The Government's Response to the Health Select Committee Report on Top-up fees

Presented to Parliament by
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Introduction

1. The House of Commons Health Select Committee published its report on "Top-Up fees" on Tuesday 12 May 2009. This Command Paper sets out the Government's response to the conclusions and recommendations of that report. We are very grateful for the attention and insight that the Committee has brought to this important and complex issue.

2. On 17 June 2008, the Secretary of State for Health asked Professor Mike Richards, the National Clinical Director for Cancer, to review the policy for patients who wished to buy additional drugs privately. During the course of the review, Professor Richards spoke to over 2,000 patients, members of the public, NHS staff, NHS managers and other stakeholders.

3. On 4 November 2008, the Secretary of State published Professor Richards' findings, and accepted the report's recommendations, announcing a significant package of measures to improve access to treatments through the NHS. These measures included:

   - improving the timeliness of National Institute for Health and Clinical Excellence (NICE) appraisals;
   - NICE giving its appraisal committees more explicit flexibility in appraising high cost end of life treatments for rarer conditions;
   - improving local decision making on funding drugs and treatments; and
   - new and more flexible pricing arrangements and a more systematic approach to the use of patient access schemes, as a result of the 2009 Pharmaceutical Price Regulation Scheme (PPRS), which came into effect on 1 January 2009.

4. These measures mean more drugs will be available to NHS patients, more quickly. In turn, this will reduce the demand from patients for additional private care. The Government's priority is to ensure that all drugs which offer benefit to patients and represent a good use of taxpayers' money are available free at the point of need to NHS patients.

5. In the context of these wider measures, the Secretary of State accepted Professor Richards' recommendation that those few patients who do still wish to pay for additional private care should not have their NHS care withdrawn. The Secretary of State agreed with Professor Richards that any additional private care should be delivered separately from NHS care, in order to safeguard the founding principles of the NHS against the development of a two-tier system.

6. The Government is grateful to the Health Select Committee for supporting the immediate end to the practice of withdrawing funding from NHS patients who purchase additional private care.

7. This document responds to each of the Committee's conclusions and recommendations which are printed in bold.

Key Issues

The nature and scale of the problem

The NHS faces growing pressure to make available an increasing number of expensive medicines to patients. The NHS uses two main methods to determine whether or not drugs are sufficiently cost-effective to be funded by the NHS: nationally through the National Institute of Health and Clinical Excellence (NICE) and locally through Primary Care Trusts (PCTs). A number of criticisms were made about the system for making drugs available on the NHS. These related to:

- **The NICE process for appraising drugs for end-of-life conditions, both in respect of the speed of appraisals and the failure to approve drugs of some clinical effectiveness, which were not demonstrably cost-effective.**
- **The inconsistencies in the decisions made by PCTs in assessing exceptional funding requests.**
- **The decision by some NHS trusts to withdraw care for patients who chose to purchase additional drugs. (Paragraph 26)**

The Department has accepted the Health Committee's criticism made in 2008 that NICE had, in some cases, been too slow in appraising new drugs. We welcome the Department's commitment that the maximum time between a drug's referral to NICE for evaluation and its availability for prescription will be six months. (Paragraph 27)

8. We welcome the Committee's support of the commitment the Government and NICE have made to improve the timeliness of appraisal guidance. We would like to clarify this commitment. Currently, the appraisal process itself takes longer than six months. However, by identifying appraisal topics while drugs are still in development and referring them to NICE before they are

licensed for use in the UK, we can ensure that the NHS has timely advice on their use.

9. The appraisal timetable outlined by the Secretary of State on 4 November 2008 will see draft or final guidance available within six months of licensing for about half of the drugs appraised through the Single Technology Appraisal process, where guidance is issued during 2009. In 2010, draft or final guidance for all new cancer drugs will be available within six months, on average, of a drug being licensed. This will reduce the period in which a significant new drug is available on the market without NICE guidance.

Of the twenty-one cancer drugs that NICE considered between 2007-08, the organisation did not approve funding for five on the grounds that they were not cost-effective. Some witnesses argued that patients had been denied drugs that would have benefited them. While some of them do give significant benefits (measured in QALYs) to certain individual patients, they are only marginally, or not at all, beneficial to the great majority of patients. (Paragraph 28)

10. We do not believe the NHS should stand in the way of the small number of patients who wish to purchase separate private care, as long as that care is never subsidised by the NHS.

11. NICE uses a range of costs-per-QALY to inform its guidance, to allow for the innovative nature of a drug and for patient need.

12. However, NICE recognises the value society places on treatments for terminal diseases that offer a significant extension to life. NICE appreciates that, where these treatments are for small numbers of patients, they may fall outside the normal cost-effective parameters for a positive NICE appraisal. This is why NICE issued supplementary guidance to its appraisal committees that gives more explicit flexibility in the appraisal of such treatments.

However, owing to the increased number of drugs that it was due to consider over coming months, Professor Richards stated that without closer working between Government and industry, some will fail the cost-effectiveness tests that are currently used and that "it may well be that higher numbers of patients will be placed in a position where their clinician feels that they could benefit from a drug that will not be funded by the NHS". (Paragraph 29)

13. The Government believes that it is important that access to medicines reflects their value to patients, a point made by the Office of Fair Trading in its report on the PPRS. That is why the new PPRS, agreed between the Government and industry, includes two specific measures to increase access to medicines and ensure that value is better reflected in pricing:

- new and more flexible pricing arrangements that will enable drug companies to supply drugs to the NHS at lower initial prices, with the option of higher prices if value is proven at a later date; and
- a more systematic approach to the use of patient access schemes, which allow drug companies to offer discounts or rebates, reducing the effective cost of a drug to the NHS.

14. The PPRS also recognises the importance of the pharmaceutical industry to healthcare and the development of medical advances. In order to encourage research and reward innovation, a package promoting the uptake of cost-effective, innovative treatments is a key part of the scheme for the first time.

15. These measures, together with NICE's flexibility in appraising end of life treatments for rarer conditions, will mean that a greater number of cost-effective drugs and treatment will be available to NHS patients, more quickly. In turn, this will minimise the demand from patients for additional private care.

Currently there are around 15,000 applications per annum made to PCTs for exceptional funding for drugs. The number of cases considered by each PCT varied widely between 1 and 1,000. It is, however, unclear how many of these cases were subsequently approved. For cancer drugs, it is estimated that about three quarters of the 3,000 requests were approved although this figure varied greatly between PCTs. (Paragraph 30)

There were a number of criticisms about the lack of information made available by PCTs to patients about the appeal process and the justification for the PCT's final decision. PCTs themselves estimated that only just over half of patients and the public were aware of the decisions they had taken. (Paragraph 31)

16. The NHS Constitution, launched in January 2009, makes clear that patients have the right to drugs and treatments that have been recommended by NICE for use in the NHS, if they are clinically appropriate.

17. It also says that patients have the right to expect local decisions on funding of other drugs and treatments to be made rationally, following a proper consideration of the evidence. This applies to all local decision-making regarding funding of drugs and treatments that have not, or have not yet, been appraised by NICE. If PCTs decide not to fund a drug or treatment that the patient and doctor feel would be right for the patient, they should explain that decision.

18. This right is further underpinned by:

- *Defining guiding principles for processes supporting local decision making about medicines*, published in January 2009. These principles are designed to help PCTs improve the consistency and quality of local

decision making. Principle 6 directly addresses the establishment of an appeals process and Principle 8 addresses communication with stakeholders including the wider NHS, patients and the public;

- *Supporting rational local decision-making about medicines and treatments: A handbook of good practice guidance*, published by the National Prescribing Centre in March 2009. This gives detailed advice on the practicalities of the appeals process and how to communicate with patients, and emphasises that PCTs should make all relevant documentation available to patients; and
- new statutory *Directions to Primary Care Trusts and NHS trusts concerning decisions about drugs and other treatments 2009*, which came into force on 1 April 2009. The Directions require PCTs to give reasons for their decisions and provide written information to patients when requested.

Some patients whose funding requests for drugs had been rejected by PCTs had decided to purchase the drug privately. Although we were told that NHS rules about purchasing treatment were clear, PCTs had applied them inconsistently. Some PCTs withdrew funding for patients who chose to purchase additional treatment, while other PCTs effectively subsidised the private treatment. (Paragraph 32)

19. The Government acted immediately to issue new guidance on additional private care for the NHS as soon as Professor Richards' review highlighted the extent of local variation. This guidance was subject to consultation, following which final guidance was published on 23 March 2009. The final guidance highlights that:

    - NHS patients should not have their NHS care withdrawn if they choose to buy additional private care, as long as the private element of care can be delivered separately from NHS care; and
    - patients must pay the full cost of the additional private care, as the NHS should never subsidise private care.

Professor Richards stated that the failure of the NHS to meet the demand for expensive drugs, and concerns that some PCTs had withdrawn NHS funding for patients who had purchased drugs, had combined to result in a loss of confidence in the system for making drugs available on the NHS. Despite much media coverage of the issue, Professor Richards estimated that there were only 18 cases where PCTs had withdrawn NHS funding for patients who had purchased additional drugs. (Paragraph 33)

20. This is an issue that has only affected patients in very rare circumstances. The Department does not hold specific data on this but Professor Richards' review did not suggest that it has affected more than a very small number of people. However, it was important that we acted quickly to ensure that, in future, no patients will lose their entitlement to NHS care because of a decision to purchase additional private care.

Addressing the problem

Since 2008 the Department made a number of decisions to increase access to drugs. In November 2008, the Department accepted immediately all Professor Richards' recommendations and made a number of decisions to implement the recommendations:

- **Where patients continued to purchase private drugs, the Department published for consultation draft guidance in January 2009 about how to separate NHS and private treatment. Final guidance was implemented on 23 March 2009.**
- **In December 2008 the Department introduced supplementary guidance to NICE Appraisal Committees to make available a greater range of more expensive drugs to a greater number of NHS patients and thereby reduce the need for patients to buy drugs privately.**
- **Other measures, including the new renegotiated PPRS aimed at reducing the price of drugs, have been introduced. (Paragraph 54)**

21. As the Committee has recognised, the Government acted immediately by announcing a set of measures to respond to Professor Richards' findings.

22. The Government fully supports the supplementary guidance to NICE appraisal committees as developed and published by NICE in December 2008.

The consequences of separating NHS and private treatment

The Richards Report was generally well received by witnesses to our Inquiry. Particularly welcome was the statement that NHS care should not be withdrawn from the very small number of patients who purchased additional drugs as long as that care was delivered separately. However some of our evidence expressed concerns about the risks, consequent on the Report, of potential disadvantages to NHS patients including the formation of a two-tier system. (Paragraph 77)

Although the Department was confident that its final guidance to NHS trusts would enable the separation of NHS and private care, one witness told us that in practice "it would be a little naive to believe that there would be complete separation. It is simply not possible". We believe that separation will be harder to achieve in practice than the Department claims. We believe it would be wrong for very seriously ill patients to be moved from an NHS ward to a different location so as to administer a privately paid for drug separately. This undoubted disruption to a patient's quality of life just to meet some bureaucratic requirement would not only endanger the patient's care but would be unjust.

We are very concerned that two patients with the same condition on the same NHS ward might receive different treatments because one patient could afford it and the other could not. This must not be allowed to happen except in the circumstances described in the Department's final guidance. (Paragraph 78)

23. The public engagement that Professor Richards conducted as part of his review demonstrated that many people feel strongly that NHS care should not routinely be provided alongside additional private care. There was a strong sense that blurring the boundaries between NHS and private care by allowing them to happen at the same time and place would undermine the founding principles of the NHS and could be the first step on the road to a two-tier system.

24. Separation between NHS and private care is important in order to uphold the principle that the NHS should not subsidise private care, and to ensure that NHS patients are never charged for their NHS care.

25. Professor Richards recommended that, with appropriate safeguards, the separate care approach can be delivered safely and effectively. For example, the NHS in the North East, an area of relatively little private provision, has shown that it is possible to put in place safe, sensible arrangements, which allow patients to have additional private treatment under the care of one clinical team.

26. In response to consultation, the final guidance for additional private care gave several case studies, which cover non-drug interventions such as physiotherapy and surgery, as well as drug interventions. The guidance recognises that in exceptional cases there may be overriding concerns of patient safety that mean private and NHS care cannot be provided separately.

27. A core theme of the guidance is that it is vital to maintain the highest standards of professional practice and clinical governance where patients have purchased additional care privately.

Ensuring that treatment for patients is provided with good continuity of care between the NHS and the private sector will require close collaboration and sharing of information between NHS and private clinicians. This will not be easy and will only be achieved by the establishment of excellent working practices and the goodwill of clinicians. (Paragraph 79)

28. Professor Richards' review considered these issues and his report was clear that this approach can be delivered safely and effectively. It is already the case that many care pathways involve patients moving between different clinical teams, and doctors' professional responsibilities ensure that this is done safely and effectively.

29. The guidance for additional private care states explicitly that protocols should be in place to ensure effective risk management and coordination between NHS and private care at all times. Furthermore, NHS Trusts and Foundation Trusts should have clear policies in place to ensure effective implementation of this guidance in their organisations.

30. This includes protocols for working with other NHS or private providers where the NHS Trust or Foundation Trust has chosen not to provide additional private care.

We are surprised that the Department has not made any estimate of the costs associated with separating NHS and private care yet felt able to describe them as "not a major problem". Other witnesses argue the costs may be significant. We were told by Professor Sikora that it might be possible to establish a tariff for treatments so that the true costs of separation can be identified and so ensure that the NHS does not subsidise private care. It will be important to establish costs without introducing an expensive bureaucratic system. (Paragraph 80)

31. The Government has it made clear that the NHS should never subsidise private care with public money. We believe the best way to achieve this is by setting clear principles that can be applied by local NHS organisations, rather than by attempting to construct national costing systems for private healthcare.

32. The guidance on additional private care highlights that there should be as clear a separation as possible of funding, legal status, liability and accountability between NHS care and any private care that a patient receives. In particular it says that:

    - "the NHS should not subsidise the private element of care; and
    - the patient should meet any additional costs associated with the private element of care, such as additional treatment needed for the management of side effects".

We are concerned that the affirmation of the guidelines regarding the separation of NHS and purchased drugs will establish a precedent that would open up the possibility of a "core service" emerging in the NHS obliging patients to co-fund aspects of their treatment or to go without. We reinforce Professor Richards' call on the Government to clarify its policy in respect of the arrangements, which apply to the separation of NHS and private care in relation to non-drug interventions, including devices and procedures. (Paragraph 81)

33. The guidance makes clear that "the fact that some patients also receive private care separately should never be used as a means of downgrading the level of service that the NHS offers".

34. More generally, the Government is committed to a comprehensive NHS, and we have published the NHS Constitution to enshrine and sustain that commitment. For example, the principles of the Constitution highlight that:

    - the NHS provides a comprehensive service, available to all;
    - access to NHS services is based on clinical need, not an individual's ability to pay. NHS services are free of charge, except in limited circumstances sanctioned by Parliament;
    - the NHS aspires to the highest standards of excellence and professionalism; and
    - the NHS is committed to providing best value for taxpayers' money and the most effective, fair and sustainable use of finite resources. Public funds for healthcare will be devoted solely to the benefit of the people that the NHS serves.

35. In the Health Bill currently before Parliament we are taking legislation to underpin the Constitution. The powers in the Bill would ensure that:

    - all providers of NHS care in England must have regard to NHS Constitution;
    - the Secretary of State must review the constitution every ten years after full consultation;
    - any government that wishes to alter the principles or values of the NHS, or the rights, pledges, duties and responsibilities in the Constitution will have to engage in a full and transparent debate with the public, patients and staff; and
    - in addition, the principles in the Constitution may only be changed in accordance with regulations agreed by Parliament.

36. On the issue of scope, the Government's guidance for additional private care applies to all secondary and specialist healthcare in England and not simply drugs. To emphasise this, the guidance includes case studies about surgery and physiotherapy, as well as about medicines.

The potential consequences of the proposals to make more drugs available

The challenge of making new, often expensive, treatments available quickly to patients within limited resources is faced by health systems around the world. The Department and NICE have introduced two significant initiatives which are aimed at increasing the availability of expensive drugs: the provision of supplementary guidance for end-of-life treatments to NICE Appraisal Committees and the introduction of a new Pharmaceutical Price Regulation Scheme. (Paragraph 110)

While Professor Rawlins denied claims that NICE had raised its end-of-life cost per QALY threshold to £70,000, he accepted that NICE Appraisal Committees would be more flexible in the way they appraised expensive treatments. In effect the QALY threshold has been raised for end-of-life drugs. We believe that the decision by NICE to raise its cost per QALY threshold for end-of-life drugs is both inequitable and an inefficient use of resources. By spending more on end-of-life treatments for limited health gain, the NHS will spend less on other more cost-effective treatments. (Paragraph 111)

NICE said that that it was important to place clear limits on the numbers of patients who would benefit from the new guidance because the NHS could not afford to apply the guidance for all conditions. However, given that increasingly new drugs are 'designer' technologies for small subgroups of patients many new products can be viewed as treatment for 'rare' diseases. We believe that the definition of subgroups of patients suffering from rarer cancers as "small populations" is too woolly and needs more clarity. There is a clear danger that the new arrangements will lead to the system becoming unaffordable as pharmaceutical companies target new drugs on subgroups of diseases. (Paragraph 112)

37. NICE's independence is vital and it would be wrong for the Government to pre-empt its appraisal committees. NICE quantifies the cost-impact of all its appraisals, including those of drugs that are positively appraised as a result of the new end of life flexibilities. Any additional costs arising from the new flexibilities NICE applies to certain drugs given near to the end of life are not likely to be very significant in the context of the £11billion the NHS spends annually on drugs. We are, however, confident that spending on this front will be affordable.

Although we consider it proper that the public's view on how NHS resources are spent is taken into account, we are not convinced that NICE's method of doing so is the right one. We recommend that more research is undertaken to determine whether NICE's favoured method of using citizens' juries and "willingness-to-pay exercises" is the best way of taking into account the public's view on this matter. (Paragraph 113)

38. NICE has recognised that the public places a special value on treatments that prolong life for people whose life expectancy is poor. This view is echoed by NICE's Citizens Council. In order to improve access to such products, NICE provided its appraisal committees with supplementary advice on the appraisal of drugs for the treatment of rarer, end of life diseases. The guidance sets out the circumstances in which the appraisal committees should exercise their discretion to recommend use of medicines proven to extend life for terminally ill patients.

39. The development of this supplementary guidance is a matter for NICE as an independent body. NICE engaged with the NHS and clinicians in the development of this advice, including the criteria to be used in the identification of such treatments. Additionally, the advice was subject to a five-week consultation before being published in January 2009.

40. We understand that NICE will review this supplementary guidance in June 2009 and will take the Committee's report into account as part of this review. NICE has also informed the Government that it will be writing in response to the specific points made by the Committee on its supplementary guidance. NICE will provide the Committee with a copy of its letter.

We welcome the Department's guidance to PCTs for more transparency in the way that they deal with exceptional funding requests for treatments. All decisions on exceptional funding should be consistent with this guidance and PCTs should provide a clear and easily intelligible explanation to patients giving the reasons for any decision to approve or reject an exceptional funding request. PCTs must inform patients of the reasons for rejecting their exceptional funding request. (Paragraph 114)

41. As outlined above, advice on how to deal with exceptional funding requests for treatments and how to communicate these decisions to patients is outlined in:

    - *Defining guiding principles for processes supporting local decision making about medicines*, published by the Government in January 2009; and
    - *Supporting rational local decision-making about medicines and treatments: A handbook of good practice guidance*, published by the National Prescribing Centre, March 2009.

42. The *Directions to Primary Care Trusts and NHS trusts concerning decisions about drugs and other treatments 2009* require PCTs to take into account any guidance and principles issued by the Secretary of State for Health in relation to this work. Thus, the Directions place a responsibility on PCTs to be consistent with current guidance.

43. The Directions also outline the duties of PCTs with regard to giving reasons for rejecting or accepting requests for particular health care interventions. The Directions require PCTs to provide a written statement on these decisions, if requested.

Despite the Department's proposals, inconsistencies will remain between PCTs about whether or not they fund certain treatments. Those PCTs which do not fund them are likely to come under severe pressure to do so through the exceptional funding request process. (Paragraph 115)

44. As a result of the various measures we have put in place, we expect to see significant improvements in PCTs' decision-making processes, including increased transparency and consistency in the way decisions are made. This will mean that patients and the public can be very clear about what the NHS is offering them by way of access to drugs and how decisions about funding are taken. These improvements underline our commitment to address public concerns about the perceived postcode lottery in access to drugs.

45. Complete uniformity in service provision is neither achievable nor necessarily desirable, as individual PCTs have different population needs. Local PCT commissioning and funding arrangements will always reflect this. However, we expect PCTs to work collaboratively where appropriate in deciding whether it is appropriate to fund certain drugs or treatments.

The Department maintains that the proposals are affordable if the NHS carries out more disinvestments in technologies which might be effective but which have been superseded by other more cost-effective drugs. In our 2001 and 2008 reports into NICE we called for more effort to be put into disinvesting in obsolete technologies. We are extremely disappointed that little progress seems to have been made in this area. (Paragraph 116)

46. The Government agrees that this is an important aspect of NICE's work but disagrees that the Institute has not made progress in this area. In September 2006, the Government and NICE announced an optimal practice review programme of work aimed at providing guidance for the NHS on interventions of dubious benefit. As part of this programme, NICE produces three different products;

- ineffective practice reviews;
- recommendation reminders; and
- commissioning guides.

47. Since the programme was established, NICE has published:

- two ineffective practice reviews which are published as clinical guidelines and relate to surgery for otitis media with effusion and antibiotics for respiratory infections;
- 30 recommendation reminders which highlight potentially cost saving recommendations from existing guidance; and
- 25 commissioning guides to support NHS commissioners to deliver NICE's guidance effectively.

48. It is important to recognise that, in carrying out its mainstream work, NICE implicitly considers whether existing treatments are ineffective, of limited benefit or have been superseded by a more cost-effective treatment. This is especially true of NICE's clinical guidelines, which make a large number of recommendations relating to a whole pathway of care. The clinical guidelines set out which interventions and treatments do or do not represent a cost-effective use of NHS resources. For example, NICE's clinical guideline on pre-operative testing made 15 recommendations about tests that should not be routinely carried out on patients who are admitted to hospital for surgery. NICE estimates that there are around 150 such recommendations each year.

49. That is why we recognise the importance of NICE's recommendation reminders in the area of optimal practice. Each month, NICE highlights four such recommendations from existing guidance and provides a costing template to calculate local savings.

50. NICE also manages the entry of many new technologies to ensure NHS resources are optimally targeted, commonly to sub-groups of the populations described in the licensed indication. For 55% of the 342 different technology/indication combinations appraised to the end of January 2009, recommendations were made to target the technologies to particular groups of patients.

The Department has introduced a new Pharmaceutical Price Regulation Scheme (PPRS) which it claims will reduce the cost to the NHS of purchasing drugs. The new PPRS will also place greater emphasis on risk sharing schemes which it has re-termed as patient access schemes. As we noted previously in our 2008 report into NICE, we have serious concerns about the effectiveness of risk sharing schemes where they place the burden of proving the success of the scheme on the NHS and not on pharmaceutical companies. We repeat the recommendation we made in our 2008 report into NICE that risk-sharing schemes be used with caution and that the risks should be borne by the company concerned. (Paragraph 117)

51. As described above, the new PPRS includes two specific sections to increase access to medicines and ensure that value is better reflected in pricing. These are:

- new and more flexible pricing arrangements that will enable drug companies to supply drugs to the NHS at lower initial prices, with the option of higher prices if value is proven at a later date; and
- a more systematic approach to the use of patient access schemes, which allow drug companies to offer discounts or rebates which reduce the effective cost of a drug to the NHS.

52. Patient access schemes and flexible pricing can be helpful in providing patients with consistent access throughout the NHS to new innovative drugs that may not have been given a positive NICE appraisal. The intention behind these schemes is to make drugs available to the NHS on a more cost-effective basis, to the benefit of NHS patients.

53. The Government agrees, however, that such schemes should be the exception rather than the rule, not least because they are likely to place additional administrative burdens on the NHS. We are working with NICE to establish a new process for validating patient access scheme proposals with the NHS before they are considered as part of a NICE appraisal.

54. Patient access schemes under the PPRS are not the same as the local "risk-sharing" schemes that operate between some NHS trusts and manufacturers. Patient access schemes in themselves are not designed to place the burden of proof on the NHS, as the Committee's recommendation might suggest. Rather, they are designed to enable the drug to be made available to the NHS on a more cost-effective basis. None of the patient access schemes so far validated by the Government for incorporation into a NICE appraisal places the burden of proving the "success" of a scheme on the NHS.

55. An example of this is the Lucentis scheme for wet-acute macular degeneration. This caps the cost of the drug at 14 doses per eye, so that the manufacturer and not the NHS will cover the costs of the drug if the patient has not recovered by the fourteenth dose. The NHS does not bear the burden of the risk in this scheme.

56. The combined impact of the new NICE end of life flexibilities and the new PPRS can be seen already. Two drugs – sunitinib for renal cell carcinoma and lenalidomide for multiple myeloma have both been positively appraised due to both the new flexibilities and the inclusion of a patient access scheme in each appraisal. Therefore, patients are already benefiting from increased access to important new drugs.

We are surprised by the statement made by Dr Harvey, Director and Head of Medicines Pharmacy and Industry, Department of Health, that the risk sharing scheme evaluated by Sheffield University has been a success as other sources, including our 2008 report into NICE, have indicated that it has been a costly failure. The first published results of the evaluation of the process by the Sheffield group were much delayed and offer little evidence of cost effectiveness. (Paragraph 118)

57. The results of the first evaluation point from this scheme have not yet been published. Sheffield University's School of Health and Related Research (ScHARR) acted as scheme co-ordinator for the initial recruitment phase to the 10-year risk-sharing scheme for drugs for multiple sclerosis. ScHARR published a paper in January 2009, which outlines its experience "…of

undertaking the monitoring study for the initial phase of this innovative scheme, the practical, scientific and political challenges encountered...".

58. ScHARR carried out some preliminary analyses of scheme data and concluded that it was too early to make judgements about the cost-effectiveness of the drugs. The report highlighted a number of methodological difficulties, which are being addressed by the scheme's independent Scientific Advisory Group. The Group has undertaken an extensive programme of work to resolve various issues, including proposals to develop an alternative natural history comparator. In the meantime, we will be publishing a peer-reviewed paper outlining the results of the first formal analysis of scheme data over the autumn of 2009. Patients continue to receive the treatment as judged appropriate by their clinicians in accordance with the rules of the scheme.

Conclusions

In conclusion, although we are not convinced by the arguments that dismiss the threats of establishing a two-tier system or that separation of patients is practicable for only a part of their treatment, we can see no transparent way of rapidly alleviating the problem other than Professor Richards' Option 3. We recommend that every effort is made to minimise the numbers of patients involved by:

- **Speeding up the NICE process.**
- **Increasing the work on disinvestment on the least useful other treatments.**
- **Standardising PCTs' Exceptional Funding Request procedures including the communication of decisions and the reasons for them to patients and families.**
- **Instructing NICE to issue brief, understandable, accessible and well publicised explanations for lay people to explain the reasons for refusing funding for drugs, to give patients and their relatives clearly spelt out information upon which they can base their decision about paying for some but not all medicines. (Paragraph 119)**

59. The Government agrees the priority should be to increase access to treatments within the NHS and minimise the need for patients to purchase additional private drugs. The measures announced by the Government, including the new NICE flexibilities, will achieve this.

60. The measures we have put in place will reduce the period in which a significant new drug is available on the market without NICE guidance but PCTs have no guidance from NICE to inform their decisions about funding.

61. The Government will continue to work with NICE to identify opportunities for disinvestment through NICE's optimal practice review programme.

62. The *Directions* now in force require PCTs to give reasons for their decisions and provide written information to patients when requested. These are further underpinned by guiding principles and good practice guidance.

63. The NHS Constitution sets out a new right for patients to make choices about their NHS care and to information to support these choices. This is underpinned by recommendation 13 of Professor Richards' review that patients should be given access to balanced written information on the benefits, risks and, where appropriate, cost of treatments.

64. The Government is pursuing a number of initiatives to help deliver reliable, accurate information to patients and the public. Taken together, these initiatives will provide quality assured processes for the production of clinical evidence and patient information, and will provide patients with relevant information at the right time in their care pathway.

65. We will work with NICE, voluntary organisations and cancer specialists to ensure that these schemes are able to deliver the information that patients need to make decisions about their care.

We recommend that the Department monitors the implementation of the Report's recommendations by funding research to gather evidence about:

- **The actual degree and modes of separation of care achieved by different trusts with and without existing private facilities.**
- **The support of consultants, especially those who do not normally undertake private practice, and other staff for the scheme.**
- **The effects on PCTs' ability to fund other established, essential treatments for other conditions that do not have the benefit of NICE guidance.**
- **The numbers of patients applying to pay for extra drugs. (Paragraph 120)**

66. We agree it is important to monitor the implementation of Professor Richards' review by funding research. Recommendation 11 of Professor Richards' report was that the Department of Health should take a lead on commissioning a national audit of demand for unfunded drugs and on the outcome of treatments. We are currently considering the scope and timing of such an audit; however, we are not aware that such a complex audit has been done before, in the UK or internationally. Pilot work is therefore expected to start later this year, and these recommendations from the Committee will be considered as part of this work.

We recommend that the Department also actively addresses the problems of prioritisation by initiating open discussions about NHS treatments or services that should be reduced or not provided. (Paragraph 121)

67. The Government has established NICE as an independent body responsible for the development of robust, evidence-based, national guidance on whether treatments represent an effective use of NHS resources. Through its optimal practice review programme, NICE performs its role with a high degree of openness and transparency, which provokes essential discussion of these issues.

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