>54</sup> but to date few older therapies have been covered by NICE guidance. Professor Appleby stated that more should be done to encourage PCTs in this regard:

The idea that if something is not killing you and may be doing you some good it is probably worth having is not how NICE or the NHS should operate. The area of disinvestment is a much more difficult area to go into, and that is why I believe PCTs need a lot more support. NICE could do more on that front, and I am sure it wants to.<sup>55</sup>

90. Research has suggested that PCTs are keen to improve commissioning to exclude less effective approaches.<sup>56</sup> It appears however that PCTs lack the information necessary to allow them to make decisions about disinvestment. Dr Tim Crayford of the ADPH told us that disinvestment information would be very useful for PCTs.<sup>57</sup> Professor Devlin confirmed:

PCTs are not always well informed of some of the data that could support them in decisions regarding either investment or disinvestment.<sup>58</sup>

91. Others argued that encouraging PCTs to disinvest in ineffective treatments should go hand in hand with a more general focus on cost-effectiveness. Professor Raymond MacAllister from University College Hospital told us:

There are clear areas of investment in health at present where I believe we are pretty wasteful....<sup>59</sup>

92. Questioned about this by the Committee, Mr Dillon denied that there was a “raft” of obsolete therapies being prescribed by clinicians.<sup>60</sup> He told us that NICE had not been able to identify any widespread use of out-of-date technologies:

The fact is that they are just not there in the way that people think they are. The health professionals do not indulge routinely and profitably in things that have absolutely no value whatsoever, to a level that would make it possible for us to say that there is a whole raft of things that should be stopped altogether.<sup>61</sup>

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53 Ev 180

54 Ev 4

55 Q 550

56 Q 527

57 Q 340

58 Q 529

59 Q 177

60 Q 655

61 Q 655

He added, however, that there were some cases of medicines being overused in inappropriate circumstances. In such cases, NICE could issue advice to ensure such treatments were used correctly.

93. NICE's argument is disingenuous. There are numerous interventions used in the NHS which have few benefits. The Cochrane Collaboration is discovering them all the time.<sup>62</sup> Of course, few are as Mr Dillon said "of absolutely no value whatsoever"<sup>63</sup>, but this is far too severe a test and certainly not one used when NICE performs its original health technology appraisals.

**94. It seems to us appropriate that topics are selected for interventional procedures, clinical guidelines and public health guidance. It is not appropriate, however, to limit technology appraisals to selected, often new and expensive, products. Instead, as we recommend below, all new drugs should be assessed.**

95. Witnesses were concerned that NICE's focus on acute treatments, in particular medicines, could skew NHS spending towards selected new and expensive (NICE-approved) drugs for acute illness. We discuss this further in the next chapter.

96. In our previous report we recommended that NICE give more emphasis to examining old technologies to encourage disinvestment. This the organisation has failed to do as fully as we expected. Its statement that few interventions have absolutely no benefit may be true but is irrelevant. Many treatments currently used are not cost-effective as many studies attest. NICE should adopt a similar standard of cost-effectiveness in assessing such treatments as it uses in its technology appraisals. The organisation must now give more emphasis to disinvestment. One approach would be to undertake more MTAs, which would reveal the existing treatments that provide poor value for money.

### **Quality-adjusted life years**

97. NICE requires a unit of measurement which enables it to compare different drugs or interventions. The unit it uses is the QALY. A QALY combines information about the benefits of a drug or intervention to provide a measure of both the extension of life and the improvement in quality of life.

98. According to NICE, the use of QALYs assumes that the extension of life and quality of life can be captured, for instance, with EQ5D.<sup>64</sup> This uses a visual analogue score and patient responses on:

- physical mobility;
- ability of patients to look after themselves;

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62 Cochrane Library: <http://www.cochrane.org/index.htm>. See also *BMJ Clinical Evidence*; *BMJ* 10 November 2007

63 Q 655

64 An assessment tool known as EQ5D or the EUROQOL, accessible at [www.euroqol.org](http://www.euroqol.org). A visual analogue score refers to a situation where patients respond to a question using a visual scale where one end of the spectrum represents, for instance, no mobility at all and other full mobility.

- ability to carry out normal activities of daily living;
- absence of pain and discomfort; and
- absence of anxiety and depression.<sup>65</sup>

99. A single QALY would indicate one year in perfect health. The value of a year in less than perfect health would be a fraction (eg. 0.5) of a QALY. Improvements in length and quality of life are referred to as fractions of a QALY.

100. To assess cost-effectiveness, the QALY score is integrated with the price of treatment using the incremental cost-effectiveness ratio (ICER). This represents the change in costs in relation to the change in health status. The result is a 'cost per QALY' figure, which allows NICE to determine the cost-effectiveness of the treatment.

101. NICE has stated that it uses a "threshold range" to determine whether the cost per QALY of a treatment offers value for money. It provides its advisory bodies with a framework for decision-making as follows:

- Below an ICER of £20,000 per QALY, judgements about the acceptability of a technology as an effective use of NHS resources are based primarily on the cost effectiveness estimate.
- Above an ICER of £20,000 per QALY, judgments about the acceptability of the technology as an effective use of NHS resources are more likely to make more explicit reference to factors including the degree of uncertainty of the ICER, 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 ICER of £30,000 per QALY the case for supporting the technology on these factors has to be increasingly strong. Recommendations for interventions costing more than £20–£30,000 per QALY must be explained.<sup>66</sup>

102. The system is an attempt to bring consistency to the measurement of health outcomes across different specialties and for different conditions. The cost per QALY does not indicate the price of annual treatment with the medicine. An expensive treatment may have a low cost per QALY if it brings significant benefit to patients; likewise, a cheaper treatment may have a high cost per QALY if the degree of benefit is relatively low. The table below, taken from an article by Professors Nancy Devlin and David Parkin, shows the authors' estimated cost per QALY for selected health interventions assessed by NICE.<sup>67</sup>

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65 Ev 8

66 Ev 8

67 Devlin N and Parkin D, *Health Economics* 2004, 13: 435–452

Intervention	Cost per QALY (£)
Smoking cessation	430
Asthma inhalers	5000
Zanamavir (for at risk groups)	20,400
Stents	25,000
Zanamavir (for all suitable patients)	38,000
Orlistat	46,000

**Table 2.**

*Devlin and Parkin, Health Economics 2004*

103. Both QALYs themselves and the way in which they are used were widely criticised in the evidence submitted to the inquiry. Some witnesses complained that NICE was too rigid in its assessments of cost-effectiveness and was over-reliant on the use of QALYs.<sup>68</sup> Some claimed QALYs failed to take into account other, equally valid, information and that the use of QALYs led to guidance that was unfair to particular groups of patients.<sup>69</sup>

104. In contrast, other witnesses argued that a method of comparing different treatments in an objective way which took account of longevity and quality of life was essential.<sup>70</sup> The Institute added that QALYs should “only inform, and not determine, NICE guidance”.<sup>71</sup>

### QALY assumptions

105. Many criticisms centred on the assumptions that are included in the calculation of QALYs. It was argued that assumptions about what exactly constituted the ‘quality’ for which life years are adjusted involved value judgements, such that the factors included in a QALY and the weight given to them may vary. Mr Steve Winyard from the Royal National Institute for Blind People (RNIB) described the difficulty in making such value judgments in relation to sight, or sight loss:

Whom do you ask? You want to measure the importance of sight to quality of life. Does one ask the sighted public, or people who are in the process of losing their sight, or do you ask those who lost their sight five years ago, for example? You will get different answers from those different groups. Which questions does one ask?<sup>72</sup>

106. Medicines manufacturers felt that the weighting given to different factors included in QALY calculations could be inappropriate. As Dr Richard Barker from the Association of the British Pharmaceutical Industry (ABPI) put it, “A model...is only as strong as the assumptions”.<sup>73</sup> Dr Rafiq Hasan from Novartis agreed:

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68 Q 608

69 Ev 164

70 NICE 105, 106

71 Ev 8

72 Q 615

73 Q 387

Sometimes one questions the assumptions that go into some of the models. We would welcome a broader debate about some of the assumptions that go into those models.<sup>74</sup>

107. Some professional groups also had little confidence in the use of QALYs. Dr David Anderson from the Old Age Faculty of the Royal College of Psychiatrists told us that experts often did not understand how some QALY-based decisions were made, and believed the process to be somewhat arbitrary:

When clinicians hear about health economic analyses many of them see it as made-up stuff. You just take some data and create an equation that is based on assumption after assumption... You fiddle about with an equation and come out with a number. If you want you can fiddle about with it some more and come out with a different number.<sup>75</sup>

108. Other professionals questioned the consistency of QALYs. Professor Raymond MacAllister from University College Hospital stated:

Depending on how complex the model is one can get an answer that can be inconsistent from one assessment to another.<sup>76</sup>

He warned that clinicians could easily lose faith in QALY-based economic assessments if they had just “one or two examples” of where the process had led to a seemingly inaccurate result. Moreover, he suggested that there was widespread suspicion of the use of the QALY as used by NICE:

The health economic assessment is a dark art for a clinician. It does not take much for someone who is a clinician, or even someone who is interested in drug policy, to be made sceptical about some of the outputs.<sup>77</sup>

### *Bias*

109. Witnesses argued that the use of QALYs could lead to bias against treatments for long-term, chronic conditions. Lower costs per QALY—and therefore greater likelihood of NICE approval—were claimed to be associated with treatments for acute conditions. Mr Winyard told the Committee:

Drugs that extend life will always achieve higher values ...

The use of QALY values puts people with long-term conditions at a disadvantage over people with life-threatening conditions.<sup>78</sup>

110. Witnesses contended that the reason for this skew towards acute illness was because quality of life measures were not rated as highly as other factors.<sup>79</sup> Drug manufacturers

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74 Q 606

75 Q 230

76 Q 187

77 Q 208

78 Ev 212

agreed that QALYs did not attribute sufficient importance to quality of life data. Dr Barker told us that, although QALYs were “a necessary tool”, they were insensitive to changes in quality of life that, although small, were important to patients:

The current process makes no distinction between an improvement of 0.2 of a QALY to 0.4 versus 0.8 to 1.0. If you are at the 0.2 level and you have a really poor quality of life, a doubling of your quality of life might be more valuable than a 20% increase or just over for a patient with a less severe condition.<sup>80</sup>

### Support for QALYs

111. On the other hand, several witnesses provided strong support for QALYs, pointing out that QALYs were necessary to ensure consistency when evaluating different treatments for different conditions. Professor Stirling Bryan from Birmingham University stated:

For many conditions the use of a QALY works well and allows for a common yardstick to be used in comparing very different conditions and... Thus, it is possible to ensure some degree of consistency when making decisions on therapies for heart disease and treatments for arthritis.<sup>81</sup>

112. Dr Tim Crayford of the ADPH described the QALY as “a great leveller”. He agreed that it should not be the only decision-making tool, but stated:

I think that that methodology, which looks at the potential benefit, the time at which it occurs in someone’s life and how long people might benefit, some sense of that going into an equation to help us weigh up whether some things are more cost-efficient than others, is a very good idea... some sort of measure that looks at the length of benefit and the size of benefit is absolutely necessary.<sup>82</sup>

113. While Professor Rawlins agreed that there were “uncertainties” about the details of the use of QALYs, he maintained that there was no better way of doing the work:

The QALY is a measure that allows us to compare the value, the health gain, of one intervention for one condition with another intervention for another condition. It has been researched extensively. There are hundreds and hundreds of articles about it. It has been investigated in Europe, in North America, in South America, even in China, and it is a reasonably robust approach to utility; in other words, the health gain.<sup>83</sup>

Mr Dillon added:

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79 Eg. those more clearly related to mortality or morbidity

80 Q 397

81 NICE 105

82 Q 316

83 Q 85

We believe QALYs are the best tool around at the moment to enable you to [understand the costs that will be foregone for other treatments] and it is important that that is applied consistently.<sup>84</sup>

114. NICE officials, and the Department of Health, also stressed that the cost per QALY calculation was not the only basis for NICE guidance, or even for its assessment of cost-effectiveness. Professor Rawlins told us that, “not everything can be expressed necessarily in QALYs”.<sup>85</sup>

115. Since the QALY will necessarily remain at the heart of NICE’s work, it is important to make the economic models they are used in as accurate and robust as possible. Professor Bryan recommended following up specific guidance after it had been promulgated to test whether the predictions of the model used in the cost-effectiveness analysis were borne out in reality:

If the conclusion was that there was a high degree of correlation between the predicted and actual results that would give us greater confidence in the modelling work.<sup>86</sup>

### *Wider economic benefits*

116. When NICE was established, the Department of Health insisted that the organisation should not take into account the economic benefits of treatment to carers, or savings related to benefits, tax allowances or productivity. Many witnesses argued that the exclusion of societal gains compromised the validity of QALY-based cost-effectiveness calculations.<sup>87</sup>

117. Professor Peter C Smith from York University told us that the system had first been developed as it had for very understandable reasons but thought that changes could now be made to incorporate the wider benefits of treatment:

[NICE] has been wise to keep the definition quite narrow and constrained so that everyone knows what it is trying to achieve with its figures, but I would hope that as we gain experience its methodology could begin to embrace broader benefits, and broader costs, associated with treatments.<sup>88</sup>

Others agreed.<sup>89</sup> The Academy of Medical Sciences stated:

NICE [should] take the overall burden of disease into account, to include societal costs to patient carers, unemployment costs or the expenditure of social services.<sup>90</sup>

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84 Q 116

85 Q 85

86 Q 196

87 Winyard, S: *The cost of sight loss in the UK*. RNIB campaign report 23 August 2004

88 Q 177

89 Ev 29, 37

90 Ev 23

118. NICE told us that it could consider personal social service costs, but was specifically precluded from considering costs such as those related to employment or disability by the existing legislation:

In our clinical guidance programmes, we take into account the cost to the NHS in personal social services. That is because our statutory instruments limit us to that perspective. It would be possible, if Parliament wished us to, to take a broader economic perspective, but that, to be honest, is your decision, not ours.<sup>91</sup>

119. The Minister agreed that there was “a real issue and a debate to be had” on the subject of including the broader costs to society in NICE’s cost-effectiveness assessments. She warned, however, of the potential bias of such considerations:

We need to be careful that we do not skew decisions away from certain groups within our community towards others...If we required [NICE] to consider the costs of not returning to work, would that skew away from the elderly and the long-term chronic diseases where people have no chance of returning to work because of their conditions? It seems to me that is the essential challenge and it is raised in other issues as well, such as the cost of care. It is difficult.<sup>92</sup>

**120. We heard much criticism of the use of QALYs. Some of the criticisms seem to be the special pleading of disappointed parties. It is vital that a method which allows comparison of the benefits and costs of different treatments for different conditions is used in cost-effectiveness evaluations. However, it is also vital that the system is accurate and reflects the real costs to society and the benefits to patients. We recommend that:**

- **Research is undertaken to follow up specific guidance to see whether the predictions of the cost-effectiveness analysis are borne out in practice;**
- **Wider benefits and costs, such as costs borne by carers and social care services, be more fully incorporated into NICE’s assessment. We were told that this would have to be a decision for Parliament.**

A discussion of the changes to the QALY threshold which would be needed to accommodate an earlier evaluation appear later in the report.

## **Evidence**

121. NICE must examine all the available evidence on a therapy or intervention during the development of its guidance. This includes meta-analyses, randomised clinical trials, observational studies, case studies and other types of evidence that are in the public domain.<sup>93</sup>

122. Witnesses referred to a number of problems regarding the evidence used by NICE:

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91 Q 87

92 Q 772

93 Technical terms mentioned here are defined in the glossary

- The lack of suitable evidence;
- The design of clinical trials;
- Poor use of the available evidence; and
- Lack of access to all the evidence.

### *Lack of suitable evidence*

123. Some types of evidence are considered more valuable than others for the assessment of medical treatments or procedures. For instance, a well-designed clinical trial involving large numbers of patients with an appropriate control group is more likely to yield robust results than a smaller scale observational study. NICE ‘grades’ evidence presented on this basis, as follows:

- Level 1 evidence (the highest quality) includes meta-analyses and systematic reviews of randomised controlled trials;
- Level 2 evidence includes high quality systematic review of case-control or cohort studies with a low risk of bias;
- Level 3 evidence includes non-analytic studies such as case reports;
- Level 4 evidence includes expert opinion or consensus.

In its guidance, NICE indicates the ‘strength’ for the basis of each recommendation.

124. NICE informed us that it was rare for all the evidence on a particular topic to be of a high standard:

The best available evidence is rarely (if ever) complete. It may be of poor quality, lack critical elements, or both.<sup>94</sup>

Evidence may be lacking in some specific areas because less research is done in the first place. Relatively few studies are undertaken in the field of public health for example. Professor Rawlins described this as an “information gap”<sup>95</sup> that could give other interventions an advantage, simply because more related research existed.

125. Even when the evidence exists, it may not present the relevant facts. Professor Carl Klaxton of York University stated that this was a particular problem for medicines:

There is constant frustration about the relevance and quality of the clinical trial evidence. As to its relevance, there are inappropriate comparators, primary endpoints that are not easily linked to ultimate health outcomes and a shorter follow-up than we would like to see. There are inclusion and exclusion criteria that do not match the NHS.<sup>96</sup>

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94 Ev 7

95 Q 316

96 Q 491

126. Professor Rawlins stated that there were several difficulties with the evidence provided by medicines manufacturers around or soon after launch. The first concerned a lack of health-related quality of life data. The second was the lack of information on comparator drugs, as most medicines are compared to placebo in clinical trials. The third, related concern was the lack of evidence of effectiveness compared to other classes of drug.<sup>97</sup> Finally, poor cost effectiveness data is a particular problem:

The much greater problem we have actually than all of that is the cost effectiveness, and the problem with cost effectiveness, to be honest with you, is that companies have not had experience of having to do sophisticated cost effective analyses, and they are on a steep learning curve.<sup>98</sup>

### *Design of clinical trials*

127. The type of clinical trial that drug companies undertake in order to gain a licence for their medicine does not necessarily provide the kind of information which is required for a consideration of cost- and clinical effectiveness for the NHS. Mr Ian Beaumont from Bowel Cancer UK recommended that NICE base its recommendations on the type of data more usually supplied by clinical trials:

Instead of finding the QALY and the overall survival rate, it would look at things like progression-free survival and disease-free survival for people after surgery. If NICE's appraisals and reviews were more in line with the way drugs actually worked then their method of evaluating them would be more meaningful.<sup>99</sup>

128. Other witnesses argued that the opposite should happen, ie. that clinical trials should move into the "real world" and aim to provide data that is more relevant for NICE. Dr Kiran Patel, a cardiologist from the South Asian Health Foundation, told the Committee that clinical trials often had design faults that meant they were not applicable to routine practice:

A lot of the trials concerned with myocardial infarction do not include people over the age of 80, but it is the over 80s who come into our coronary unit day in day out. If one applied evidence-based medicine down the line one would conclude that people over 80 are not represented in any clinical trials and therefore they should not be given any treatments within those trials. Clearly, we do not do that.<sup>100</sup>

He added that the patients who are most likely to receive medicines for heart disease have one or more comorbidity. Such patients are not included in clinical trials, again limiting the applicability of the evidence.

129. Moreover, even when drug companies make some effort to provide relevant evidence, the data generated is often difficult to use. For example, inappropriate surrogate (indirect)

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97 Q 667

98 Q 669

99 Q 263

100 Q 273

markers of efficacy are employed rather than direct measurements of patient-reported symptom improvement.<sup>101</sup>

130. Many witnesses argued that closer collaboration between NICE and medicines manufacturers during the drug development process was needed to improve the design of clinical trials.<sup>102</sup>

131. This has been highlighted before. Closer working between NICE and the manufacturer was a key recommendation of the Cooksey report. Dr Barker told us that there had been movement towards greater cooperation already:

In terms of better dialogue earlier in the process...so that the company knows what, in a sense, it needs to prove (in other words, what will be regarded as an economically sound case for this medicine), we are already in dialogue with NICE about how we might do that, how we might introduce it earlier in the process of development—not ten to 12 years before but maybe a year or two before launch—a dialogue on what the company ought to be able to prove.<sup>103</sup>

132. The better design of trials to take account of cost-effectiveness would be particularly effective if it occurred in all countries in which large-scale clinical trials take place.

133. NICE agreed that discussion with pharmaceutical companies during the development of drugs could be beneficial, particularly in reference to the ‘conditional licensing’ recommendations of the Cooksey report. Professor Rawlins stated:

We are discussing with other interested parties, particularly the MHRA...what sorts of arrangements we might have to put in place to enable us to look at the cost-effectiveness of drugs at an early stage. I do not think this is at all impossible and there are examples of really early drugs which we have looked at in the past where we have found it possible to do this, perhaps being a little bit more imaginative about the approaches we take.<sup>104</sup>

134. In addition, NICE could introduce incentives to encourage drug companies to design appropriate trials. NICE currently publishes a broad grading of the evidence it has received (see paragraph 123), on the basis of number of patients involved, whether a control group is used etc.; an alternative or additional measure would be to publish a detailed assessment of the quality of the research it receives.

### *Poor use of evidence*

135. Some witnesses thought that NICE did not use evidence properly, even when it was available. They claimed that the high value placed on certain types of research meant that the results of many useful studies were discounted. Lifeblood: The Thrombosis Charity blamed NICE’s “overly-prescriptive formulaic approaches to data gathering” for ignoring

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101 NICE 114. For example, trials of anti-arrhythmic medication might measure the reduced number of heart beats as opposed to patient-reported improvement or reduced mortality.

102 Q 263, 370, Ev 86

103 Q 387

104 Q 641

larger studies, with admitted design faults, while considering smaller randomised controlled trials with stricter protocols.

136. Witnesses also claimed that the studies which were ignored were more likely to be independent (ie. funded by an academic or other non-commercial source) than other, large-scale and expensive clinical trials. Given that the results of clinical trials are more likely to be designed and funded by the pharmaceutical industry and to favour the sponsor or manufacturer,<sup>105</sup> witnesses feared that this would lead to bias in guidance in favour of new products over older medicines or other, perhaps non-pharmacological, interventions. As Professor Mike Richards, the National Cancer Director, stated:

It is not just about drugs. There are a lot of other innovations that can save lives. Making sure people are aware of healthy life-styles factors, getting screening programmes introduced and early diagnosis are extremely important. Remember that we have to look at the whole of that.<sup>106</sup>

### Access to evidence

137. While NICE has access to published data, it does not have automatic access to the information relating to a particular therapy which is not in the public domain. Unpublished research, which the MHRA may use, is not necessarily available to NICE.

138. This Committee recommended in its first Report on NICE that all information be shared between the MHRA and NICE. This has happened to a limited extent. The Institute itself stated that the sharing of summary information with the MHRA, and increased use by manufacturers of the European Medicines Evaluation Authority (which, when licensing, publishes a discussion of the studies considered online<sup>107</sup>), has improved its access to trial information.

139. Nonetheless, witnesses maintained that more could be done to improve access to information, stating, “the decisions that NICE make are just as important as those of the licensing authority and it should have similar powers”.<sup>108</sup>

140. Some witnesses proposed the mandatory registration of all clinical trials to improve NICE’s access to relevant material further. The problem of the non-publication of negative trial results has been highlighted many times in the past, not least by this Committee in its inquiry into the *Influence of the pharmaceutical industry*. As Professor Claxton stated:

There have been a number of examples where trials that have been conducted have been withheld from an appraisal. Those trials are always ones that show the product in a poor light.<sup>109</sup>

Professor Rawlins agreed that more should be done to ensure registration:

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105 Q 210, 516, Lexchin et al, *BMJ* 2003;326:1167–1170

106 Q 31

107 Known as the European Public Assessment Report

108 Q 494

109 Q 494

There is increasingly registration of clinical trials. I do not think it goes as far as it should go.<sup>110</sup>

Professor Adrian Towse of the Office of Health Economics endorsed this view:

As far as concerns the voluntary registration scheme, it seems to me that it is the last chance saloon. The industry must get its act together and disclose what trials are going on; otherwise, sooner or later somebody will impose some requirements on them.<sup>111</sup>

141. Professor Jon Nicholl from Sheffield University told us that at least trials taking place within the NHS now have to be registered:

The key first step is registration and that is being addressed at the moment. All trials that are started which involve NHS patients or premises need to be registered. Quite rightly, the results of all those trials need to be made publicly available.<sup>112</sup>

142. However, fundamental problems remain. Notably, NICE has to make public the evidence it bases its recommendations on, but some of the unpublished data it might wish to use is confidential for commercial reasons.

**143. NICE does not have all the information it needs to assess and compare treatments. First, while access to EMEA documents and other changes have improved NICE's access to information, it still does not have access to all the relevant information which is available. Secondly, clinical trials undertaken by pharmaceutical companies understandably focus on generating data about the drug's efficacy and safety, which is required for the licensing process; such trials are not usually designed to generate the type of data on cost-effectiveness which NICE requires. Third, in some areas, without commercial sponsors, notably public health and many physical and psychological therapies, there is little research about the cost-effectiveness of different interventions.**

**144. We recommend that NICE be granted the right to see all the evidence the MHRA uses when making its decisions. We appreciate that this would mean that there would be some commercial-in-confidence material that NICE could not make public when it published its guidance.**

**145. We welcome the fact that both NICE and drug companies are aware that they need to collaborate closely to ensure that clinical trials are undertaken with the needs of NICE appraisal in mind. The Government should encourage all countries in which large-scale clinical trials take place to adopt a similar policy. We support the mandatory registration of all clinical trials so that the results of all negative trials are accessible. We recommend that NICE assesses and reports the quality of the research it receives.**

**146. More publicly funded research should be undertaken to assist the development of public health guidance and other areas without commercial sponsors.**

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110 Q 671

111 Q 495

112 Q 493

### Expert involvement

147. NICE Appraisal Committees, evidence review groups (ERGs), which assess whether there is adequate evidence to allow technology appraisals to take place, and guideline development groups (GDGs) include people from a range of backgrounds. ERGs and GDGs ought to include relevant experts. Appraisal Committees, in contrast, are unlikely to contain those with expertise in the area covered by the particular product or approach under consideration since their membership does not alter with each guideline or appraisal.

### Appraisal committees

148. Many witnesses were critical of appraisal committees' lack of relevant expertise.<sup>113</sup> Dr Anderson from the Royal College of Psychiatrists, stated:

Appraisal committees extraordinarily exclude experts deliberately. NICE has made that its policy.<sup>114</sup>

The charity Leukaemia CARE told us:

There are no consultants with a knowledge of haematological cancers sitting on any of the committees who pass judgement on blood cancer products.<sup>115</sup>

The charity added that “use of appropriate expertise on the appraisal committees” would help develop “an equitable and homogenous health service that is not just fair to all who have a need to use it, but is *seen* to be fair to all”.

149. Professor Rawlins explained that Appraisal Committees might not include experts as members, but experts did play a role in their deliberations. Experts were invited to attend committee meetings on specific days, to answer questions and provide clarification to the members:

The appraisal committee always has at its meeting experts who are not members, not voting members, they are there to help the committee understand the issues, but the committee itself makes the decision and it has to be like that really.<sup>116</sup>

150. Witnesses said that NICE did not use experts well, but that affiliating experts with Appraisal Committees for the duration of the consideration of a topic could improve matters:

**Charlotte Atkins:** ...this Committee has advisers who stay with it for the whole period of an inquiry and they advise it and give it the value of their expertise. What I suggest is that [experts] can simply be professional advisers to the committee.

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113 Q 231, Ev 25

114 Q 231

115 Ev 131

116 Q 686

**Dr Beverley Hunt**<sup>117</sup>: What you describe is an excellent idea. I had not thought of it in that way. You have someone there for most of the process but he or she is not a member of the committee but sits in. It sounds a very logical way forward.<sup>118</sup>

### *Guideline development groups*

151. GDGs present a different problem. Although these groups include experts, some witnesses thought that not all GDGs included the most relevant experts; as a result the guidance produced was flawed. Dr Beverley Hunt of Lifeblood: The Thrombosis Charity [speaking of the GDG which examined venous thromboembolism (VTE)] stated:

The problem is that on that committee there are no experts....Therefore, they will review what the statisticians have said and come out with an answer which is usually there or thereabout, but they do not have an understanding of the nuances or the papers in the area.<sup>119</sup>

Some witnesses claimed that the wrong emphasis was placed in the VTE guideline on mechanical methods compared to chemical methods because of a lack of relevant expertise.<sup>120</sup> Dr Hunt and Professor Roger Atkins of the British Orthopaedic Association (BOA) explained that the guidance was not as relevant to clinical practice because the right experts were not included in the guideline development process.<sup>121</sup>

152. We questioned NICE about the use of experts in GDGs. Professor Rawlins denied that experts were not included in the process. He stated:

We always have experts in the condition but we also have more generalists to give a broader view and we also include two patients or, in the case of children, the parents of patients with the particular condition.<sup>122</sup>

He added that it would not be possible to include more than a certain number of participants in any group, but that additional experts could be called when necessary:

So we have a broad base and, of course, the guideline development group...often does invite other experts in other areas. If you try to get everybody on, then the whole thing becomes unmanageable.<sup>123</sup>

### *Conflicts of interest*

153. Witnesses claimed that the Institute did not include experts on its Appraisal Committees because it “believes that they have a vested interest” in the guidance

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117 From Lifeblood: The Thrombosis Charity

118 Q 595

119 Q 579

120 Q 595, 597

121 Q 590

122 Q 91

123 Q 91

produced.<sup>124</sup> However, most specialists working in a particular field of medicine have undertaken work for a manufacturer or been linked to the pharmaceutical industry in some other way during their careers.

154. Dr Anderson from the Faculty of Old Age Psychiatry at the Royal College of Psychiatrists admitted that many experts would have links to the industry, but he denied that this would affect the group's decision:

We all live in a world where we face conflicts of interest every day of our lives. There must be a process to manage that and to declare those interests, but it is much better to have an informed group looking at the most complex problems in healthcare than a range of specialists who do not even know what Alzheimer's disease is until they have spent six months in the group.<sup>125</sup>

Other witnesses recommended that as long as any conflicts of interests were declared this should not preclude the inclusion of experts.<sup>126</sup>

155. NICE officials maintained the need for strict rules regarding conflicts of interest, however. Professor Rawlins stated:

we have a very clear statement of conflicts of interest. If they are clearly personal financial specific interests then [members of advisory bodies] must not just declare them but not take part in the proceedings. When it comes to outside experts or organisations—and it might be organisations as well as experts—then we require them to tell us what their interests are and those are recorded.<sup>127</sup>

Mr Dillon added:

In circumstances where a member of an independent advisory body has a conflict in relation to a particular topic, they do not take part in the business of formulating the recommendation.<sup>128</sup>

He stressed that it was not “inevitable” that every expert had received funding from drug companies during their careers.

**156. Many witnesses thought that too few experts with the relevant detailed expertise were involved in the process of producing guidance. Since they have a permanent membership, Appraisal Committees are unlikely to have such experts. They do consult experts, but this is unsatisfactory because such experts appear for the day alone. We therefore recommend that Appraisal Committees appoint specialist advisers, without voting rights, to work with the Committee throughout consideration of a technology appraisal or clinical guideline. This will improve guidance and ensure public and patient confidence in the system. Decisions about which experts should be appointed**

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124 Q 231

125 Q 233

126 Ev 25

127 Q 103

128 Q 105

should remain the responsibility of NICE following consultation with the appropriate clinical bodies.

### *Involvement of the pharmaceutical industry*

157. NICE's Scottish equivalent has a more "collaborative relationship" with the pharmaceutical industry during medicines technology appraisal than NICE. Companies can rework elements of their submissions and resubmit when necessary.<sup>129</sup> Some witnesses suggested that such an SMC-type approach should be adopted by NICE.<sup>130</sup>

158. Medicines manufacturers argued that their representatives should have a greater role in the evaluation process. Dr David Brickwood from Johnson & Johnson thought that at the very least pharmaceutical companies should have a similar role to patient organisations:

Stakeholders such as patient organisations and clinicians are invited into those [Appraisal Committee] sessions, but, in the case, particularly, of single technology appraisals, where the company probably has the greatest knowledge of the development of the product and probably spent ten years developing it, then the company is excluded from that process.<sup>131</sup>

Others recommended that the manufacturer should have more opportunity to consult with NICE. Many claimed that the Institute should provide more feedback to the industry on the assessment of its products.

159. In particular, some pharmaceutical companies want access to the economic models used by NICE. This was the focus of the judicial review that took place in 2007 (see Chapter 2). The manufacturers of the Alzheimer's drug donepezil (Aricept), Eisai and Pfizer, demanded access to a "fully transparent working version of the calculations used in the cost effectiveness model for independent evaluation and comment". NICE refused to let the companies have access to a usable version of the modelling tool; instead a 'read-only' version was released.

160. Eisai stated that without access to the model "full and proper investigation of the conclusions reached" by NICE were impossible, and the manufacturers could not:

check the accuracy of the formulae used in the model...;  
 assess the overall quality/ validity of the model;  
 assess the sensitivity of the model to the inputs used; and  
 test the model using alternative inputs and combinations of inputs, where those used by the Assessment Group are controversial.<sup>132</sup>

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129 Q 374, 371

130 Ev 23, 24

131 Q 370

132 NICE 118

The company added that “The economic models used by NICE are highly complex and errors readily occur”, making access to the model necessary so that consultees can comment properly and, “maintain confidence in the approach adopted by NICE”. Other companies agreed with the stance taken by Eisai and its marketing partner Pfizer.<sup>133</sup>

161. NICE defended its position as follows:

The companies get all the spreadsheets. What they cannot do is to put the numbers in they want to see how that would change the conclusion that they want...<sup>134</sup>

Mr Dillon suggested that if companies so wished, they could reconstruct the model with the figures provided by NICE and change the assumptions themselves.<sup>135</sup>

### Consultation

162. There are many opportunities for stakeholders to participate in the development of NICE guidance, for example through consultation on the assessment report, appraisal consultation document and final appraisal determination. However, some witnesses complained that NICE did not take sufficient notice of consultees’ point of view once it was submitted.<sup>136</sup> Mr Beaumont from Bowel Cancer UK stated:

NICE appears to pay lip service to patient groups and the views we put forward, including our most recent submission to NICE which was the most comprehensive we had ever provided, did not appear to be taken into consideration. We believe strongly that there should be greater engagement between NICE and patient groups.

Professor Roger Atkins of the BOA and others also argued that submissions were virtually ignored:<sup>137</sup>

When we received the draft document at the turn of 2006 the BOA, specialist societies and the group I chair wrote a 17-page document of commentary, including the latest evidence, and received no reply. We emailed the chairman of [the National Collaborating Centre for Acute Care (NCCAC)] on a number of occasions and received no reply.

163. Some witnesses complained that responding to NICE consultations was time consuming.<sup>138</sup> Small charities found that the number of consultations taking place across the UK was a particular problem; we were told that it was difficult to respond individually to all NICE equivalent organisations in England, Scotland and Wales.<sup>139</sup> The relatively short period of consultation added to the problem.<sup>140</sup>

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133 Ev 183, 222

134 Q 681

135 Q 684

136 Q 224

137 Q 612, 240

138 Ev 113

139 Ev 135

140 Responses to NICE consultations must be given within 4 weeks. See Annex

164. Not all patient groups agreed with this viewpoint, however. Some praised the many opportunities for consultation during the guidance development process. Breakthrough Breast Cancer stated that it:

...welcome[d] the move by NICE to run consultations on many of their processes... This demonstrates a willingness to be efficient and inclusive.<sup>141</sup>

Dr Kiran Patel also told us:

If you look back 10 years, the access which organisations such as ours had in terms of policy-making and developing guidance was more or less zero. NICE has afforded us an opportunity to access the decision-making process as a stakeholder and we have utilised that very well. From our point of view we have had very good access at all levels of the process.

165. NICE is proud of the amount of consultation that takes place during the development of its guidance. Professor Rawlins described consultation as, “a very, very important quality control safeguard mechanism in our processes”. He added:

We would be very, very reluctant to stop doing that, it is a very important part of our process.<sup>142</sup>

166. The Institute denied that consultees’ submissions were ignored.<sup>143</sup> It told us that it was not common practice to respond individually to every submission made to the consultation process. Instead, consultees and detailed responses to all comments they made were listed on the Institute’s website once guidance was published.

167. NICE did accept that it was disappointed about some aspects of its consultation processes, however. In particular, while PCTs have always been able to contribute through the NHS Confederation,<sup>144</sup> since 2002 they have asked for their input during the development of technology appraisals.<sup>145</sup> Unfortunately, they do not often respond to consultation. Mr Dillon stated:

What we have discovered over the years is that the extent to which those PCTs actively get involved is quite low. That is disappointing and it is as much our responsibility as it is the community of PCTs to do something about that. We are concerned.<sup>146</sup>

He added that the NHS Confederation was discussing with PCTs proposals for new arrangements which would allow them to contribute to NICE’s work more efficiently:

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141 Ev 62

142 Q 170

143 Q 700

144 Q 364

145 Ev 14

146 Q 705

It is absolutely in our interests and it is the interests of good quality guidance and it is the right thing to do and we are very happy to work with the Confederation to make that work better.<sup>147</sup>

168. **The wide consultation which takes place during the development of NICE guidance is greatly valued. While we agree that it is difficult for some organisations to respond within the often brief time limits, we recognise that a long consultation period would slow the guidance production time further. Nevertheless, the situation would be improved if NICE were to give interested stakeholders greater warning of forthcoming consultations, to allow them to organise their resources in time to respond effectively.**

169. **Some consultees complain that their views are ignored. We understand that NICE does not have the resources to respond individually to each consultee. NICE could, however, issue a standard response to inform every consultee how it will respond and setting out how the system works.**

## Appeals

170. Appeals against NICE decisions can be made by interested parties when, “the Institute has failed to act fairly, has exceeded its powers or has formulated guidance which cannot reasonably be justified”.<sup>148</sup> Appeals may not be made on other grounds, such as the interpretation of the evidence; they are based on process alone. NICE does not permit new information to be introduced during the appeal stage. The process is based on English law.

171. Appeals are held in public by a panel of three non-executive directors of NICE or two non-executive directors plus an NHS clinician. An individual with experience in the relevant industry (chosen following consultation with the relevant trade associations) and a lay representative are also present on the panel.

172. The number of appeals against NICE decisions has not increased significantly since the Institute’s establishment. However, a high proportion of recommendations are appealed against each year, perhaps reflecting the dissatisfaction with elements of the evaluation process described above. Most appeals relate to technology appraisals.<sup>149</sup> There was considerable criticism of the appeals system.

173. Appeals are not always made on valid grounds. The South Asian Health Foundation told us that appeals could be considered as “a reasonable and appropriate strategy for the pharmaceutical industry...to challenge unfavourable decisions from NICE”. Professor Rawlins agreed that the industry sometimes used appeals in this way:

Sometimes we have what the industry have themselves described as “gaming appeals” where we have done a class appraisal of several different drugs in the same class and where one manufacturer is appealing the fact that we have not picked his out as the best. Then we have to hear the appeal. They have made an appeal and our

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147 Q 706

148 NICE terms of reference: <http://www.nice.org.uk/niceMedia/pdf/DirectionFromSecretaryOfState2005.pdf>

149 See Chapter 2 for details

rules are that we listen to the appeal. In those sorts of appeals, we have always rejected them.

174. Professor Claxton added that the increasing requirement for the NICE and its committees to prove their case during appeals contributed to “a dangerous situation”. He stated:

Many of the appeals have been upheld and so [the Appraisal Committee’s] ability to say no has been somewhat undermined...That starts to shift the burden of proof back onto the institute and the independent groups but within a process where they do not have the resources and time to do a full independent analysis.<sup>150</sup>

175. Some witnesses argued that changes to aspects of the technology appraisal would reduce the number of appeals made. Dr Anderson of the Royal College of Psychiatrists claimed that changing the make-up of the appraisal committees, for example, would mean that appeals were less likely:

If you had informed appraisal groups they would not be so contentious even though they made tough decisions, and I do not believe you would have as many appeals against them.<sup>151</sup>

176. This view was echoed by manufacturers. They also thought that closer working with NICE during the assessment process would reduce the numbers of appeals. Novartis told us:

We believe the degree of challenge to NICE decisions would be reduced considerably if a more inclusive approach were taken during the assessment process, enabling a dialogue from the beginning on the appropriate patient population in whom treatment may be cost effective, on methods, on assumptions, on data quality and on modelling techniques.

177. There appears to be strong dissatisfaction with the appeal system itself, however. Many witnesses bemoaned its limits, indicating that appeals should be possible on grounds beyond those currently permitted by the legislation. While some appeals are upheld, large-scale changes are very rare.<sup>152</sup> As Mr Gray from GlaxoSmithKline stated, the limits of the process explain why so many appeals meet this fate:

When you look at quite why so many appeals appear to fail, it is because in many ways you have appealed about something that is not being assessed in the appeal—and, big surprise, lots of things do not get through appeal.<sup>153</sup>

178. To improve the appeals system it was proposed that:

- The introduction of new evidence be permitted;

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150 Q 499

151 Q 279. See paragraph 156

152 According to NICE, small changes of wording are often all that is required

153 Q 387

- NICE make more effort to explain the reasons for the initial decision; and
- The appeals system be made independent of NICE.

### **New evidence**

179. Witnesses argued that NICE should permit new evidence to be presented at an appeal as it may give potentially useful information about the treatment in question. Mr Beaumont called the Institute’s refusal to accept additional evidence during appeal, “a cop-out”.<sup>154</sup> We were informed of many examples of the exclusion of seemingly valuable new evidence from the appeals process. For instance, Merck Serono highlighted a case where new evidence “which validated the use of particular assumptions within its economic modelling” came to light after draft guidance rejecting the drug was published. The new evidence could not be presented during the appeal. The appeal itself delayed guidance publication; the company claimed that this delay in combination with the exclusion of new evidence meant that the final guidance was possibly mistaken:

This [new] information could not be assessed by NICE. Therefore a total of 21 months elapsed with no possibility of any new evidence being considered for nearly 18 months. This new evidence could mean that drugs originally given negative guidance might be given a positive guidance.<sup>155</sup>

180. On the other hand, allowing additional evidence at the appeal stage would extend the process significantly, and might discourage companies from producing high quality trial data at the time of first assessment. It might also risk more “gaming” appeals.

### **Better communication**

181. We were informed that better explanations of the reasons for rejecting appeals could improve the process and lead to fewer appeals being brought.<sup>156</sup> NICE has often been accused of communicating poorly with the general public and manufacturers, about this and other areas of its work.<sup>157</sup> As Mr Dillon told us:

I am not sure we have been as successful in engaging the general public.<sup>158</sup>

182. Professor Raymond MacAllister thought that better communication strategies could also improve the public’s perception of the appeals process and of Institute more generally:

When one says “no” obviously one will be unpopular unless one says clearly why one is saying that. At times I believe that the reason why it says “no” is not clearly stated. It is very easy for those who do not like the “no” to marshal considerable forces in the

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154 Q 281

155 Ev 154

156 Q 386

157 Ev 91, 92, 113

158 Q 648

press and elsewhere to attack that decision and many times the criticism is very unfair. A more clear description of why “no” has been said would be helpful.<sup>159</sup>

### **Independence**

183. The appeals process is handled internally by NICE. This may add to the dissatisfaction and perceived unfairness experienced by appellants. Some witnesses felt that the lack of independence limited the validity of the appeals procedure<sup>160</sup>, as Mr Beaumont from Bowel Cancer UK told us:

It is crazy that the people who made the decision in NICE then sit in judgment on themselves in the appeal. What possible motive would they have to change their minds when they would be regarded as having got it wrong in the first place? The appeals process in NICE should be independent.<sup>161</sup>

Many manufacturers agreed. Dr Brickwood from Johnson & Johnson stated:

We would advocate that the committee and its structure has a total independence from NICE.<sup>162</sup>

**184. We note the pressure to change the grounds for appeal, but consider changes might cause more problems than they solved. Allowing additional evidence at the appeal stage would extend the process significantly, and might discourage companies from producing high quality trial data at the time of first assessment. It also might risk more “gaming” appeals. We make recommendations in the next section which we expect will lead to fewer appeals being brought in the first place.**

### **Speed of guidance**

185. NICE technology appraisals take between 9–12 months (STA) and two years (MTA). Clinical guidelines and public health guidance take longer. The evidence we received indicated that the speed of technology appraisals was of great concern.<sup>163</sup> Mr Winyard described the appraisal process as “wretchedly slow”.<sup>164</sup> The Minister agreed:

Is there an issue about speed? Yes, and everybody recognises that.<sup>165</sup>

### **NICE blight: the consequences**

186. Manufacturers, clinicians and patients groups alike highlighted problems related to the delay in access to treatment caused by the relatively long period between licensing and

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159 Q 221

160 See Ev 30

161 Q 283

162 Q 384

163 See Ev 23, 24

164 Q 638

165 Q 769

publication of NICE guidance. We heard many complaints about this situation, which is often described as ‘NICE blight’.

187. Clinicians are less likely to prescribe a medicine during the period between licensing and the issue of guidance as they may either prefer to wait or may be specifically forbidden to prescribe the medicine by their PCT. Whether or not patients have access to the drug at this point therefore depends on where they live. Mr Winyard from the RNIB pointed out:

In some areas...patients will be treated but in others they will be turned away. For example, in Staffordshire there appears to be a blanket ban on treatment [for macular degeneration] and in many areas patients have to go blind in one eye before the primary care trust will consider treating the second. This is fundamentally wrong.<sup>166</sup>

188. Delayed guidance harms patients who are waiting for treatment. Dr Crayford stated:

The bigger problem with the delay in producing guidance, say from the time which the drug comes to the market, is more the effect it has on patient groups and patients’ expectations. That is the bigger and more difficult thing to handle [compared to financial effects on PCTs].<sup>167</sup>

189. On the other hand, it is possible to prescribe licensed medicines before the relevant NICE guidance is published. It is very difficult to take a medicine off the ‘available list’ once it had already been widely prescribed. This clearly creates problems if the NICE guidance eventually finds that it is not cost-effective. Rapid assessment of a medicine and determination of how it should be used helps to avoid these problems.<sup>168</sup>

190. Delays also bring the risk that the assessment will no longer be relevant by the time clinicians are able to use it. Dr Kiran Patel stated that:

There is a real danger that by the time some NICE guidance is published it is outdated.<sup>169</sup>

He echoed the views of many, stating that, “We need to have a mechanism to speed [guidance development] up”.

191. NICE itself has recognised the need for faster guidance. It introduced its STA process for precisely this reason and has clearly stated that it would like to issue guidance earlier than at present. Professor Rawlins told us:

We have the ambition of trying to make sure that the NHS has advice around about the time of launch—it cannot necessarily be at launch, but within a month or two or three.<sup>170</sup>

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166 Q 604

167 Q 308

168 This was highlighted to us during our visit to the SMC

169 Q 286–7

170 Q 665

### *Faster guidance*

192. Witnesses argued that faster guidance could be issued if:

- NICE adopted a similar system to that in place in Scotland; or
- Reduced the rigour of its evaluation.

### *A Scottish process*

193. The STA has increased the speed of NICE guidance; however, it remains slower than the process used as standard by the SMC. Patient groups told us that patients benefited from the swifter process in place in Scotland.<sup>171</sup> The speed of the Scottish system, and the fact that the SMC evaluates all new medicines immediately, means that ‘approved’ medicines are available to Scottish patients sooner than to their English counterparts.

194. Professor Rawlins pointed out that the SMC procedure was shorter than that of NICE because it did not involve the same consultation and appeals processes. Mr Dillon also indicated that the guidance documents issued by the SMC are much less detailed than those of NICE:

What NICE does is to identify precisely the circumstances in the form of the clinical advice that we offer that the drug should be used in. So we would identify specific populations, we would identify specific features of the way that the drug should be administered and the circumstances in which it should be used. That just takes longer to do.<sup>172</sup>

The Minister lent her support to the evaluation system used by NICE. While she admitted it was a longer process than that of the SMC, she added:

We, as ministers, prefer the process of NICE, we think it is more robust, transparent, it is being improved with certain considerations moving to public session and it is respected, therefore, internationally as the right way to proceed.<sup>173</sup>

Mr Dillon added, however, that NICE would be willing to consider measures to reduce the delay to guidance release:

I am concerned for any delay... it is our job, along with the Department of Health’s and the departments involved in the topic selection process, to minimise this as much as we can. I am sure that there is more that we can do to achieve that.<sup>174</sup>

195. The ABPI told us that there is a period of four months on average between licensing of a medicine by the MHRA and launch of that medicine on to the market.<sup>175</sup> A shorter evaluation, along the lines of that of the SMC, could fit into this gap.

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171 Q 638

172 Q 662

173 Q 769

174 Q 663

175 Information supplied by the ABPI (NICE 72A)

### *A less rigorous process*

196. NICE technology appraisals are widely considered to be thorough. However, some witnesses argued that a shorter, less rigorous process which examined all drugs would bring many benefits. Dr Crayford told us that PCTs would benefit from a faster system that evaluated all medicines, even if it was less stringent than the current procedures:

We obviously need a degree of scientific rigour...[but] in order to broaden [the process] (and I would argue that needs to be broadened considerably), it needs to become less rigorous and provide us swiftly with best quality clinical evidence for a much wider range of clinical interventions.<sup>176</sup>

197. Witnesses pointed out that the uncertainty of cost-effectiveness at this early point could be mitigated through the use of a lower cost-per-QALY threshold, which could be revised later. Professor Smith told us:

one could negotiate a lower price for an interim period until further research was forthcoming, and so on. There are lots of imaginative possibilities to speed up the process and protect the NHS from making adverse decisions in a rush but also to encourage companies to continue to come forward with new treatments.<sup>177</sup>

198. Professor Rawlins denied that a “quick and dirty” evaluation was the solution. He told the Committee that such a move would reduce the value of the appraisal process and risk making recommendations that would wrongly guide the use of resources in healthcare:

We rely and depend on the rigour of our system not just to withstand the law and all that type of thing but to actually make sure that we are fair to everybody who uses the National Health Service, because saying yes to something that is cost ineffective will deprive other people of cost effective care, and saying things that are cost ineffective when they are cost effective really will have the opposite effect and that would be wrong.<sup>178</sup>

199. We agree with the Minister that the time taken to publish guidance is a serious cause for concern. In our view, the delay between medicines licensing and guidance publication does indeed ‘blight’ healthcare delivery by the NHS. The reduced access to those licensed medicines that are eventually proven to be cost-effective before NICE’s assessment takes place is unacceptable. It is also unacceptable that Scottish patients have access to new medicines while English patients do not.

**200. A shorter, less in-depth initial evaluation of medicines at an early point would be useful. It is important that clinicians have access to independent information about new therapies as soon as they are available. However, a quick, in-depth, fully consultative evaluation for all new medicines by the time of launch is not possible. We therefore recommend that NICE should examine all new medicines for their indications as set out in the marketing authorisation. Assessment should be carried out**

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176 Q 329

177 Q 191

178 Q 665

during the period between licensing and launch. It should be brief and published prior to, or at the time of, launch. There should be no formal appeal process and only limited consultation. These brief assessments should be followed by a larger scale multiple technology appraisal for selected products (an MTA or STA as appropriate) at a later date, when more evidence is available. The technology appraisal should include current levels of consultation. The guidance issued at this later stage should be definitive, overriding that issued earlier.

201. Since providing an evaluation of all drugs at launch will be a more rough and ready process, it would be inappropriate to use the same threshold range as the full assessment. One of the aims of the new process is to ensure that treatments which are obviously cost effective are available at an earlier stage than at present. We therefore recommend that a threshold below the current range be used in these early assessments. This could be raised for individual products in special circumstances, for instance where no other treatment exists. At the time of the full assessment, the cost per QALY threshold could increase.

## 4 Affordability and rationing

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202. NICE aims to promote good health and prevent or treat ill health through the use of treatments which are both cost-effective and clinically effective. If NICE provides guidance that NHS organisations cannot afford, it has failed to achieve this aim. The evidence we received revealed that many PCTs find it difficult to adopt NICE technology appraisals and clinical guidelines because of their cost implications. Witnesses had the following concerns:

- NICE’s threshold is not evidence based;
- The threshold is higher than that of other NHS organisations;
- As a result, there is a risk that PCTs give other possibly cost-effective therapies, which have not been assessed by NICE, a low priority;
- Although NICE and PCTs have taken some steps to address the situation, there has to be a re-examination of the NICE threshold; and
- If the threshold is wrong, NICE’s role in rationing is compromised.

### Criticisms of NICE

#### *NICE’s threshold is not clear and is not evidence-based*

203. The incremental cost-effectiveness ratio (ICER), or cost per QALY, which NICE uses to determine cost-effectiveness in its technology appraisals was described earlier. The cost per QALY is one indication of whether a treatment or procedure will be approved by NICE.

204. As we have seen, Professor Rawlins told us that NICE employs a threshold of £20-30,000. Some research has indicated that it could be even higher.<sup>179</sup> Professor John Appleby from the Kings Fund told the Committee:

Some fudging is going on as far as NICE and others are concerned, not just about what the threshold is but how it is applied.<sup>180</sup>

205. There is clearly confusion about the cost per QALY threshold. Witnesses questioned whether there was any evidence to support the level that appears to be used. Professor Devlin told us that, “the threshold has no explicit basis or location in evidence”.<sup>181</sup> Others agreed that it was “arbitrary”.<sup>182</sup> Professor Smith confirmed:

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179 Q 540

180 Q 531

181 Q 531

182 Ev 102, 152

[NICE] has had to undertake its work in the absence of secure information about the most appropriate cost-effectiveness ‘threshold’ at which to approve new technologies.<sup>183</sup>

206. Professor Rawlins admitted that the threshold was not based on “empirical research” as no such research existed anywhere in the world. He told us instead that the threshold was:

...really based on the collective judgment of the health economists we have approached across the country. There is no known piece of work which tells you what the threshold should be.<sup>184</sup>

207. No public discussion has ever taken place of the suitability of the threshold used. The American Pharmaceutical Group pointed out that the threshold has “never been the subject of public debate or Parliamentary approval”.<sup>185</sup> Cancer Research UK also argued that the threshold should be discussed openly and the reasons for its level should be determined in consultation with interested organisations:

The public should know where the £30,000 figure came from and why it is set at that level...Government [should] enter a debate with stakeholders about what threshold is appropriate for a country such as the UK and for the NHS in the future.

The pharmaceutical company Bristol Myers Squibb agreed that, “it should be much clearer how the threshold is determined”.<sup>186</sup>

208. The cost per QALY threshold used by NICE does not appear to have changed over time. The range it uses now of £20,000 to £30,000 is the same as it was in 1999. Witnesses claimed that this was further evidence of its arbitrary nature.<sup>187</sup> If it had kept pace with NHS-specific inflation, then in 2007 it would be £28,000 to £42,000.<sup>188</sup> Professors Devlin, Appleby and Parkin claimed:

By whatever means the threshold is determined, it should be adjusted over time. NICE appears to have operated the same threshold or threshold range since 1999.<sup>189</sup>

209. The stability of the cost per QALY threshold over a period when NHS spending has increased significantly indicates that there is little or no relationship between it and the NHS budget. Witnesses claimed that the two should be directly related. Professor Bryan told us that:

The issue of affordability and the overall size of the budget cannot be unrelated to the appropriate level of threshold.<sup>190</sup>

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183 NICE 104

184 Q 644

185 Ev 32

186 Ev 67

187 Ev 62, NICE 103

188 NICE 103

189 NICE 103

### **NICE's threshold compared to the threshold used by other NHS organisations**

210. Inevitably, in view of the lack of any firm justification for it, witnesses expressed concern about the current threshold range. A number of witnesses claimed that the threshold should be higher than its current level, to ensure that patients received the best treatments.<sup>191</sup> For instance, Breakthrough Breast Cancer stated:

If NICE does not review its current cost thresholds there is a danger that the NHS will not keep up-to-date with treatment advances and that patients in England and Wales will not be offered the most effective treatments for their conditions.<sup>192</sup>

211. Manufacturers and patient groups in particular argued that certain treatment areas should have higher associated cost per QALY thresholds. It was claimed that orphan drugs (drugs for rare conditions) should continue to benefit from a higher cost per QALY threshold.<sup>193</sup>

212. NICE argued that the current cost per QALY threshold was appropriate. In particular, Professor Rawlins maintained that a lower threshold would have negative effects on the treatments available to patients:

The truth of the matter is if we halve the broad threshold, we would have declined as cost-ineffective most of the new drugs we have looked at and I do not think that is what people want.<sup>194</sup>

213. Other witnesses, in contrast, expressed a fear that the threshold used by NICE was too high, and that the efficiency of the NHS could be reduced as a result.<sup>195</sup> These witnesses questioned whether the threshold, or the threshold range, currently in use was in line with spending decisions made by PCTs about treatments not assessed by NICE.<sup>196, 197</sup>

214. Professor Smith told us that his research had compared the cost per QALY threshold in funding decisions made by PCTs to that used by NICE in its technology appraisals.<sup>198</sup> Using data from the Department of Health's programme budgeting system, Professor Smith showed that the cost to PCTs of "a life saved" was around £13,100 for cancer patients and £8,000 for heart disease patients. Adjustment for the quality of the year of life saved (to make the figures comparable to the ICER used by NICE) indicated a cost of around £19,100 for cancer and £12,000 for circulatory disease. Professor Smith stated:

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190 Q 182

191 Ev 154

192 Ev 62

193 Ev 85, 110

194 Q 645

195 NICE 103, Appleby et al, *BMJ* 2007; 335:358–359

196 Q 176

197 Q 551. Professor Appleby suggested that NICE did not consider individual PCT variation in delivery costs.

198 This work was undertaken by Professor Smith and colleagues as part of the Quest for Quality and Improved Performance, a five-year initiative of The Health Foundation

Although these figures are rudimentary, they do suggest that the existing cost of a QALY secured in these programmes of care may be lower than many commentators have assumed....

A threshold closer to £20,000 is more appropriate than £30,000 on the basis of the evidence that we have been able to uncover.<sup>199</sup>

215. Further research by the same group evaluated three more areas of care: respiratory disease, gastro-intestinal conditions and diabetes. A comparison of the figures for all five areas, unadjusted for quality of life, indicated that the cost of a “life year saved” for respiratory problems was lower than cancer and circulatory disease and higher for gastro-intestinal conditions and diabetes.

216. Professor Rawlins claimed that this was evidence that NICE used an appropriate threshold. He cited the later work by Professor Smith stating, “His more recent and more extensive work indicates that we are in about the same sort of ballpark”.<sup>200</sup> However, Professor Smith maintained that his results actually suggested PCTs used a cost per QALY threshold closer to £20,000 than £30,000.<sup>201</sup>

217. Although this research implies that PCTs make use of a cost per QALY threshold, other witnesses claimed that most decisions by PCTs were not made on the basis of a specific threshold. Professor Appleby told the Committee that “every decision seems to be separate”.<sup>202</sup> Dr Anderson from the Royal College of Psychiatrists, the Hepatitis C Trust and others, questioned the basis of PCT decision-making more generally.<sup>203</sup>

## Consequences

### *Crowding out*

218. Since guidance given in technology appraisals must be funded within three months, too high a threshold would cause significant financial problems for local health organisations. Professor Smith described the significance of the threshold [the cost per QALY above which a treatment is unlikely to be approved] for the NHS:

If NICE adopts too liberal a threshold, it may approve technologies that drive out more cost-effective conventional treatments...if NICE adopts a threshold that is too strict it may inhibit the development and adoption of cost-effective new treatments.

Professor Stirling Bryan told us:

A serious concern on the threshold topic is how the band of £20,000 to £30,000 has been arrived at and whether it has been set at too high a level. These worries are

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199 Q 184

200 Q 643

201 Martin S, Rice N, Smith PC: Further evidence on the link between health care spending and health outcomes in England: <http://ideas.repec.org/p/chy/respap/rp32.html>

202 Q 545

203 Q 275, Ev 115

driven, in part, by the mandatory nature of NICE technology appraisals guidance ...An important consequence of applying this threshold is, therefore, that local NHS organisations will find it even more difficult to remain within budget.

Moreover, while a lower threshold would deprive patients of treatments which were just marginally useful, it would thereby free up funding for more cost-effective interventions.<sup>204</sup>

219. Positive NICE technology appraisals must be funded within three months of publication. As NICE evaluates more and more medicines and procedures using the current threshold, there is a risk that treatments which NICE has not examined, or other areas of healthcare, will be 'crowded out' as PCTs are forced to prioritise NICE-evaluated approaches.<sup>205</sup> Patients with conditions not yet covered by guidance may therefore receive cheaper or less up-to-date therapies than patients who receive treatments which have been the subject of NICE appraisal. As the ADPH stated:

The disadvantaged group is the majority of patients who have conditions that are not covered by NICE, and in particular those who, unbeknown to them, are denied interventions because the funding has been diverted to implement NICE's recommendations for others.<sup>206</sup>

220. Many witnesses thought that areas of spending not evaluated by NICE, which were foregone as a result of NICE guidance, might represent better value for money, particularly in the long-term, than those evaluated. These could include older, established and often cheaper medicines, or public health measures. Professor Bryan told us:

[My research] suggests that people have to displace things that they perceive to be of greater value as a result of the NICE guidance. There is a perception, whether or not it is the reality, that that is the case.<sup>207</sup>

221. The NHS Confederation agreed. It stated that many of the treatments examined by NICE were only just within the defined limits of cost-effectiveness and that other areas of care suffered because of their implementation:

As a result the paradox arises that NHS funding is mandated for a marginally cost effective drug and local NHS organisations may have to achieve this by not spending on treatments which may be very much more effective and could benefit more people.<sup>208</sup>

222. An example, which highlights the difficulties caused by the mandatory funding of drugs over other types of therapy, concerns the new thrombolysing drug Actilyse (alteplase). Alteplase was the subject of an STA that was published in June 2007. Dr Nigel Dudley, a consultant in elderly and stroke medicine, argued that the appraisal should not have been published ahead of NICE's stroke guidelines or the National Stroke Strategy.

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204 NICE 103

205 Q 302

206 NICE 111

207 Q 183

208 Ev 180

Many areas that lacked far more basic stroke services had to purchase this expensive drug before addressing other, more pressing issues:

The priority given to Alteplase means that those who have shouted loudest...have gained in this particular case at the expense of other parts of an underfunded stroke service...

money that has to be spent by law on thrombolysis for patients aged 80 and less will not be available to spend on patients of all ages in rehabilitation units or early supported discharge services who would benefit.<sup>209</sup>

223. The problem is exacerbated by the exclusion of some particularly expensive treatments from the payment by results tariff. The increase in costs associated with NICE guidance is incorporated into the uplift to the tariff, determined every year by the Department of Health.<sup>210</sup> Exclusion of drugs from the tariff means they are paid for separately after specific approval by PCTs, which is usually automatic if following NICE guidance. The overall effect is that there is an incentive for hospital clinicians to use NICE-approved new technologies, since such an approach brings in extra income to the trust, rather than weigh up the relative value against other more established forms of treatment covered within the tariff price. While this encourages the uptake of new technologies, it also means higher costs for PCTs.

## What should be done

### *Measures to be taken by NICE and PCTs*

224. Some steps have been taken to help PCTs afford NICE guidance and provide funds for treatments which NICE has not assessed and, we were told, others could be taken, including:

- Better communication of NICE's programme of work to PCTs;
- Inclusion of the cost of all drugs in the tariff; and
- Better evidence about cost- and clinical-effectiveness in all areas to enable PCTs to prioritise treatments more effectively.

225. The Department and NICE have attempted to help PCTs plan for the expenses associated with NICE guidance by providing information on NICE's work programme and new treatments in general.<sup>211</sup> NICE has also started developing commissioning guides and templates.<sup>212</sup>

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209 NICE 123

210 Q 20

211 Q 21

212 Q 47. There may be problems with some cost templates though. We were told of concerns with data contained in the costing template for PCT commissioners that accompanied the clot-busting drug Alteplase appraisal published in June 2007. NICE 123

226. Some argued that NICE could do more. The Academy of Medical Sciences recommended that better communication between NICE and PCTs could improve the situation:

...close communication between NICE and PCTs so that Trusts are financially prepared for the provision of new treatments...Advance preparation of all PCTs would reduce inconsistencies between those that provide a treatment and those that do not.<sup>213</sup>

227. As we have seen, the fact that high-cost drugs are not included in the tariff means that there is no incentive for hospital doctors to consider whether they represent value for money. Inclusion of all NICE-approved therapies within the tariff would share the financial risk and encourage caution since the average uplift could be kept closer to what is affordable in general. The Minister agreed that this approach should be considered:

The final roll-out does not cover all specialties until 2007–08. Obviously that could include high cost drugs and it could give greater certainty to PCTs. Overall it would not increase the funding. This is something, bearing in mind the roll-out finally through 2007–08, we are prepared to keep under consideration.<sup>214</sup>

228. Other witnesses told us that the limited evidence available on unassessed areas of healthcare meant that PCTs had difficulties deciding where to reduce spending as a result of mandatory NICE guidance.<sup>215</sup> This has led some PCTs to come together to pool knowledge to determine how best to commission in the areas of care that have not been assessed by NICE. Professor Devlin stated that the lack of information available on the value for money of these areas had prompted some PCTs to attempt to provide this evidence themselves:

One of the aspects of PCT decision-making that we found was a desperate demand for information on what services were poor value for money, what should it be disinvesting from, what would be the appropriate responses to cost pressures and so on. We found instances of PCTs working together to try to create some sort of information and evidence base on which to do that.<sup>216</sup>

229. Dr Lise Llewellyn, Chief Executive of Berkshire East PCT, told the Committee that this approach had been taken in her area:

Collectively PCTs have got systems and processes where they work together. In my patch we have got a public health unit that works across PCTs so that where there are concerns or queries about treatments or types of drugs, et cetera, we actually try and take a collective decision.<sup>217</sup>

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213 Ev 24

214 Q 762

215 Q 548

216 Q 550

217 Q 299

### Setting the threshold

230. Some witnesses claimed that the current NICE threshold was simply unsustainable.<sup>218</sup> As PCTs are forced to fund more technologies, the resources left over for PCTs to use as they see fit, will shrink so much that they are unable to tailor healthcare delivery to the needs of their own areas. Dr Kiran Patel told us:

There needs to be a level of local PCT freedom to do what is appropriate for its population.<sup>219</sup>

231. NICE is clearly concerned about the threshold it uses; a feasibility study is currently being undertaken by Professors Appleby and Devlin jointly with the Institute to examine the cost per QALY of decisions taken by PCTs to invest or disinvest in treatments. This will allow comparison of the cost of PCT decisions with those recommended by NICE.<sup>220</sup>

232. Witnesses argued that the problems outlined above could be mitigated if the threshold of cost-effectiveness was set independently of NICE. The Institute was certainly not established, nor constituted, to make political decisions of this kind.<sup>221</sup> Some suggested that the threshold should be set by Parliament.<sup>222</sup> Professors Devlin and Appleby argued that the NHS should be given independence on this matter in a manner similar to that of the Bank of England on interest rates. NICE, PCTs and other purchasing bodies would then be required to adopt this threshold. Professor Devlin told us that this could lead to a fairer and more efficient system:

We are suggesting that NICE's threshold is not just a matter for NICE alone: it is not just NICE's business. If NICE makes a mandatory decision that PCTs must implement it completely alters the bundle of services which PCTs can afford to deliver. That affects the services that all patients can potentially consume or benefit from, so NICE's threshold should have an input from the sector and a much wider range of expertise.<sup>223</sup>

233. Professor Rawlins spoke out against this suggestion, however, stating that this would substitute one system lacking in evidence for another, potentially less informed, one:

My own view is an independent body would have exactly the same difficulties we have had. They would have to use judgment about it because there is no empirical basis.<sup>224</sup>

He added that NICE had commissioned research on the subject, which would be reported towards the end of 2007.

218 Ev 243

219 Q 271

220 NICE 103. Initial research has revealed that a larger scale study is possible, but that determining the basis of marginal decisions (ie. cases where it is less obvious, or the data are less clear, regarding investment or disinvestment in technologies) will be difficult to unravel.

221 See Annex 1 for NICE's terms of reference

222 Culyer A et al. *Journal of Health Services Research & Policy* 2007;12;56. Cited Ev 77; Ev 118

223 Q 533

224 Q 645

## Rationing

234. The question of the threshold used by NICE is key to any discussion of healthcare rationing. Ensuring that guidance is affordable to purchasers is vital if NICE is successfully to perform its vital role of helping to ensure that the NHS's budget is spent as cost-effectively as possible. The resources of the NHS are limited, and the funding increases of the last few years will not be repeated in 2008. The cost of new health technologies, including medical treatments and diagnostic and surgical procedures, is rising. At the same time, the population of the UK is ageing and with age comes increasing healthcare needs. As Professor Rawlins told us, "every single developed country has got the same problem".<sup>225</sup> Demand outstrips resources; as a result rationing occurs in all healthcare systems.<sup>226</sup> Several witnesses highlighted the consequent need for a true public discussion of healthcare rationing. As Professor Stirling Bryan told us:

I also believe there is a need for a more open and frank public conversation about rationing in healthcare. If one is to get the public engaged and supportive of NICE one needs to be much more open and frank with them about resource limits, how decisions are made and the fact that there are very difficult judgments to be made in terms of healthcare priority-setting and rationing and that NICE is part of that.<sup>227</sup>

Dr Keith Syrett, from the University of Bristol, also stated:

There remains a need to engage in a comprehensive debate about rationing in the NHS in order to secure public acceptance of the authority of limit-setting bodies such as NICE.<sup>228</sup>

He stressed that, "it is the function of government, not of NICE, to initiate such a public debate".<sup>229</sup>

235. To date, however, politicians have shied away from such a discussion, perhaps because it is uncomfortable to talk about the 'cost' of a life (ie. the threshold that should be used), or the maximum that should be paid to improve a patient's quality of life. The subject of cost does not sit easily with the concept of a NHS that is "free at the point of need". Professor Nancy Devlin stated:

It is a very difficult debate to get going in the current climate. A big policy issue within the NHS has been improving patient choice. Patients should be able to choose what treatments they get, where and when. That sort of theme around responsiveness and individualisation of treatment alongside a debate on rationing in the health service is somewhat uncomfortable, is it not?<sup>230</sup>

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225 Q 648

226 Maynard et al, *BMJ* 2004; 327: 227–229

227 Q 222

228 Ev 271

229 Ev 271

230 Q 556

236. The problem is exacerbated by the threat of legal action, which has been used to override PCT rationing decisions, resulting in patients receiving the treatment they desire. On rare occasions, as we have seen, Ministers have intervened to achieve the same aim. Such actions may fuel public perception that rationing is wrong and that denial of treatment is in a sense cheating the patient. Dr Daphne Austin, a public health consultant, claimed:

The perception that it is wrong to deny treatment is fuelled by the fact that PCTs frequently step down when there is a real threat of legal action. This is interpreted as an acknowledgement of the PCT being ‘in the wrong’.<sup>231</sup>

237. Mr Dillon agreed that NICE should play a role in discussions about rationing, and that “the realities of decision making” should be communicated:

I would quite like NICE to contribute to a broader debate about why it is necessary sometimes for those controversial decisions to be taken, and I think that has to be a debate that involves government with its stewardship responsibilities for the health service.<sup>232</sup>

Dr Austin concluded that without such a discussion, achieving affordability—and therefore determining the right threshold—was unlikely:

Until we can accept that not all needs can be met we will continue to have a distortion in health service priorities and, ironically, fail to get value for money overall.<sup>233</sup>

238. The Minister agreed that rationing was necessary, and that NICE should play a part in public discussion of the subject:

in a cash limited system we clearly cannot pay for absolutely everything so there needs to be an approach which attempts to prioritise on evidence what is available. NICE has a role to play in that in starting to shift the debate on to a more robust footing that is about what the evidence tells us.<sup>234</sup>

She added that “transparency and engaging” were needed to communicate to the public that not every medical treatment or procedure is available for them through the NHS. She also told us that “managing expectations”, through communication and better understanding of local needs, was necessary.<sup>235</sup>

## Recommendations

**239. The threshold or ceiling NICE employs (measured in pounds sterling per QALY) to decide whether a treatment is cost-effective, and so should be available in the NHS, is**

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231 Ev 241

232 Q 654

233 Ev 244

234 Q 716

235 Q 717

not based on empirical research. Nor is the threshold directly related to the NHS budget, since the threshold has remained constant while the budget has increased hugely since 1999.

240. The threshold used by NICE does not take into account the funding decisions made by PCTs generally. For interventions not assessed by NICE, PCTs appear to use thresholds which vary from treatment to treatment but for the most part seem to be lower than the NICE threshold.

241. Many PCTs struggle to afford to implement NICE technology appraisals, as well as clinical guidelines. As more interventions are evaluated it is feared that the position will become unsustainable. Funding is essentially ring-fenced for technology appraisals, leaving PCTs little room for manoeuvre in their budgets to reflect local needs and priorities.

242. A number of steps were proposed by witnesses to alleviate the situation. To improve coordination between NICE and PCTs, we support the wider use of implementation consultants, who would provide information both from NICE to the PCTs and from the PCTs to NICE.

243. There must be incentives for clinicians to be very careful about the use of expensive drugs. We recommend that current exclusion of high-cost drugs from the payment by results tariff be reviewed.

244. It is difficult for individual PCTs to decide which areas to prioritise and in which to reduce spending when their expenditure rises as a result of new NICE guidance. In the absence of NICE guidance on disinvestment, we recommend that groups of PCTs should work together to determine appropriate areas of spending in consultation with the public. Such groups should also examine existing treatments to determine which are not cost-effective.

245. While the measures listed above would mitigate the problems PCTs face, the fundamental problem which has to be addressed, according to several witnesses, is NICE's cost-effectiveness threshold. Given the uncertainties, for example about the thresholds used by PCTs, we are not in a position to decide authoritatively whether the current threshold, or threshold range, is appropriate. We recommend that more work similar to that undertaken by Professor Smith and colleagues at York University takes place on the thresholds used by NICE. We are encouraged that NICE has commissioned its own research in this area.

246. During the inquiry, doubt was cast on whether NICE alone should continue to determine the level of the threshold. We consider the present situation is unsatisfactory. We recommend that a separate body, with representation from NICE, the Department, PCTs and others should set the level, or range, to be used. NICE's threshold should be closely linked to that used by PCTs. The threshold should also relate to the size of the NHS budget. The new body should decide whether orphan drugs continue to be treated differently from other treatments.

**247. Demand for NHS services will always exceed the ability to meet it. Not every treatment can be provided to every person. NICE has a vital role to play in the rationing arrangements and, working with Government, should make clear to the public how and why such decisions are made.**

## 5 Implementation

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248. If NHS organisations do not take up its recommendations, NICE's work is pointless. The implementation of guidance is therefore a major challenge to NICE and the Department of Health.

249. NICE produces several different types of guidance which are implemented in different ways:

- Technology appraisals (both single and multiple), which PCTs are under a legal obligation to implement within three months. They are a core standard assessed by the Healthcare Commission [a core standard is an area NHS organisations must implement];
- Clinical guidelines, which organisations are expected to work towards adopting. They are considered developmental standards by the Healthcare Commission [a developmental standard is an area organisations must take steps towards implementing];
- Public health guidance, which organisations should work towards adopting. This is also a developmental standard.

### Who is responsible for implementation?

#### *The Department of Health and NHS organisations*

250. While NICE has a role to play in implementation, in that it must issue guidance that it is possible to follow, that is affordable and that is acceptable to clinicians, it is not its job to ensure that organisations take its advice. It is up to the Department of Health and individual NHS organisations to ensure implementation of guidance. The Department is obliged to make adequate funding available for the implementation of technology appraisals and other types of guidance. Departmental officials argued that PCTs and other purchasers should be able to afford to adopt NICE guidance because of the large increases in NHS resources that have occurred in the past few years.<sup>236</sup>

251. The Department, through SHAs, has also to provide management support for local organisations to help them implement NICE guidance. Dr Felicity Harvey told us:

there is a role for the Strategic Health Authorities in understanding where their Primary Care Trusts are in terms of taking a view as to how they implement this guidance.<sup>237</sup>

252. The implementation of positive technology appraisals (ie. appraisals of products that NICE recommends for use in the NHS) on medicines and public health guidance is chiefly

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<sup>236</sup> Mr Simon Reeve, policy lead for NICE at DoH, stated: 'In terms of your question about affordability, if you have £1.2 billion cumulative pressure (ie. the cost of implementing NICE guidance) and in the same period the cash flow within funding has been over £40 billion, that pressure accounts for about 3% of the growth (Q 19)

the responsibility of PCTs. The implementation of clinical guidelines and appraisals of interventional procedures is likely fall within the remits of both primary and secondary care organisations. Local organisations are required to make funding available for positive technology appraisals almost immediately and should work towards funding other types of guidance. Witnesses indicated that implementing NICE guidance involved more than just allocating resources, however. Dame Gill Morgan stated:

To say it is just £1.2 billion [ie. the cost to the NHS of the products recommended by NICE technology appraisals] I think underplays the complexity of the decisions that have to be made at local level.<sup>238</sup>

## NICE

253. Since the publication of the Health Committee's first report on NICE, the Institute has made considerable efforts to improve the implementation of guidance. It has published a guide, *How to Implement NICE Guidance*, and has issued costing templates to help PCTs plan for the expense associated with guidance. Professor Rawlins stated that pilot studies of commissioning guides were underway:

We have started to develop commissioning guides to accompany our clinical guidelines. This is advice to commissioners on what service provisions they should be contracting for with their providers. As a pilot we have developed five or six and we plan to do more next year. In principle, we would like resources...to expand that role.<sup>239</sup>

254. The implementation directorate at NICE has created a database on the uptake of positive technology appraisals to help commissioners. Of the 89 appraisals that had been published by November 2006, 47 had at least one study on uptake (either commissioned by NICE or in external literature) documented within the database.

255. Mr Dillon told the Committee that regional 'implementation consultants' were now working with the NHS at a local level to improve the adoption of NICE guidance:

Take a look at Birmingham City Hospital, where I was recently, and their approach to introducing technology appraisals, for example. We are seeding that good practice through those implementation consultants around the NHS.<sup>240</sup>

256. Furthermore, NICE has tried to improve training for clinicians, and future clinicians, in the use of evidence-based guidance. Mr Dillon stated:

we decided to talk to those who design the curricula for medical undergraduates and other health professional education, because we believe it is important that those who are in the early stages of the training understand the benefits and the limitations of evidence-based guidance; not that they learn by rote the guidance that NICE has

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238 Q 309

239 Q 640

240 Q 132. There are now five implementation consultants in post, covering the East of England, the North, the West, the South West, and London & South East

produced but that they are in a position, when they come out and start practising, to look at it as part of the support that is available to them.<sup>241</sup>

## To what extent is NICE guidance implemented

257. We received mixed evidence about the implementation of NICE guidance. The Department told us that uptake of NICE guidance was increasing. A review carried out by the Healthcare Commission in 2005/2006 showed that 84.6% of organisations reported full compliance and only 4.6% did not meet this standard. A more recent pilot study showed that 90% of trusts reported “good, excellent or fair implementation” of clinical guidelines and 88% reported a similar result for public health guidance.<sup>242</sup> Officials also cited a report on the ‘Review of NHS Usage of Cancer Drugs Approved by NICE’ in 2006.<sup>243</sup> It:

...showed significant progress in reducing variation in access to NICE approved cancer drugs across the country in the past two years. The report concluded that there has been a 47% increase in use of key cancer drugs since the last assessment in 2004, and that geographic variation in the use of these drugs has decreased.

Professor Richards confirmed that patchiness in implementation had reduced for some treatments. He told us of the “unacceptable variation between cancer networks” that existed in 2004. The Department asked SHAs to develop action plans to tackle the problem. He stated that this approach was successful:

We repeated the study in 2006, after an 18 month gap, and what we saw were two things that mattered. First of all, there was a major increase in the uptake of drugs. The average increase in uptake was 47% so a huge increase in an 18 month period. Equally importantly the variation between networks had decreased for each and every drug.<sup>244</sup>

258. On the other hand, there is evidence that the rate of uptake of guidance may be considerably lower than the Department has estimated. Research by the Audit Commission revealed that NICE guidance was not implemented systematically throughout the NHS and only 25% of PCTs assessed by the Commission could verify that implementation of NICE technology appraisals took place within three months.<sup>245</sup>

259. Witnesses told us that implementation was patchy. The Ethical Medicines Industry Group spoke for many others, stating:

In spite of considerable efforts by NICE to dedicate resource to working with the NHS and other stakeholders to improve implementation of its guidance, implementation remains slow and patchy, denying patients access to medicines that have been found to be clinically and cost effective.<sup>246</sup>

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241 Q 132

242 Q 659

243 Department of Health, September 2006. *Usage of cancer drugs approved by NICE*

244 Q 40

245 Audit Commission, September 2005. *Managing the implementation of NICE guidance*

246 Ev 103

260. The ADPH told us that PCTs in their response to the Healthcare Commission may “fudge” the true position of implementation of NICE guidance. Dr Crayford stated:

a lot of NICE guidance is relatively complex, and simply there are not the measurement tools available in the NHS to ascertain whether or not NICE guidance or guidelines are being implemented to the Nth degree. The honest truth is probably that we do not know, to a very precise measure, to what extent NICE guidance is being implemented.<sup>247</sup>

261. Nevertheless, overall there appear to be fewer problems with the implementation of technology appraisals. Although the Audit Commission found that there were particular difficulties in implementing guidance for high-cost drugs,<sup>248</sup> many witnesses indicated that guidelines on products evaluated as part of STAs or MTAs were implemented fairly swiftly overall.<sup>249</sup> Implementation of technology assessments within three months was clearly a challenge for the health service,<sup>250</sup> but this requirement meant more uniform access to products and services.<sup>251</sup>

262. The implementation of clinical guidelines, however, appears to be more variable. A study conducted in 2004 showed that, for four conditions, only 40 per cent of patients received care that reflected the best practice as described in NICE guidelines.<sup>252</sup> While the situation may have improved since then, the evidence we received revealed much dissatisfaction about implementation of guidelines. Help the Aged told us that clinical guidelines were “poorly implemented”.<sup>253</sup> Dr Tom Marshall, from the University of Birmingham pointed out that the implementation of guidelines was dependent on the decisions of individual GPs.<sup>254</sup> Beat, the eating disorders charity, told us of the ambivalence of GPs towards implementing the guideline on eating disorders, which was published in January 2004, adding:

Given the vital role of GPs in diagnosing and providing access to secondary and specialist care—this ambivalence and sense of burden that NICE guidance places does need to be addressed with some priority.<sup>255</sup>

Diabetes UK also referred to the “difficulties” of implementing NICE clinical guidelines. The patient group stated:

There are inherent difficulties with the position of NICE as their recommendations are neither mandatory but neither are they insignificant in their weight.

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247 Q 328

248 Audit Commission, September 2005. *Managing the implementation of NICE guidance*

249 Q 328

250 Q 319

251 Q 730

252 Hanies et al. *Bulletin of the WHO* 2004; 82: 724–732

253 Ev 112

254 Ev 268

255 Ev 54

263. Take-up is often slow.<sup>256</sup> Best practice is not spread evenly across the country.<sup>257</sup> Variance in rates of guidance implementation has led to criticism of PCTs that do not fund certain treatments and of NICE and the Department for not ensuring that guidance is followed. We discuss these issues below.

## What are the barriers to implementation

264. Witnesses indicated that there were a number of significant barriers to the implementation of guidance. According to NICE, inadequate resources, the lack of a clear organisational process and disagreement with the recommendations were the main barriers to implementation of clinical guidelines. Here we discuss the organisational difficulties and clinical attitudes which have led to the variable implementation of NICE guidance.

### Organisational difficulties

#### Lack of resources

265. Lack of funding is widely seen as the principal reason for the limited implementation of guidance. The Audit Commission assessed the extent to which PCTs made funding available for the implementation of guidance. The Commission found that 85% of survey respondents said that funds for implementing technology appraisals were insufficient. Lack of money or access to necessary resources was cited as one of the main barriers to implementation among NHS bodies. The Audit Commission also reported that recent financial pressures meant that some trusts lacked the capacity to manage change and that better financial management was needed to improve implementation of NICE guidance.<sup>258</sup>

266. Many witnesses agreed that a lack of resources was responsible for the uneven implementation of NICE guidance in England.<sup>259</sup> The ADPH stated that for this reason the implementation of public health guidance affecting primary care services was particularly likely to be slow:

For guidance affecting primary care, there is a tension between promoting implementation and guarding against overspend of budgets, especially on prescribing. The net effect in most PCTs is half-hearted endorsement of NICE guidelines, with agreement that it is the right direction of travel, but little active encouragement and especially where new resources are required.<sup>260</sup>

267. While Professor Mike Richards denied that PCTs refused to fund medicines, he admitted that a lack of resources had played a role in the failure to implement cancer guidelines in the past:

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256 Ev 46, 171

257 Ev 46, 68, 182

258 Audit Commission, September 2005. *Managing the implementation of NICE guidance*

259 Ev 43, 52

260 NICE 111

It is not ...that the PCTs were not funding the drugs. There had been some problem about PCTs not necessarily funding enough of the other costs, like the costs of the nurses and pharmacists because obviously you need more of those to deliver the drugs. There was a problem at a local level with forward planning.<sup>261</sup>

### *NHS organisations' ability to implement guidance*

268. A point made by a number of witnesses was that NHS organisations differed greatly in their capacity to deliver services. Dr Felicity Harvey of the Department of Health told us:

the difficulty you do have with a clinical guideline is that you have delivery at different positions in different parts of the country.<sup>262</sup>

Mr Dillon agreed. He told us that it was “inevitable” that some areas of the health service would take longer to implement recommendations than others for this reason:

For any disease or condition one part of the NHS might, for all sorts of reasons...be much closer to broad concordance with our recommendations when we publish a piece of guidance than in other parts of the NHS, which has not made that investment and has a much longer road to travel in implementing our recommendations.<sup>263</sup>

269. The Minister told us that it was “a challenge” to implement NICE guidance, and clinical guidelines in particular, because PCTs were in different positions at the start of the process. She added that the Department was still learning about the best ways to encourage guidance uptake:

The very nature of the [clinical] guidelines...can be very challenging because it depends where the PCTs themselves are in their experience...there is still quite a lot for us to learn about how we engage and roll those out<sup>264</sup>

She highlighted the need to balance “local priorities and national direction” but acknowledged the need to “to speed up to a standard that is across the whole country”.<sup>265</sup>

270. Those responsible for commissioning services were also blamed for poor implementation. David Anderson from the Faculty of Old Age Psychiatry at the Royal College of Psychiatrists claimed that sometimes PCTs did not implement NICE technology appraisals because those responsible were not aware of them.<sup>266</sup> The Royal College of Nursing also blamed individual commissioners, and the non-obligatory status of clinical guidelines for their limited implementation:

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261 Q 40

262 Q 755

263 Q 652

264 Q 753

265 Q 754

266 Q 274

The main element of difficulty is the apparent intransigence of some commissioners to respond positively to clinical guideline recommendations, as these do not have the same mandatory weight as technology appraisals.<sup>267</sup>

### **The attitude of clinicians**

271. Witnesses also told us that the slow uptake of treatments approved by NICE was sometimes due to the approaches of individual clinicians. The Audit Commission stated that lack of time, lack of knowledge about guidance and resistance to change among clinicians were important contributors to poor implementation.<sup>268</sup>

272. Clinicians sometimes think that NICE's guidance is inadequate. This is particularly likely when there are conflicting guidelines. This was the case for venous thromboembolism, where conflicting NICE guidance and Department of Health guidelines were published at roughly the same time.

273. There are obviously circumstances when clinicians have legitimate grounds for ignoring or rejecting NICE guidance. The publication of new evidence after NICE has published its guidance can undermine the validity of that guidance. There may also be specific cases in which NICE guidance is inappropriate due to the characteristics of the patient. As Professor Rawlins stated, "guidelines are guidelines; they cannot cover 100% of patient interactions".<sup>269</sup>

274. Professor Michael Schlander, from the Institute for Innovation and Valuation in Health Care in Germany, stated that guidance may be "more likely to be adopted when there is strong professional support...guidance needs to be clear and reflect the clinical context". The Royal College of Nursing told us:

To some extent, implementation of NICE guidance often depends on whether the clinicians want to use it...<sup>270</sup>

275. NICE officials and the Department of Health agreed that "clinical engagement" was needed to ensure the effective uptake of NICE guidance.

### **How to improve implementation**

276. While the uptake of some guidance seems to be improving, there is more that could be done. The recommendations we made earlier in the report to improve the evidence base for NICE's assessments, for re-examination of the cost-per-QALY threshold and for better use of experts should lead to improvements in implementation.

277. In addition, we received evidence recommending the following changes, particularly in relation to clinical guidelines:

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267 Ev 204

268 Audit Commission, September 2005. *Managing the implementation of NICE guidance*

269 Q 653

270 Ev 204

- Better planning and sanctions;
- Improved measurement of progress;
- Better clinical engagement;
- Greater involvement of the Royal Colleges and other professional bodies;
- Clarity of guidance status;
- Mandating elements of clinical guidelines.

### **Better planning and sanctions**

278. PCT managers have limited time and resources. NICE clinical guidelines may recommend significant changes to areas of service delivery. The NHS Confederation argued that the Department could help PCTs improve their implementation of clinical guidelines by offering more advice on prioritisation and planning:

Departmental expectations of implementation for NICE products needs to consider how organisations such as a NHS trust and its PCT partner(s) can prioritise which guidelines and within individual guidelines which recommendations to implement first and where business planning is required to progress new or additional resource.<sup>271</sup>

279. AstraZeneca argued that inspection by the Healthcare Commission should be combined with “joined-up financial incentives/penalties for lack of implementation”.<sup>272</sup> The ABPI also told us that there were few sanctions that could be taken when NHS organisations failed to take up guidance. It suggested that the lack of sanctions contributed to the problem of poor implementation.<sup>273</sup>

280. Others agreed that SHA managers had few powers to improve implementation at present.<sup>274</sup> Dr Harvey told us that when PCTs do not take up NICE guidance...:

This is where we would expect the Strategic Health Authorities to be taking management action.<sup>275</sup>

It was not made clear exactly what this management action would involve, however. Indeed, it is perhaps more likely that SHAs would take action to reduce cases of overspending than encourage PCTs to spend more money on implementing NICE guidance.

281. The Minister told us that more could be done to manage the organisations that are slow at taking up guidance:

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271 Ev 181

272 Ev 53

273 Ev 47

274 Q 326

275 Q 55

I would need to consider and discuss with my officials whether there is an enhanced role that the strategic health authorities might be able to play in addressing the timeframe problem that you are identifying.<sup>276</sup>

### **Improved measurement of progress**

282. While some witnesses stressed that inspection by the Healthcare Commission and inclusion of NICE guidance in its core and developmental standards represented a “very powerful tool”<sup>277</sup>, others thought that more could be done. AstraZeneca claimed that the implementation of NICE guidance was not a priority for the Healthcare Commission. Professor Richards stressed the need to assess and record progress:

The way in which we can make progress... is through measurement, through audit.<sup>278</sup>

283. Dr Fiona Adshead, Deputy Chief Medical Officer, agreed. She added that using different ways of assessing the impact of guidance was important.<sup>279</sup>

284. It was argued that self-assessment by PCTs of their implementation of NICE guidance was not adequate.<sup>280</sup> The Healthcare Commission should conduct more in-depth inspections of this element of practice. The charity Help the Aged told us that this was particularly important for clinical guidelines, as they had “no real monitoring or performance by SHAs or the Healthcare Commission”.<sup>281</sup> The Medical Technology Group stated that measuring implementation:

...does not appear to be a sufficiently high priority for the Healthcare Commission.<sup>282</sup>

### **Clinical engagement**

285. As we discussed above, ensuring strong support for guidance among healthcare professionals would improve levels of implementation. Better use of appropriate experts in the appraisal of treatments and development of clinical guidelines, as recommended earlier, should increase the sense of ‘ownership’ among clinicians. This in turn should improve levels of implementation.

### **A role for the Royal Colleges and other professional organisations**

286. Greater involvement of the Royal Colleges and other professional organisations in encouraging implementation could also increase the uptake of NICE guidance. The

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276 Q 757

277 Q 326

278 Q 44

279 Q 46

280 Ev 43, 66

281 Ev 112

282 Ev 150

Multiple Sclerosis (MS) Society drew the Committee's attention to an audit of the NICE guideline for MS which was carried out by the Royal College of Physicians in association with the MS Trust to encourage the uptake of its recommendations.

287. Dr Llewellyn told us that professional organisations could play a role in improving implementation of NICE guidance on the uptake of new technologies as well as disinvestment from old approaches:

I do think there is something about...nationally encouraging colleges, as part of their remit of looking at professional standards, *et cetera*, to look at NICE, to look at the implementation, but also to look at the implementation of disinvestment decisions.<sup>283</sup>

288. In addition, the approval of trusts as training organisations (eg. teaching hospitals) could be linked to uptake of guidance.

### **Clarity of guidance status**

289. It appears that patients and the public are sometimes not aware that only approved technology appraisals are mandatory and that the NHS is not under any obligation to implement other types of guidance within a specific timeframe. This is partly because of the terminology used by NICE: the term 'guidance' is commonly employed for all types of advice given by the Institute, and does not differentiate between that which is obligatory and that which is not.

290. This has led to confusion about the status of the different types of guidance issued by NICE, and elevated expectations among patients of the type of treatment that they will receive. For example, in vitro fertilisation (IVF) is the subject of a clinical guideline. NICE recommended that PCTs should provide three cycles of IVF to eligible patients. Many patients therefore believe that the NICE guideline means that they should have access to three cycles of IVF through the NHS.<sup>284</sup> However, PCTs are not obliged to fund this number of cycles and many do not. Access to such treatment therefore varies widely across the country. The National Infertility Awareness Campaign told us of the disappointment faced by many couples as a result:

It is incredibly frustrating for the one in seven couples affected by difficulties in conceiving that more than three years after the publication of the NICE fertility guideline, huge inequalities in access to NHS funded treatment continue to exist. This is not what patients were promised and many feel let down.<sup>285</sup>

Members suggested that clearer terminology could mitigate this problem.

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283 Q 351

284 Ev 172

285 Ev 174

### ***Mandating elements of clinical guidelines***

291. There was widespread dissatisfaction with the limited implementation of some clinical guidelines. Some witnesses thought that there were elements of certain clinical guidelines that were equally, or more, important than the topics assessed as technology appraisals.<sup>286</sup> It would be impossible for all organisations to implement all the recommendations contained within a clinical guideline. As Mr Dillon stated:

it is difficult to [implement clinical guidelines over a period of time] because a guideline might contain 30 or 40 recommendations and could involve for individual parts of the NHS very significant changes.<sup>287</sup>

292. Nonetheless, it seems illogical that technology appraisals must be implemented while eminently sensible elements of clinical guidelines are not obligatory. An example of what might be done relates to the guidelines on VTE. The risk assessment for all hospital patients for VTE which is included in the NICE guidelines on the subject, could be mandatory whereas other aspects of the guidelines could remain as guidelines. Questioned about this subject, the Minister told us that it was “a reasonable proposition”<sup>288</sup> that sections of clinical guidelines should be mandatory.

### **Recommendations**

293. Despite both the efforts of NICE and other organisations to improve implementation and inspections by the Healthcare Commission to determine levels of implementation, NHS bodies respond to NICE guidance at different rates.<sup>289</sup> This means that new technologies are not available to all patients and the highest standards are not used throughout the NHS.

**294. There need to be additional measures to improve the implementation of clinical guidelines. There should be more help for PCTs to implement guidelines. We recommend that the Department ensure that PCTs are aware of the assistance that is available and develop other ways of helping PCTs to plan and prioritise clinical guidelines.**

**295. Better measurement of guidance implementation is also needed. Self-assessment is not enough. We recommend that the Healthcare Commission conduct more in-depth inspections of this element of practice.**

**296. Improvements to the system of evaluating medicines and greater involvement of experts in the technology appraisal and guideline development processes should also result in guidance that is more acceptable to clinicians.**

**297. We also recommend greater involvement of Royal Colleges and other professional organisations in ensuring implementation. For instance, the approval of trusts as**

<sup>286</sup> Ev 176, Q 592

<sup>287</sup> Q 653

<sup>288</sup> Q 761

<sup>289</sup> See IVF example above

training organisations could be linked to uptake of guidance. Elements of clinical guidelines, particularly those covered by technology appraisals, such as risk assessment of VTE patients, should be mandatory.

298. To combat public confusion over the status of technology appraisals and other types of guidance, we recommend:-

- Recommendations made following technology appraisals should be referred to as ‘NICE directives’; and
- Everything else should be referred to as guidelines or guidance.

299. Greater involvement of PCTs in NICE assessments and a re-examination of the NICE cost per QALY threshold, which we recommend above, would produce guidance which NHS organisations find more affordable.

## 6 Drug pricing

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### Current system

300. The Pharmaceutical Price Regulation Scheme (PPRS) is a mechanism for determining the profit made by drug companies through the sales of branded medicines to the NHS. The scheme is negotiated every five years by the Department of Health and the Association of the British Pharmaceutical Industry (ABPI). The broad outlines of the Scheme are published but the details of any negotiations are not reported, with most information involved treated as commercial in confidence. The objectives of the PPRS are to:

- Secure the provision of safe and effective [branded] medicines for the NHS at reasonable prices;
- Promote a strong and profitable pharmaceutical industry capable of sustained research and development that will lead to the future availability of new and improved medicines; and
- Encourage the efficient and competitive development and supply of medicines to pharmaceutical markets in this and other countries.

301. There are three main operating principles:

- A profit cap with banded upper rates of return for each company for the total ‘package’ of medicines it sells to the NHS;<sup>290</sup>
- Drug prices to be set by the company with freedom to determine the prices of drugs at launch without negotiation with the NHS;
- Price modulation (companies may increase the prices of some products provided they reduce the prices of others such that the expected overall cost to the NHS is the same).<sup>291</sup>

Allowances for innovation and marketing expenses are built into the PPRS. Generic preparations are not considered under the scheme.

302. Various problems with the PPRS have been reported. For instance, it has been argued that the profit cap has no effect and profit repayments are extremely rare. Companies may report higher assessed costs of drug development rather than higher profits. There have also been complaints about the lack of transparency of the scheme.<sup>292</sup>

303. The perceived limitations of the PPRS in achieving value for money for the NHS, in addition to the lack of independent review of the scheme since its establishment over 50

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290 This means that if companies make profits above a certain level, they must refund the NHS a certain percentage of the income

291 This enables companies with product portfolios to adjust to competitive circumstances—ie. their revenues would otherwise be falling—at cost neutrality to the NHS

292 This was covered in our report on *The influence of the pharmaceutical industry*, Fourth Report of Session 2004–05, HC 42–I

years ago, led the Office of Fair Trading (OFT) to examine the PPRS in detail. The OFT produced a report on the subject, which was published in February 2007.

### The OFT's findings

304. The report highlighted the fact that, under the PPRS, two drugs for the same condition that have a very similar level of benefit to patients, may vary by up to 500% in price (ie. the difference between a branded drug and a generic version). This indicates that value to the patient has no effect on the price of the product. It also found that the profit cap had a negligible impact, with only 0.01% of payments affected. The system of price cuts was criticised as ineffectual. Simeon Thornton, formerly of the OFT, who led the investigation, told us that the findings indicated that the PPRS was no longer “fit for purpose”.<sup>293</sup>

305. The report concluded that a system of “value-based pricing” whereby each drug would be reviewed and priced “in accordance with the clinical benefits it produces relative to an appropriate comparator” would offer better value for money to the NHS. The OFT’s recommendations for such a system included:

- all new active substances would be assessed to determine cost-effectiveness before they came onto the market (ex ante) through a fast track appraisal process;
- the existing stock of drugs would be assessed on a rolling basis through ex post reviews;
- risk-sharing contracts could be agreed in principle if there was insufficient information at the time of launch to reach a robust view on the cost effectiveness of a drug;
- different pricing arrangements could be negotiated to accommodate situations in which value differs by indication and / or subgroup;
- The price of branded drugs should fall once a generic version enters the market.

The OFT recommended that NICE should play a key part in the new system of value-based medicines pricing.

### Government response

306. The Government issued an ‘interim response’ to the report. It neither accepted nor rejected the proposals for a system of value-based pricing of drugs. It stated that the PPRS had been effective in the past, had contributed to a “stable pricing regime” and had helped “sustain a strong pharmaceutical and bioscience industry”. It added, however, that:

we agree with OFT that encouraging cost-effective prescribing is crucial and we will continue to explore further opportunities in this area.<sup>294</sup>

307. The Government agreed that better mechanisms were needed to ensure that the NHS paid a fair and affordable price for medicines. It added that the uptake of cost-effective

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293 Q 435

294 [www.gnn.gov.uk/environment/fullDetail.asp?ReleaseID=304783&NewsAreaID=2](http://www.gnn.gov.uk/environment/fullDetail.asp?ReleaseID=304783&NewsAreaID=2)

medicines needed to increase. However, the response indicated that any new pricing system must “encourage research and reward innovation”. The system must also provide a stable market for industry.

308. Discussion between the Government and the pharmaceutical industry is ongoing. The Minister could not tell us when the Government’s final response to the OFT’s report would be published. She said negotiations were ongoing:

We have given our interim response and it would be inappropriate for me to go further because of the negotiations starting on the PPRS. I feel I am caught here between a rock and a hard place...

I am not trying to be unhelpful, I am saying I can only lay out the principles. I am acknowledging the points you are making but we now need to proceed with those detailed negotiations.<sup>295</sup>

## Arguments

309. The evidence we received revealed much support for the OFT’s recommendations in principle, but many voiced concerns about how such a system would work in practice. The main issues were:

- Value for money;
- The effect of value-based pricing on innovation;
- How value would be determined and whether enough evidence is available at launch to make such a judgement;
- The risk of lower prices for medicines, and consequent effect on the pharmaceutical industry.

## *Value for money and innovation*

310. Mr Thornton, who led the OFT’s investigation, told us that the PPRS does not reward companies for producing the best medicines and as such does not ensure value for money for the NHS. Although it encourages innovation, the PPRS does not send the right ‘signals’ to drug developers:

PPRS profit and price controls take no account of the value to patients of the drugs companies are producing.<sup>296</sup>

311. Professor Adrian Towse of the Office of Health Economics agreed that the PPRS encouraged the development of new medicines but admitted that it had little effect on the price of medicines relative to each other. This means that the cost of similar drugs may vary greatly and the production of drugs that are valuable to the NHS and those that are not are both rewarded:

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295 Q 741, 742

296 NICE 119

[The PPRS] does not incentivise efficient relative prices. That is not its job.<sup>297</sup>

312. Some witnesses argued that innovation should be encouraged for its own sake, as it was likely to lead to overall benefit to patients in the long term. Medicines manufacturers in particular claimed that small improvements in product profiles were necessary in order to achieve significant gains over time. Professor Peter C Smith told us that innovation should be promoted in certain circumstances:

There may however be reasons beyond health benefits why one may want to accept certain therapies, for example perhaps for the research benefits that it confers and the promise of longer-term benefits in terms of cheaper therapies in future.<sup>298</sup>

313. However, many witnesses told us that the current system did not promote the development of drugs that would most benefit NHS patients, yet there was an expectation that products would be purchased simply because they were new. Professor Karl Claxton from York University stated:

It is rather like saying that everybody should be compelled to buy the first generation iPod on the basis that if they do maybe Apple will produce a product that people will buy without being compelled to do so. It over-incentivises innovation in things that are not of value to the healthcare system.<sup>299</sup>

314. Professor Towse argued that innovation and development in areas of unmet need should be encouraged through better prescribing, rather than changing the pricing system. If prescribers gave patients medicines that were both valuable to the patient in terms of health benefit and represented value for money for the NHS, this would indirectly encourage the development of better, and possibly cheaper, medicines. Increased demand would lead to increased supply of the type of products needed by the health service. He told us:

You do not need to force down prices; you just need doctors to change their prescribing.<sup>300</sup>

He claimed that we need “a better version of what we currently have”<sup>301</sup> rather than a completely new system.

### **Adequate evidence for price determination**

315. A system of value-based pricing would require evaluation of products individually at the time of launch. We have already discussed the limited public evidence that is available and the associated difficulty of assessing medicines at this point. There was much concern that the value of new products would not be demonstrated fully, and that this would lead to

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297 Towse A, *Health Economics* 2007; 653–665

298 Q 180

299 Q 439

300 Q 440

301 Q 459

adoption of a lower than justified price. Professor Towse told us it would be “hugely constraining” to provide all the necessary information before launch:

It is unrealistic to assume that everything must be on the table before the drug is launched. We should not allow ourselves to get the NHS into a position where that is the case. We must be able to collect good quality evidence once the product is launched.<sup>302</sup>

316. Others agreed that the value of some products was unlikely to be clear just after products were licensed.<sup>303</sup> Dr Richard Barker from the ABPI stated that determining value at launch would be difficult in the real world:

So [the OFT’s] proposal of value-based pricing sounds very straightforward but I think anybody who has looked at their recommendations very closely will realise that it is actually very hard to do in practice.<sup>304</sup>

317. Professor Jon Nicholl from the University of Sheffield told us that there would always be uncertainty about the benefits and risks of medicines at launch. Moreover, it might not be possible to resolve these uncertainties, even with additional research.<sup>305</sup>

318. As we discussed earlier, the provision of better evidence at launch would allow more effective evaluation of whether medicines are appropriate for use in the NHS. If the benefits are proven, it was argued that it would be easier to determine a suitable price.<sup>306</sup> Professor Claxton recommended that a product’s price should be linked to the evidence that proves its efficacy:

A link between price and the value of evidence provides incentives for manufactures to invest in the type of evidence needed by the NHS early in the development of their products.<sup>307</sup>

319. On the other hand, it must be possible to determine appropriate prices for medicines using the information that is currently available. Public authorities in France and Australia carry out medicines pricing and, most importantly, medicines manufacturers themselves are able to determine prices they believe to be suitable for their products.

### ***The effect of lower prices***

320. Companies were concerned that a value-based system would drive down the prices they are able to charge for their products. Professor Towse admitted that companies currently charged, “what they think the market will bear and what returns they can get”.<sup>308</sup> This would not be possible with value-based pricing.

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302 Q 457

303 Q 428

304 Q 429

305 Q 461

306 Q 522

307 NICE 116

308 Q 441

321. Some witnesses argued that lower drug prices would be a positive outcome.<sup>309</sup> Dr Kiran Patel told us:

If drugs were cheaper we would not be having a lot of the debates that we have today.<sup>310</sup>

322. On the other hand, Professor Claxton stated that drug prices overall need not fall, if the value of medicines to the NHS is proven:

It is perfectly possible that the overall NHS spend on branded drugs may increase, particularly if new and valuable pharmaceuticals which can command higher prices are developed.<sup>311</sup>

### Generics

323. The inclusion or exclusion of generic drugs in cost-effectiveness analyses could affect prices charged under a value-based system. The industry argued that the price of branded, on-patent drugs should not be compared to generics.<sup>312</sup> New, branded, products are likely to be considerably more expensive than similar generic versions, and therefore appear poor value for money. At the same time, if a drug is innovative and represents incremental value to patients, there will be no generic version. According to Mr Thornton it makes no sense to exclude generic medicines from cost-benefit analyses of new products:

Given the limited resources the NHS has at its disposal, it cannot afford, on grounds of both efficiency and fairness to patients, to ignore relevant comparators on the grounds that they are ‘too cost effective’. If the best available treatment is a generic, then treatments must demonstrate their benefits in relation to the generic to receive higher prices...<sup>313</sup>

He added that consideration of cheaper alternatives was necessary to give companies the right incentives to invest in areas of greatest medical need.

### Threat to industry

324. Medicines manufacturers have threatened to move their research, development and manufacturing interests elsewhere, possibly in response to the prospect of lower prices. Some witnesses dismissed these threats as “red herrings”, however.<sup>314</sup> Mr Thornton stated:

there are lots of explicit drivers for where investment takes place, such as having good clinical trial networks, financial incentives, tax incentives and a well skilled labour force. There is a question as to whether that sort of threat is really credible.<sup>315</sup>

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309 Q 186

310 Q 284

311 NICE 116

312 Q 398

313 NICE 119

314 Q 485

315 Q 485

Professor Nicholl added:

There is every incentive for the companies to continue to conduct R&D here. As to why they are against [a value-based pricing] scheme, a rather sinister voice whispers to me that perhaps the products are not quite as effective as they would have us believe.<sup>316</sup>

325. On the other hand, UK prices for medicines may affect prices in other countries. The UK medicines market represents only 3% of global sales, so the direct effect of changes to how the NHS pays for medicines on overall revenue would be small. However, many other countries use the UK as a reference point for setting their own prices. Witnesses estimated that such countries constituted about 25% of global demand for medicines.<sup>317</sup>

326. Drugs manufacturers indicated that they might prefer not to bring products to market in the UK rather than risk many other countries adopting a lower price.<sup>318</sup>

### Risk sharing and drug pricing

327. The OFT report recommended that the problems of price setting with limited data and uncertainties about treatments at launch could be mitigated using risk-sharing schemes. The financial risks associated with uncertainty due to lack of evidence would be split between the NHS and the manufacturer. As Professor Peter Smith told us:

There are three [possible] approaches to take. One is to wait and do more research; one is to take a provisional decision that one may reverse in the future; and the other is in some way to get the companies to share the risks of accepting the drug early.<sup>319</sup>

328. Key to effective risk-sharing is the adequate measurement of the effects of treatment following its introduction to the NHS. Witnesses told us that “monitoring studies” should be conducted to determine whether the NHS or the manufacturer should pay for the treatment. According to Professor Jon Nicholl, an initial price may be set, based on best estimates of efficacy:

At a later date when the monitoring study is concluded, the cost-effectiveness of the treatment can be recalculated, and the appropriate NHS price of the drug reviewed. If the treatment is less cost-effective than previously estimated there may be reimbursements paid for earlier treatments, as well as a change in the price paid for future treatments.<sup>320</sup>

329. Professor Claxton agreed that a lower price could be set initially, while such studies are carried out, to ensure that the NHS does not lose out financially:

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316 Q 487

317 Q 505

318 Q 421

319 Q 190

320 NICE 117

If the type of evidence required cannot be provided with provisional approval, then price ought to be reduced so that the cost-effectiveness of the drug is not uncertain.<sup>321</sup>

330. However, there are problems with this approach. They include the difficulty of imposing clinical trial conditions in the real world of the NHS. According to Professor Nicholl, the universal availability of drugs used under risk-sharing schemes means that there are no comparator groups and no controls. This means that the uncertainties observed initially are not resolved:

One cannot collect evidence which says that the outcomes for patients who are being treated by the treatments being made available under a risk-sharing scheme are better than for patients not being treated by those drugs. In turn the consequence is that we cannot resolve the uncertainties in the cost and effectiveness as we try to do in the first place.<sup>322</sup>

331. Such studies are expensive, and who should pay must be determined. Professor Nicholl and others stated that they should be publicly funded, possibly with a charge to industry to cover the cost of acquiring the information or consideration of the cost when determining the price of a product.<sup>323</sup>

332. NICE and the Department of Health have recently accepted a risk-sharing scheme for the multiple myeloma drug Velcade (bortezomib)<sup>324</sup> and the OFT report indicated the positive aspects of risk-sharing. However, significant problems have been observed with such schemes in the past. The scheme to evaluate the benefits and risks of beta interferon and glatiramer for multiple sclerosis does not appear to have been successful (see box below for details). Witnesses warned that “we should be wary of rushing into this as a generalised mechanism”.<sup>325</sup>

333. Some treatments may be more suitable for risk-sharing than others. Bortezomib, for example, lends itself to such a scheme because there is a protein marker that indicates whether a patient has responded to the drug or not.<sup>326</sup> Dr Barker told us that such markers were likely to be more common in the future:

increasingly we are developing markers of response and so it will be more practical for medicines to be given with a marker of response as a diagnostic so that everybody has the confidence, the patient has the confidence, the clinician has the confidence, the NHS has got the confidence that this is being given to a patient for whom it will work.<sup>327</sup>

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321 NICE 116

322 Q 510

323 Q 516, 522

324 The NHS will pay for patients who respond to treatment; the manufacturer will meet the cost of treatment for patients who do not respond

325 Q 413

326 *Ibid*

327 *Ibid*

334. Professor Nicholl also indicated that risk sharing schemes would be suitable to determine the relative prices of two treatments where patients and clinicians have not yet decided which treatment is preferable.<sup>328</sup>

#### **Example of the multiple sclerosis risk-sharing scheme**

In January 2002, NICE refused to recommend beta-interferon and glatiramer for patients with multiple sclerosis as the cost per QALY for treatment was judged to be too high. The manufacturers of the drugs came to an agreement with the Department of Health whereby the drug could be used in the NHS as part of a long-term trial. It was agreed that if the drug was less effective than £36,000 per QALY, industry would recompense the NHS.

The evaluation is to take 10 years. Sheffield University signed a contract with an MS organisation, four medicines manufacturers and the Department of Health (industry division) for an initial three years. Sheffield researchers wrote a three-year report which was submitted last year. The next seven year contract then went to tender and Sheffield did not bid. In evidence to the Committee, Professor Jon Nicholl, who led the study, suggested that there were problems with the scheme.

Details of the scheme have not been publicised and the report from Sheffield is not in the public domain, but there are indications that the study will not yield reliable information about the beneficial effects of the drugs.<sup>329</sup>

Professor Nicholl told us that the evidence obtained from schemes that used historical controls, such as the MS scheme, was “weak”. He stated that his university had decided not to bid for the rest of the contract because, “we were less certain about the ability of the scheme to deliver the science that we hoped it would.”<sup>330</sup>

#### **Box 4.**

335. An alternative to risk sharing is NICE’s recommendation that technologies may be used in the NHS but only in the context of research. This recommendation is made when therapies or procedures do not have strong enough supporting evidence but appear to be promising. It has been argued that an ‘only in research’ recommendation is a more suitable way of dealing with uncertainty than risk-sharing, because the technology is given in a controlled manner and is not open to all prescribers. It is therefore possible to gain more robust evidence.<sup>331</sup> The approach is supported by NICE’s Citizens Council. However, it is hard to implement an ‘only in research’ recommendation. For instance, there is no way of ensuring that the work is done and there are no criteria for when the recommendation should be made. Such a recommendation is therefore not attractive to Appraisal Committees.

### **Potential role of NICE in drug pricing**

336. Altering the drug pricing system in line with the OFT’s recommendations could mean large-scale changes for NICE. The expertise of the Institute would be used in helping to determine the prices of drugs. As a consequence, it would be required to advise on the clinical and economic value of medicines prior to launch.

328 Q 511

329 RSM

330 Q 515

331 Chalkidou K et al, *Journal of the Royal Society of Medicine* 2007, 100: 453–460

337. Witnesses thought that NICE already carried out some of the work that would be necessary to introduce a system of value-based pricing.<sup>332</sup> Professor Claxton stated that a stronger version of the current technology appraisal process could be used:

NICE is already conducting the kind of analysis that would underpin such negotiation...I believe that existing processes would have to be strengthened. The principles that underpin the STA are correct and provide a suitable basis, but at the moment all the appraisal committee has to do is decide whether or not it believes that at a given price the product is cost-effective. Is it above or below a certain threshold? Here the demands will be a little greater.<sup>333</sup>

338. Some witnesses indicated that there would be benefits of involving NICE in price negotiations beyond the NHS paying a higher price for valuable medicines and lower price for less beneficial drugs. Dr Crayford stated that such a move could also reduce the perception within the NHS that new products cost too much. He told the Committee:

There is certainly a lot of cynicism in the NHS more widely about the cost ...and that may go some way to ameliorate that.<sup>334</sup>

339. The OFT report recommended that NICE could work with the SMC and the All Wales Medicines Strategy Group to advise on the value of all new medicines.<sup>335</sup> Some witnesses wondered if this arrangement in the devolved health services was workable.<sup>336</sup>

340. Other witnesses thought that NICE should not be involved in drug pricing at all. Dame Gill Morgan from the NHS Confederation stated that the issues of price and cost-effectiveness should be kept separate:

The Appraisal Committees are set up to do the scientific analysis about the effectiveness, the risks and the cost benefit. If you start to bring price into that as a variable, I think it would so change the discussion and the debate in those groups that you would not get the decision about cost-effectiveness which I think is a jewel in the crown of NICE.<sup>337</sup>

341. While Mr Dillon stated that he was “intrigued” at the possibility that NICE could become involved in drug pricing<sup>338</sup>, Professor Rawlins warned that no changes should be introduced that would reduce the standards of evaluation used by the Institute. He added that any changes to the system should be introduced gradually:

we would not wish in any way to change our standards or robustness or anything like that, and, secondly, I do not think it is possible to suddenly do it. It is not just the amount of money we have to set up the committees and the staff and everything, but

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332 Q 502

333 Q 458

334 Q 358

335 Q 463

336 *Ibid*

337 Q 360

338 Q 712

there just are not enough health economists around or that sort of expertise around in the country...it would be a massive workload<sup>339</sup>

342. Elsewhere in the world, the organisation that determines best use of medicines is also closely linked with price determination. In France, for example, the Transparency Commission assesses all new medicines, once licensed, before a price is set. Recommendations from this group are passed on to the Economic Committee for Health Products, which negotiates with industry to fix the price of drugs and devices.

## Recommendations

**343. Given that discussions between the Government and the pharmaceutical industry on future drug pricing arrangements are already underway, we do not make any firm recommendations on how a future system should operate. However, we agree with the Government that better mechanisms are needed to ensure that the NHS pays a fair and affordable price for medicines. Any change to the system of medicines pricing is likely to have profound consequences for NICE and the Institute should be involved in any changes that might affect how it works. Moreover, it should be funded for the alterations in practice it might be required to make.**

**344. We recommend that risk-sharing schemes be used with caution. They should not be used as a catch-all in cases of uncertainty over a drug's benefit. The Department must bear in mind the evidence that will be foregone in such cases. Uncertainty would be better addressed by the careful design and performance of a publicly funded randomised controlled clinical trial. Better use should be made of NICE's 'only in research' recommendation in this regard.**

**345. The short evaluation of all medicines at launch, which we recommended earlier, could be established in such a way that negotiations on drug pricing could be incorporated into the process. The NICE evaluation process could also take account of potential improvements in subsequent data about clinical and cost effectiveness, and its consequences for product pricing.**

## 7 Conclusions

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346. Healthcare budgets in England as elsewhere are limited. Difficult decisions have to be made about priorities. Patients cannot expect to receive every possible treatment. In other words rationing is essential. Dr Imogen Evans echoed the views of many witnesses when she stated, “the cost of healthcare weighs heavily on all national economies, and the need to set priorities is inescapable”. Moreover, it would be sensible if any choices to be made were based on robust evidence, with the aim of offering patients the best available treatments. In this context NICE evidently has a key role to play.

347. The evidence we received indicated that since it was established in 1999 NICE has achieved much. Its work has led to a greater concentration on cost- as well as clinical effectiveness. Its evaluation processes are generally robust, and are well-regarded worldwide. However, there are failings.

348. The evaluation process was criticised on a number of grounds:

- The speed of publication of guidance is a major problem; licensed medicines are often not prescribed while PCTs and clinicians wait for NICE to make a decision. Delayed access to products later found to be both cost and clinically effective harm both patients and the NHS.
- Topic selection is also unsatisfactory. Selecting topics for assessment is appropriate for clinical and public health guidance, but not for technology assessments. While there needs to be an assessment of all new drugs, it is very important not to skew spending to expensive secondary care at the expense of public health and other clinical guidance. There is also far too little emphasis on disinvestment. Here we found NICE’s responses to our questions disingenuous. While few older treatments may do no good at all, many will not be cost-effective.
- The wider benefits of treatment to society, for example to carers, are not included in NICE’s cost : benefit analyses; and
- NICE often does not have all the information it needs to make a full assessment. It does not have access to all the information the MHRA uses and clinical trials are usually designed without NICE’s work on cost-effectiveness in mind.

349. **To improve the evaluation process, we recommend that:**

- **All drugs be assessed at the time of licensing, so that clinicians can prescribe useful and cost-effective products as soon as they are launched;**
- **There be more emphasis on disinvestment;**
- **Our last report on NICE recommended that the legislation be changed to accommodate the need to ensure that assessments of products take account of the wider benefits to society; we make the same recommendation here;**

- **NICE have access to the same material used by the licensing body, clinical trials be registered and there should be closer working between NICE and the industry to enable these early assessments to take place.**

350. **The affordability of NICE guidance and the range, measured in cost-per-QALY, it uses to decide whether a treatment is cost-effective is of serious concern. The threshold it employs is not based on empirical research and is not directly related to the NHS budget, nor is it at the same level as that used by PCTs in providing treatments not assessed by NICE, which tends to be lower. Some witnesses, including patient organisations and pharmaceutical companies, thought NICE should be more generous in the cost per QALY threshold it uses, and should approve more products. On the other hand, some PCTs struggle to implement NICE guidance at the current threshold and other witnesses argued that a lower level should be used. However, there are many uncertainties about the thresholds used by PCTs. Accordingly we cannot authoritatively at this stage recommend a change in NICE threshold. Nevertheless, we recommend that it be reviewed. We do recommend that an independent body determine the threshold used when making judgements of the value of drugs to the NHS.**

351. **The implementation of non-mandatory guidance is variable. This is due to a variety of causes, including the threshold used by NICE for determining cost-effectiveness, lack of clarity about the status of guidance, lack of involvement of PCTs in the development of guidance, clinicians' disagreement about the worth of some NICE guidelines. This is a particular problem when clinicians believe that NICE has not used the right experts or when different guidelines from other organisations are published about the same time as NICE guidance.**

352. **To improve the implementation of NICE guidance we recommend:**

- **More help for PCTs to implement guidance;**
- **Better assessment of the level of uptake;**
- **That PCTs should play a larger role in the development of guidance;**
- **Better use of experts in the development of guidance;**
- **A change in the terminology used by NICE, to clarify to patients what they can and cannot expect by right from their local NHS organisation; and**
- **That some elements of clinical guidelines be made mandatory.**

353. **Changes to the system of medicines pricing may follow the OFT's report on the PPRS. NICE should be involved in any new system, and to ensure full involvement in any such process, NICE should be adequately resourced.**

354. **We recommend that risk-sharing schemes be used with caution. They should not be used as a catch-all in cases of uncertainty over a drug's benefit. Uncertainty would be better addressed by the careful design and performance of a publicly funded randomised controlled clinical trial. Better use should be made of NICE's 'only in research' recommendation in this regard.**

355. NICE does a vital job in difficult circumstances. The development of more and more health technologies and procedures, coupled with rising patient expectations and the ageing population, is going to make it even more difficult in the future. NICE can improve and we have recommended improvements. In the past it has changed and we are sure it can do so again. Given the difficult environment, NICE requires the backing of the Government. Ministers must support NICE, not seek to undermine it. NICE must not be left to fight a lone battle to support cost- and clinical effectiveness in the NHS.

## Conclusions and recommendations

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1. We note that it is not the role for Ministers to directly or indirectly seek to influence the NICE decision-making process. (Paragraph 68)
2. It is clear that the environment in which NICE operates has changed considerably since the Institute was established in 1999. It is also clear that there is a vital role for NICE in the rationing of healthcare and in encouraging best clinical practice. In the future the role of NICE will be ever more important and demanding with new expensive drugs and a slower rate of growth in NHS expenditure. There remains, however, concern about aspects of how NICE does its job. (Paragraph 79)
3. It seems to us appropriate that topics are selected for interventional procedures, clinical guidelines and public health guidance. It is not appropriate, however, to limit technology appraisals to selected, often new and expensive, products. Instead, as we recommend below, all new drugs should be assessed. (Paragraph 94)
4. Witnesses were concerned that NICE's focus on acute treatments, in particular medicines, could skew NHS spending towards selected new and expensive (NICE-approved) drugs for acute illness. (Paragraph 95)
5. In our previous report we recommended that NICE give more emphasis to examining old technologies to encourage disinvestment. This the organisation has failed to do as fully as we expected. Its statement that few interventions have absolutely no benefit may be true but is irrelevant. Many treatments currently used are not cost-effective as many studies attest. NICE should adopt a similar standard of cost-effectiveness in assessing such treatments as it uses in its technology appraisals. The organisation must now give more emphasis to disinvestment. One approach would be to undertake more MTAs, which would reveal the existing treatments that provide poor value for money. (Paragraph 96)
6. We heard much criticism of the use of QALYs. Some of the criticisms seem to be the special pleading of disappointed parties. It is vital that a method which allows comparison of the benefits and costs of different treatments for different conditions is used in cost-effectiveness evaluations. However, it is also vital that the system is accurate and reflects the real costs to society and the benefits to patients. We recommend that:
  - Research is undertaken to follow up specific guidance to see whether the predictions of the cost-effectiveness analysis are borne out in practice;
  - Wider benefits and costs, such as costs borne by carers and social care services, be more fully incorporated into NICE's assessment. We were told that this would have to be a decision for Parliament. (Paragraph 120 )
7. NICE does not have all the information it needs to assess and compare treatments. First, while access to EMEA documents and other changes have improved NICE's access to information, it still does not have access to all the relevant information which is available. Secondly, clinical trials undertaken by pharmaceutical companies

understandably focus on generating data about the drug's efficacy and safety, which is required for the licensing process; such trials are not usually designed to generate the type of data on cost-effectiveness which NICE requires. Third, in some areas, without commercial sponsors, notably public health and many physical and psychological therapies, there is little research about the cost-effectiveness of different interventions. (Paragraph 143)

8. We recommend that NICE be granted the right to see all the evidence the MHRA uses when making its decisions. We appreciate that this would mean that there would be some commercial-in-confidence material that NICE could not make public when it published its guidance. (Paragraph 144)
9. We welcome the fact that both NICE and drug companies are aware that they need to collaborate closely to ensure that clinical trials are undertaken with the needs of NICE appraisal in mind. The Government should encourage all countries in which large-scale clinical trials take place to adopt a similar policy. We support the mandatory registration of all clinical trials so that the results of all negative trials are accessible. We recommend that NICE assesses and reports the quality of the research it receives. (Paragraph 145)
10. More publicly funded research should be undertaken to assist the development of public health guidance and other areas without commercial sponsors. (Paragraph 146)
11. Many witnesses thought that too few experts with the relevant detailed expertise were involved in the process of producing guidance. Since they have a permanent membership, Appraisal Committees are unlikely to have such experts. They do consult experts, but this is unsatisfactory because such experts appear for the day alone. We therefore recommend that Appraisal Committees appoint specialist advisers, without voting rights, to work with the Committee throughout consideration of a technology appraisal or clinical guideline. This will improve guidance and ensure public and patient confidence in the system. Decisions about which experts should be appointed should remain the responsibility of NICE following consultation with the appropriate clinical bodies. (Paragraph 156)
12. The wide consultation which takes place during the development of NICE guidance is greatly valued. While we agree that it is difficult for some organisations to respond within the often brief time limits, we recognise that a long consultation period would slow the guidance production time further. Nevertheless, the situation would be improved if NICE were to give interested stakeholders greater warning of forthcoming consultations, to allow them to organise their resources in time to respond effectively. (Paragraph 168)
13. Some consultees complain that their views are ignored. We understand that NICE does not have the resources to respond individually to each consultee. NICE could, however, issue a standard response to inform every consultee how it will respond and setting out how the system works. (Paragraph 169)

14. We note the pressure to change the grounds for appeal, but consider changes might cause more problems than they solved. Allowing additional evidence at the appeal stage would extend the process significantly, and might discourage companies from producing high quality trial data at the time of first assessment. It also might risk more “gaming” appeals. We make recommendations in the next section which we expect will lead to fewer appeals being brought in the first place. (Paragraph 184)
15. A shorter, less in-depth initial evaluation of medicines at an early point would be useful. It is important that clinicians have access to independent information about new therapies as soon as they are available. However, a quick, in-depth, fully consultative evaluation for all new medicines by the time of launch is not possible. We therefore recommend that NICE should examine all new medicines for their indications as set out in the marketing authorisation. Assessment should be carried out during the period between licensing and launch. It should be brief and published prior to, or at the time of, launch. There should be no formal appeal process and only limited consultation. These brief assessments should be followed by a larger scale multiple technology appraisal for selected products (an MTA or STA as appropriate) at a later date, when more evidence is available. The technology appraisal should include current levels of consultation. The guidance issued at this later stage should be definitive, over-riding that issued earlier. (Paragraph 200)
16. Since providing an evaluation of all drugs at launch will be a more rough and ready process, it would be inappropriate to use the same threshold range as the full assessment. One of the aims of the new process is to ensure that treatments which are obviously cost effective are available at an earlier stage than at present. We therefore recommend that a threshold below the current range be used in these early assessments. This could be raised for individual products in special circumstances, for instance where no other treatment exists. At the time of the full assessment, the cost per QALY threshold could increase. (Paragraph 201)
17. The threshold or ceiling NICE employs (measured in pounds sterling per QALY) to decide whether a treatment is cost-effective, and so should be available in the NHS, is not based on empirical research. Nor is the threshold directly related to the NHS budget, since the threshold has remained constant while the budget has increased hugely since 1999. (Paragraph 239)
18. The threshold used by NICE does not take into account the funding decisions made by PCTs generally. For interventions not assessed by NICE, PCTs appear to use thresholds which vary from treatment to treatment but for the most part seem to be lower than the NICE threshold. (Paragraph 240)
19. Many PCTs struggle to afford to implement NICE technology appraisals, as well as clinical guidelines. As more interventions are evaluated it is feared that the position will become unsustainable. Funding is essentially ring-fenced for technology appraisals, leaving PCTs little room for manoeuvre in their budgets to reflect local needs and priorities. (Paragraph 241)

20. A number of steps were proposed by witnesses to alleviate the situation. To improve coordination between NICE and PCTs, we support the wider use of implementation consultants, who would provide information both from NICE to the PCTs and from the PCTs to NICE. (Paragraph 242)
21. There must be incentives for clinicians to be very careful about the use of expensive drugs. We recommend that current exclusion of high-cost drugs from the payment by results tariff be reviewed. (Paragraph 243)
22. It is difficult for individual PCTs to decide which areas to prioritise and in which to reduce spending when their expenditure rises as a result of new NICE guidance. In the absence of NICE guidance on disinvestment, we recommend that groups of PCTs should work together to determine appropriate areas of spending in consultation with the public. Such groups should also examine existing treatments to determine which are not cost-effective. (Paragraph 244)
23. While the measures listed above would mitigate the problems PCTs face, the fundamental problem which has to be addressed, according to several witnesses, is NICE's cost-effectiveness threshold. Given the uncertainties, for example about the thresholds used by PCTs, we are not in a position to decide authoritatively whether the current threshold, or threshold range, is appropriate. We recommend that more work similar to that undertaken by Professor Smith and colleagues at York University takes place on the thresholds used by NICE. We are encouraged that NICE has commissioned its own research in this area. (Paragraph 245)
24. During the inquiry, doubt was cast on whether NICE alone should continue to determine the level of the threshold. We consider the present situation is unsatisfactory. We recommend that a separate body, with representation from NICE, the Department, PCTs and others should set the level, or range, to be used. NICE's threshold should be closely linked to that used by PCTs. The threshold should also relate to the size of the NHS budget. The new body should decide whether orphan drugs continue to be treated differently from other treatments. (Paragraph 246)
25. Demand for NHS services will always exceed the ability to meet it. Not every treatment can be provided to every person. NICE has a vital role to play in the rationing arrangements and, working with Government, should make clear to the public how and why such decisions are made. (Paragraph 247)
26. There need to be additional measures to improve the implementation of clinical guidelines. There should be more help for PCTs to implement guidelines. We recommend that the Department ensure that PCTs are aware of the assistance that is available and develop other ways of helping PCTs to plan and prioritise clinical guidelines. (Paragraph 294)
27. Better measurement of guidance implementation is also needed. Self-assessment is not enough. We recommend that the Healthcare Commission conduct more in-depth inspections of this element of practice. (Paragraph 295)

28. Improvements to the system of evaluating medicines and greater involvement of experts in the technology appraisal and guideline development processes should also result in guidance that is more acceptable to clinicians. (Paragraph 296)
29. We also recommend greater involvement of Royal Colleges and other professional organisations in ensuring implementation. For instance, the approval of trusts as training organisations could be linked to uptake of guidance. Elements of clinical guidelines, particularly those covered by technology appraisals, such as risk assessment of VTE patients, should be mandatory. (Paragraph 297)
30. To combat public confusion over the status of technology appraisals and other types of guidance, we recommend:-
  - Recommendations made following technology appraisals should be referred to as ‘NICE directives’; and
  - Everything else should be referred to as guidelines or guidance. (Paragraph 298)
31. Greater involvement of PCTs in NICE assessments and a re-examination of the NICE cost per QALY threshold, which we recommend above, would produce guidance which NHS organisations find more affordable. (Paragraph 299)
32. Given that discussions between the Government and the pharmaceutical industry on future drug pricing arrangements are already underway, we do not make any firm recommendations on how a future system should operate. However, we agree with the Government that better mechanisms are needed to ensure that the NHS pays a fair and affordable price for medicines. Any change to the system of medicines pricing is likely to have profound consequences for NICE and the Institute should be involved in any changes that might affect how it works. Moreover, it should be funded for the alterations in practice it might be required to make. (Paragraph 343)
33. We recommend that risk-sharing schemes be used with caution. They should not be used as a catch-all in cases of uncertainty over a drug’s benefit. The Department must bear in mind the evidence that will be foregone in such cases. Uncertainty would be better addressed by the careful design and performance of a publicly funded randomised controlled clinical trial. Better use should be made of NICE’s ‘only in research’ recommendation in this regard. (Paragraph 344)
34. The short evaluation of all medicines at launch, which we recommended earlier, could be established in such a way that negotiations on drug pricing could be incorporated into the process. The NICE evaluation process could also take account of potential improvements in subsequent data about clinical and cost effectiveness, and its consequences for product pricing. (Paragraph 345)
35. To improve the evaluation process, we recommend that:
  - All drugs be assessed at the time of licensing, so that clinicians can prescribe useful and cost-effective products as soon as they are launched;
  - There be more emphasis on disinvestment;

- Our last report on NICE recommended that the legislation be changed to accommodate the need to ensure that assessments of products take account of the wider benefits to society; we make the same recommendation here;
  - NICE have access to the same material used by the licensing body, clinical trials be registered and there should be closer working between NICE and the industry to enable these early assessments to take place. (Paragraph 349)
36. The affordability of NICE guidance and the range, measured in cost-per-QALY, it uses to decide whether a treatment is cost-effective is of serious concern. The threshold it employs is not based on empirical research and is not directly related to the NHS budget, nor is it at the same level as that used by PCTs in providing treatments not assessed by NICE, which tends to be lower. Some witnesses, including patient organisations and pharmaceutical companies, thought NICE should be more generous in the cost per QALY threshold it uses, and should approve more products. On the other hand, some PCTs struggle to implement NICE guidance at the current threshold and other witnesses argued that a lower level should be used. However, there are many uncertainties about the thresholds used by PCTs. Accordingly we cannot authoritatively at this stage recommend a change in NICE threshold. Nevertheless, we recommend that it be reviewed. We do recommend that an independent body determine the threshold used when making judgements of the value of drugs to the NHS. (Paragraph 350)
37. To improve the implementation of NICE guidance we recommend:
- More help for PCTs to implement guidance;
  - Better assessment of the level of uptake;
  - That PCTs should play a larger role in the development of guidance;
  - Better use of experts in the development of guidance;
  - A change in the terminology used by NICE, to clarify to patients what they can and cannot expect by right from their local NHS organisation; and
  - That some elements of clinical guidelines should be made mandatory. (Paragraph 352)
38. We recommend that risk-sharing schemes be used with caution. They should not be used as a catch-all in cases of uncertainty over a drug's benefit. Uncertainty would be better addressed by the careful design and performance of a publicly funded randomised controlled clinical trial. Better use should be made of NICE's 'only in research' recommendation in this regard. (Paragraph 354)

## Glossary

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ADPH	Association of Directors of Public Health
AMD	Age-related macular degeneration
APBI	Association of the British Pharmaceutical Industry
BOA	British Orthopaedic Association
ERG	Evidence review group
GDG	Guideline development group
HTA	Health technology appraisal
ICER	Incremental cost effectiveness ratio
Meta-analysis	Combined analysis of several studies
MHRA	Medicines and Healthcare Products Regulatory Agency
MS	Multiple sclerosis
MTA	Multiple technology appraisal
NICE	National Institute for Health and Clinical Excellence
PCT	Primary Care Trust
PHIAC	Public Health Interventions Advisory Committee
PPRS	Pharmaceutical Price Regulation Scheme
OFT	Office of Fair Trading
OIR	Only in research
QALY	Quality-adjusted life year
Randomised controlled trial	Trial in which participants are assigned by chance to receive experimental or control treatment
SHA	Strategic Health Authority
SIGN	Scottish Intercollegiate Guidelines Network
SMC	Scottish Medicines Consortium
STA	Single technology appraisal
VTE	Venous thromboembolism

## Annex 1: Functions of NICE

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The functions of NICE, as described in its terms of reference<sup>340</sup>, are as follows:

- b) to appraise the clinical benefits and the costs of such health care interventions as may be notified by the Secretary of State and to make recommendations;
- c) to develop guidelines providing advice on good practice in the management of such diseases and conditions as may be notified by the Secretary of State;
- d) to provide such information and instruction on the implementation of the recommendations and guidelines referred to in sub- paragraphs (a) and (b) above to persons employed in activities connected with the health service as may be conducive to their efficiency in relation to such employment;
- e) to include in the recommendations and guidelines referred to in sub-paragraphs (a) and (b) above guidance on clinical audit criteria;
- f) to disseminate, as appropriate and via an appropriate range of media, such recommendations and guidelines referred to in sub-paragraphs (a) and (b) above to the health service and to the general public;
- g) to look into and consider, for the purpose of advising the Secretary of State with regard to possible improvements in the provision of health services and in the effective use of available resources, such other matters as may be notified by the Secretary of State;
- h) to provide information on medicines and prescribing through the National Prescribing Centre (a);
- i) to consider all interventional procedures notified to the Institute;
- j) to identify any interventional procedure notified to the Institute for which an assessment by the Institute as to whether it is safe and efficacious for use in the health service is unnecessary;
- k) to identify any interventional procedure notified to the Institute that does not fall within sub-paragraph (b) for which a comprehensive review of the evidence is required to enable the Institute to evaluate the procedure in accordance with sub-paragraph (e);
- l) to assess the extent to which any interventional procedure notified to the Institute that does not fall within either of sub-paragraphs (h) or (i) is safe and efficacious for use in the health service and to issue guidance to the health service on the use of such a procedure;
- m) to evaluate the safety and efficacy of any interventional procedure identified under sub-paragraph (j) for the purposes of use in the health service and to issue guidance to the health service on the use of such a procedure.

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340 NICE terms of reference: <http://www.nice.org.uk/niceMedia/pdf/DirectionFromSecretaryOfState2005.pdf>

Regarding public health...

- a) to develop, maintain and disseminate an evidence-base for effective public health action on health improvement and the reduction of inequalities in health in line with the Secretary of State's priorities for health as notified from time to time;
- b) to provide guidance on the development and setting of standards for public health and health promotion programmes and practice and support their implementation;
- c) to provide guidance on the means for improving the capability and capacity of organisations, systems and the wider public health workforce to deliver health improvement and reduce inequalities in health;
- d) to advise and assist in relation to such other matters as may be notified by the Secretary of State from time to time;
- e) to disseminate, as appropriate and via an appropriate range of media, the guidance referred to in sub-paragraphs (b) and (c) above.<sup>341</sup>

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<sup>341</sup> For the purpose of this direction "to evaluate" in sub-paragraph (l) shall include a comprehensive analysis of the available academic and clinical evidence and the collection and analysis of any new clinical data that may be made available by the health service.

## Annex 2: Guidance development processes

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### Interventional procedure appraisal process

- A procedure is notified to NICE by a clinician (most common), other individual or group. Once approved, all notified procedures are detailed on the website;
- Interest is registered by individuals and organisations;
- An overview of the procedure is prepared. The independent Interventional Procedures Advisory Committee (IPAC) considers the procedure. IPAC takes advice from at least three specialist advisers nominated by relevant professional organisations to determine whether to produce guidance;
- A consultation document on the safety and efficacy of the procedure is produced, and a consultation period of four weeks takes place;
- The final interventional procedures document is produced following consideration of the consultation findings;
- Formal approval of the guideline by NICE, followed by notification of consultees. If there is a problem with the guidance, consultees may request changes at this point;
- Guidance is issued by NICE if no challenges are made.

### Clinical guidelines development process

- Guideline topic is referred to NICE by the Department of Health;
- Stakeholders, such as healthcare professionals and patient groups, register their interest;
- The scope of the guideline is prepared by the NCC commissioned to develop the guideline. An independent guideline review panel, plus registered stakeholders, may feed into this process;
- A guideline development group, comprising patient and carer group representatives, health and technical professionals, is established;
- Assessment of the evidence by the guideline development group leads to the production of a draft guideline;
- Consultation on this guideline takes place. Registered stakeholders may comment on the draft, and the independent guideline review panel conducts a review;
- Following finalisation of the recommendations from the guideline development group, the NCC produces the final guideline;
- Guidance is issued, following formal approval by NICE.

## **Public health guidance development process**

### ***Public health intervention guidance***

- Topics are referred by the Department of Health;
- Stakeholders register interest. These groups are consulted throughout the process;
- Scope of the guidance, and details of the review process, are prepared. Consultation takes place at this point;
- Evidence is reviewed by NICE or another research body. This includes an economic assessment of the intervention. Stakeholders may comment at this point;
- Draft intervention guidance is prepared by the Public Health Interventions Advisory Committee (PHIAC);
- A one-month consultation period on the draft guidance takes place;
- Fieldwork is carried out to test the draft guidance, including meetings with professionals who have not contributed to the guidance. A technical report on the basis of these meetings is submitted to PHIAC;
- Final guidance is produced by PHIAC, considering the technical report and consultation findings;
- Peer review, and guidance is issued to the NHS

### ***Public health programme guidance***

The process for the development of public health programme guidance is similar to that of intervention guidance, except that a programme development group is established before the scope of the guidance is prepared. This group takes the place of PHIAC, and is involved in production of the draft guidance, review of the technical report and consultation findings and production of the final guidance.

# Formal Minutes

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**Monday 17 December 2007**

Members present:

Mr Kevin Barron, in the Chair

Charlotte Atkins  
Jim Dowd  
Sandra Gidley

Dr Doug Naysmith  
Dr Howard Stoaite  
Dr Richard Taylor

Draft Report (National Institute for Health and Clinical Excellence), proposed by the Chairman, brought up and read.

*Ordered*, That the Chairman's draft Report be read a second time, paragraph by paragraph.

Paragraphs 1 to 355 read and agreed to.

Annex and Summary agreed to.

*Resolved*, That the Report be the First Report of the Committee to the House.

*Ordered*, That the Chairman make the Report to the House.

*Ordered*, That embargoed copies of the Report be made available, in accordance with the provisions of Standing Order No. 134.

Written evidence was ordered to be reported to the House for printing with the Report.

Written evidence was ordered to be reported to the House for placing in the Library and Parliamentary Archives.

[Adjourned till 10 January 2008 at 9.30 am

## Witnesses

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### Thursday 17 May 2007

**Dr Felicity Harvey**, Head of Medicines, Pharmacy and Industry Group,  
**Simon Reeve**, Head of Clinical & Cost Effectiveness in MPI,  
**Dr Fiona Adshead**, Deputy Chief Medical Officer, and  
**Professor Mike Richards**, National Cancer Director, Department of Health

**Professor Sir Michael Rawlins**, Chairman, and **Andrew Dillon**, Chief Executive, National Institute for Health and Clinical Excellence

### Thursday 28 June 2007

**Professor Peter C Smith**, Centre for Health Economics, University of York,  
**Professor Raymond MacAllister**, Chair, Use of Medicines Committee, UCHL,  
and **Professor Stirling Bryan**, Health Services Management Centre,  
University of Birmingham

**Ian Beaumont**, Director of Press, PR and Public Affairs, Bowel Cancer UK,  
**Dr Kiran Patel**, Chairman of Trustees, South Asian Health Foundation, and  
**Dr David Anderson**, Chair, Faculty of Old Age Psychiatry, Royal College of Psychiatrists

### Thursday 12 July 2007

**Dame Gill Morgan**, Chief Executive, NHS Confederation, **Dr Tim Crayford**,  
President, Association of Directors of Public Health, and **Dr Lise Llewellyn**,  
Chief Executive, Berkshire East PCT

**Dr Richard Barker**, Association of the British Pharmaceutical Industry,  
**Eddie Gray**, General Manager and Senior Vice-President, GlaxoSmithKline  
(GSK), and **Dr David Brickwood**, Vice-President, International and  
Government Affairs – Europe, Johnson & Johnson

### Thursday 11 October 2007

**Simeon Thornton**, formerly of the OFT, **Professor Karl Claxton**, University of  
York, **Professor Adrian Towse**, Office of Health Economics, and  
**Professor Jon Nicholl**, University of Sheffield

### Thursday 18 October 2007

**Professor Nancy Devlin**, Department of Economics, City University, and  
**Professor John Appleby**, Chief Economist, The King's Fund

**Dr Beverley Hunt**, Medical Director, Lifeblood: The Thrombosis Charity,  
**Professor Roger Atkins**, British Orthopaedic Association, and  
**Dr Anita Thomas**, Chair of the Chief Medical Officer's VTE Implementation  
Working Group

**Steve Winyard**, Head of Policy and Campaigns, Royal National Institute of  
Blind People, and **Dr Rafiq Hasan**, Director of Market Access, Novartis

### Thursday 8 November 2007

**Professor Sir Michael Rawlins**, Chairman, and **Andrew Dillon**, Chief  
Executive, National Institute for Health and Clinical Excellence

**Rt Hon Dawn Primarolo**, a Member of the House, Minister of State for  
Public Health, **Dr Felicity Harvey**, Head of Medicines, Pharmacy and Industry  
Group, and **Dr Sunjai Gupta**, Deputy Director, Public Health Strategy, Social  
Marketing and Sexual Health, Department of Health

## List of written evidence

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The following memoranda were published as *National Institute for Health and Clinical Excellence: Written evidence*, HC 503–II, Session 2006–07

- 1 Department of Health (NICE 01)
- 2 National Institute for Health and Clinical Excellence (NICE 71)
- 3 Academy of Medical Sciences (NICE 39)
- 4 Alzheimer’s Society (NICE 70)
- 5 American Pharmaceutical Group (NICE 89)
- 6 Amgen (NICE 58)
- 7 Archimedes Pharma (NICE 68)
- 8 Arthritis and Musculoskeletal Alliance (ARMA) (NICE 97)
- 9 Association of British Healthcare Industries (NICE 85)
- 10 Association of the British Pharmaceutical Industry (NICE 72)
- 11 AstraZeneca UK (NICE 33)
- 12 Beat (NICE 06)
- 13 BioIndustry Association (NICE 93)
- 14 Bowel Cancer UK (NICE 38)
- 15 Breakthrough Breast Cancer (NICE 60)
- 16 Bristol-Myers Squibb Pharmaceuticals Limited (NICE 51)
- 17 British Association for Counselling and Psychotherapy (NICE 92)
- 18 British Medical Association (NICE 95)
- 19 British Society for Rheumatology (NICE 53)
- 20 Cancerbackup (NICE 42)
- 21 Cancer Research UK (NICE 67)
- 22 Roy Castle Lung Cancer Foundation (NICE 24)
- 23 Continence Foundation (NICE 23)
- 24 Cystic Fibrosis Trust (NICE 43)
- 25 Deltex Medical Group (NICE 87)
- 26 Diabetes UK (NICE 78)
- 27 Joint Epilepsy Council of the UK and Ireland (NICE 40)
- 28 Ethical Medicines Industry Group (NICE 79)
- 29 European Medicines Group (NICE 84)
- 30 FEmISA (NICE 49)
- 31 GlaxoSmithKline (NICE 86)
- 32 Help the Aged (NICE 11)
- 33 The Hepatitis C Trust (NICE 29)
- 34 Improving Surgical Outcomes Group (NICE 75)
- 35 Institute for Innovation & Valuation in Health Care (NICE 18)
- 36 Johnson & Johnson (NICE 74)
- 37 KCI Medical UK (NICE 14)
- 38 Kidney Cancer UK (NICE 90)
- 39 Leukaemia CARE (NICE 64)

- 40 Lifeblood: The Thrombosis Charity (NICE 35)
- 41 Lilly (NICE 94)
- 42 25% ME Group (NICE 19)
- 43 The ME Association (NICE 28)
- 44 Medical Technology Group (NICE 52)
- 45 Medtronic Ltd (NICE 15)
- 46 Merck Serono (NICE 77)
- 47 MS Society (NICE 65)
- 48 Motor Neurone Disease Association (NICE 25)
- 49 Myeloma UK (NICE 30)
- 50 UK Myeloma Forum (NICE 96)
- 51 National Childbirth Trust (NICE 27)
- 52 National Infertility Awareness Campaign (NICE 44)
- 53 National Osteoporosis Society (NICE 69)
- 54 National Rheumatoid Arthritis Society (NICE 82)
- 55 NHS Confederation (NICE 73)
- 56 Novartis Pharmaceuticals UK Limited (NICE 59)
- 57 Pfizer Limited (NICE 47)
- 58 The British Psychological Society (NICE 81)
- 59 Rarer Cancers Forum (NICE 34)
- 60 Roche (NICE 55)
- 61 Royal College of Midwives (NICE 62)
- 62 Royal College of Nursing (NICE 100)
- 63 Royal College of Physicians of Edinburgh (NICE 45)
- 64 Royal College of Paediatrics and Child Health (NICE 66)
- 65 Royal College of Psychiatrists (NICE 22)
- 66 Royal National Institute of the Blind (NICE 63)
- 67 Sanofi-aventis (NICE 57)
- 68 Schering Health Care Ltd (NICE 56) (now Bayer Schering Pharma)
- 69 ScotME (NICE 91)
- 70 Servier Laboratories Limited (NICE 41)
- 71 Sirtex Medical (NICE 54)
- 72 South Asian Health Foundation (NICE 12)
- 73 Specialised Healthcare Alliance (NICE 80)
- 74 Wyeth Pharmaceuticals (NICE 88)
- 75 Daphne Austin (NICE 20)
- 76 Professor Michael Barkham (NICE 83)
- 77 Dr Imogen Evans, Mrs Hazel Thornton and Sir Iain Chalmers (NICE 21)
- 78 Professor Malcolm Hooper (NICE 07)
- 79 Dr Chris Hyde (NICE 76)
- 80 Doris Jones
- 81 Gay Lee (NICE 03)
- 82 Michael Lee (NICE 13)
- 83 Professor Ragnar Lofstedt & Frederic Boudier, King's College London (NICE 31)
- 84 Dr Tom Marshall, University of Birmingham (NICE 05)

- 85 Dr Catherine Meads, University of Birmingham (NICE 08)
- 86 Sandra Simkin (NICE 50)
- 87 Dr Keith Syrett, University of Bristol (NICE 48)
- 88 Peter Telford (NICE 32)
- 89 David Thomson (NICE 16)
- 90 John Walsh (NICE 09)
- 91 Miss Rose A Woodward (NICE 98)
- 92 Pelvic Pain Support Network (NICE 04)

## List of further written evidence

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The following written submissions were received after the publication of *National Institute for Health and Clinical Excellence: Written evidence*, HC 503–II, Session 2006–07. They are reproduced with the Oral evidence in Volume II of this Report.

- 1 Department of Health (NICE 01A)
- 2 Department of Health (NICE 01B)
- 3 Royal College of Psychiatrists (NICE 22A)
- 4 Lifeblood: The Thrombosis Charity (NICE 35A)
- 5 Novartis (NICE 59A)
- 6 National Institute for Health and Clinical Excellence (NICE 71B)
- 7 National Institute for Health and Clinical Excellence (NICE 71C)
- 8 National Institute for Health and Clinical Excellence (NICE 71D)
- 9 National Institute for Health and Clinical Excellence (NICE 71E)
- 10 Association of the British Pharmaceutical Industry (ABPI) (NICE 72A)
- 11 Graph by Association of the British Pharmaceutical Industry (ABPI) (NICE 72B)
- 12 Peter C Smith, University of York (NICE 104)
- 13 Professor Stirling Bryan, University of Birmingham (NICE 105)
- 14 Professor Tony Culyer, Institute for Work & Health, Toronto (NICE 106)
- 15 Professor Raymond MacAllister, UCL (NICE 108)
- 16 Association of Directors of Public Health (NICE 111)
- 17 National Cancer Research Network (NICE 115)
- 18 Professor Karl Claxton, University of York (NICE 116)
- 19 Professor Jon Nicholl, University of Sheffield (NICE 117)
- 20 Eisai Ltd (NICE 118)
- 21 Simeon Thornton (NICE 119)
- 22 British Orthopaedic Association and Orthopaedic Specialist Societies (NICE 121)
- 23 Dr Anita J Thomas (NICE 122)
- 24 Dr Nigel Dudley, St James's University Hospital, Leeds (NICE 123)

## List of unprinted evidence

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The following memoranda have been reported to the House, but to save printing costs they have not been printed and copies have been placed in the House of Commons Library, where they may be inspected by Members. Other copies are in the Parliamentary Archives, and are available to the public for inspection. Requests for inspection should be addressed to, e-mail [archives@parliament.uk](mailto:archives@parliament.uk), Parliamentary Archives, Houses of Parliament, London SW1A 0PW (tel. 020 7219 3074). Opening hours are from 9.30 am to 5.00 pm on Mondays to Fridays (apart from public holidays).

Derek Enlander (NICE 02)

Robert Parkinson (NICE 10)

North London ME Network (NICE 17)

Invest in ME (NICE 26)

ME Association (NICE 28A)

Roche (NICE 55) (Appendix)

Bayer Schering Pharma (NICE 56A)

Mrs Josephine Hyde-Hartley (NICE 61)

Archimedes Pharma Ltd (NICE 68A)

ScotME (NICE 91)

Lilly (NICE 94A)

Alison M Beaney (NICE 99)

Multiple Sclerosis Trust (NICE 101)

Dr Fergus Macbeth, Director, National Collaborating Centre for Cancer (NICE 102)

Professor Stephen Birch and Dr Amiram Gafni, McMaster University, Ontario (NICE 107)

Beating Bowel Cancer (NICE 109)

Celgene Limited (NICE 110)

SMC presentation to the Health Committee (NICE 113)

Arthritis Care (NICE 124)

## Reports from the Health Committee

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The following reports have been produced by the Committee in this Parliament. The reference number of the Government's response to the Report is printed in brackets after the HC printing number.

### Session 2006–07

First Report	NHS Deficits	HC 73 (Cm 7028)
Second Report	Work of the Committee 2005–06	HC 297
Third Report	Patient and Public Involvement in the NHS	HC 278 (Cm 7128)
Fourth Report	Workforce Planning	HC 171 (Cm 7085)
Fifth Report	Audiology Services	HC 392 (Cm 7140)
Sixth Report	The Electronic Patient Record	HC 422 (Cm 7264)

### Session 2005–06

First Report	Smoking in Public Places	HC 436 (Cm 6769)
Second Report	Changes to Primary Care Trusts	HC 646 (Cm 6760)
Third Report	NHS Charges	HC 815 (Cm 6922)
Fourth Report	Independent Sector Treatment Centres	HC 934 (Cm 6930)

The following reports have been produced by the Committee in the 2001–05 Parliament.

### Session 2004–05

First Report	The Work of the Health Committee	HC 284
Second Report	The Prevention of Thromboembolism in Hospitalised Patients	HC 99 (Cm 6635)
Third Report	HIV/AIDS and Sexual Health	HC 252 (Cm 6649)
Fourth Report	The Influence of the Pharmaceutical Industry	HC 42 (Cm 6655)
Fifth Report	The Use of New Medical Technologies within the NHS	HC 398 (Cm 6656)
Sixth Report	NHS Continuing Care	HC 399 (Cm 6650)

### Session 2003–04

First Report	The Work of the Health Committee	HC 95
Second Report	Elder Abuse	HC 111 (Cm 6270)
Third Report	Obesity	HC 23 (Cm 6438)
Fourth Report	Palliative Care	HC 454 (Cm 6327)
Fifth Report	GP Out-of-Hours Services	HC 697 (Cm 6352)
Sixth Report	The Provision of Allergy Services	HC 696 (Cm 6433)

### Session 2002–03

First Report	The Work of the Health Committee	HC 261
Second Report	Foundation Trusts	HC 395 (Cm 5876)
Third Report	Sexual Health	HC 69 (Cm 5959)
Fourth Report	Provision of Maternity Services	HC 464 (Cm 6140)
Fifth Report	The Control of Entry Regulations and Retail Pharmacy Services in the UK	HC 571 (Cm 5896)

Sixth Report	The Victoria Climbié Inquiry Report	HC 570 (Cm 5992)
Seventh Report	Patient and Public Involvement in the NHS	HC 697 (Cm 6005)
Eight Report	Inequalities in Access to Maternity Services	HC 696 (Cm 6140)
Ninth Report	Choice in Maternity Services	HC 796 (Cm 6140)

**Session 2001–02**

First Report	The Role of the Private Sector in the NHS	HC 308 (Cm 5567)
Second Report	National Institute for Clinical Excellence	HC 515 (Cm 5611)
Third Report	Delayed Discharges	HC 617 (Cm 5645)