



House of Commons
Health Committee

Top-up fees

Session 2008–09

Volume II

Written evidence

*Ordered by The House of Commons
to be printed 22 January 2009*

The Health Committee

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Written evidence

Memorandum by the Department of Health (TF 01)

IMPROVING ACCESS TO MEDICINES FOR NHS PATIENTS

1. The Department welcomes the Health Select Committee's interest in the Review, led by Professor Mike Richards, into policy for patients who wish to buy additional private drugs, and the Government's response to this Review. This note represents the Department's written evidence to the Committee. It sets out the background to the Review, and a summary of the action the Government is taking in response. It also provides the Committee with the key documents they may wish to read.

2. In June, the Secretary of State for Health asked Professor Mike Richards, National Clinical Director for Cancer, to review policy for NHS patients who wish to buy additional drugs privately. The Secretary of State felt that a review was necessary because a number of cases of patients having NHS care withdrawn had come to his attention, and he felt that there may be unacceptable variations in the way in which existing guidance was being implemented.

3. During the review, Professor Richards spoke to over 2,000 patients, members of the public, NHS staff and managers, and other stakeholders. He received over 400 submissions to the Review mailbox, and held a number of patient workshops with charities such as Macmillan Cancer Support, and the Long Term Conditions Alliance.

4. On 4 November, the Secretary of State published Professor Richards' report and accepted the report's findings.¹ On the same day, the Secretary of State set out the Government response to the Review in an oral statement to the House of Commons. The text of the Secretary of State's statement can be found at Annex A.

5. The package of measures the Secretary of State announced on 4 November will make available a greater range of more expensive drugs to more patients on the NHS. This will reduce the need for patients to seek additional drugs on a private basis. These measures included:

- supporting the National Institute for Health and Clinical Excellence's (NICE's) proposal for greater flexibility in appraising more expensive drugs for terminally-ill patients;
- working closely with the pharmaceutical industry to agree new and more flexible pricing arrangements to increase access to new drugs, including lower initial prices (with the option of higher prices if value is proven at a later date) and patient access schemes;
- working with NICE to ensure that timely appraisal guidance is available on significant new drugs; and
- improving the quality and consistency of local decisions by setting out core principles and detailed good practice guidance to guide Primary Care Trusts on the funding of new drugs where no NICE guidance is in place, in addition to encouraging collaborative working between PCTs.

6. The Committee will want to note that since the Secretary of State's statement, final agreement has now been reached between the Government and the pharmaceutical industry on these new, more flexible pricing arrangements. As a result, patients will continue to benefit from cost-effective, innovative drugs. This deal is expected to deliver savings of around £350 million for the UK in 2009–10, and around £550 million per year thereafter. The final Pharmaceutical Price Regulation Scheme (PPRS) agreement will be published shortly, at which point the Department will provide the Committee with a copy.

7. For those patients who may still wish to buy additional private care, the Secretary of State accepted Professor Richards' recommendation that these patients should not have their NHS care withdrawn, as long as the private care can be delivered separately.

8. To support this, the Secretary of State published revised guidance on 4 November which sets out this principle, and makes clear how the NHS should handle these situations. The guidance is out for consultation until 27 January 2009, and the Department welcomes any views as part of this consultation on how it might be improved.²

9. Due to the need to end uncertainty for patients and the NHS as soon as possible, the Secretary of State has made clear that, with immediate effect, the NHS should not withdraw treatment in these circumstances. David Nicholson, the NHS Chief Executive, wrote to all relevant NHS chief executives on 4 November asking them to begin applying the guidance immediately.³

10. The Department will then publish final guidance, taking into account views from the consultation, early in 2009.

December 2008

¹ http://www.dh.gov.uk/en/Publicationsandstatistics/Publications/PublicationsPolicyAndGuidance/DH_089927

² http://www.dh.gov.uk/en/Consultations/Liveconsultations/DH_089926

³ http://www.dh.gov.uk/en/Publicationsandstatistics/Lettersandcirculars/Dearcolleagueletters/DH_091033

SECRETARY OF STATE FOR HEALTH'S ORAL STATEMENT TO THE HOUSE OF COMMONS ON 4 NOVEMBER 2008

The Secretary of State for Health (Alan Johnson): With permission, Mr Speaker, I should like to make a statement about Professor Mike Richards's review of current policy concerning NHS patients who wish to pay for additional private drugs.

I start by paying tribute to Professor Richards and his review team for the diligence with which they have conducted this review. The report is informed by evidence from thousands of patients, carers and clinicians across the country, and it was completed with necessary urgency but without in any way compromising its thoroughness. The time scales that I set were deliberately challenging, because this issue is causing great concern and distress to a number of patients and their families.

The review's terms of reference were to examine current policy relating to patients who choose to pay privately for drugs that are recommended by their clinician but not funded by the NHS and who, as a result, are required to pay for the NHS care that they would otherwise have received free of charge. I also asked Professor Richards to make recommendations on whether policy or guidance could be clarified or improved.

Professor Richards quickly identified the underlying causes of the problem. His review starts from the fundamental principle that the NHS provides a universal and comprehensive service to all its patients, free at the point of need. During the Second Reading of the NHS Bill in 1946, Nye Bevan described the financial anxiety endured by people seeking medical help as the "first evil" that the NHS must vanquish. Yet Professor Richards's report shows that access to certain treatments on the NHS, particularly drugs for the terminally ill, is inconsistent, and as a result a very small number of patients feel that they have to pay for additional treatment and are worried that in doing so they will jeopardise their entitlement to NHS care. His report makes recommendations not only to revise the guidance for those exceptional and rare circumstances, but to improve access to certain drugs on the NHS, reducing the need for such patients to resort to private care. His recommendations are accompanied by proposals put forward by Professor Sir Michael Rawlins, chair of the National Institute for Health and Clinical Excellence, to employ greater flexibility in the appraisal of certain treatments, specifically those relating to drugs for the terminally ill. I accept Professor Richards's recommendations in full and today I can announce two immediate developments to make drugs more available on the NHS to those who could benefit from them.

Since it was established in 1999, NICE has ensured not only that many thousands of patients benefit from access to the latest treatments, but that the taxpayer gets value for money. NICE provides wholly independent and scientifically rigorous assessments of the latest medicines and treatments and is widely admired across the world for its work. Its guidance on drugs and treatments is internationally respected, and many other countries are adopting similar models.

The price of the drugs that NICE assesses is a matter for the manufacturers, but I can confirm that I am working closely with the pharmaceutical industry on new and more flexible pricing arrangements that will increase patients' access to new drugs. Those arrangements will include enabling 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. The new arrangements will also build on patient access schemes already developed for particular drugs. That will help to ensure that patients can access medicines that would not otherwise have been deemed cost-effective by NICE. I expect to be able to say more about that soon.

In addition, NICE has for some time been concerned about its ability to take into account the premium that society places on helping those with terminal illnesses. Professor Sir Michael Rawlins has written to me to outline a new, more flexible approach to the appraisal process in order to address those concerns. NICE proposes to introduce more flexibility in the evaluation of higher-cost drugs, which have been shown to extend the lives of terminally ill patients, and where the less common nature of a particular condition may mean that the more flexible pricing arrangements we are discussing with the industry are not in themselves sufficient. The proposal will be implemented immediately while NICE holds a full consultation

Those two measures will ensure that more treatments, which in the past patients may only have been able to access privately, will be available free on the NHS. The work of NICE is necessarily complex, but as we highlighted in the cancer reform strategy, it sometimes takes too long. Its appraisal guidance has often not been available for two years or more after a drug has been licensed. Steps have already been taken to address that, with, for example, fast-track procedures to appraise automatically significant new cancer drugs, but the time it takes NICE to publish guidance can lead to significant local variation.

In response to Professor Richards's report, I am today setting out a new timetable to speed up the appraisal process for all drugs evaluated by NICE. In 2009, draft or final guidance will be available within six months of licensing for about half of the drugs that are being appraised through the fast-track single technology appraisal programme. In 2010, draft or final guidance for all new cancer drugs will be available within six months, on average, of a drug being licensed. We will work with NICE to explore further ways of speeding up the appraisal process with the intention of announcing the outcome of our work early in the new year.

All of those measures will dramatically reduce the instances where a drug is available on the market but primary care trusts have no guidance from NICE on which to base their decisions about funding. However, we cannot totally eliminate instances of primary care trusts having to make decisions in the absence of NICE guidance.

Professor Richards has found that patients and professionals are often unclear about who takes such decisions or how they make them. Patients and their families are often left bewildered about why a particular treatment has been denied, despite what they see as a clear clinical need. As we highlighted in the NHS constitution, we need to do more to improve the quality and consistency of the decisions, especially in any interim period before NICE's guidance is released.

We will therefore publish a set of core principles to inform the way in which primary care trusts make decisions about funding new drugs. It is essential that the process, as well as the decisions, is clear and accessible to patients and the public so that, if a patient is denied access to a particular treatment, the reasoning behind the decision is transparent and consistent. We will publish detailed good practice guidance for primary care trusts about the processes that they should have in place to make decisions about funding new drugs and handling exceptional cases.

Better local decision making, a faster appraisal process, fairer pricing and greater flexibility in evaluating certain treatments will address the main issues that have fuelled the demand from some patients for additional private treatment. However, it would be unreasonable to expect the NHS to fund every single new treatment, and we cannot guarantee that we will completely eradicate the circumstances in which a small minority of patients wish to pay for a drug that the NHS does not provide.

The vast majority of people to whom Professor Richards spoke during his review thought that, in such circumstances, it would be wrong to take away a patient's entitlement to NHS care because they had purchased additional treatment privately. Professor Richards recommends that that practice should end and I agree. It will end with immediate effect.

However, Professor Richards is also clear that there must be tight controls to prevent the NHS from becoming a two-tier system. Today, we will publish draft revised guidance, to take immediate effect, to ensure that the NHS is clear about how to deal with the rare occasions—which, with the implementation of the proposals already mentioned will be even rarer—when patients opt to fund additional drugs privately. The guidance will make it clear that NHS care should never be withdrawn and that administering private care should take place separately from NHS care to ensure that NHS funds are never used to subsidise private care. In short, there is no question of patients who can pay more being able to access a different and higher level of NHS treatment, but neither should any patient who has at some point paid for private treatment lose their entitlement to NHS care.

Today's draft guidance sets out that, in such circumstances, private treatment should be provided in a private facility so that it is always clear whether a particular treatment is NHS or private. That removes any confusion about what constitutes a single visit or single episode of care. It will also remove any question of a two-tier system, whereby those who pay for additional care have their private care subsidised by the NHS.

The NHS must, by definition, continually evolve and improve to fulfil rising expectations and offer the latest advances in medical care to all our citizens, irrespective of wealth, class, ethnicity or disability. The debate has been critically important to the NHS and the values that underpin it. I believe that the measures that I outlined today will, in enabling the NHS to exercise its natural compassion, help secure those values for the future.

We are fortunate in Britain to have a health system that provides a comprehensive service for all patients, based on clinical need, with public funds for health care devoted solely to the benefit of the people it serves. The report by Professor Richards enhances those principles and I commend the statement to the House.

Memorandum by the Cystic Fibrosis Trust (TF 02)

TOP-UP FEES

Whilst the Cystic Fibrosis Trust regrets the need for this debate, as in an ideal world necessary drugs and treatment would be provided in full by the National Health Service, we recognise that there will inevitably be occasions where this is not the case. We would therefore strongly support the right of an individual to top up their treatment as necessary and appropriate, and when possible. We recognise that this could easily create a disparity in care, and we in the Cystic Fibrosis Trust would always work hard to draw this to the attention of the appropriate authorities and to minimise it.

We would like to give one or two examples of just how important we think it is that top-up fees should be permitted without any jeopardy to subsequent NHS care.

One such case was a young man seriously ill in Bristol and on the transplant list for new lungs. His health was deteriorating rapidly and his consultant feared he would not survive long enough to be able to take advantage of a transplant. He recommended the use of a relatively new antibiotic, TOBI, which is one of the more expensive drugs used to treat Cystic Fibrosis patients at around £10,000 per annum. Many Cystic

Fibrosis patients in the UK are offered this drug, but the Primary Care Trust in which this young man lived refused to pay for it. His desperate mother set about raising the money via various fundraising initiatives. The drug was purchased and administered, resulting in a marked improvement in the young man's condition. When, some two months later, a transplant was possible he was well enough to undergo it and it was successful. He is now doing extremely well. However, had he been denied NHS treatment as a result of his mother's intervention, he would not have been offered the transplant and would almost certainly have died by now. Conversely, had she not bought the drug for him, he may well not have lived long enough or been well enough to take advantage of the operation. This sort of situation puts families in an intolerable position. The family concerned were not wealthy. The mother had raised the necessary £10,000 for one year's supply of the drug, and as he only needed two months worth, she donated the remaining funds to the Cystic Fibrosis Trust.

Other examples of Cystic Fibrosis patients supplementing the care given by the NHS include providing themselves with oxygen in a more convenient form. Many of those with Cystic Fibrosis who need oxygen are young people who try to keep up with their studies, their work and their social lives in spite of their deteriorating health. If they are only offered non-portage oxygen in heavy cylinders they immediately become housebound, and their quality of life is severely curtailed. The Cystic Fibrosis Trust is pleased to report that the provision of oxygen to those with Cystic Fibrosis has improved markedly over the last few months, and as a result most people with Cystic Fibrosis are given portable oxygen via the NHS. However, before this was the case, again had those who upgraded their oxygen supply in this way been disadvantaged in terms of the rest of their Cystic Fibrosis treatment, they would be at a huge disadvantage. Again, they would have been placed in a no-win situation.

Overall, our concern is that in areas where consultants find it difficult to prescribe particular drugs because of the reluctance of PCTs to pay for them, they may well be tempted not to prescribe them and not even to mention them to the patients concerned. It is only the most determined and passionate of doctors who will take on PCTs in these kind of circumstances in order to do the best for their patients.

We recognise that those who are told that a particular drug would be of benefit to them but that it is not affordable is the exception rather than the rule. Most simply never hear of what might be offered if they lived somewhere else. Many consultants describe the situation where they are able to prescribe drugs for patients in certain parts of their catchment area but not patients who come from other areas. This kind of disparity is unacceptable, and we hope that with the new commissioning arrangements will soon become a thing of the past. However, the Cystic Fibrosis Trust strongly endorses the right of patients with Cystic Fibrosis to upgrade their care and to pay for drugs or treatments that may be of help to them that are denied by the NHS without compromising the rest of their treatment.

December 2008

Memorandum by Dr Ian Mackenzie (TF 03)

THE PURCHASE OF ADDITIONAL DRUGS BY NHS PATIENTS ("TOP-UP FEES")

EXECUTIVE SUMMARY

NHS consultants who recommend that patients should consider self-funding of additional drugs must declare any relevant interests regarding the recommended drug.

1. Many NHS consultants providing oncology and cancer care receive funding directly or in kind from the pharmaceutical companies who manufacture cancer drugs.

2. Most national and international conferences are sponsored by the pharmaceutical industry that funds the flights, accommodation and hospitality for the consultant delegates. New cancer drugs are discussed and promoted at such conferences before formal clinical trials are published. Other consultants act as advisers to the companies or receive fees for lecturing.

3. It is essential that patients who initiate a conversation with a NHS consultant about a drug (which they may have been made aware of through the media or patient group) or indeed are recommended by a NHS consultant to seek a drug not normally funded by the NHS, are informed of any self-interest that the consultant has, either as a result of receipt direct payment by the company which makes the drug or from the receipt of payments in kind from the company. This is not made clear in the Department of Health consultation guidance but does need to be highlighted.

December 2008

Memorandum by the Rarer Cancers Forum (TF 04)

TOP UP FEES

1. EXECUTIVE SUMMARY

1.1 The Rarer Cancers Forum is a patient-led charity that offers advice and information to people with rare or less common cancers. During 2008 we have undertaken detailed work to assess the extent of problems relating to unfunded cancer treatments.

1.2 We welcome Professor Richards' review and the decision by the Committee to launch an inquiry into the issue. We are pleased to have the opportunity to submit written evidence. We would also welcome the opportunity to provide oral evidence.

1.3 In our submission we make a number of recommendations which the Committee may wish to consider as part of its inquiry:

- Clear guidance on PCT exceptional decision-making process should be issued by the Department of Health. Our submission sets out the issues which should be addressed in the guidance.
- PCTs should act swiftly to introduce collaborative decision-making processes, ensuring that they have access to appropriate levels of expertise for making funding decisions for treatments for often extremely rare conditions.
- Improving the timeliness of NICE guidance is crucial. Given the problems NICE has encountered in the past in publishing guidance within six months of a new treatment becoming available in the UK:
 - NICE should be required to report annually to Parliament on the time it has taken to appraise each new treatment and, where the six month deadline has been missed, be required to explain the reasons for this delay.
 - Where NICE has not published guidance within six months of a treatment's launch, it should signpost NHS organisations to other guidance which should be adhered to until NICE guidance has been published.
 - In the event that NICE begins conducting an appraisal on a certain treatment and fails, within six months of the treatment becoming available in the UK, either to issue guidance or to signpost the NHS to interim guidance, it should be fined by being required to pay for each patient to receive the treatment on which it has failed to issue guidance, provided that the treatment has been recommended by a NHS doctor.
- The proposed NICE scheme for end of life treatments is to be welcomed. In the interests of clarity and transparent governance we request that this commitment be strengthened to include a full review of the scheme 18 months after it is put in place (ie June 2010).
- NICE and the Department of Health should also urgently assess the case for increasing the cost-per-QALY threshold to reflect inflation.
- There should be an assessment of the costs of drugs in European countries, compared with the UK.
- The Department of Health should investigate ways in which the discrepancy between the funding of cancer services in England and other European countries can be addressed. If the Government believes that English levels of funding for cancer services are appropriate, it should publish the reasons why it believes the higher, European levels of funding are inappropriate.
- The "separate care" arrangements proposed by Professor Richards appear, when considered alongside the other recommendations made in his report, to be a fair and pragmatic solution to a difficult issue. We suggest that the Department of Health should commission a follow-up report in the autumn of 2009 to assess progress with implementation of the revised guidance.

2. INTRODUCTION

2.1 The Rarer Cancers Forum is a patient-led charity that offers advice and information to people with rare or less common cancers and to their families and friends. The charity facilitates supportive networking, raises awareness of rare and less common cancers and works to ensure that people with rarer cancers have access to the best possible services.

2.2 A number of treatments for rarer cancers have not been routinely funded by the NHS, resulting in patients having to rely on exceptional funding processes or—ultimately—purchasing drugs privately (often referred to as "topping up") to secure life sustaining treatment.

2.3 The Rarer Cancers Forum has supported many patients who have found themselves in this situation. We therefore undertook detailed investigation into the issue, resulting in the publication of two reports—Taking Exception and Exceptional England?—which quantified the extent of the problem and suggested solutions.

2.4 We welcome the publication of Professor Richards' report and fully support the recommendations he makes. In particular, we welcome the focus on addressing the underlying causes of the problems relating to access to medicines, as well as the specific issue of "topping up."

3. THE EXTENT OF THE PROBLEM

Demand for unfunded cancer treatments

3.1 There is significant demand for unfunded drugs. Taking Exception revealed that, for cancer alone:⁴

- There are approximately 3,000 applications for exceptional funding for cancer patients annually.
- Of these applications, approximately 2,200 cases are approved.
- Approximately 800 cancer patients are denied funding for treatment through the exceptional cases process every year, leaving them with little option but to consider "topping up" if they can afford it.

3.2 Applications tend to relate to three categories of treatments:

- Those which NICE is appraising, but has yet to publish a final recommendation.
- Those which are licensed for very rare conditions or which are being used for a similar condition outside the license ("near label") and so will never be appraised by NICE.
- Those which have been appraised by NICE and not recommended for use in the NHS.

3.3 Current requests for exceptional funding are likely to be a significant underestimate of the total latent demand for unfunded drugs, as many clinicians and patients will be deterred from submitting an application if they see little chance of success.

3.4 If a less restrictive attitude were adopted towards funding cancer treatments, we estimate that—rather than the existing 2,200 patients who receive funding for cancer treatments through the exceptional cases process at present—4,840 patients would receive such cancer treatments after one year, 7,163 patients after two years, and 10,602 patients after three years.⁵

Variations in access to cancer treatments

3.5 There are also significant inequalities in access to cancer medicines. There is a 180-fold variation in the number of exceptional funding requests received by PCTs. This suggests that either some PCTs are particularly generous in their funding of cancer medicines which have not been approved by NICE, meaning that very few treatments ever get to the stage where exceptional requests for funding are made, or that some PCTs are discouraging clinicians from using the exceptional funding route.

3.6 There is also a "postcode lottery" in the proportion of applications for cancer treatment accepted and rejected. Of the 62 PCTs from which we received directly comparable information on this issue, 11 PCTs approved all the requests made to their exceptional cases panel, while two PCTs approved none of the requests.⁶

The costs of unfunded cancer treatments

3.7 Many unfunded cancer treatments have a high unit cost. Our research suggests that the average cost of an unfunded cancer treatment is £20,821.⁷ It is logical to conclude that the cost of "topping up" to an individual would be similar.

3.8 However, the overall cost of unfunded cancer treatments is relatively modest in the context of wider expenditure on healthcare. The total cost to the taxpayer of funding the 800 rejected exceptional cases applications is just £16.7 million a year.⁸ Even if latent demand were met, the overall cost of funding additional treatment for 10,602 patients would be approximately £175 million—a figure which represents just 10% of the current NHS surplus.⁹

⁴ Rarer Cancers Forum, *Taking Exception: An audit of the policies and processes used by PCTs to determine exceptional funding requests*, August 2008, page 30.

⁵ Rarer Cancers Forum, *Exceptional England? An investigation of the role of Primary Care Trusts in making cancer medicines available through exceptional cases processes*, October 2008, page 29–30.

⁶ Rarer Cancers Forum, *Taking Exception: An audit of the policies and processes used by PCTs to determine exceptional funding requests*, August 2008, page 26.

⁷ Rarer Cancers Forum, *Exceptional England? An investigation of the role of Primary Care Trusts in making cancer medicines available through exceptional cases processes*, October 2008, page 41–42.

⁸ Rarer Cancers Forum, *Exceptional England? An investigation of the role of Primary Care Trusts in making cancer medicines available through exceptional cases processes*, October 2008, page 29.

⁹ Rarer Cancers Forum, *Exceptional England? An investigation of the role of Primary Care Trusts in making cancer medicines available through exceptional cases processes*, October 2008, page 30.

4. MINIMISING THE NUMBER OF PATIENTS WHO MAY NEED TO “TOP UP”

4.1 We support the concept of a “comprehensive” NHS, as set out in the draft NHS Constitution. However, it is clear that, if large numbers of patients are denied life-sustaining treatment, this principle will be eroded. It is therefore imperative that urgent steps are taken to minimise the extent of the unfunded drugs problem.

Improving PCT decision-making processes

4.2 Taking Exception revealed the unacceptable variations which exist in the processes used by PCTs to determine exceptional funding applications. We therefore wholeheartedly support Professor Richards’ recommendations 2, 3 and 4 which will, if implemented properly, lead to considerable improvements in the quality, consistency and timeliness of processes used.

4.3 The guidance on PCT exceptional decision-making process to be published by the Department of Health should make clear that:

- PCTs should adhere to a clear, national definition of what constitutes “exceptionality”, and a framework for including social criteria in any assessment of exceptionality should be set out by the Department of Health and debated in Parliament. Equally, the terms given to describe “exceptional cases” and “exceptional cases panels” should be standardised across the country.
- PCTs should be required to have clear, written information describing their exceptional cases processes, to publish this information on their websites, and to proactively provide this information to patients who are subject to the processes.
- Staff of the NHS’s Patient Advice and Liaison Services should be trained in how to support those patients who are subject to exceptional cases processes.
- Patients or their representatives have a right to attend the panel meeting at which their case is discussed, and to make their own representations to panel members.
- Exceptional cases panels should reach decisions relating to cancer treatments speedily, so as to ensure that the maximum 31-day treatment target for cancer therapies is not breached. We suggest that panels should reach their initial decision within a maximum of five working days, with appeals having to be heard within an additional five working days.
- Exceptional cases panels should be constituted in the same way in each PCT. They should include at least an equal number of clinicians and administrators, lay representation and, where they consider cancer treatments, an oncologist. The decision-making processes of panels should be standardised and should include rules related to quorate committees and the method by which decisions are reached when members disagree.
- Patients should receive prompt and clear written explanations setting out why their case has been approved or rejected.
- PCTs’ adherence to the guidance should be assessed as part of the World Class Commissioning assurance framework currently being developed and implemented by the Department of Health. Given the potential for unacceptable variations to occur in exceptional cases processes in different areas of the country, we suggest that a process of peer review is also initiated, enabling different PCTs to learn from each other and share good practice.

4.4 PCTs should act swiftly to introduce collaborative decision-making processes, ensuring that they have access to appropriate levels of expertise for making funding decisions for treatments for often extremely rare conditions, which will clearly not be available in every PCT.

Making NICE appraisals more timely

4.5 The treatments which are subject to the most exceptional funding requests are those for which lengthy NICE technology appraisals are underway.¹⁰ It is imperative that steps are taken to make NICE appraisals more timely and we therefore welcome Professor Richards’ first recommendation.

4.6 Several attempts have already been made to speed up the development of NICE guidance. Despite these attempts, many appraisals are still subject to significant delays.¹¹ Further measures are therefore needed to ensure the availability of timely guidance. Given the problems NICE has encountered in the past in publishing guidance within six months of a new treatment becoming available in the UK:

- NICE should be required to report annually to Parliament on the time it has taken to appraise each new treatment and, where the six-month deadline has been missed, be required to explain the reasons for this delay.

¹⁰ Rarer Cancers Forum, *Taking Exception: An audit of the policies and processes used by PCTs to determine exceptional funding requests*, August 2008, page 28.

¹¹ *Hansard*, 4 November 2008, Column 371W.

- Where NICE has not published guidance within six months of a treatment’s launch, it should signpost NHS organisations to other guidance which should be adhered to until NICE guidance has been published. This other guidance could include that issued by the Scottish Medicines Consortium, by a cancer network, by the medical Royal Colleges, or simply a published, consensus opinion from leading clinicians.
- In the event that NICE begins conducting an appraisal on a certain treatment and fails, within six months of the treatment becoming available in the UK, either to issue guidance or to signpost the NHS to interim guidance, it should be fined by being required to pay for each patient to receive the treatment on which it has failed to issue guidance, provided that the treatment has been recommended by a NHS doctor.

Proposed NICE scheme for treatments for rarer, end of life conditions

4.7 There is a persuasive case for varying the cost-per-QALY threshold according to both severity of disease and the rarity of the condition in question. Recommendation 10 of *Exceptional England?* stated that: “NICE should employ greater flexibility in assessing the cost-per-QALY for patients who are near the end of life.” It is therefore greatly encouraging to see NICE acting on this suggestion with the proposed scheme, and we warmly welcome it.

It will, however, be important to keep the operation of the scheme under review so as to ensure that it:

- Successfully makes available more innovative treatments for conditions such as metastatic rarer cancers, addressing the emerging inequality in treatment provision between England and Europe.
- Delivers timely advice to the NHS on new treatments for advanced, rarer cancers.
- Ensures fair access to treatment for patients, a fair price for manufacturers and fair value for the NHS.

4.8 We note that NICE is proposing to “put arrangements in place for an independent evaluation in due course”.¹² In the interests of clarity and transparent governance we request that this commitment be strengthened to include a full review of the scheme 18 months after it is put in place.

Other measures for NICE to consider

4.9 NICE’s proposed scheme is a welcome step forward. Alongside this, NICE and the Department of Health should urgently assess the case for increasing the cost-per-QALY threshold to reflect inflation. As the Committee was told in its inquiry into NICE last year, “by whatever means the threshold is determined, it should be adjusted over time”.¹³

4.10 The NICE cost-effectiveness “threshold” has been frozen since 1999, representing a real-terms increase in the height of the cost-effectiveness hurdle which treatments must clear each and every year since then. If it had been adjusted to reflect inflation indices, then treatments with a cost-per-QALY of up to £49,000 would now be considered cost-effective.¹⁴

The role of the pharmaceutical industry

4.11 The pharmaceutical industry has a responsibility to play a part in ensuring that the treatments it develops are available to NHS patients. We support Professor Richards’ recommendation that flexible pricing mechanisms should be explored as part of the Pharmaceutical Price Regulation Scheme (PPRS) and we welcome the subsequent announcement on this issue.

4.12 However, the issue of relative drug prices is worthy of further exploration and there should therefore be an assessment of the costs of drugs in European countries, compared with the UK.

Ensuring appropriate funding for cancer services

4.13 In the longer term, future issues with the availability of cancer treatments could be addressed by reconsidering the level of funding given to cancer services. The Department of Health’s own figures show that France and Germany spend 51% and 79% more per head respectively on cancer services than England.¹⁵ Increasing funding for cancer services only to the level of France would result in substantial increased funding for cancer services, providing over £2 billion extra across England.¹⁶

¹² NICE, *Consultation on appraising end of life medicines*, November 2008.

¹³ Health Committee, *National Institute for Health and Clinical Excellence*, 10 January 2008. HC 27-1

¹⁴ Rarer Cancers Forum, *Exceptional England? An investigation of the role of Primary Care Trusts in making cancer medicines available through exceptional cases processes*, October 2008, page 27.

¹⁵ Department of Health, *Cancer Reform Strategy*, 3 December 2007, page 121.

¹⁶ Rarer Cancers Forum, *Exceptional England? An investigation of the role of Primary Care Trusts in making cancer medicines available through exceptional cases processes*, October 2008, page 23.

4.14 The extra spending on cancer services in France and Germany is made possible, not because they spend more on healthcare *per se*, but because they commit a far greater proportion of their healthcare budget to support cancer services: the 5.6% of England's NHS budget spend on cancer services, for example, compares with 7.7% of France's public healthcare budget, and 9.6% of Germany's.¹⁷

4.15 The Department of Health should investigate ways in which the discrepancy between the funding of cancer services in England and other European countries can be addressed. If the Government believes that English levels of funding for cancer services are appropriate, it should publish the reasons why it believes the higher, European levels of funding are inappropriate.

5. CLARIFYING WHEN AND HOW A PATIENT MAY PURCHASE ADDITIONAL DRUGS

5.1 We agree with Professor Richards that the overwhelming priority should be to ensure that patients are not put in a position where they may have to "top up." We hope that this will be the primary focus of policymakers' efforts over the coming years.

5.2 Nonetheless, existing guidance on when and how a patient may purchase additional treatment is confused, resulting in significant differences in practice. We therefore welcome the decision to clarify the existing guidance on the basis that no patient should lose their entitlement to NHS care they would have otherwise received simply because they opt to purchase additional treatment.

5.3 The "separate care" arrangements proposed by Professor Richards are, when considered alongside the other recommendations made in his report, a fair and pragmatic solution to a difficult issue.

5.4 Given the previous inequalities in implementation, and the potential impact that these have on public confidence in the NHS, we suggest that the Department of Health should commission a follow-up report in the autumn of 2009 to assess progress with implementation of the revised guidance.

December 2008

Memorandum by the Royal College of General Practitioners (TF 05)

TOP-UP FEES

1. The College welcomes the opportunity to contribute evidence to the Parliamentary Health Committees inquiry into Top-up fees.

2. The Royal College of General Practitioners is the largest membership organisation in the United Kingdom solely for GPs. It aims to encourage and maintain the highest standards of general medical practice and to act as the "voice" of GPs on issues concerned with education, training, research, and clinical standards. Founded in 1952, the RCGP has over 36,000 members who are committed to improving patient care, developing their own skills and promoting general practice as a discipline.

3. The College submitted evidence to Professor Mike Richard's review of the consequences of additional private drugs for NHS care in August 2008. Following on from the publication of Professor Mike Richard's report, we are submitting evidence to two further related consultations: the National Institute for Clinical Excellence's (NICE) consultation on Appraising End of Life Medicines and the Department of Health's consultation on draft guidance on NHS patients who wish to pay for additional private care. You will find below an outline of the arguments, ideas and suggestions raised by our members in response to these three consultations. We hope that these aid the Health Committee with their inquiry and we would be willing to provide further evidence, both oral and in writing.

The potential threat to equity

4. Several members have raised concern that the introduction of co-payments might undermine the founding principles of the NHS—that care should be provided on the basis of need rather than the ability to pay. Some have considered the introduction of co-payments to be the "thin end of the wedge", marking a significant step towards the gradual privatisation of the NHS, and have expressed a fear that as new treatments become more costly a two-tier health system might develop similar to those in other countries.

¹⁷ Department of Health, *Cancer Reform Strategy*, 3 December 2007, page 121.

The impact of top ups on patients and their families

5. Whilst the College would support patients being involved in the decision making process about their care, some members have suggested that patients and their families might become “medicalised” and be exploited into purchasing ineffective treatments privately through desperation. This would result, it has been argued, to a similar situation as occurs in the United States, where individuals and their families feel obliged to spend all their resources on treatments. We know that the vast majority of our lifetime NHS expenditure is in our last six months of life. Passing this burden on to families, shortly to be bereaved is too great an emotional and financial pressure for individuals to bear. Families will face dilemmas, such as whether to sell their house or not, for the chance of a few more months of life of their loved one. Patients will have to be protected from these burdens when they are at their most vulnerable.

The administration of private drugs and the cost of treating complications which might arise from private treatment

6. Some members have objected to the prospect of patients accessing private treatment and additional care whilst simultaneously having their care administered by the NHS. It has been argued that the additional staff time to administer complex drugs and the monitoring involved in treatment will create significant costs for the local service and, if these costs are not borne by the patient, others will lose out. Furthermore, it has been suggested that if individuals want to purchase drugs and require monitoring, any additional monitoring and prescription, including echocardiograms and bloods, should be paid for privately. Training would also be required for staff who administer the new drugs and it is unclear where the funding for this would come from. There will be an increase in demand for oncology inpatient and outpatient services as patients may live a few months longer. This needs planning and appropriate funding.

7. It has also been questioned whether patients should be expected to be treated by the NHS for complications which might arise from new, unproven treatments. The extra drugs will cause their share of side effects some of which will cause additional hospital stays. It is not unreasonable to ask who will pay for this treatment—whether this will be the taxpayer or the patient.

Observations on the current system

8. Others have argued that at the inception of the NHS it was accepted that private medical practice could continue alongside NHS practice. This meant acceptance of the ability to provide such services, but also that patients could use both systems. College respondents to Professor Mike Richard’s review highlighted that fact that millions of patients have used private providers for surgery and other procedures, such as IVF and diagnostic tests and patients have often elected for private consultations in order to get advice from the consultant or to ensure that their operations are performed by a named consultant of their choice. At no point in the past has the NHS ever tried to refuse treatment to patients who have had private surgery; IVF for example is often private, but the NHS provides antenatal and postnatal care.

Personal responsibility

9. It was noted that the rules on co-payments, as they stood at the time of Professor Mike Richard’s review, were inconsistent with the government’s view that patients should take responsibility for their own health and should be involved in the decision making process about their treatment.

Preserving the rights of patients

10. The College would empathise with any patient who finds themselves in a position where they are forced to consider whether to fund their own treatment. Some College respondents to Mike Richard’s review felt strongly that if patients want to pay for other treatment they are exercising their autonomy. All patients, it was argued, have contributed to basic NHS provision through taxation, and so through the purchase of top-ups, they should not forfeit their right to NHS treatment.

Protecting the autonomy of doctors

11. It was suggested that the rules on top-ups at the time of Professor Mike Richard’s review conflicted with the duty of a doctor to do their best for their patients. The role of a doctor, it was argued, should be to use all the resources available for each individual patient and to give their patients reasonable information to enable them to make their own decisions.

Implications for treatment if co-payments were prohibited

12. Some respondents feared that if rules were imposed that prohibit co-payments, a situation might arise whereby patients conceal private treatment in order not to forfeit their NHS treatment, leading to deterioration in the clinical relationship between patient and doctor and placing the patient at greater risk of complications associated with drug interactions.

Possible solutions to the co-payments debate

13. In response to Professor Mike Richard's review, the College suggested that the situation could be improved if action was taken to support and strengthen NICE to ensure that effective drugs were made available to all more rapidly. It was however recognised that NICE would still be likely to reject the funding of some drugs or treatments on the grounds of cost-effectiveness (but which nevertheless may confer some benefit to some patients). Therefore, it was suggested that Baroness Finlay's model¹⁸ be adopted, whereby a small number of drugs or devices are approved for co-payments and sanctioned according to the following criteria:

- The drug or device is listed as one for which copayment is allowed.
- To ensure that hopes are not raised unrealistically, the patient will have discussed the risks and likelihood of failure as well as success with their clinician.
- The clinician should have a reasonable belief—supported by peers—that the anticipated benefits for the patient of the unfunded drug outweigh the benefits of other treatment.
- Patients who are unable to participate in a clinical trial should be willing for their treatment and its outcomes to be recorded on a register and be potentially available for research.

14. Whilst it may not allow the level of clinical freedom and patient choice that some may desire, it was argued that the advantage of Finlay's model was that it would offer a workable solution that would preserve equity and justice for prescribing in the NHS.

The drug appraisal process

15. The College welcomes proposals to speed up the NICE appraisal processes so that new cancer drugs are made available to more patients more quickly. We would also like to see a review of less effective drugs so that further resources could be freed up and better invested to benefit all patients. The new funding guidance for PCTs will also help remove the postcode lottery where patients in some areas have been allowed to pay for their drugs in exceptional circumstances.

16. We are in favour of NICE considering the approval of drugs costing more than £30,000 per QALY gained when these drugs have been shown to prolong length and quality of life in terminal illness.

17. Some College members have, however, voiced concern that an increase in the NICE threshold might mean that many new treatments, if they fall into the threshold, will become mandatory. The danger of this is that money will be shifted to these new drugs and away from other treatments. From a commissioning point of view, if drugs are introduced without warning it can lead to a distortion of health priority with funding being taken out of services which are not protected by NICE labels. It is likely that the government will face pressure to channel resources into cancer and palliative care. It is therefore imperative that NICE and the MHRA remain free from political influence and only operate in the interests of the patient.

18. I acknowledge the contributions of Dr Sandy Taylor, Dr Penny Cox, Professor Tony Avery, Dr Andrew Ross, Dr Clare Gerada, Dr K Sivakumar, Dr Mei Ling Denney, Professor Amanda Howe, Dr David McKinlay, Dr David Millson, Dr Andrew Spooner, Dr Pauline Brimblecombe, RCGP Tamar Faculty (Dr Steve Watkins, Surgeon Rear Admiral Ralph Curr, Dr Nick Bradley, Dr David Bossano, Dr Colan Robinson), Dr Jonathan Orrell, Dr Bryn Neal, Dr David Jewell, Dr Ben Jackson, Dr Kevin Ilsley, Dr Martin Wilkinson, Professor Bob Berrington, Dr Alison Miles and RCGP East Anglia Faculty towards the above comments. While contributing to this response, it cannot be assumed that those named all necessarily agree with all of the above comments.

Dr Maureen Baker
Honorary Secretary of Council

December 2008

¹⁸ BMJ 2008;337:a527.

Memorandum by Breakthrough Breast Cancer (TF 06)

TOP-UP FEES

1. INTRODUCTION

1.1 Breakthrough Breast Cancer welcomes the Health Committee Inquiry into top up fees. As rising numbers of advanced cancer treatments are developed, the issue of what the NHS can, and should, pay for is receiving more and more attention.

1.2 There is concern that the NHS may be unable to afford to provide new treatments as they come onto the market and the National Institute for Health and Clinical Excellence (NICE) will become less likely to recommend treatments for use on the NHS if they are not viewed as cost effective.

1.3 One proposed solution is to allow patients to top-up their NHS healthcare by paying for additional treatment, such as drugs that are currently unavailable on the NHS (eg because they are awaiting NICE guidance). Such an option has many potential consequences. Some commentators have suggested that top ups will advance the creation of a two-tier health system between those with, and without, the ability to pay. Another concern is that allowing top-ups may serve to undermine the current role of NICE, as the purveyor of rational evidence-based guidance for the NHS, and additional costs could be incurred by the NHS if expected to deliver associated treatments and care for a patient who has “topped up”. Alongside these issues is an increased focus on patients being able to exercise choice in their treatment options or to obtain care abroad if they wish to do so.¹⁹

1.4 As this is a multifaceted issue peppered with complex ethical questions, Breakthrough recognised the need to identify the views of its stakeholders. In order to do this, a Citizens Jury²⁰ was held in September 2008 involving over 30 people affected by breast cancer. Many of the views and issues raised during the event are also pertinent to the Health Committee Inquiry and are reflected and quoted in this memorandum.²¹

2. EXECUTIVE SUMMARY

3.1 Much of the debate on top ups during the Citizens Jury organised by Breakthrough was polarised, with strong arguments both for and against. However, a number of key issues were identified by participants—including the need to ensure that:

- Demonstrably effective drugs are available to all who need them based on clinical need, not cost.
- New drugs and treatments are made available as quickly as possible and the role of NICE supported.
- Drug pricing is both fair and transparent.
- High quality patient information is available on the quality of clinical evidence relating to a particular treatment, including the benefits, risks and limitations of treatment.
- Patients at the end of their lives do not feel forced to challenge funding decisions to access drug treatments at a time when they are least able to do so.
- There is greater clarity over PCT Exceptional Case Review processes—with national guidance.

3.2 The proposals outlined by Professor Richards in his recent report,²² go some way in addressing the needs of patients. However, Breakthrough believes there are some issues that still need to be addressed. It is now essential that the proposals are implemented consistently across the NHS to ensure that clarity is provided on this complex issue.

¹⁹ The proposed European Union (EU) Health Services Directive, which may allow people to pay to have treatments abroad and then be reimbursed later by the NHS, adds another layer of complexity to this debate. This system may operate on a top up basis, where patients could get a certain proportion of the cost of a treatment reimbursed by the NHS, but make up the difference themselves. What impact, if any, this will have on the NHS or how the EU Directive will be balanced alongside the NHS Constitution or, indeed, the founding principles of the NHS is currently unclear.

²⁰ A Citizens Jury is a unique approach to qualitative research that enables participants to discuss complex issues. The Citizens Jury model was selected to explore stakeholders’ perceptions of top-ups broadly, rather than to answer specific questions conclusively. Jurors heard from a variety of experts, deliberated the evidence in small facilitated groups and participated in the cross-examination of experts by sharing views and asking questions. The experts, known as Expert Witnesses were identified for their professional expertise on the issue and covered the medical, economic, workforce, legal/ethical and political principles which underpin the issue of top-ups.

²¹ Breakthrough’s recommendations submitted to the review of the consequences of additional private drugs for NHS care can be found in Appendix 1.

²² *Improving access to medicines for NHS patients*, Professor Mike Richards, November 2008.

3. ABOUT BREAKTHROUGH BREAST CANCER

3.3 Breakthrough Breast Cancer is the UK's leading breast cancer charity and is committed to fighting breast cancer through research, campaigning and education. Breakthrough campaigns for policies that support breast cancer research and improved services, as well as promoting breast cancer education and awareness amongst the general public, policy makers, health professionals and the media. Breakthrough has also established the UK's first dedicated breast cancer research centre in order to achieve our vision of the future free from the fear of breast cancer.

3.4 Breakthrough works closely with healthcare professionals, patient advocates and researchers. This memorandum incorporates the views of Breakthrough and members of the Campaigns and Advocacy Network (Breakthrough CAN), made up of over 1,100 individuals and organisations. Many members of Breakthrough CAN have personal experience of breast cancer as well as being involved in working alongside their local NHS to deliver better services and treatment for people affected by this disease and their families.

4. THE VIEWS OF PEOPLE AFFECTED BY BREAST CANCER

A two tier health system

4.1 There is no doubt that the issue of whether patients should be allowed to top up their NHS care is complex with numerous political, medical, ethical and economic arguments both for and against. Therefore, minimising the need for top ups is an important aspiration of Professor Richards' report *Improving access to medicines for NHS patients*.

4.2 The possibility that the introduction of top up payments could increase inequalities, leading to a two-tier health system undermining the founding principles of the NHS, caused considerable controversy amongst participants at Breakthrough's Citizens Jury. Whilst some participants felt that that core principles on which the NHS was founded are absolutely fundamental, others felt that the NHS is already two tier (with local variations in care delivered) so the introduction of top ups would have little effect.

"Founding principles are fundamental to the NHS."

"... Like it or not, we already have a two tier health system because we allow private healthcare in this country, so I don't see why buying drugs is very different."

"If equality is the central principle of the NHS, why are prescriptions free for some and not for others and why are there such large local variations?"

4.3 However, any move to further embed or possibly establish new inequalities was considered unacceptable by some participants as is the possibility that the NHS would only be able to offer a "core service" rather than what patients clinically need. Particular concerns were raised around whether allowing top ups would be a "slippery slope" towards an American insurance-based healthcare system.

"Top ups are the thin end of the wedge"

"The worry is that it becomes a system for educated middle class people who are informed about their options."

"It's down to who can and can't afford it and I feel uncomfortable with that."

4.4 It is essential that top ups are carefully monitored within the NHS to ensure that their use is minimised.

Impact on the NHS

4.5 Top ups differ from private healthcare in that if paying privately, the patient will pay for all the associated costs of their treatment (eg administration, staff time, hospital stays etc). Avastin, a treatment for breast cancer, is an example of where not only the costs of the drug itself need to be considered, but that of the chemotherapy drug paclitaxel which it has to be used in combination with, and the associated costs of administration, staff time, hospital stays etc. Whilst paclitaxel is recommended by NICE as an option for the treatment of advanced breast cancer where initial chemotherapy has failed or is inappropriate, Avastin has not yet been recommended for use in the NHS as a treatment for breast cancer.

4.6 Should patients wishing to top up their care (in this example with a drug not approved for use in the NHS) also pay the associated costs of paclitaxel—a treatment the patient may not otherwise receive? How will other associated costs be covered? Participants at Breakthrough's Citizen Jury felt strongly that patients should only pay for the cost of the drug itself. However there was also general confusion over what "associated costs" include (do they include drugs used in combination, staff time, administration?) and just how much money was involved—which many participants felt needed to be balanced against the cost savings that could potentially be made from allowing patients to top up their NHS care.

4.7 This is an issue that still requires considerable clarification. Recommendation 8 of Professor Richards' report asks the Department of Health to make it clear that no patient should lose their entitlement to NHS care they would otherwise have received because they wish to top up their care. However, it is not clear, using the Avastin example above, whether a patient would receive paclitaxel on the NHS (which they might not otherwise need) or whether other associated costs of treatment are covered.

4.8 It has also been proposed by Professor Richards that individuals may top up their care while continuing to be treated as an NHS patient for other elements of care, as long as the two elements of care are provided separately (eg using a designated hospital with private facilities, making use of homecare provision or designating an area of an NHS hospital for patients for the delivery of top up treatment). Breakthrough has heard from healthcare professionals that they are particularly concerned about how this will be implemented in practice—for example, whether continuity of care will be maintained, the practicalities of finding a separate area for care (and whether services will have to be reconfigured to accommodate this) and patient safety (ie concerns over the safety of delivering treatments not usually used) have all been raised.

Access to drugs and treatments

4.9 As a charity funding around £12 million worth of research each year in the UK, Breakthrough is keen to ensure that new advances in treatments are translated into patient benefit as quickly as possible.

4.10 The focus of research for cancer treatment is increasingly on more specialist, complex, targeted therapies—this approach has the potential to greatly benefit patients by making treatments more targeted and possibly reducing side effects. The technology used to develop these therapies is also more sophisticated and subsequently more expensive. In addition, many treatments are being designed to work as additive treatments (in addition to an existing standard therapy), further adding to treatment costs. In addition, as treatments become more targeted, the potential market from which pharmaceutical companies can recoup costs is also reduced.

4.11 In addition to clinical effectiveness, the cost of new drugs and treatments is at the heart of the top ups issue, raising questions over whether the NHS cancer drugs budget should be increased and whether current drug prices are competitive. In 2007, the Office of Fair Trading raised concerns that some drug prices were significantly out of line with patient benefits.²³ Similarly, at Breakthrough's Citizens Jury, concerns were raised over how drug prices are currently negotiated and set, and whether allowing top ups would reduce the need for drug companies to price competitively.

"If I was the only customer I would negotiate a good price, so why doesn't the NHS do this?"

"... the question is, are the drugs actually worth the money?"

"Drug companies have some responsibility here too—they should be pressured to come in with more reasonable prices."

4.12 Recommendation 5 in Professor Richards report *Improving access to medicines for NHS patients* outlines the intention to promote more flexible approaches to the pricing and availability of new drugs. However, during the Citizens Jury, whilst there was general support for the principle of exploring such schemes, questions were raised regarding whether they provide a possible solution for the NHS as they may distort doctors' views on which drugs to prescribe and do not necessarily address issues around competitive pricing. There was also concern that the risk sharing schemes currently in operation²⁴ have not been evaluated.

The role of NICE

4.13 Overall, participants in Breakthrough's Citizens Jury were supportive of the need for rational evidence-based guidance and prioritisation in the provision of NHS services and the role of NICE in assessing clinical effectiveness. However, questions were raised over the speed at which NICE is able to issue guidance, given the current pace of advances in cancer treatment, and transparency of its decision-making processes.

4.14 How cost effectiveness is assessed by NICE using Quality Adjusted Life Years (QALY) was also highlighted. A number of questions were raised over whether the unofficial QALY threshold of between £20,000 and £30,000 was appropriate, realistic and up-to-date.

"The concept of NICE is good, but too much of its resources are taken up with admin and paperwork."

"We need to 'bolster up' NICE to make it quicker and give the public more confidence in its processes and decisions."

"It seems ridiculous that [the QALY] hasn't kept pace with inflation."

²³ <http://www.offt.gov.uk/news/press/2007/29-07>

²⁴ For example, in 2007, in order to optimise access to the bone marrow cancer drug bortezomib (Velcade), NICE issued draft guidance recommending that the NHS should provide funding for all suitable patients with multiple myeloma, but that the drug's manufacturer should refund the cost of treatment in patients showing "a minimal or no response".

4.15 With overall support for NICE, there is concern that allowing top ups could undermine its role—to allow some patients to have non-NICE approved treatments or interventions (should they be willing to pay for them) could be seen as disregarding advice regarding clinical effectiveness. However, whether drugs are available on the NHS is not wholly the preserve of NICE. In order to appraise the clinical and cost effectiveness of individual drugs, NICE relies on evidence from pharmaceutical companies. However, there have been recent examples where appraisals have been terminated because no submission has been made by the manufacturer.²⁵ This caused particular concern amongst participants at the Citizens Jury, who felt it was unacceptable.

4.16 Similarly, and despite a directive requiring PCTs to provide funding for NICE approved drugs within three months,²⁶ there is evidence to suggest that NICE guidance is not being implemented. In a recent Health Services Journal article, Baroness Young highlighted that in last year's Healthcare Commission annual rating, the core standard on whether organisations comply with NICE technology appraisals was the seventh least complied with part of the assessment. There were 43 NHS organisations that did not meet, or did not supply sufficient information of meeting the standard in 2006–07.²⁷

The role of PCTs

4.17 The current processes for Exceptional Case Reviews (ECRs) organised at PCT level across the NHS were highlighted as particularly concerning by participants at Breakthrough's Citizens Jury, who felt that such systems need to be far more transparent and consistent to maximise patient benefits. Breakthrough hopes that the draft NHS Constitution (as part of the Health Reform Bill) will commit to ensuring transparency in PCT decision making. It is essential in order to rebuild public confidence in this process that these processes are clear and detailed plans on how this commitment should be met set out as soon as possible.²⁸

“There needs to be a national process for making decisions or better quality decision making by PCTs based on national guidelines.”

“There should be some kind of central instruction to PCTs about how they make the decisions . . . Patients should be allowed to attend the meetings and there should be more transparency about the whole process.”

Patients at the end of their lives

4.18 The importance of the issue of top ups is perhaps best highlighted by the fact that some patients currently affected by this issue are seriously ill, often at the end of their lives. For people diagnosed with secondary (metastatic) breast cancer, the aim of treatment is to prolong and hopefully increase the quality of their life. When asked, such patients expressed different views on whether, if able, they would top up their NHS care.

“. . . However unfair top up charges might be, as a patient I am not prepared to have my treatment chances dictated by accountants who make their decision based on population studies. As an individual and a stage 4 cancer patient with a limited life span. . . anything that prolongs life is welcome.”

“My life wouldn't be worth saving at the expense of the lives of 20–30 other people.”

4.19 Allocating life-extending drugs in the NHS is not simple, and participants raised questions around whether the current processes (eg NICE approval and PCT Exceptional Case Reviews) inadvertently discriminate against life extending drugs by trying to “put a price on time”. The current consultation being undertaken by NICE on providing supplemental advice to Appraisal Committees assessing medicines which extend life at the end of life is particularly welcome to help address some of these concerns.²⁹

Information and communication

4.20 Increased public awareness of new interventions and treatments, via the media and internet, has resulted in greater demand. However, in some cases the benefits of treatment may be over inflated or only of benefit to a very specific sub-group of patients. Participants in the Citizens Jury highlighted that patients need much more high quality information about the true value of individual treatments.

“Clearer communication is crucial as many patients don't realise that what is being heralded as a 'wonder drug' may not be appropriate for their specific type of cancer or possibly that it won't be available for several years.”

“Communication and information just isn't there. How would I know whether a drug would be beneficial?”

²⁵ The NICE Single Technology Appraisal of Avastin in combination with paclitaxel for the first-line treatment of metastatic breast cancer was terminated in June 2008 because no evidence submission was received from the manufacturer or sponsor of the technology.

²⁶ http://www.dh.gov.uk/en/Publicationsandstatistics/Publications/PublicationsLegislation/DH_4083088

²⁷ Health Services Journal, 18 August 2008.

²⁸ Draft NHS Constitution (pages 2–3).

²⁹ <http://www.nice.org.uk/newsevents/infocus/EndOfLifeMedicinesConsultation.jsp>

4.21 It is clear that communicating with patients and providing them with high quality and realistic information is vital to ensure they understand the quality of clinical evidence relating to a treatment, what the risks and benefits are, and importantly, the limitations of treatment. This was raised as particularly important by participants in the Citizens Jury, especially for patients at the end of their lives who may be desperate and willing to try anything even if that means significant financial difficulties.

4.22 In addition it is vital that clinicians are supported in accessing up to date information. It is absolutely essential, as outlined in recommendation 12 of Professor Richard's report, that the Department of Health consider how patients can best access this information. There is also no doubt that healthcare professionals play an incredibly important role in communicating the benefits and limitations of treatment options.

4.23 Throughout the consultations Breakthrough has held on the issue of top ups, similar concerns about access to clinically effective treatments, the transparency of current decision-making processes and the importance of high quality patient information have been consistently raised. Breakthrough believes that it is only by addressing these fundamental issues, that the Government can successfully meet the needs of patients and their families. There is a need to improve the current system to ensure that demonstrably effective drugs and treatments are available to all who need them based on clinical need.

December 2008

APPENDIX 1

RECOMMENDATIONS SUBMITTED BY BREAKTHROUGH BREAST TO THE REVIEW OF THE CONSEQUENCES OF ADDITIONAL PRIVATE DRUGS FOR NHS CARE

Breakthrough Breast Cancer welcomes the Government's review into the consequences of additional private drugs for NHS care and the opportunity to input and represent the views of people affected by breast cancer. There is no doubt that the issue of whether patients should be allowed to top up their NHS care is extremely complex with numerous political, medical, ethical and economic arguments both for and against. However, the current situation where patients have effectively been "caught in the middle" is unacceptable.

Based on the views of people affected by breast cancer, Breakthrough does not believe that there is a simple "yes" or "no" answer to this issue. There is a real danger that by focusing on top ups, and whether they should or shouldn't be allowed in the NHS, the current debate will centre around the wrong issues. Will top ups solve the problem or further complicate it? Top ups cannot be considered in isolation, but raises a number of other important issues that must be considered by the Review.

Throughout the consultations Breakthrough has held to inform this submission to the Review, similar concerns about access to clinically effective treatments, the transparency of current decision-making processes and the importance of high quality patient information have been consistently raised. Breakthrough believes that it is only by addressing these fundamental issues, that the Government can successfully meet the needs of patients and their families.

There is a need to improve the current system to ensure that demonstrably effective drugs and treatments are available to all who need them based on clinical need.

The following recommendations—based on the views of people affected by breast cancer—should be taken forward by the Review to address these issues:

1. The founding principles of the NHS must be preserved so that care is based on clinical need not ability to pay.
2. The scale of this issue should be clearly defined and clarification provided on the extent to which top-up payments in the NHS are currently affecting patients and their families.
3. Systems should be established to collect data on drug usage (including clinical outcomes and side effects). This information should be made publicly available and facilitated by the National Cancer Intelligence Network (NCIN).
4. An urgent review of the cancer drugs budget should be undertaken. As part of this review it must be ensured that the NHS is making the most efficient and effective use of its resources.
5. The current system of pharmaceutical drug pricing should be reviewed, ensuring that vital research and development in the UK is not disincentivised. The opportunity presented by the current renegotiation of the PPRS must be fully explored with the Association of the British Pharmaceutical Industry (ABPI) and further options for reducing, where appropriate, drug prices in the UK fairly considered by all parties.
6. The role of NICE should be fully supported and wherever possible refinements made to the current technology appraisal process to ensure guidance is issued to the NHS as quickly as possible. As part of this, pharmaceutical companies must be encouraged, in the strongest possible terms, to submit evidence to NICE regarding the clinical and cost effectiveness of drugs being appraised, and PCTs must implement guidance issued by NICE immediately.
7. Quality Adjusted Life Year (QALY) levels must be regularly updated (by an independent body and including full consultation with patients and the public) to take into account rising costs and up to date assessments of quality of life measures.

8. PCT Exceptional Case Review processes should be changed, and national guidance provided to ensure consistency and provide clarification for patients. This guidance should include recommendations on the appropriate membership of Exceptional Case Review committees.

9. Options for PCTs to work together to agree guidance on drugs pre-license and pre-NICE approval should be fully explored and good practice shared by bodies such as the London New Drugs Group.

10. It is vital that all patients receive realistic and clear information on the true benefits, risks and limitations of new treatments, to make fully informed decisions regarding their treatment and that clinicians are supported in accessing up to date information. The NHS, charities, pharmaceutical companies and NICE should work together to ensure that this information is available. In addition, training for healthcare professionals in communicating complex health information is necessary.

11. The outcomes of the Review must be clearly, and transparently, communicated to ensure that it is understood. In particular, clarity is needed over the terms used in the current debate and misunderstandings that exist around the definitions of “top ups”, “co-payments” and “episode of care” must be addressed.

12. A full explanation, upon publication, of the basis on which the outcomes of the Review have been derived should be provided along with practical and consistent implementation plans.

13. Pending the outcomes of the review, provision must be made to ensure that no patients are denied access to NHS care if, on the advice of their doctor, they choose to pay for clinically effective drugs not currently available to them. This statement is not indicative of Breakthrough’s support for the status quo, but rather demonstrates the need for urgent reform to tackle the issues that have caused this debate.

What is clear is that any decision or clarification of the policy on this issue must have the needs of all patients and the founding principles of the NHS—that treatment is based on clinical need not ability to pay—at its heart.

Memorandum by the Royal College of Radiologists (RCR) (TF 07)

TOP-UP FEES

The RCR welcomed the thoughtful and measured report by Professor Mike Richards: Improving access to medicines for NHS patients.

Through the Joint Collegiate Council for Oncology (JCCO) we look forward to working with Professor Richards and the National Institute for Health and Clinical Excellence to implement the recommendations in the report.

We do not have any other comments at this stage.

Dr Jane Barrett
Vice-President and Dean of the Faculty of Clinical Oncology

December 2008

Memorandum by the UK Commissioning Public Health Network (UKCPHN) (TF 08)

TOP-UP FEES

EXECUTIVE SUMMARY

- The proposal to strengthen the separation between private and public health care practice is to be welcomed. The Department of Health’s consultation document on patients wishing to seek private treatment both protects a citizen’s right to access NHS care on an equitable basis and protects the NHS from subsidising private patients.
- The second proposal coming arising out of Professor Richard’s Review which mandates preferential treatment for interventions which are aimed at those with poor prognosis. The effect of this will be that the NHS will spend an increasingly greater proportion of its funding on treatments which have the least effect, and proportionately less on other patient groups.
- The recommended policy change must be seen in the wider priority setting context as well as the prevailing economic climate.
- The QALY threshold should be set at a threshold which our healthcare system can afford. It cannot be based on the “willingness to pay” unless this is reflected in taxation levels and allocation of funds across different Government Departments. If the threshold for some patient groups is liberalised then it has to be tightened for other patients groups. While the UKCPHN opposes the proposal for liberalising the threshold for certain groups, basic economics dictate that if this policy

is to be pursued, then NICE should also be consulting on a lowering of the QALY threshold for all other treatments or for selected patient groups. As presented the policy change does not make economic sense. A whole system approach is essential.

BACKGROUND INFORMATION ABOUT THE UKCPHN

1. The UKCPHN is a professional network of senior public health doctors and other practitioners working for the NHS either in primary care trust commissioning or specialised commissioning teams.

2. The group was first established in 2002 and now has almost 200 members. These members are drawn from all 10 England specialised commissioning teams and just over half the PCTs in England. As such the members represent a significant proportion of Consultants in Public Health actively working in commissioning.

3. The group has provided the model for the Department of Health's newly funded English Public Health Network for Commissioning under the auspices of the National Institute for Innovation and Modernisation.

4. The Group's particular interest is priority setting and has, amongst its members, individuals recognised as having specialist knowledge and experience in this field.

THE MIKE RICHARDS' REVIEW AND THE PURCHASE OF ADDITIONAL DRUGS BY NHS PATIENTS

5. The demand for healthcare will always outstrip the ability of nations to meet the needs of their citizens. In this context all healthcare systems must have the ability to define the limits of what will be provided and in doing so exclude certain services and treatments.

6. Centrally funded public healthcare systems that aim to provide comprehensive healthcare and which operate alongside a lesser private healthcare sector have been shown to provide the best overall health outcomes for their citizens.

7. Individuals within the UK have always been allowed to combine NHS and private treatment. For instance, patients can pay for private physiotherapy for osteoarthritis but still consult their GP for that problem and be referred within the NHS for surgery. Similarly, patients can have a private diagnostic test to confirm a diagnosis but have their treatment on the NHS as long as they are not given preferential treatment. It follows, therefore, that a patient with cancer who wishes to have a cancer drug privately can continue to access, in parallel, NHS cancer care which would normally be available to all cancer patients in the public sector.

8. Department of Health Guidance: *A code of Conduct for Private Practice* from 2004 makes it clear that private patients do not forego their rights to normal NHS care and that they can access NHS care on the same basis as any other patient.

9. What is not allowed is both NHS and private contribution in a single visit (ie outpatient or inpatient attendance). The Richards' Review resulted from patients being denied NHS cancer care if they chose to pay for a cancer drug privately, because the drug was not available routine on the NHS either because it was not considered cost effective by NICE (The National Institute for Health and Clinical Excellence) or primary care trusts. There has been a serious misinterpretation of the existing code of conduct that was widespread across all sectors of the NHS. Clinicians, trusts and primary care trusts have all made mistakes. So the clinician who advised a patient that if he wanted to pay for an Alzheimer's drug he would have to pay for all his care; and the PCT who told a patient that if they wanted to pay for a cancer treatment they would also have to pay for their palliative care were not merely overzealous—they were outright wrong. While it is recognised that there are some grey areas, they are relatively uncommon.

10. In this context, the recommendation of the Richards' Review relating to the relationship between the NHS and the private sector has been to reinforce and clarify the existing position. No policy change has been recommended but rather the aim is to eradicate misunderstanding and misinterpretation of the existing law and policy and reinstate the existing rights of citizens, regardless of income, to access NHS care to which they always had a right.

11. This has already been translated into new guidance by the Department of Health which is currently under consultations. (Guidance on NHS patients who wish to pay for additional private care—A consultation).

12. The UKCPHN completely supports this particular outcome of the Review in that it has ensured a clear demarcation between private and public healthcare. Our response to the Department of Health's Consultation is therefore supportive.

13. However it needs to be understood that the recommended position does not in fact allow "top-up funding" in the true sense of the word. True top up funding (as talked about before the Review reported) is a form of co-funding in which the patient contributes to part of their healthcare in a NHS setting. Requests from patients to be allowed to do this have been increasing because they can only afford part of the cost of

private care. So for example they will offer to pay for the drug or medical device and ask for the NHS to pay for the associated service costs. Related to this are requests from individuals who have started to fund their care in the private sector and then run out of money and ask the NHS to pick up ongoing costs.

14. If allowed, this model of funding would result in taxpayers subsidising private patients. Professor Richards, while stating it important that the NHS did not subsidise private patients, did not adequately explain the potential risks to the NHS if such a move were allowed.

15. The risks to the NHS are profound and the UKCPHN highlighted the four main ones in their response to Professor Richards' original consultation. These are:

- 15.1 The NHS would lose its credibility. Having stated that a treatment was not sufficiently effective or cost-effective to be funded by the NHS or not affordable, it would then allow NHS outpatient and inpatient capacity to make the treatment available to those who could pay. Doing this would mean that the NHS is not merely be tacitly supporting private practice but facilitating it.
- 15.2 The NHS would have made a policy decision which positively contributes to worsening inequalities through its subsidies to private patients. Subsidisation is inevitable because: patients will not pay the full costs of the additional activity to the NHS, there will be rising medico-legal costs (as a result of accepting payment for experimental treatments), both providers and commissioners will need to invest in additional bureaucracy to administer and monitor this complex system, and finally, the NHS will have to pick up the tab when patients run out of funds. In order to pay for these costs the NHS will have to take money from the rest of the population they serve. It would result in the poor subsidising those who are better off. This is an untenable position for an organisation such as the NHS.
- 15.3 Some NHS services, where capacity cannot be readily increased because of scarce skills, may be put at risk.
- 15.4 The final risk relates to the long term consequence of a two-tier system not being acceptable to the public. The UKCPHN predicted, based on the experience of Medicare, that within a short period of time the public would find co-funding unacceptable and lead to a call for "everything to be made available to everyone". This would remove the inequality of access treatment by ability to pay only for a specific set of treatments (mostly the high profile which industry interests) because in a fixed budget system rationing will merely be displaced to other areas of care. Funding decisions are more likely than ever to be based on the phenomena of "he who shouts loudest" and be biased in favour of services for which there is private sector provision. This is not a basis for reasonable priority setting.

16. The rest of this submission relates to the last risk because it is no longer a potential risk but an actual proposal coming out of the Richards's Review.

PREFERENTIAL TREATMENT FOR END OF LIFE MEDICINES IN THE NHS

17. Whilst the Richards' Review has strengthened the separation between private and public healthcare, Professor Richards has indicated this should not create problems for patients because another recommendation of the Review has been to ensure that the NHS makes more drugs available. Professor Richards has, in short, recommended near unfettered access for cancer drugs which will have to be extended to all treatments for all patients with a poor prognosis. It is difficult to see how, if this policy is implemented, the NHS will be able to avoid funding treatments on demand. It is even more concerning that such a proposal should come at a time of economic difficulty for the country. High cost drug inflation currently runs at about 10%. NHS funding cannot keep pace with this and so the result will be a growing percentage of the NHS budget will be spent on high cost drugs, many of which represent relatively poor value for money.

18. In accepting this proposal the Department of Health has asked the National Institute for Health and Clinical Excellence to rapidly consult on increasing the threshold for end of life treatments which results in the NHS giving preferential treatment to patients that have a poor prognosis and/or an uncommon disease.

19. The UKCPHN opposes this recommended change. The effect will be to give priority largely to treatments that extend life by a matter of weeks or months, as these will be the overwhelming majority of treatments falling under this policy.

20. The proposed change also professes to address the issue of uncommon/rare conditions by setting a prevalence level of 7,000 people per year or less. However this figure will probably include most treatments as drugs are often licensed for sub-group of patients with common cancers. Patient numbers for at least two of the major health technology appraisals for cancer of the colon are 7,000 or less.

21. Even if this policy did only apply to uncommon disorders the policy should be opposed. PCTs, the UKCPHN and many health economists consider that such an approach, however attractive on the face of it, would be unsustainable because it would eventually lead to a breakdown in any notion of a threshold, thereby making the application of value for money potentially redundant within the NHS.

22. Few individuals outside primary care organisations fully appreciate the scale of priority setting that takes place. For the 2004–05 commissioning round, one PCT had to prioritise a list of cost pressures and service developments amounting to £46 million across the full range of services down to the £5 million of new resources available. On this list were £8 million of mandatory NICE treatments (mostly drugs) and GP prescribed drugs (which are also mandatory). With the requirements to fund more and more drugs, the higher than inflation costs of many drugs and a worsening economic situation globally, Professor Richards’ recommendations will distort priorities even further towards pharmaceutical products and interventions of marginal benefit.

23. In considering cancer alone, such a policy seems irrational. The poorer cancer related health outcomes experienced by the UK are not merely a result of funding cancer drugs but are related to a complex range of issues including health behaviour, compliance, stage at presentation and the availability of radiotherapy. Furthermore it is unlikely that increasing spending on treatments for patients with metastatic or particularly aggressive disease are likely to improve five year survival rates but might comprise the NHS’s ability to deliver other aspects of care and meet the challenges of an ageing population and increase in chronic disease and disability across the age groups.

24. In relation to a the NICE threshold it should be appreciated that:

24.1 The current NICE indicative threshold of £20,000–£30,000 per Quality Adjusted Life Year (QALY) is not based on the UK’s ability to fund all services and treatments falling under that level. Many experts in the field of health economics are of the view that the threshold of affordability should be in the region £12,000–£15,000. Because of the way in which NICE is structure there is already a preferential bias in favour service developments which are looked at by NICE.

24.2 The threshold should relate to the overall resources of the NHS. As the financial settlement of the NHS either relatively increases or decreases the QALY threshold should also be changed.

24.3 “Willingness to pay” does not relate to what Society can afford. There is insufficient evidence on public perception on the appropriate balance of priorities for healthcare funding. Without the wider context it would be misleading to suggest that the public supports this particular proposal. Put another way are we sure that the public fully understands the implications of such a change and particularly are they prepared to accept cuts or failure to fund particular other services as a result of this policy change? Our experience is that the decisions people make in hypothetical settings are different to those when tested with actual examples of priority setting in which they are placed in the same position as PCTs.

24.4 In a system with a fixed budget if some patients groups are given preferential treatment (by raising the QALY limit) then it goes without saying that the QALY limit for other patient groups should be lowered. NICE is only consulting on a higher QALY limit for those with terminal illnesses but it is not consulting on where the limit will be lowered. It is right that Society’s values need to be embedded into NHS decision making but this cannot be done in a piecemeal way. If politicians and the public wish resource allocators to give preferential treatment to certain patients then they will also need to say which services and treatments should be reduced or not provided at all. Society cannot keep pretending that it can afford everything. The NHS needs political leadership on this issue as a matter of urgency.

25. The UKCPHN therefore argues that the proposed supplementary advice to NICE’s Appraisal Committees is misguided, unnecessary and will have serious and detrimental effects for patients elsewhere in the NHS.

26. The proposal itself is likely to be inflationary and encourage pharmaceutical companies to focus on researching drugs with modest improvements in survival and pricing these at a level that will fit within the proposed new guidance. We know this phenomenon already occurs at the lower NICE threshold levels.

27. In our view, choices concerning end of life care needs greater consideration. Recent audits, reports and academic papers about the use of interventions in patients with a poor prognosis emphasises there should be cause for concern about how many patients are exposed to unpleasant treatments without a sound understanding of what the benefits of treatment actually are. PCTs have long been concerned about the communication of prognosis and proper informed consent in treatment for those with cancer of poor prognosis.

RECOMMENDATIONS

28. That the Department of Health consultation document: Guidance on NHS patients who wish to pay for additional private care is supported. This is the fairest option for our Society.

29. That the Health Select Committee asks for more careful consideration of the proposals to alter the NICE threshold for patients with a poor prognosis. The effect of implementing this would result in unfettered access to treatments of any kind, and of whatever value, for patients with poor prognosis and restrict access to other patient groups. In our view it is not possible to increase priority for one group without lowering priority for other groups and if this change is wanted then NICE’s threshold for all other patient

groups should be reduced below £20,000. The full effects of this policy change were neither considered nor properly costed by Professor Richards. More work is needed before a properly informed decision can be made on such a significant policy change.

December 2008

Memorandum by Cam Donaldson (TF 09)

TOP-UPS FOR CANCER DRUGS

Fiscal pressures are prevalent throughout the world's health care systems. Under such strain, it is natural to opt for "solutions" which appear to make intuitive sense. The latest innovation in this regard is top-ups for expensive cancer therapies here in the UK. Funny how politicians always look for alternative words for what are essentially "user charges". A popular one, emanating from insurance circles is "copayments", and another warm and fuzzy one is "diversifying the revenue stream". But they are all different forms of user charge. The question then is whether such charges are a solution to the problem at hand. On this, I think it is fair to say that this is one area of policy on which there is evidence and virtually unanimous agreement amongst the experts. User charges are an idea which is intellectually dead, but keeps coming back to threaten our publicly-funded health care systems, and have thus been classed as a policy zombie.³⁰

On the face of it, it seems very powerful to say "Why not let people spend their hard-earned money in whatever way they like? Surely this relieves the public system of a burden rather than placing one upon it?" Neither of these is true, unfortunately. In the case of top-ups, people will still have to have care in hospitals, provided by nurses and physicians. The issue, then, of whether it is worth providing such (often unproven) treatments relative to how else our public monies could be spent, will always arise. If the drugs are indeed effective to some degree, and if people can self-prescribe, stump up the cash for the drugs and get them off the Net, self-administer and self-monitor as to how the drug has worked, then it could be argued that top-ups might have little impact on publicly-funded health care. All these things take publicly-funded resources (mainly carers' time, space in hospitals etc) which may be diverted from someone else because a person was able to come up with the cash. Therefore, health care commissioners and national bodies, such as NICE, will have to engage. Top-ups will not have made the problem go away, but will actually very likely complicate it, as those who are willing and able to pay gain greater access whilst consuming public resources.

Some may say that a move to top-ups simply reflects the more mixed public-private funding arrangements of continental Europe, often seen as better than what exists in the UK. However, despite many recent references to such countries as success stories, they tend to have more problems with cost control than we have in the UK precisely because of these mixed funding regimes. Given the recent states of their economies, they are also struggling to maintain these systems.

More general issues are raised beyond cancer. Once such a policy is introduced for one major disease where will it stop? One can see it coming in for equally-unproven dementia drugs, for example. The evidence on user charges would then say that we will be in a situation of escalating costs as well as threats to equity in health care delivery.

Costs would escalate because the proposal for user charges suffers from a fallacy of composition; that is, assuming what happens at the level of the individual will happen at the level of a whole system, in this case the health care system. There is no doubt (and studies have shown this) that some users will respond to charges by reducing demands on the health care system. But costs will not be saved. Why? Because physicians, faced with reduced demand by one group, will simply respond by providing more services to those who present. Combine that with the fact that those who choose not to present are likely to be poorer and more in need of care, and we end up with a system incurring the same costs and meeting less need than before.

One only has to look to the USA to see this. The USA makes the most widespread use of user charges in its health care system, and yet it faces the most difficulties with control of health care costs of any country in the world. Studies there have shown that when demand on the system is reduced it is indeed poorer people whose demands go down. And they suffer. Demand for needed care is reduced just as much as demand for unneeded care. So, user charges are a blunt instrument. Not only that, if reduced demand for needed care develops into a more serious problem later on, that episode will end up costing the system more.³¹

User charge proponents always say that we can declare some people eligible for exemption from user charges, and so reduce the unfairness. But why create a whole new (and costly) bureaucracy for this? Also, with those who are exempt from user fees being able to access care as before and those who are not exempt also being able to access care, how is this going to reduce pressure on the system? There is no doubt there are some things currently available in health care that we should be asking patients to provide for

³⁰ Evans RG, Barer ML, Stoddart GL, Bhatia V. *Who Are the Zombie Masters, and What Do They Want?* Centre for Health Services and Policy Research, University of British Columbia, December 1993.

³¹ Tamblyn R, Laprise R, Hanley JA, Abrahamowicz M, Scott S, Mayo N, Hurley J, Grad R, Latimer E, Perrault R, McLeod P, Huang A, Larochelle P, Mallet L. *Adverse effects associated with prescription drug cost-sharing among poor and elderly persons.* *Journal of the American Medical Association* 2001; 285: 2328-2329.

themselves. For example, hospitals are not hotels, and some optional, non- health related services could be scaled back, charged for or brought by patients themselves. However, as a general policy, user charges would be disastrous. They would not reduce demand but they would compromise the equity objectives of our health care system. In countries with progressive taxation systems, and given the association between income and health, all that would happen would be a transfer of income from the poor and sick to the rich and healthy.

If we want to control health care costs and at the same time reduce the moral dilemmas that budget-busting drugs pose for society, we should remember that most decisions in health care are taken by providers. Governments have to take courage and target the supply side of this complex market. The keys are (1) to sort out inefficiencies in the NHS, which allow for more (useful) care to be provided and (2) to recognise, as a society, that the quid pro quo for having a publicly-funded system is that we cannot provide everything (which politicians do not seem able to admit publicly) and so we need an open and honest debate, as a society, about what the criteria for provision should be. This still keeps things within public funding without (initial) resort to higher taxation. However, if we decide more funding is indeed required, then why not through increased taxation?

It is wrong, unfair and ineffective to try and limit consumer and patient access through user fees, and also to dress it up as actually enhancing access. Instead let us continue to find effective ways to involve clients and the public more generally in health system decision-making and in making good choices about health and health care. If we want to limit access to supply-led services, let us talk to the health care professionals who manage and provide health care. If we want a financing mechanism consistent with what we want to achieve in health care, there is another way; taxation.

Despite my personal opinions, as an economist, I am comfortable with individuals having the right to spend their own money on top-ups as long as the proponents of such policies come clean and admit that we are now changing the resource allocation system within the NHS towards one based, at least in part, on willingness and ability to pay, and that the politicians who introduce any such scheme seek societal approval for it at the ballot box.

Cam Donaldson

Director, Institute of Health and Society
Health Foundation Chair in Health Economics
National Institute for Health Research Senior Investigator
Newcastle University

Research Professor
University of Calgary

December 2008

Memorandum by the Royal Pharmaceutical Society of Great Britain (TF 10)

TOP-UP FEES

We welcome the opportunity to make a submission to the Select Committee's Inquiry on top-up fees. We have focussed primarily on pharmacy practice in England in line with the remit of the Inquiry.

BACKGROUND

The Royal Pharmaceutical Society of Great Britain (RPSGB) is the professional and regulatory body for pharmacists in England, Scotland and Wales. It also regulates pharmacy technicians on a voluntary basis, which is expected to become statutory under anticipated legislation.

The primary objectives of the RPSGB are to lead, regulate, develop and represent the profession of pharmacy.

The RPSGB leads and supports the development of the profession within the context of the public benefit. This includes the advancement of science, practice, education and knowledge in pharmacy. In addition, it promotes the profession's policies and views to a range of external stakeholders in a number of different forums.

Following the publication in 2007 of the Government White Paper *Trust, Assurance and Safety—The Regulation of Health Professionals in the 21st Century*, the Society is working towards the demerger of its regulatory and professional roles. This will see the establishment of a new General Pharmaceutical Council and a new professional body for pharmacy in 2010.

RPSGB POLICY ON TOP-UPS

On 6 November 2008, the RPSGB Council agreed the following policy in relation to top-ups:

1. The need for top-ups should be minimised as far as possible: the focus should be on improving access to effective medicines for NHS patients. Top-ups should therefore be the exception, not the rule.
2. A number of measures could help improve access to medicines for NHS patients, including:
 - speeding up NICE appraisals; however there are risks associated with rapid assessments, especially where there are insufficient published data to base the assessment on;
 - reforms to NICE procedures to improve the availability of drugs used near the end of life that do not meet current cost effectiveness criteria;
 - risk and cost-sharing schemes with the pharmaceutical industry;
 - value or performance based pricing agreements with industry (The above two measures could be included in the new PPRS. RPSGB is collecting innovative examples including cost and risk sharing schemes);
 - Phase IV clinical trials of new drugs in use, which will generate the data NICE needs to make quicker, but still robust, decisions on cost-effectiveness; and
 - improving the prediction of who will benefit from expensive new medicines through patient stratification and the development of bio-markers—the RPSGB is commissioning work in this area.
3. Local decision making processes on funding exceptional prescribing including off label drugs,³² need to be improved to provide prompt, consistent, robust and transparent decisions.
4. There will still be circumstances in which medicines are not available under the NHS so there will be a need to provide unbiased, accessible information on these medicines for any patients who might be considering whether to access them privately. Measures to protect the public from misleading claims are also needed.
5. Pharmacists will play a key role in advising patients and helping them access trustworthy sources of information in these situations.

We hope our submission will be of use to the Inquiry and look forward to seeing the final report.

The RPSGB would be happy to give oral evidence at the Committee's hearings in the New Year.

Steve Churton
President

December 2008

Memorandum by UNISON (TF 11)

TOP-UP FEES

EXECUTIVE SUMMARY

UNISON welcomes a number of recommendations contained within Professor Mike Richards' recent report, as well as NICE's plans to allow more drugs to be funded by the NHS, and news that the Department of Health has taken steps to bring down the cost charged by pharmaceutical companies for drugs. Unfortunately, however, more significant than all of these moves are the recommendations within the Richards report to permit the use of top-ups for those that can afford to pay for treatment over and above their NHS entitlement. This policy *volte-face* threatens to undermine the founding principles of the NHS and creates problems where practical implementation is concerned. A preferable approach would have been to give the other changes recommended by the review a chance to have an impact before considering anything more wide-ranging.

INTRODUCTION

1. UNISON is the major trade union in the health service and the largest public sector union in the UK. We represent more than 450,000 healthcare staff employed in the NHS, and by private contractors, the voluntary sector and general practitioners. There is also a wider interest in the NHS among our total membership of more than 1.3 million people who use, or have family members who use, health services.

³² Off label prescribing is where a medicine is used for a condition or in a manner that it has not been licensed for. This is common practice in some specialities such as paediatrics where few medicines are licensed for use in children.

2. UNISON's members are in the caring business—whether as nurses, occupational therapists, paramedics, or others supplying public services—and spend time with patients and service users, a small number of whom find themselves stranded by the current system, such as the high-profile recent case of Linda O'Boyle. In 2006 UNISON successfully sought a judicial review on behalf of Elisabeth Cooke, a UNISON member suffering from breast cancer who had been denied Herceptin by Bristol primary care trust.

3. UNISON welcomes the chance to respond to this review and has been one of the most vocal commentators on the whole top-ups debate throughout the second half of 2008, including providing evidence to Mike Richards' review and taking part in one of the citizens' juries on this subject, organised by Breakthrough Breast Cancer. Given the remit and word limit of this inquiry, we do not intend to simply repeat our evidence to the Richards review. Rather we will focus instead on the potential fall-out from some of the decisions taken in this area of health policy.

SOME POSITIVES

4. As a result of the Richards review a number of positive steps have been taken. Within the main Improving access to medicines for NHS patients report the vast majority of recommendations are sensible and represent a progressive response to the vexed question of top-up payments. Examples of this include a number of recommendations that UNISON had called for: re-emphasising the aspects of Lord Darzi's *High quality care for all* report to improve the timeliness of the NICE decision making process and the clarification of patient rights enshrined in the draft NHS Constitution;³³ and ensuring cooperation and transparency in the way PCTs work as a means of trying to establish a greater consistency of approach.

5. The report called on the Department of Health to work with NICE to assess affordable measures to make available drugs used near the end of life that do not meet existing cost-effectiveness criteria. In response to this, NICE's own proposals to consider drugs that cost in excess of its threshold of £30,000 per quality-adjusted life year should ensure that there will be less demand from patients at the end of life to top-up their NHS entitlement.

6. The report also called on the Department to work with the pharmaceutical industry to promote more flexible approaches to the pricing and availability of new drugs. UNISON is glad to see that already action has been taken in this area with a new deal on drug pricing announced in mid-November that will mean a 3.9% cut in the cost of drugs sold to the NHS from February 2009 and a further reduction the following year. It is also good to see that companies would only be paid higher prices once the value of their product has been proven with patients. Short of pharmaceuticals being part of the publicly funded health system, this represents a realistic response to limiting the excesses of drug-pricing. UNISON would, however, call upon the Department to monitor this relationship with the pharmaceutical companies very carefully and apply greater pressure on the industry to further reduce the costs of drugs in the future. After all, in the immediate aftermath of the top-up decision, the Association of British Pharmaceutical Industry revealingly claimed that "growth in the medicines bill can largely be paid for by saving in the medicines bill post-patient"³⁴—the implication being that with the Department apparently saving £2.9 billion on the drugs bill over the next four years, there should be plenty left to spend on their new drugs.

TOWARDS A TWO-TIER HEALTH SYSTEM

7. Unfortunately, however, these positive steps have been dwarfed by the potentially hugely damaging decision taken by the government to accept the recommendations of the Richards review to permit patients to top-up their healthcare (with the caveat that any additional private drugs received must be delivered separately from the NHS elements of their care). UNISON has serious reservations about how this will be done in practice and what the implications for staff will be.

8. The decision changes fundamentally the nature and ethos of the NHS in which treatment is available on the basis of need, not ability to pay. Opening the NHS up to a system of co-payments—however marginally—runs the risk of core NHS provision being seen merely as something that people who cannot afford to pay extra choose to take up. Over time, there is a real danger that NHS services will come to be seen as a basic "Cinderella service" with the more elaborate or expensive treatments needing to be paid for by top-ups. Issues around the access to and take-up of UK dentistry demonstrate the problems that can be created when you move to a two-tier approach to paying for aspects of healthcare. Unfortunately the UK's record on tackling health inequalities still leaves much to be desired, and essentially permitting top-up payments transfers health resources towards the rich and away from those who cannot afford to pay.

9. There is nothing straightforward about allocating life-extending drugs. The previous system did unfortunately provide some heartbreaking cases, such as the oft-cited Linda O'Boyle example, which was why UNISON argued that the status quo was not sustainable as it was. But now it is likely that equally appalling headlines will start to appear in the popular press. Healthcare staff will find themselves in the

³³ Department of Health, *High quality care for all: NHS next stage review* final report, June 2008, p 44; Department of Health, *The National Health Service Constitution: a draft for consultation*, July 2008, pp 2–3.

³⁴ ABPI quoted in *Tribune*, "These top-up plans have a huge downside for NHS fairness", Jill Palmer, Thursday, 6 November 2008.

invidious and demoralising position of potentially giving different treatments to two patients with the same illness within the same hospital—even if not within the same ward—based on who can pay and who cannot. Equally, even where a patient is able to afford a drug privately, staff would still have to refuse that patient treatment if the patient wanted to receive it on NHS premises.

10. An ample demonstration of the potentially far-reaching effects of this decision comes from insurance companies, whom the BBC reports are already eyeing up a lucrative new market, just as UNISON warned in our submission to the Richards review. Charlie MacEwan, corporate communications director from one of the companies, WPA, said: “the potential for this market is phenomenal and could dwarf private medical insurance.”³⁵ Particularly revealing is the following quote from Dr Natalie-Jane Macdonald, director of Bupa UK Health Insurance:

“So far this has largely been a debate over principle, but I think the important thing is that this is the first time that it has come to public attention that the NHS can’t always be there for you.”³⁶

11. UNISON argued throughout the top-ups debate that the Richards review should have been merely the start of an ongoing process and part of reframing the debate beyond the often ill-informed media furore around this controversial topic. Instead the government has arrived at its decision on the basis of an informal four-month review prompted by a series of emotive headlines and one-sided media coverage. As it stands, NICE only rejects a tiny percentage of all the drugs it assesses for use on the NHS (around 5%) and since 1999 has recommended over 90% of the cancer drugs it has been asked to look at.³⁷ With the new measures the number of rejections will drop even further. So a preferable approach would have been to give the other changes recommended by the review a chance to have an impact on the system before considering whether anything more wide-ranging would be needed in the future.

OTHER ISSUES

12. There are a number of practical considerations that the decision to allow top-ups has created and which need to be carefully considered in guidance provided by the Department to PCTs and hospitals. For example, it has already been reported that PCTs could find themselves coming under pressure to refund thousands of pounds to patients who have paid for top-up treatment.³⁸ The new system could provide an extra burden on PCTs with patients potentially claiming back the money they had to pay for the rest of their NHS treatment when they had bought other drugs privately—something which the new system would now not oblige them to pay for. There are also question marks about exactly how hospitals will be able to separate physically the private elements of care from the public elements to ensure they do not breach the government’s new guidelines where patients do choose to top-up. Related to this is the issue about foundation trusts and the private patient income cap. The Department should resist any pressure from foundation trusts (or Monitor) to raise or remove the cap due to difficulties in separating patients paying for private top-ups from the rest of the NHS care they provide.

13. UNISON hopes that the Committee’s inquiry will also consider the influence of the pharmaceutical industry on the top-ups debate, as well as other pressures on the NHS where topping-up care is concerned. In the near future this is likely to come from the development of personal health budgets and particularly the piloting of direct payments for patients with long-term conditions: for example, what happens if a patient exhausts the money they have been allocated through a direct payment? And the current European Commission proposal for a Directive on cross-border healthcare could also become an issue, with patients having to pay extra for travel, accommodation and after-care when they chose to access treatment abroad.³⁹

UNISON Policy Unit

December 2008

Memorandum by the Association of Directors of Public Health (TF 12)

TOP-UP FEES

The Association of Directors of Public Health (ADPH) is the representative body for directors of public health (DPH) in the UK. It seeks to improve and protect the health of the population through DPH development, sharing good practice, and policy and advocacy programmes. www.adph.org.uk

³⁵ BBC News online, “Insurance firms eye top-up market”, Nick Triggle, 7 November 2008.

³⁶ Health Investor online, *Bupa eyes NHS top-up market*, 7 November 2008.

³⁷ Health Service Journal online, “NICE chairman hits back at critics”, Sir Michael Rawlins, 18 August 2008.

³⁸ Health Service Journal, “PCTs may face bill for top-up refunds”.

³⁹ See UNISON’s written evidence to the House of Lords European Union Committee, Social and Consumer Affairs (Sub-Committee G) inquiry: <http://www.parliament.uk/documents/upload/UNISON%20Sept%2008.doc>

EXECUTIVE SUMMARY

The ADPH welcomes this inquiry, and the opportunity it affords to explore in detail the implications of new guidance emanating from the Review *Improving access to medicines for NHS patients*.

Directors of Public Health are often on the front line in trying to justify difficult decisions around expensive drugs. We are therefore pleased that a national solution is being sought to alleviate these local problems.

However, the ADPH identifies a number of concerns arising from the enabling of top-up funding within the NHS, specifically:

1. Potential undermining of the core principles of the NHS and introduction of a two-tier national health service;
2. Risk of increased inequality and inequities, and the widening of the health inequalities gap;
3. Complexities of implementation and the additional NHS resources that will need to be given over to managing these complexities;
4. Concerns over marginal health gain and clinical effectiveness of treatments purchased.

DETAILED RESPONSE

1. *Core principles of the NHS*

- (a) the enabling of patients to purchase additional treatments has potential to undermine the core principles of the NHS, that access to care should be determined by need and that care should be free at the point of delivery.
- (b) This risks the introduction of a two-tier NHS, creating inequities within the NHS and working against the reduction/removal of health inequalities.
- (c) Individual Case Review Processes are in place within the NHS which, if strengthened in those areas where they are not working well, would help to alleviate many of the existing concerns about access to drugs.

2. *Issues of equity*

- (a) The Department of Health is working to resolve inequities that already exist within the NHS—the enabling of top-up fees will cause access to some treatments to be determined by patients' ability to pay, thereby increasing inequities within the health service.
- (b) This will be exacerbated when patients who have opted to purchase additional treatments are no longer able to fund those treatments. NHS commissioners locally are likely to come under significant pressure to support continued treatment in these instances.
- (c) In light of the above concerns, we believe that this policy should be the subject of an Equity Impact Assessment.

3. *Implementation*

- (a) Whilst providing a permissive framework and approach, the complexities of dealing with individual care are not addressed within the Review.
- (b) We are concerned that the principles of separateness are not clearly defined nor sufficiently worked through, particularly in the case of in-patients.
- (c) Also, clarification is needed in relation to non-drug treatments.
- (d) There are concerns as to the likely additional NHS clinical (and administrative) time and resources that will need to be given over to managing individual cases.

4. *Clinical effectiveness and health gain*

- (a) We welcome measures to improve the timeliness of the NICE decision making process as this has been a cause of much of the public's concerns.
- (b) There are questions over the likely health gain for patients which are likely to accrue from increased access to marginally effective drugs and treatments—individuals and families may place themselves in positions of financial hardship to fund treatments which are of limited/marginal effectiveness.
- (c) We are concerned that clinicians may be pressured into providing treatment they feel is not able to provide reasonable health gain.
- (d) Patients should have access to impartial clinical advice—where will this advice come from?

- (e) We have some concerns over the emphasis in the Review on those patients near the end of life. Whilst recognising the importance of this, we should have a concern for the quality of life for all patients, whatever their age and stage of life.
- (f) Whilst Professor Richards rejects the likelihood of the Review leading towards an insurance-based system, we do have concerns that there will be a shift in this direction, along with a consequential risk of increased prices for drugs.

Dr Frank Atherton
President

December 2008

Memorandum by the National Institute for Health And Clinical Excellence (Nice) (TF 13)

TOP-UP FEES

1. EXECUTIVE SUMMARY

The Institute understands current concerns about the availability of costly drugs in the NHS. We are taking steps to improve the timeliness of NICE guidance on new drugs by 2010; and we expect to announce, in January 2009, the results of our consultation on end of life treatments. If agreed, this arrangement would explicitly permit a more flexible approach to be taken in advising on the use of drugs for patients with rarer conditions who have a limited life-span. We are also piloting ways of providing information that will help patients make decisions on whether they should purchase additional treatments unavailable under the NHS. The Institute is optimistic that the arrangements, outlined in the 2009 PPRS for flexible pricing of new pharmaceutical products, will help ensure all patients who have a limited life-span benefit from innovation arising from pharmaceutical research and development.

2. ABOUT NICE

NICE is the independent organisation responsible for providing national guidance on the promotion of good health and the prevention and treatment of ill health. NICE produces guidance in three areas of health:

- public health—guidance on the promotion of good health and the prevention of ill health for those working in the NHS, local authorities and the wider public and voluntary sector;
- health technologies—guidance on the use of new and existing medicines and devices as well as diagnostic and therapeutic procedures within the NHS; and
- clinical practice—guidance on the appropriate treatment and care of people with specific diseases and conditions within the NHS.

NICE guidance thus helps to improve the public's health and makes access to healthcare more equitable across the country.

3. BACKGROUND: THE DEBATE ABOUT TOP-UP FEES AND THE RICHARDS REVIEW

Over the past two years there has been considerable public, professional and parliamentary concern about the position of patients who choose to pay, privately, for products that are not funded by the NHS. In particular, anxieties were expressed as to whether, in such circumstances, it was appropriate for patients to be denied their entitlement to other modalities of NHS care that they would otherwise have received. The government responded to these concerns by asking Professor Mike Richards to examine if, when and in what circumstances patients should be able to purchase additional drugs that are not funded by the NHS. Professor Richards' review analysed the factors that were putting pressure on current arrangements for making drugs available, including the part played by NICE.

The report of the review notes that most drugs are available on the NHS to all patients whose clinician thinks they could benefit. It also commends NICE's work and states that, to date, NICE has either fully or partly recommended a large proportion of the drugs it has appraised for use in the NHS. An analysis of technology appraisals of cancer drugs published between January 2004 and November 2008 bears this out:

- NICE has fully approved 13 cancer drugs for the licensed indications examined.
- NICE has approved 14 drugs for use with particular sub-groups within the examined licensed indications.
- Only in five instances has NICE recommended that drugs should not be used for particular indications—in all cases because of lack of evidence of effectiveness compared with current NHS best practice. (These drugs were all, however, approved for other indications.)

- NICE has recommended that one drug for a particular indication should be used only in the context of research.
- Four appraisals were terminated either because the manufacturer failed to provide evidence or provided inadequate evidence.

However, the report predicts that, in the future, it may become more challenging to demonstrate cost-effectiveness according to the criteria currently used, particularly in the case of drugs used near the end of life.

4. THE DEMAND FOR UNFUNDED DRUGS IN THE NHS

To estimate and analyse the demand within the NHS for unfunded drugs the report examined evidence on exceptional case requests to PCTs. It concluded that there were three categories of unfunded drug:

- Drugs on which NICE has yet to issue final guidance. The review regarded these as forming the largest category of requests. (A Department of Health survey for the review found that 43% of PCTs which responded had funded drugs of this kind.)
- Drugs that NICE will not appraise or that a clinician wished to use “off label”. (The review found there were “numerous requests” for such drugs.)
- Drugs that NICE has declined to recommend for use in the NHS. The review found that requests for these appeared to be uncommon. (13% of PCTs responding to the survey had funded drugs in this category.)

The report makes recommendations both on specific arrangements for enabling people to pay top-up fees while remaining as NHS patients for the rest of their treatment, and on measures that would alleviate or remove some of the current obstacles to the availability of drugs on the NHS. Two recommendations of the latter variety directly affect the Institute. Recommendation 1 strongly supports “the measures the government is already taking to improve the timeliness of the NICE decision making process”. Recommendation 5 urges the Department of Health and NICE to assess “what affordable measures could be taken to make available drugs used near the end of life that do not meet the cost-effectiveness criteria currently applied to all drugs”. Recommendation 5 also urges the Department of Health and pharmaceutical industry “to promote more flexible approaches to the pricing and availability of new drugs”.

In addition, the report also recommends (recommendation 13) that the Department of Health should consider how patients could be given access to information about the benefits, toxicities and, where appropriate, costs of novel treatments; and proposes that NICE, working with professional and patient groups, could develop high quality information of this kind.

5. NICE’S ROLE AND CONTRIBUTION

The Institute accepts the report’s analysis of the factors giving rise to NHS patients’ desire to have access to products for which no NICE guidance has been published. The Institute has also consulted on a proposal to supplement the advice it gives to its Appraisal Committees (the advisory bodies responsible for NICE technology appraisals) to facilitate access to end of life medicines. As a possible further contribution, we are investigating ways of providing patients with information to support informed decision-making about the purchase of additional medicines.

5.1 *Timeliness of NICE recommendations*

We have previously acknowledged to the Committee that for various reasons, not all within the Institute’s control, our guidance has sometimes failed to be available as quickly as patients or the NHS would have wished. However, we have been able to make progress on the topics already referred to us: during 2009, we expect to halve the time taken from market authorisation to publication of guidance on these topics to around 14 months.

Since our response in April 2008 to the Committee’s First Report of Session 2007–08, we have taken further action on our commitment to publish appraisals of new drugs as close as possible to their launch in the UK market and ideally within three to six months of becoming available for general use. A new topic selection process for anti-cancer drug appraisals has been introduced which will involve most being referred to NICE. Ministers have agreed to refer anti-cancer drugs every two months (rather than the current quarterly referral arrangement). In addition, under the 2009 PPRS (Pharmaceutical Price Regulation Scheme), the Department and the ABPI will jointly establish a single unified horizon scanning process to

enable the Institute to plan its work programmes. The key impact of these changes is that work on appraisals can now begin more than a year before marketing authorisation of the drug. Consequently, by 2010, draft or final NICE guidance for all new cancer drugs will be available within an average of six months of marketing.

5.2 *Appraisal of drugs for end of life*

In November 2008 the Institute consulted on a proposal to supplement the advice it gives to its Appraisal Committees to enable access to medicines licensed for terminal illnesses affecting small numbers of patients. In such circumstances the Appraisal Committees would (subject to the various provisos described below) be asked to consider recommending the use of medicines for use in the NHS even though the incremental cost effectiveness ratio is in excess of £30,000 per quality adjusted life year (QALY).

The Institute has always expected its advisory bodies to take account of circumstances in which a treatment is being offered and the nature of what it offers to those who might benefit from it, and they have recognised, in the recommendations they have made, the importance of life to those with terminal illnesses. In considering this supplementary advice, we have taken account of the approach advisory bodies have used in the past, the principles in our guide to the use of Social Value Judgements,⁴⁰ and the views of our Citizens Council on the “rule of rescue”, rare diseases and severity,⁴¹ as well as research findings.⁴²

The criteria we consulted on during November and December 2008 for using this supplementary advice are as follows:

- *Rarity*: The medicine is indicated, in its license, for patient populations not normally exceeding 7000 new patients per annum; and
- *Severity*: The medicine is indicated for the treatment of patients with a diagnosis of a terminal illness and who are not, on average, expected to live for more than 24 months; and
- *Innovation*: No alternative treatment with comparable benefits should be available through the NHS.
- *Cost-effectiveness*: The medicine must have been assessed by NICE as having an incremental cost effectiveness ratio in excess of the upper end of the range (£30,000) normally considered by Appraisal Committees to represent a cost effective use of NHS resources.
- *Available evidence*: There is sufficient evidence to indicate that it offers a substantial average extension to life compared to current treatment.

Medicines recommended for use on the basis of the criteria set out in these proposals would be subject to an appropriately designed programme of evidence development to ensure that the anticipated survival gains are evident when used in routine practice.

The period of consultation on the detail of these proposals closed on 10 December. The Institute anticipates that there will be clarification of a number of definitions used in the consultation document. We expect to apply the revised supplementary advice at the next scheduled Appraisal Committee meetings in January 2009. The Institute would be keen to supplement this current submission with the finalised document at that time.

5.3 *Developing high quality information*

As part of the Professor Richards’ review the Institute was asked to explore the possibility of producing information to help patients make informed choices about paying for treatments not recommended by NICE. We have begun to test out a standard format for explaining both the top-up treatment and the treatment normally available on the NHS, and the cost implications for the patient. The information would be drawn in part from appraisal documents, complemented by information from other sources, but

⁴⁰ NICE (2008) *Social value judgements: principles for development of NICE guidance*. Second edition. www.nice.org.uk/aboutnice/howwework/socialvaluejudgements/socialvaluejudgements.jsp

⁴¹ NICE Citizens Council Report: rule of rescue, June 2006. http://www.nice.org.uk/getinvolved/patientandpublicinvolvement/opportunitiestogetinvolved/citizenscouncil/reports/citizens_council_report_rule_of_rescue.jsp
NICE Citizens Council Report: ultra orphan drugs, February 2005.

http://www.nice.org.uk/getinvolved/patientandpublicinvolvement/opportunitiestogetinvolved/citizenscouncil/reports/citizens_council_report_ultra_orphan_drugs.jsp
Report on NICE Citizens Council meeting: Quality Adjusted Life Years (QALYs) and the severity of illness, July 2008. www.nice.org.uk/getinvolved/patientandpublicinvolvement/opportunitiestogetinvolved/citizenscouncil/reports/reports.jsp?domeid=1&mid=2C2EE003-19B9-E0B5-D4C782EABB26BFF7

⁴² Dolan P *et al* (2008) *The relative societal value of health gains to different beneficiaries*. University of Birmingham. http://www.pcpoh.bham.ac.uk/publichealth/methodology/projects/RM03_JH11_PD.shtml

Baker R *et al* (2008) *Weighting and valuing quality adjusted life years: preliminary results from the Social Value of a QALY Project*. University of Birmingham.

http://www.pcpoh.bham.ac.uk/publichealth/methodology/projects/RM03_JH12_CD.shtml
Mason H *et al* (2008) *Estimating a monetary value of a QALY from existing UK values of prevented fatalities and serious injuries*. University of Birmingham.

www.pcpoh.bham.ac.uk/publichealth/methodology/projects/RM03_JH13_CD.shtml

Mason H, Jones-Lee M and Donaldson C (2008) *Modelling the monetary value of a QALY: a new approach based on UK data*. Health Econ (2008) DOI: 10.1002/hec.

<http://www3.interscience.wiley.com/cgi-bin/fulltext/121452241/PDFSTART>

presented in a version suitable for a patient and public audience. This pilot stage has raised questions about the level of detail desired by patients, how much information about standard treatment should be provided, the best way of assessing the cost of the top-up treatment, and whether the information should also be aimed at professionals so as to support essential discussions with patients.

December 2008

Memorandum by the Royal College Of Psychiatrists (TF 14)

TOP-UP FEES

The Royal College of Psychiatrists is the leading medical authority on mental health in the United Kingdom and is the professional and educational organisation for doctors specialising in psychiatry.

We are pleased to respond to this consultation. Please note that the response was completed by the Psychotherapy faculty.

RESPONSE

Although top-up fees have received most attention in the context of NHS patients' wishes to obtain pharmaceutical treatments when their availability has varied according to where a patient lived, we believe the review needs to take into account patients access to and use of other kinds of intervention. The relevance of this is twofold.

First, with respect to the important issues of principle raised by top-up fees, interventions such as psychological therapies provide a clear example of treatments that patients have been able to procure in the private sector for many years when they could afford them, without any risk to their ongoing entitlement to NHS treatment for the same condition. At a time when these treatments are finally being discussed with the seriousness they deserve (including recent commitments to ensure all NICE guidance is resourced), it might be anomalous to adopt different principles for access to additional physical as opposed to psychological treatment procedures.

Second, the provision of psychological treatments differs from that of physical treatments in that current evidence-based guidelines are always based on a smaller body of evidence concerning the outcomes of a treatment for a specific condition, and economic analyses are less well developed than for physical interventions. Moreover, attempting to base clinical decisions on best evidence is a more complex process as other patient factors have to be taken into account. Factors known to influence the effectiveness of a psychological therapy at the level of the individual patient even include a patient's own preference for the treatment concerned. Unless the range of psychological treatments that was routinely available to all patients was relatively large (and in excess of the limited lists produced quite properly through NICE's procedures) it could be particularly difficult to justify curtailment of patients' entitlement to NHS services because they sought a psychological treatment that was likely to be effective in their individual circumstances from a private provider.

This Consultation was prepared by the Policy Administrator, Claire Churchill and approved by the Registrar, Professor Sue Bailey, and Associate Registrar, Dr Ola Junaid.

December 2008

Memorandum by the Social Market Foundation (TF 15)

TOP-UP FEES

EXECUTIVE SUMMARY

- The Social Market Foundation (SMF) is an independent, non-partisan think tank;
- SMF welcomes the decision not to remove the entitlement to NHS care if a patient purchases additional private services;
- The Richards review essentially blurs the line between public and private provision, and we need a new definition of equity to deal with this change;
- The NHS should be more open in accepting that treatments are only provided if they are effective and affordable;
- Top up fees mean that PCT accountability mechanisms should be strengthened, with an emphasis on the desirability of locally tailored services;

- SHAs should play an important role in providing information to patients about expensive new drugs; and
- The role of NICE and the overall process of approving new medicines should be more transparent.

WRITTEN EVIDENCE

1.1 The Social Market Foundation (SMF) is an independent, non-partisan think tank, developing innovative ideas across a broad range of economic and social policy. It champions policy ideas which marry markets with social justice and takes a pro-market rather than free-market approach. Our work is characterised by the belief that governments have an important role to play in correcting market failures and setting the framework within which markets can operate in a way that benefits individuals and society as a whole.

1.2 The SMF has a track record of making a strong contribution to the public and political debate on healthcare, notably through its 2004 Health Commission and ongoing Health Project which is examining the long term future of the NHS.

1.3 The SMF welcomes the report *Improving access to medicines for NHS patients*. We agree that an important goal of the NHS should be to provide access to the best treatments. We welcome the decision not to remove the entitlement to NHS care because a patient chooses to purchase additional private treatment. We also welcome the measures that will be put in place to improve the adoption and diffusion of new drugs into the UK health system. However we believe that despite this excellent report, there are still policy challenges to be overcome where there is not agreement about the best way to proceed

1.4 We argue that current definitions of equity in the NHS are not adequate to equip us to deal with the challenge of top up payments. Top up payments do not fit with conventional descriptions of equity in the NHS which have previously ruled out mixing public and private care. While additionally purchased drugs will be administered in a private setting, the continuity needed to ensure that the quality of care is not compromised effectively blurs the line between public and private provision. This ruling means that the distinction between public and private funding in the NHS is now less clear, and we need to define equity more clearly for the future.

1.5 The SMF believes that the NHS should commit itself to providing access to a high quality range of medical treatments based on need not ability to pay. However we should no longer pretend that the NHS provides all of the treatment to all of the people all of the time—the NHS should accept that it will only provide treatment where it is both effective, and within existing budgets. To adopt this more limited definition of equity would be to avoid some of the political pressure that has led to decision making not in the long term interests of the NHS and its patients, such as the rushing of Herceptin through the NICE process in response to media uproar.

1.6 *Improving access to medicines for NHS patients* calls for greater transparency in decision making at a PCT level, to help patients understand what drugs are available and why particular decisions are made. This recommendation is entirely appropriate. It should be seen in the wider context of an underdeveloped model of PCT accountability and local involvement. Currently some PCTs are very good at actively engaging with their local population, but there are many that need to improve significantly. Government should support PCTs in their decision making process, especially when it comes to designing services that are appropriate locally but where that might result in an exacerbation of the “postcode lottery”. The desirability of intended local variation should be an underlying principle of efforts to improve accountability in PCTs.

1.7 High quality information is of the utmost importance if patients are to be allowed to purchase additional private treatment. We recommend that the NHS should recognise that there will inevitably be a profusion of information sources, some reliable, others not. Controlling the flow of information to patients is beyond the capability of health providers. Therefore SHAs should have a role in compiling high quality information for patients in partnership with pharmaceutical companies and charity stakeholders. By expecting SHAs to take the lead in ensuring that patients have access to high quality information, the likelihood of patients making poor decisions as a result of unreliable information is reduced. PCTs will also have an important role in disseminating information, reflecting their status as the NHS point of contact for patients.

1.8 The SMF recommends that the legal status of information provided by individual clinicians, PCTs or SHAs be thoroughly investigated in order to reduce the chance of litigation from patients unhappy with the outcome of additional private treatment purchased on the basis of information provided by the NHS.

1.9 The SMF welcomes the emphasis placed on the need for government to work with NICE and the pharmaceutical industry to contain the cost of drugs. There is evidence that NICE has had some success in controlling health costs. However, there remains concern that the development of new medicines is likely to proceed towards drugs that are relatively unaffordable for the NHS because they are effective only in a small cohort of patients. Further, it is not always possible to tell who will benefit from a very expensive drug until it is administered to the patient.

1.10 Despite the emergence of some expensive cancer drugs, we do not agree that technological progress poses a threat to the future of the NHS itself. However we will require stronger organisations and processes to enable us to make decisions about what should and should not be provided to NHS patients in order to retain public support for the health service.

1.11 While access to new medicines can and should be improved, there remains the challenge of drugs that do not meet the NICE threshold for QALYs. We can be sure that however good our processes and however compliant the manufacturers, there will always be products to which patients want access but which NICE considers to be an unjustifiable use of NHS resources. To retain public confidence in NICE the government should invest more in explaining the necessity of a threshold, and defending the level at which it is set. Public confidence in the ability of health providers to ensure fair access to new medicines is a crucial component of overall support for the NHS. An acceptance that some things should not be available on the NHS might actually improve public confidence in the process overall.

December 2008

Memorandum by Macmillan Cancer Support (TF 16)

1. ABOUT MACMILLAN CANCER SUPPORT

1.1 Macmillan Cancer Support is a UK charity working to improve the lives of people affected by cancer. We provide practical, medical, emotional and financial support and push for better cancer care. We want to be able to reach every single person who is diagnosed with cancer and to be there for them at every stage of their cancer journey, no matter who they are, what part of the country they live in, or what type of cancer they have. We will reach them by helping to make life better, in however large or small a way, through our services.

2. EXECUTIVE SUMMARY

2.1 It is vital that as many patients as possible can access the cancer treatments that they need within the NHS. Macmillan Cancer Support believes that co-payments are a symptom of wider problems within the NHS and that we should in fact be tackling underlying issues rather than debating whether top-ups should be allowed. We believe that the Government should be trying to answer the alternative question, “how can we make clinically effective treatments available within the NHS?” Therefore our evidence to the Select Committee will focus on the solutions to the underlying causes of top-ups.

2.2 *Improving the timeliness of NICE decision making*—NICE should be sped up to ensure that patients are able to access the latest treatments in a timely way. However, decisions must be made at an appropriate time to ensure that as many treatments as possible are given a positive determination from NICE.

2.3 *Exceptional funding process and the NHS Constitution*—There are unacceptable variations in the exceptional funding process across the country. We believe that the Government should set out a framework for PCTs to ensure that there is a common standard to reduce the postcode lottery. This framework should be designed to allow PCTs to make decisions which are responsive to local health needs. Patients should also be told about the rationale for decision making.

2.4 *NICE and end-of-life medicines*—We welcome the proposal to evaluate treatments in end-of-life, for small patient populations by a new criteria. We believe that this presents the opportunity to also consider how other orphan treatments and treatments for severe diseases should be evaluated under the Technology Appraisal process.

2.5 *Pharmaceutical pricing*—We support the flexible pricing solutions agreed under the new PPRS. In addition we would like to see the Department of Health and pharmaceutical companies to continue to develop innovative pricing arrangements, such as risk sharing, to ensure that treatments are deemed cost effective by NICE.

2.6 *Information and clinician communication*—Macmillan believes that patients should be given all of the information that they need in order to make decisions about their treatment. In order to make this as good as possible, it is vital that clinicians are trained in communications to ensure that they are able to support patients in making difficult decisions and to make sure that conversations about end-of-life are had at a suitably early stage.

3. MACMILLAN’S POSITION

3.1 Prior to the publication of the report *Improving access to medicines for NHS patients* the emphasis of the debate on top-ups had been positioned in a number of newspaper articles as being around choosing between two unacceptable alternatives:

- Not allowing top-ups—“I am being denied the right to use my money to save my life.”.

- Allowing top-ups—“Encouragement for patients to incur debt, while risking the creation of a multi-tier NHS. The end of the NHS as we know it.”

3.2 Macmillan Cancer Support believes that co-payments are a symptom of wider problems within the NHS and that we need to tackle the underlying issues rather than debating whether top-ups should be allowed. We believe that the Government should be trying to answer the alternative question, ‘how can we make clinically effective treatments available within the NHS?’ which is the conclusion which strongly came out of the review conducted by Mike Richards.

4. ASSESSMENT OF THE RECOMMENDATIONS IN THE “IMPROVING ACCESS TO MEDICINES FOR NHS PATIENTS” REPORT

4.1 *Improving the timeliness of NICE decision making*

4.1.1 Macmillan supports the principle of a body such as NICE and works closely with the Institute on many projects. In May 2005, Cancerbackup⁴³ launched a “Dossier of Delay”⁴⁴ which revealed that cancer patients were waiting unacceptable lengths of time for potentially life-saving cancer treatments to be considered by NICE. Following the “Dossier of Delay” and our subsequent NICEwatch campaign⁴⁵ NICE introduced the Single Technology Appraisal process which has gone some way to speeding up decision making. However, it remains the case that the UK lags behind much of the rest of Europe: we spend less than other countries on cancer treatments, and we are slower to provide them on the NHS. These delays and barriers to treatment mean that for many people with cancer, treatments are not available in time to help them.

4.1.2 It is vital that patients are able to get access to the treatments that they need in a timely fashion. Much of the criticism about England being behind in prescribing the latest treatments is because of the delays in NICE issuing guidance. Two specific measures could be implemented to speed up the process:

- streamlining both the Single Technology Appraisal and the Multiple Technology Appraisal processes; and
- removing the time consuming steps of Ministerial referral.

4.1.3 Following the commitment in the Cancer Reform Strategy that “as a default position new cancer drugs and significant new licensed indications will normally be referred to NICE and to ensure that NICE appraisal guidance is available as early as possible”,⁴⁶ it seems to Macmillan that Ministerial referral is now obsolete for cancer treatments.

4.1.4 Although we believe that the Technology Appraisal process can be shorter, quicker appraisal times per se will not always make the best treatments available within the NHS. There must be a balance between treatments being made routinely available within the NHS quickly and decision making taking place at the most appropriate time. For example if a clinical trial is taking place, then in certain circumstances it may be better to wait for the results (as this may reduce uncertainty around clinical effectiveness) rather than to have a quick decision. By speeding up NICE the worst possible outcome for patients would be for more treatments to be turned down because of uncertainty in the evidence.

4.1.5 In addition to this we believe that it is vital that NICE consults clinicians, patients and patient groups during the decision-making process. These periods of consultation could be streamlined; however it is vital that stakeholders are able to engage in the process. Therefore we would not support any proposed system where the consultation periods were removed.

4.2 *Exceptional funding process and the NHS Constitution*

4.2.1 Earlier this year, Macmillan conducted a Freedom of Information request to all PCTs about their exceptional funding processes. The survey found that there is no consistency in the way that this process is conducted throughout the country. We are particularly concerned about a number of issues that the survey uncovered:

- *Postcode lottery*: There is no consistency across the country around the rules to determine whether a patient should be considered an exception for treatment funding. This therefore perpetuates the postcode lottery of cancer care.
- *Availability*: Only 38% of PCTs that responded said they actively promoted the process to patients.
- *Panel composition*: Only 6% said that there was an oncologist on the decision making panel, while 61% said there was administrator representation.
- *Process delays*: Only just under half of PCTs that responded (49%) said that they dealt with applications for exceptional funding (from application to decision) in a month or less.

⁴³ Cancerbackup and Macmillan Cancer Support merged on 1 April 2008.

⁴⁴ <http://www.cancerbackup.org.uk/News/Campaigns/Accessstotreatment/NICEwatch>

⁴⁵ *Ibid.*

⁴⁶ Cancer Reform Strategy, Department of Health, December 2007, p 85.

- *Criteria:* Around half of the PCT respondents (46%) said they take the patient's personal circumstances into account. Of those that said they did:
 - 36% considered age as a factor, 28% dependents, and 12% marital status,
 - 6% of PCTs said that they actively take into account local publicity/media activity in their decision making and 4% of PCTs were unsure if this influences the decision making process, and
 - 2% of PCTs that responded said they take into account the point of the financial year at which the application is received.

4.2.2 While we understand the importance of discretion when dealing with the needs of the local community, national guidance is essential to ensure that these decisions are made fairly and transparently.

4.2.3 It is also vital that there is a responsibility on PCTs to inform patients about the rationale for decision making so that patients are fully informed of the reasons if access to a treatment is turned down. The draft NHS Constitution outlines commitments to strengthen the exceptional funding process and will make PCTs responsible for informing patients why they have made their decision. These are important proposals which would be a big step forward in giving patients the information that gives them the control to understand their treatment and prognosis.

4.2.4 We hope that the NHS Constitution will ensure that the unacceptable variation across the country is reduced and that it will give patients the power and information that they need to determine whether they should pursue an exceptional funding claim. We understand that the Department of Health has commissioned the National Prescribing Centre (NPC) to develop a framework for exceptional funding to ensure that there is some uniformity across the country. As recommended in the *Improving access to medicines for NHS patients* report it is vital that the plans for the NPC project and the implementation of the NHS Constitution are outlined in full and that there is communication about how these projects are progressing.

4.3 NICE and end-of-life medicines

4.3.1 We are pleased that NICE has responded to our concerns that treatments for rarer cancers should be considered under a new set of criteria. Macmillan believes it is important that as many patients as possible are able to access clinically effective treatments within the NHS. We know that treatments which fall into the criteria outlined in the consultation which NICE is currently conducting are more likely to have high acquisition costs and correspondingly high ICERs. Therefore these new proposals would ensure that more cancer patients are able to access the treatments that they need.

4.3.2 We remain concerned, however, that the treatments NICE will consider under this new guidance may be too narrow and should be extended to consider:

- 4.3.2.1 Patients with severe diseases, such as lung cancer—The NICE Citizens' Council recommended that NICE and its advisory bodies should take the severity of a patient's disease into account when making decisions. This recommendation was agreed by the NICE Board in September 2008 to be added to the next edition of the Social Value Judgements guidance. We believe that this consultation on end-of-life treatments provides an important opportunity for NICE to implement this recommendation at the earliest possible opportunity. As the second edition of the Social Value Judgements principles was only launched in July 2008 it is unlikely that this document will be updated again for a number of years. We would therefore urge NICE to implement these recommendations on severe diseases within the new proposals for end-of-life treatments. As the recommendations stand patients with severe diseases such as lung cancer will lose out just because they have a more common cancer. This is unfair and could be considered discriminatory.
- 4.3.2.2 Patients with other rare diseases that are not for end-of-life—We believe that treatments for all orphan conditions (as categorised by the EMEA) should be considered in these new proposals, not just in the end-of-life setting. This would mean that more patients with rarer cancers are able to access clinically effective treatments, with a limited budget impact to the NHS.
- 4.3.2.3 Patients who fall just outside of the 7,000 patient population—The Institute should make the 7,000 patient population criteria guidance rather than an arbitrary cut off point. As with the QALY threshold, unless stakeholders fully understand how this cut off point has been determined it will be challenged extensively in the future. We would like the Institute to explain the rationale behind the decision to choose this as the correct number. It is essential that there is flexibility built in to the system because in certain cases there may be a compelling case for considering a treatment with a larger patient population under these new rules.
- 4.3.2.4 For treatments which significantly improve the quality of life rather than the length of a patient's life—Quality of life issues can be specific to cancer treatments and not score highly within the NICE process. The impact of issues such as the oral administration of

treatment in place of IV administration, fatigue and hair loss of patients undergoing cancer treatment and often in their final weeks of life are frequently undervalued. We would like NICE to consider these wider quality of life issues in this new system of appraising end-of-life treatments and more broadly in treatments in larger patient populations.

4.3.3 We would like to see more detail from NICE about the specific criteria which the Appraisal Committee will be measuring these treatments against. As it stands it does not seem as if there will be any cost effectiveness measures for patient populations at end of life in patient populations of less than 7,000. In order to be transparent we would like to know if there are going to be cost effectiveness measures and if so what QALY limit or other measures will be for treatments which fall in to the consultation criteria. It is crucial if the NICE process is to work effectively that stakeholders can properly scrutinise the decision making process. Without this information we believe that decision making could become inconsistent and opaque.

4.3.4 Macmillan would like NICE to consider widening the scope of its consultation to take the opportunity to improve the appraisal process for more patients and ensure that more patients are able to access treatments within the NHS.

4.4 *Pharmaceutical pricing*

4.4.1 We welcome the outcome of the PPRS negotiations to allow more flexible pricing. We believe that this along with other changes will make more treatments cost effective for use within the NHS.

4.4.2 In addition to this, Macmillan supports continued efforts by the Government to reduce the price of drug treatments. We believe that the pharmaceutical industry must work more closely with the Department of Health to construct innovative pricing solutions (such as risk-sharing) to ensure that treatments are cost-effective for use within the NHS.

4.4.3 Risk-sharing schemes can mean that if a drug is clinically effective for a particular patient then it is cost effective, but where a drug is not effective the costs are small and the NHS is reimbursed by the pharmaceutical company.

4.4.4 We believe that by engineering these systems the costs to the NHS of new treatments will be significantly reduced and mean that the pharmaceutical industry continues to invest in the UK and make their treatments available within the NHS market.

4.5 *Information and clinician communication*

4.5.1 A MORI poll conducted on behalf of Cancerbackup found that 79% of British adults would like to know about all potential cancer treatments—even if they are unavailable on the NHS.⁴⁷ We want people with cancer to be fully involved in decisions about their care. The provision of timely information and support is essential to ensure people affected by cancer are able to make informed decisions about their own care if they wish. We would like to see this information linked in to the information pathways which are currently being set up.

4.5.2 The National Confidential Enquiry into Patient Outcome and Death (NCEPOD) report *Systemic Anti-Cancer Therapy: For better, for worse?* found that chemotherapy treatment towards the end of life was inappropriate in nearly a fifth of cases.⁴⁸ In order to ensure that decisions about whether a patient should participate in drug treatment at the end-of-life it is vital that clinicians are given specific training in communications to deal with these difficult situations and also to make the vast amounts of complex information understandable to patients.

4.5.3 We want end-of-life discussions between doctor and patient to take place at an appropriately early stage in treatment and for the necessary, supported information to be in place to help patients make informed choices in a timely way. Patients nearing the end of life report confusion about prognosis and supportive care options.⁴⁹ While they need multiple opportunities to discuss end-of-life issues, oncologists rarely initiate discussions with patients about possibly being in the last year of life (so called “what if” conversations) during active treatment.

⁴⁷ *Cancer—A public priority? Attitudes towards cancer treatment in Britain*. Research Study conducted for Cancerbackup, Ipsos MORI, August 2006.

⁴⁸ *Systemic Anti-Cancer Therapy: For better, for worse?*, National Confidential Enquiry into Patient Outcome and Death, November 2008.

⁴⁹ Grunfeld E A *et al*, Perceptions of palliative chemotherapy: the view of advanced breast cancer patients, *Journal of Clinical Oncology* 2006, 24 (7), 1090–1098.

5. CONCLUSIONS

5.1 Macmillan believes that the issue of top-ups is a symptom of wider problems within the NHS. If the Government, NHS, pharmaceutical companies, and patient groups work together to reform NICE, develop new drug pricing models, issue guidance on the exceptional funding process, and reduce inefficiencies within the NHS, then we believe that many more patients will be able to get access to the cancer treatments that they require freely within the NHS. We welcome the Secretary of State's acceptance of all of the recommendations presented in the report *Improving access to medicines for NHS patients*. We now want to see detailed proposals about how these recommendations will be implemented including a detailed timetable.

December 2008

Memorandum by the Royal College of Nursing (TF 17)

TOP-UP FEES

1. EXECUTIVE SUMMARY

1.1 The Royal College of Nursing (RCN) used its response to the review of the consequences of additional private drugs for NHS care by National Cancer Director Professor Mike Richards earlier this year to assert that the Government should allow top up payments for private prescriptions for a limited time period alongside funded NHS care in order to publicly work through the wider implications of this issue.

1.2 The RCN therefore believes the Government made the right decision in lifting the ban on NHS care for those who choose to pay for additional drugs privately. However, there are still significant issues about how this principle is translated into practice, which could have a large impact on the work of nurses and how patients are cared for. We are committed to working with Government at every step of the way to ensure the best possible outcome.

1.3 More needs to be known about the recent Government announcement that under a new principle of separate care, private treatment should take place in a private facility away from NHS wards and must not be subsidised by the NHS. The RCN has serious concerns about providing topped-up care in the same setting as those receiving care on the NHS and anticipates that there will be significant issues for both nursing practice and patient safety.

1.4 The RCN has consistently highlighted concerns that consumerism in health care will bring consequences for nurses, patients and policy makers which must be carefully thought through. The RCN has also emphasised that such choice, if not properly deliberated, has the potential to exacerbate inequalities as some members of the public make choices others are not able to make. In this case, there is a clear risk of creating a business class standard of care for those who have the money.

1.5 On principle the RCN opposes the introduction of further top up payments as these can only serve to widen inequalities in access to services and disproportionately favour people who have a greater disposable income. In short we are opposed to changes in NHS systems or funding which offers qualitative improvements in care to those with the ability or means to pay more.

1.6 The RCN is also pleased to note that the Government has accepted recommendations on transparency in NICE and the need to speed up its drug approval process so that fewer people have to consider co-payments in the first place. In addition, Primary Care Trusts (PCT) need to work with each other and through Strategic Health Authorities to ensure that decisions are made quickly on the best evidence available in an open and transparent way.

1.7 PCT exemption request processes for unapproved drugs and treatments need to be urgently reviewed and mandatory national guidance drawn up which enshrines effective clinical engagement, improves public accountability and addresses the gross inequalities in entitlement the current system produces.

1.8 NICE has made a significant impact on the NHS. However that work and the remit of NICE need to be publicly explored and a new consensus obtained for the use of economic modelling in the allocation of NHS resources. Attention should be paid in the meantime to enhancing transparency of decision making and speeding up authorisations processes.

1.9 The RCN is currently reviewing the draft guidance on NHS patients who wish to pay for additional private care and will make a response to the Department of Health based on the views of RCN members by the January consultation deadline.

2. INTRODUCTION

2.1 With a membership of over 390,000 registered nurses, midwives, health visitors, nursing students, health care assistants and nurse cadets, the Royal College of Nursing (RCN) is the voice of nursing across the UK and the largest professional union of nursing staff in the world. RCN members work in a variety of hospital and community settings in the NHS and the independent sector. The RCN promotes patient and nursing interests on a wide range of issues by working closely with the Government, the UK parliaments and other national and European political institutions, trade unions, professional bodies and voluntary organisations. The RCN welcomes the opportunity to submit to this Health Select Committee investigation on top up fees.

2.2 The RCN welcomed the review of the consequences of additional private drugs for NHS care review by Professor Mike Richards earlier this year. The RCN consulted widely with its members on the issue before submitting its response to the review.

2.3 On principle the RCN opposes the introduction of further top up payments as these can only serve to widen inequalities in access to services and disproportionately favours people who have a greater disposable income. In short we are opposed to changes in NHS systems or funding which offers qualitative improvements in care to those with the ability or means to pay more. In its submission to Professor Richards the RCN stated that there was a risk that in effect, the NHS would be providing a business class service to those who could pay.

2.4 However, we recognise the unique situation that has emerged as a result of conflicting policy guidance. Prior to Professor Mike Richards' review an NHS patient could not mix and match private and public provision. If a patient opted for a private prescription because the drug was not approved by NICE for NHS funding, that patient would be treated as a private patient and thus also lose entitlement to NHS funded care related to the prescription and the primary diagnosis. This effectively meant that patients would either have to pay substantially more for their care or risk having NHS benefits and service withdrawn because they opted to pay for unapproved drugs. In our evidence to Professor Richards' review we found this to be unfair not least because the fact that the drug was unapproved did not mean that it was not effective, just that it was not approved.

2.5 Our recommendations to the review were as follows:

- allow top up payments in cancer care only for a period of one year whilst the wider impact of top up payments in the NHS is scoped out and publicly debated;
- in the interim, guidance should be developed for PCTs in handling exception requests for drugs and treatments not currently approved by NICE. RCN members indicated clearly that PCTs applied different tests in these circumstances; and
- In addition NICE processes need to be improved and speeded up and they should be involved in supporting PCTs to develop internal approval processes.

2.6 The RCN believes that the prime concern of the NHS should be the effective and appropriate distribution of limited resources to provide maximum health gain for those in need regardless of ability to pay.

2.7 The timing of this Health Select Committee Inquiry—between the publication of Professor Richards' recommendations and the consultation on the draft *guidance on NHS patients who wish to pay for additional private care* which ends in January means that the RCN submission will be informed by RCN members in advance of the Government announcement. The RCN is still working thorough implications with its membership ahead of the January deadline.

3. DEFINITION OF “TOP-UPS”

3.1 Patients have topping up their NHS care for a number of years. The public are able to buy hearing aids; orthopaedic braces; wheelchairs and other mobility aids; eye care; dental care; and even nursing care all whilst receiving some element of NHS-funded care.

3.2 The RCN considers that a top-up payment is a payment made to purchase products or services in addition to those already provided under the NHS. For example it has long been the case that NHS patients can buy a more expensive type of hearing aid privately but have both the testing and the fitting of the device carried out by the NHS. At a recent BMA conference a consultant orthopaedic surgeon stated that he regularly treats patients for sports injuries who pay for physiotherapy or sports braces privately.⁵⁰ Some NHS hospitals also allow women to pay for a one-to-one midwife during labour.

⁵⁰ *The Sunday Times* (2008) “We’ve paid into the system all our lives. Why has the NHS turned on us?”.

4. POTENTIAL IMPLICATIONS OF TOPPING UP CARE ON THE ROLE OF NURSES

4.1 The RCN is currently consulting its members on the implications and practicalities of the implementation of the separate care principle in the clinical environment. The RCN anticipates that there will be significant issues for both nursing practice and patient safety.

4.2 For example, physically moving a patient to a separate facility, either within the hospital or to another building, may create discomfort for the patient or at worst could potentially result in a deterioration in their condition. In addition a particular drug therapy regime may require administration at particular times which will dictate when a patient has to be moved and this would then have implications for how a nursing team, primarily caring for NHS patients, manages and organises care.

4.3 The RCN is keen to explore the extent to which a hospital can re-designate a facility from NHS to private. There is need to investigate the possible implications of a single side room being re-designated as a private facility for certain periods of the day when a top-up drug is administered but then reverted to an NHS facility.

4.4 The RCN is seeking clarification on the issue as there may be persuasive patient safety reasons for doing this. There is potential that two patients in adjoining side rooms who are being cared for by the same nursing team to be an NHS and private patient. This could present challenges for the management of nursing care and relationships between staff and patients.

5. PRIMARY CARE TRUSTS AND DRUG APPROVAL PROCESSES

5.1 The RCN has welcomed the announcement from government to improve quality and consistency of local decisions by setting out core principles to guide primary care trusts on the funding of new drugs, where there is no NICE guidance in place.

5.2 The evidence we received for the Professor Richards' review from our members suggested that the one cause of unhappiness amongst the public who have genuinely enquired about different treatment options available to them is the manner in which the enquiries have been handled by PCT's.

5.3 The RCN told the Richards review that some PCTs appear to have a robust process for dealing with requests for unapproved drugs or therapies—often called exemption requests which are publicly accessible and give a clear statement of principles upon which any decision will be based.

5.4 The role of the PCT in justifying public confidence that the decision has been clinically driven as well as financially informed is of particular importance. The RCN understands that PCTs currently do not have to spell out what is meant by exceptional circumstances provided the policy genuinely recognises the possibility of there being an overriding clinical need and provides for each request to be considered on its own merit.

5.5 In the case *Rogers v Swindon PCT*⁵¹ the court decided it was permissible for a PCT to carry out a cost/benefit analysis when deciding what treatment to fund. Set against the legal requirement for PCTs to break even, it is clear that PCTs are required to consider the cost of all treatments before agreeing to fund them.

5.6 If it is accepted that these decisions should be made by PCTs on behalf of the public, staff responsible for making such decisions need to be confident that they do so with the backing of government, the professions, the media and indeed, the public. As such these decisions need to be based on the highest possible degree of public, professional and human scrutiny.

5.7 We believe that it is vitally important that clinical judgement, accurate weighing of evidence and due consideration of the implications for the patient in question should top the list of considerations rather than cost although we recognise that this is important.

5.8 Allowing a patient's exception request has implications beyond simply paying for the drug. In the case of Trastuzumab (Herceptin), we understand that additional clinical tests, staff development and changes to policies and procedures all impacted upon resource allocations beyond the cost of funding the drug itself.^{52, 53} In addition, in the event of an adverse reaction to an exemption drug or treatment, the costs of emergency or critical care can be substantial.

5.9 Similarly the implications for a refusal to fund treatment go beyond cold appraisal of the facts or a de facto affordability argument. A decision to refuse to fund treatment for a person in fear for their life or indeed fearing a painful or debilitating death has a profound impact on the person concerned, their families and on society as a whole.

⁵¹ [2006] EWCA Civ 392, [2006] All ER (D) 181 (April).

⁵² In the case *Rogers v Swindon PCT* it was identified that Herceptin is given by a loading dose followed by a further 17 doses given at three week intervals. The estimated cost (including VAT) of the course of treatment was £26,328.22, which Mrs Rogers was unable to pay. This included operational costs, screening and the drug itself.

⁵³ In an article in the BMJ specialist clinicians estimated drug costs alone for one NHS trust would be £1.9 million and when added to the cost of administration and operation, would lead to the closure of other services—see Barrett, A *et al* (2006) *How much will Herceptin really cost?* BMJ 333:1118-1120 (25 November).

6. ROLE OF NICE

6.1 The RCN welcomed announcements by the Government to support NICE's proposal for greater flexibility in appraising more expensive drugs for terminally ill patients, to work closely with the pharmaceutical industry to agree new and more flexible pricing arrangements to increase access to new drugs and to speed up the NICE appraisal process for new drugs so that they become available to patients more quickly.

6.2 The role of NICE could be enhanced to incorporate consideration of applications for top up payments as suggested by the Institute for Public Policy Research.⁵⁴ For example they could have a role in assisting PCTs in any exemptions decision making processes where NICE has not already ruled or where there is a newly emerging or disputed clinical evidence base. However this would be an additional role for an organisation already heavily committed to expanding its role and speeding up its processes. Such moves should be carefully considered as part of a longer term plan and should arise out of a public decision to endorse the general approach ie that a cost value basis is the right way to ration limited resources within the NHS.

December 2008

Memorandum by Breast Cancer Campaign (TF 18)

THE PURCHASE OF ADDITIONAL DRUGS BY NHS PATIENTS

1. INTRODUCTION

1.1 Breast Cancer Campaign welcomes the opportunity to submit evidence to the House of Commons Health Committee inquiry on the purchase of additional drugs by NHS patients. The inquiry aims to assess the repercussions of the recent review of this issue that was conducted by Professor Mike Richards, National Clinical Director of Cancer, which looked at the purchase of private drugs for NHS care. This paper reflects Breast Cancer Campaign's response to *Improving access to medicines for NHS patients: a report for the Secretary of State for Health* by Professor Mike Richards, and has been prepared specifically for the Committee's inquiry.

1.2 Breast Cancer Campaign specialises in funding innovative world-class research to understand how breast cancer develops, leading to improved diagnosis, treatment, prevention and cure. We aim to be the leading specialist in breast cancer research across the UK and the Republic of Ireland, making a significant impact on breast cancer for the benefit of patients. We currently fund 105 projects throughout the UK worth over £13.5 million.

1.3 Top-up payments refer to the practice of allowing patients to pay for medication privately whilst still receiving free treatment from the health service. To inform the charity's view on this issue, Breast Cancer Campaign has consulted scientists that currently receive research funding from the charity.

2. THE PURCHASE OF ADDITIONAL DRUGS ON THE NHS

2.1 Professor Mike Richards's review of access to additional treatments for NHS patients gathered information from stakeholders and sets out a number of recommendations to improve access to treatments, quickly and at a cost the NHS can afford. Although particularly pertinent to cancer treatments, these recommendations apply to all conditions.

2.2 The concerns that Breast Cancer Campaign highlighted to Professor Richards centred on three key areas. Firstly, top-ups would exacerbate inequalities within the NHS, as those without sufficient private means or without medical insurance would still not be able to access the treatment recommended by their clinician. Secondly, top-ups would not in themselves help to address the wider issue of access to increasingly expensive treatments. And thirdly, top-ups could instead act as a disincentive for NICE to pass future treatments for NHS use.

Increasing the number of drugs available on the NHS

2.3 In Professor Richards's comprehensive report, we are pleased to see that some of these concerns have been addressed, particularly with regards to increasing access to clinically effective treatments. It is important that NICE issues guidance of high quality in a short timeframe to ensure patients get access to new treatments quickly. We therefore agree with Recommendation 1 in support of the Government's current commitment to improve the speed of NICE's decision-making processes.

⁵⁴ Farrington-Douglas, J and J Crabtree (2008) *Topping up: Should it be allowed in the NHS?* NHS Confederation Debate Papers—17 June.

2.4 We also welcome Recommendation 5, which encourages the Department of Health to examine how drugs used near the end of life but which do not meet NICE's cost-effectiveness criteria can become available in an affordable way. Breast Cancer Campaign is contributing to the Department of Health consultation on this issue, "Appraising end of life medicines". These drugs, which would be used by a very small proportion of patients, are unlikely to be made unavailable on the NHS without modifying the current criteria on cost-effectiveness. The consultation proposes raising the Quality Adjusted Life Year (QALY) threshold to enable these patients to receive end of life drugs, a proposal that we are supportive of.

2.5 Recommendation 5 also encourages the Department of Health to develop a more flexible approach to the Pharmaceutical Price Regulation Scheme with the pharmaceutical industry. Since the Richards Report was published, the PPRS has been successfully renegotiated to ensure greater access and reduced costs to the NHS. The agreement should enable drugs to be provided to the NHS at lower initial prices, with the option of higher prices if value is proven at a later date. This is a welcome step towards increasing the quantity of treatments available without causing adverse financial effects on the NHS.

Establishing clear guidance on purchasing additional drugs

2.6 The interpretation and application of guidance on the purchasing of additional treatment has been inconsistent across the NHS. This has unfortunately meant that in some cases, patients have had their NHS care withdrawn as a result of opting to buy an additional treatment. Recommendation 8 makes it clear that a patient's entitlement to NHS care should not be lost in the event that additional treatment is purchased. While we do not believe that patients should have their NHS care removed for purchasing additional treatments, we have fundamental concerns that allowing the purchasing of additional treatments will undermine equality.

2.7 We welcome the guidance to clinicians that they should exhaust all reasonable avenues for securing NHS funding before a patient considers purchasing additional drugs. However, we do have some reservations on Recommendation 9, which clearly states that patients should be allowed to receive additional private drugs as long as these are delivered separately from the NHS elements of their care. Though the intention appears to be to separate private elements from NHS care by making the point of delivery of these drugs clearly separate, the reality is that these measures will still undermine the principle of equality within the NHS. NHS patients without the resources available to pay for these additional drugs will still not receive access to them.

Concerns on implementation

2.8 We are also concerned by the omission of guidance on how the delivery of these additional drugs will work in practice. Without a clear indication of how this policy should be implemented, the Government will be shifting the responsibility to Strategic Health Authorities, creating a disparity between SHAs in how this new provision is incorporated into current practices.

2.9 The crucial issue of what will happen to a patient should their funding run out during the course of their treatment has been left unanswered in the Richards Report. Recommendation 7 clearly states that it should be the role of the Department of Health "to clarify how the NHS should handle situations where a patient wishes to purchase additional treatment" and that this should be done "to ensure consistency in practice across the NHS". Without clear guidance on how to respond when a patient runs out of the funding needed to continue their treatment, there is a serious danger that this will lead to the very inconsistency in practice across the NHS that the Richards Report seeks to avoid.

3. SUMMARY

3.1 In reviewing the difficult issue of whether to allow patients to purchase additional treatments, Professor Richards has presented a set of recommendations that should ensure that access to treatments is widened. Increasing the speed of the appraisal process by NICE for new drugs, in addition to the new PPRS agreement announced recently, are important steps to allowing patients greater access to new drugs, whilst acknowledging the finite resources at the NHS's disposal.

3.2 Providing NHS patients with the option of purchasing additional drugs weakens the notion of equality within the NHS. In order to ensure that this policy is implemented in a consistent manner, the Government needs to provide clear guidance on how these private drugs should be delivered, in addition to clarifying the course of action hospitals should take if a patient's funding runs out prior to completion of their additional treatment.

December 2008

Memorandum by the King's Fund (TF 19)

THE PURCHASE OF ADDITIONAL DRUGS BY NHS PATIENTS (TOP-UP FEES)

The King's Fund seeks to understand how the health system in England can be improved. Using that insight, we help to shape policy, transform services and bring about behaviour change. Our work includes research, analysis, leadership development and service improvement. We also offer a wide range of resources to help everyone working in health to share knowledge, learning and ideas.

1. OVERVIEW

1.1 In its evidence to the Inquiry conducted by Professor Mike Richards into the consequences of additional private drugs for NHS care, The King's Fund acknowledged that the government had to resolve a conflict between two views of the fairness. However, The King's Fund suggested that if patients chose to purchase a drug not funded by the NHS they should not, as a consequence, be denied the NHS care they would ordinarily have received. This was also the view taken by Professor Richards in his report and by the government in its response. (Recommendation 8)

1.2 Richards' recommendations and the Department of Health consultation document are based on the principle that NHS resources should be devoted solely to NHS patients and should not be used to subsidise private care. They therefore propose that patients who choose to pay for part of their care privately will also be required to pay all associated costs. Again this is in line with the recommendation in our submission to the Richards review. In order that such patients are not denied care they would ordinarily have received, Richards suggests that the private and public elements of care will need to be not only clearly identified but also kept separate. In effect, this strengthens existing guidance on this matter.

1.3 At the same time the Richards' review has aimed to contain the demand for private purchase of additional drugs by:

- speeding up NICE processes (Recommendation 1);
- negotiating with the pharmaceutical industry, seeking to introduce more pricing flexibility (Recommendation 5);
- ensuring that the scope for NHS funding is fully explored before purchase of private drugs is considered (Recommendation 9); and
- ensuring that patients are made fully aware of the clinical and financial implications of doing so (Recommendations 12 and 13).

We agree with all these recommendations.

1.4 In addition, the possibility, raised in the Richards review, that certain patient groups near the end of life could have the health benefits of any potential drug treatment weighted more highly than other patient groups is also designed to reduce demand for topping up by making drugs that would ordinarily fall above NICE's "acceptability range" more cost effective. We comment further on this below.

1.5 In its consultation paper, the Department of Health seeks to define a line between publicly and privately funded care on the basis of "separation". This is designed to ensure that the costs of administering drugs paid for privately fall entirely on the patient (or the insurer) and that the NHS itself does not pay for care that has been assessed by NICE as being not cost effective. There is a variety of ways in which "separateness" could be created but the Department has chosen a physical concept of separateness.

1.6 A commonly used argument for physical separation is that it would be undesirable for patients on the same NHS ward to be receiving different levels of treatment. Using a "specially designated area within an NHS trust" for private care could, Richards suggests, constitute separateness. But it is unclear whether guidance along these lines will secure against patients in the same ward being treated differently—the notion of "separateness" is not precise enough to ensure it. But even if physical separation was clear, this would not ensure that patients would be unaware of the differences in treatment being offered. The real argument in favour of physical and thereby temporal separation is to ensure that NHS and privately funded care can be clearly identified and are not mixed up.

1.7 As an example of something that would fall outside the permitted scope for top-ups, the Department's consultation paper uses the example of an improved lens for cataract surgery—on the grounds that the extraction of the cataract and the insertion of the lens must be carried out during the same operation and hence in the same place. In other words, separation of publicly and privately funded care in a physical sense is not achievable. However, as the Richards review and its recommendations are confined to drugs this is a somewhat strange choice of example.

1.8 The example does, of course, illustrate how "separation" could be achieved in a financial sense. Although the operation is one procedure, it would be easy to allow the improved lens to be paid for by the patient and the rest of his or her care provided by the NHS. This point has already been recognised by, for example, Age Concern, which is seeking clarification for other similar situations. There are likely to be more arguments for allowing patients to pay privately for elements of their care in cases where financial separation

is easily defined, as in the case of upgraded implants, aids, and devices. The current rules around spectacles already permit patients both to mix NHS lenses with private frames and to buy more expensive glasses than NHS vouchers will cover.

1.9 The Richards report concludes (Recommendation 14) that the government should confirm how situations in which patients wish to purchase additional non-drug interventions should be handled. We agree with this.

2. OTHER ISSUES

2.1 The Richards report (Recommendation 5) proposes that the Department of Health together with NICE should [re]consider the value to be attached to extra time at the end of life.

2.2 The government is right to look again at the value attached to giving extra time to patients with life-threatening conditions. If media coverage and political interest is anything to go by, there does appear to be public support for the idea that a patient who has been given just a few months to live may well regard the time left to them as particularly precious and that it is reasonable for the state to place additional value on that time compared to say someone who can expect to have say 20 years left. However, more work does need to be done to explore public attitudes and to have a wider and more considered debate than hitherto. There does not seem to have been much research in this area—the Review identified just two studies on patients' notional willingness to pay for an extended, or improved, quality of life.

2.3 However, any such move to introduce differential valuations of life must be taken cautiously. For NICE to move away from the principle that a “QALY is a QALY is a QALY”—that is, treating everyone's lives of equal value—represents a significant step. The central ethical question here is who should decide any differential valuation?

3. VARIATIONS

3.1 The Richards report makes a number of recommendations (1,3,10) designed to reduce variations between PCTs in their decisions on exceptional cases. We agree with most of these. However, the proposed collaboration between PCTs within SHAs or care networks, while offering the possibility of more rigorous and robust assessments, still leaves room for differences to emerge across the country as a whole, which will be challenged and which in a national health service may be hard to justify.

3.2 Making a case for exceptional treatment must inevitably involve the use of information specific to the case concerned. The proposals for a more transparent process will clearly help to promote some degree of consistency as they will allow both patients and clinicians to see what information and arguments have been used in similar cases.

3.3 If the government is successful in reducing the number of patients seeking access to non-approved drugs on the NHS, a national appeals system may be feasible. But if administrative practicalities suggest that local arrangements should be retained, then a system more accountable to the operation of the courts may be preferable. This would allow precedents and case law to be developed and used nationally and would reduce (though not eliminate) the scope for variation between areas.

3.4 The issue of individual hospitals choosing to top up NHS care on behalf of their patients—as one foundation trust is currently doing—should also be addressed, particularly where it is not just individual clinicians making exceptional case decisions based on the clinical circumstances of each patient. Although topping up is within their vires, and hospitals would not be able to bill their PCTs for the funding involved, they are, arguably, using NHS funds and introducing a form of inequity that the Richards' proposals are designed to reduce.

4. DRUG APPROVALS

4.1 The Richards report (Recommendation 1) supports the measures already being taken to speed up the approval process. The example of the Scottish Medicines Consortium does suggest that at least some decisions can be made more quickly than they are now. But the risk is that speedier appraisals may be of lower quality as less information will be available. If a speedier process is adopted then it will be necessary to ensure that effective arrangements are in place for monitoring the effectiveness of the drugs concerned when they are in day-to-day use.

4.2 However, there is a case for a more radical policy that would eliminate the time drugs are “in limbo” by formally adopting a stage 4 approach to all new drugs. This would mean that they could be used as soon as they are licensed but that they should, immediately after licensing, be subject to systematic evaluation until a definitive evaluation was possible. This would have the advantage of allowing speedy take-up of new drugs but at the same time ensuring that when they are evaluated the financial and clinical evidence base for doing so is much more substantial than is the case now. In principle PCTs are already allowed to pay for drugs that have not been assessed by NICE, but there are no arrangements for monitoring the impact of their decisions. Our proposal would ensure that this interim period was used effectively to generate the information required for a full appraisal to be carried out when the evidence base is adequate.

5. AUDIT

5.1 Recommendation 11—that the use of non-approved drugs should be audited—is sensible and is in line with what we suggest above. However, the government needs to make clear what the purpose of such an audit is: is it intended to provide information that might be used by NICE to review its position; to help clinicians identify the patients who might benefit from non-approved drugs and hence make the case for them being treated as exceptional cases; or to provide patients with better information on the cost and clinical implications of deciding to pay for them?

5.2 It may be possible to support this process through targeted research. NICE evaluations aim to identify the overall benefit of using a drug over the population it is applied to. Typically, however, the benefits of a drug are unevenly distributed, with some people gaining a lot and others, nothing. If those likely to benefit could be better identified in advance—as is the case now in some instances as the Richards report notes (para 2.19)—the cost-effectiveness of the drug concerned would be transformed. The government has recently begun to support, through its Biomarkers programme, research that aims at the more effective identification of the patients who have a good chance of benefiting from a particular intervention. It would be desirable to ensure that this programme is linked to the proposed audit process—or even better our proposed stage 4 approach.

6. THE NEEDS OF PATIENTS

6.1 Recommendation 11 in the Richards report proposes that the Department of Health should consider the needs of patients for soundly based information. We agree with this but consider that the government should give more consideration to those patients who do opt for expensive top-ups. Although the proposals for better informed consent are sound, they do not deal with the financial risk to patients of their treatment being more successful than forecast. A stop-loss provision should be introduced to protect such patients from severe financial hardship. This would be justifiable in cost-effectiveness terms as longer-than-expected survival would mean that the drug concerned was cost effective for this particular patient.

7. CONCLUDING COMMENT

7.1 Although we agree with the broad approach the government has taken to minimise the number of patients seeking to top up their care in the sense defined in the Richards report, this, and the government's response to it, represent the beginning rather than the end of a debate about access to treatments that the NHS decides not to fund.

7.2 The current economic climate and the prospect of a sharp reduction in the rate of growth of the NHS budget from 2010 onwards combined with continuing technical progress in medicines and other technologies means that the number of occasions when patients wish to supplement their NHS care by paying for additional or superior treatments may grow, despite the measures Richards proposes to limit them. It is important therefore that any “solution” is designed for the future, as well as to meet current difficulties.

December 2008

Memorandum by Novartis Oncology (TF 20)

THE PURCHASE OF ADDITIONAL DRUGS BY NHS PATIENTS

EXECUTIVE SUMMARY

Novartis Oncology welcomes the inquiry by the Health Select Committee into the purchase of additional drugs by NHS patients or “top-up” fees.

In Professor Mike Richards' report and the subsequent Department of Health draft guidance on additional private care, it is made clear that additional drugs purchased from a private provider should be administered separately from NHS elements of care.

This management of patient care between private and NHS services creates a further set of challenges which must be addressed to ensure consistent and fair application of the recommendations. These include continuity of care for the patient, the additional cost of drugs and related administration and accommodation charges to be paid by patients, and variable pricing by private institutions. In addressing these challenges, it is vital that the Government clarifies the definition of an “episode of care” within the NHS so that it is possible to differentiate between a “top-up” treatment provided in a private setting, and NHS care provided free at the point of delivery.

National policy guidance for PCTs and clinical governance standards for health professionals should also be considered to ensure that the recommendations in Professor Mike Richards' report are implemented fairly across the NHS. Robust auditing and quality monitoring systems will be required to enable this to happen. The Government should also review the implementation of Professor Mike Richards' recommendations at least annually and report these findings to Parliament.

1. Novartis Oncology aims to improve and extend the lives of cancer patients by developing innovative therapies for the treatment of a range of cancers. Novartis Oncology has seven key marketed brands in the UK, as well as several new compounds in development.

2. In its response to Professor Mike Richards' consultation in the summer, Novartis Pharmaceuticals stated that the concept of "top-up" payments runs counter to the principles of the NHS, and cautioned that a decision to allow the purchase of additional drugs alongside NHS care must take into account the complex consequences of such a move, and put in place appropriate safeguards to ensure there would be no unintended negative impact for patients.

3. In Professor Mike Richards' report and the subsequent Department of Health draft guidance on additional private care, it is made clear that additional drugs purchased should be administered in a private setting. The separation of NHS and private care in the administration of additional drugs raises a number of issues which need to be addressed in order that the new policy can be implemented consistently and fairly for patients.

4. Current Government guidance indicates that an "episode of care" should be the determining factor in deciding whether a patient can mix public and private health care provision. In line with the principles of the NHS, this guidance states that patients may not mix public and private services within the same "episode of care". However, current Government guidance on what constitutes an "episode of care" within the NHS is unclear. As a result, there is currently no universal framework for determining when a patient is able to pay for additional drugs alongside NHS services. The Government must review all legislative and policy opportunities to rectify this problem as soon as possible, and establish clear guidance so that PCTs are obliged to treat all NHS patients equitably.

5. Transfer to private facilities may result in a lack of continuity of care for patients, particularly where NHS consultants do not also act in a private capacity. Clinical governance standards should be considered to ensure that health professionals manage patient care effectively between private and NHS care settings. Professional bodies should work with the Department of Health to establish clear clinical guidelines and health information protocols to ensure seamless integration between the public and private sector.

6. The patient will need to gain additional consent for the treatment from a clinician in the private setting following transfer by NHS clinician to private care, incurring unnecessary additional consultation costs for the patient.

7. The cost of paying for the administration of additional drugs in the private setting will substantially increase the overall cost of additional care to the patient beyond the purchase of additional drugs. This will further limit the number of people who can realistically afford additional drug treatment. Different private facilities are likely to charge different rates, including both the direct costs required for administering the drugs, and costs relating to the utilisation of a private facility, which could vary dramatically. This could lead to a double "postcode lottery" for cancer patients, firstly in terms of whether the local NHS organisation will fund their treatment, and secondly the variable cost of purchase and administration of additional drugs in the private treatment centres locally available to them. Variations in different types of ancillary costs across private providers may create regional inequalities in access to and affordability of additional drug treatment.

8. This situation could be improved through a move towards a standardised or regulated pricing and referral system for additional drug treatment in the private setting overseen by an independent regulator.

9. Professor Mike Richards' report emphasises that the recommendations on reforming NHS and NICE processes for appraising and providing new drugs, in particular changes to technology appraisals and PCT exceptional case committees, should minimise the need for patients to pay for additional drugs. The Department of Health should therefore consider appropriate auditing mechanisms for regularly monitoring the uptake of privately funded drugs and the quality of care delivered to patients between care settings. Given the degree of public interest in this issue and the potential impact on the principles of the NHS, Ministers should report these findings to Parliament once a year for debate and scrutiny.

Memorandum by the NHS Confederation (TF 21)

TOP-UP FEES

The NHS Confederation is the only independent membership body for the full range of organisations that make up today's NHS. We represent over 95% of NHS organisations. We have a number of Networks which represent sector-specific services including Foundation Trust Network, Primary Care Trust Network, Mental Health Network, Ambulance Services Network and the NHS Partners Network, which represents independent (commercial and not-for-profit) healthcare providers of NHS care.

We also have dedicated teams and steering groups working on behalf of our acute trusts and independent sector providers.

The NHS Confederation welcomes the opportunity to give evidence to the Health Select Committee on top-up fees. This evidence sets out our views, based on feedback from our members and our ongoing work programme.

KEY POINTS

- The review, *Improving access to medicines for NHS patients*, provides a reasonable solution to a difficult issue. It keeps the scope of the solution within narrow confines that should prevent the development of a multi-tiered NHS.
- Our members were against the idea of top-up fees in principle but recognised the need for a solution for people who wanted additional care. They also thought that the rule that suggested that a patient should lose their entire entitlement to NHS care was unsustainable. The solution of providing separate private care is a sensible alternative. There are however, a number of significant practical implications.
- Practical issues that need to be considered include pricing, achieving separation, collection of payment, what happens when patients run out of money clinicians with ethical objections and the private patient income cap.
- Monitor should be allowed to treat income from separate care as outside the private patient income cap.
- NICE's processes must be speeded up to increase access to new and effective drugs.
- There is scope for improving the way that exceptional case panels work.
- Further consideration has to be given to NICE proposals to extend access to end of life care drugs.
- Patients must be given the best possible advice from clinicians and NICE about the actual effectiveness of drugs.
- The top-ups, or separate care, issue is only one part of a much wider debate about the efficacy of treatments, as well as what is feasible for the NHS to fund with a finite amount of money.

INTRODUCTION

1. We were pleased by the outcome of the review which seems to have found a sensible way around a difficult issue.

2. The view of most of the members consulted by the NHS Confederation was that they would rather there were no top ups at all allowed in the NHS. Reasons given ranged from concerns about equity, the possibility of hazards for the long term integrity of the NHS system based on social solidarity and the potential for practical difficulties outweighing any potential benefits of the system.

3. Despite these concerns members realised that such a position is not sustainable, not least for the reason that the principle has already been partly surrendered in a number of areas of NHS treatment. The solution of separate care avoids a number of the hazards associated with a top up payment system.

4. The NHS Confederation has done extensive work in identifying some of the practical concerns associated with top-up fees. A number of these have been answered in the Richards' Review, but there are several that still need to be worked out.

PRACTICAL QUESTIONS

5. There are a number of practical questions that need to be asked to aid implementation of the review. These include:

- 5.1 Who should pick up the costs where patients run out of money or show that they have benefited from the drug?
- 5.2 Issues around collecting payment, particularly when the patient has died.

- 5.3 Clinicians who do not want to get paid to provide private care.
- 5.4 The ability to create appropriate alternative accommodation
- 5.5 How the price for these drugs should be set—which costs should be included? For example, should it include a risk premium for the costs of predictable complications?

PRIVATE PATIENT INCOME CAP

6. One of the most significant practical implications identified by the NHS Confederation is that of the private patient income cap.

7. NHS Foundation Trusts are limited in the amount of income they can generate through private treatment. This is set out in the initial legislation governing foundation trusts. The cap is calculated based on how much of the trust's income was generated by private patient work in the financial year ending on 31 March 2003 (or the year before it became a foundation trust if it didn't exist in 2003).

8. If separate care treatments are considered as private patient income, a significant number of foundation trusts would be unable to offer them without a change in the law. Many of the trusts that are likely to find themselves offering this service are close to or at their private patient cap ceiling.

9. The high cost of the drug component and the need to avoid being seen to make a profit from this type of care means that other forms of private patient activity would be more attractive as these generate a surplus which is then applied for the benefit of NHS patients.

10. This means that offering separate care in Foundation Trusts that are close to their private patient income cap would violate one of the fundamental principles of the review—it would disadvantage NHS patients.

11. There seem to be a number of possible solutions:

- 11.1 Designate separate care as a NHS service that is fully charged for. This seems to undermine the distinction that Professor Richards makes between separate care and top ups and so is not desirable.
- 11.2 Revise the approach to setting the private patient income cap. This would require difficult changes in primary legislation and while we believe this will need to be looked at in future this is probably not the issue that should trigger this review.
- 11.3 Sub-contract all care of this sort to other providers—this has implications for continuity of care and, potentially, safety. It also means that patients lose their ability to choose.
- 11.4 Allow Monitor to treat income from separate care as outside of the private patient income cap. This would entail making a case by case adjustment to each Trust's target. The grounds for this would be that this situation was not foreseen at the point at which the income cap was set.

ACCESS TO DRUGS AND NICE

12. The NHS still provides free, comprehensive access to cost effective treatments and it is important to remember that 54 out of 59 cancer treatments assessed by NICE have been approved.

13. The demand from patients to pay out of pocket for new drugs will fall if improvements are made to the speed of NICE appraisals.

14. We support the Government's proposal to look at exploring risk sharing models with drug companies where they are paid based on their effectiveness, and that they share the risk of new, sometimes marginal treatments. However, the bureaucratic burden of running these schemes is a potential issue

15. There is also scope to improve the transparency and equity of exceptional case panels, which decide on whether drugs are made available to individual patients. The NHS Confederation is working closely with the National Prescribing Centre to help improve current guidelines. Some PCTs are already working together to minimise the problem of perceived postcode lotteries by sharing policies and operating panels across a number of organisations, as well as sharing responsibilities for appraising new drugs and technologies.

END OF LIFE MEDICINES

16. The terms of the NICE review on end of life medicines define those conditions covered as “not normally exceeding” 7,000 diagnoses per year in England. This equates to around 137 people per 1 million population (or between 13 and 178 patients per PCT). Depending on the costs of the drugs this may be financially significant.

17. If the effect is to increase expenditure on end-of-life drugs then, as cash-limited organisations, PCTs will need to reduce spending elsewhere. There is some concern that treatments/interventions/services that are more cost effective but not subject to NICE guidance will lose out to pay for high cost end-of-life drugs.

18. There is some evidence from public engagement exercises run by PCTs and cancer networks that when given an opportunity to consider and discuss priority-setting and funding issues in detail, members of the public emphasise the importance of factors including cost-effectiveness and quality of life, and do not necessarily prioritise length of life. It is not clear what evidence there is that the public wish to prioritise greater expenditure on drugs that extend life for a matter of months for small numbers of patients.

19. We would expect that NICE will research public views on this issue. However, this would appear to be a case in which the public estimation of the utility to the patient is particularly likely to be subject to significant distortion from the well documented cognitive biases introduced by asking people to imagine a possible future state: particularly where it is foreign to them and potentially unpleasant. Whilst there is legitimacy in the public having a view about how as a society we value our ability to intervene to extend life this should not overshadow the actual experience of the patient and the utility that they gain from this. We are persuaded that the standard methods for calculating QALYs do not take this problem sufficiently into account. How this experience can be effectively and ethically researched is undoubtedly a challenge but getting a better understanding of this issue will be important for this and other public policy decisions.

INFORMATION

20. A further concern is that many of the drugs in the category covered by separate care are very expensive, and may only have limited effectiveness. We need to be very careful that patients are not given false hope about the effectiveness of drug treatments.

21. It will be important to make sure that people get the best possible advice from clinicians, NICE and others about evidence based and likely effectiveness of drugs before investing significant amounts of money in them.

22. These are very difficult issues and this policy initiative, whilst potentially helpful, will not bring to an end the ongoing debate on priority setting and what we as a society should agree to fund. There needs to be an acknowledgement that the NHS has a finite amount of money to invest and focussing on drug treatments may be potentially misleading to the public.

December 2008

Memorandum by the Association of British Insurers (TF 22)

THE PURCHASE OF ADDITIONAL DRUGS BY NHS PATIENTS (“TOP-UP FEES”)

The ABI is the voice of the insurance and investment industry. Its members constitute over 90% of the insurance market in the UK and 20% across the EU. They control assets equivalent to a quarter of the UK’s capital. They are the risk managers of the UK’s economy and society. Through the ABI their voice is heard in Government and in public debate on insurance, savings, and investment matters.

CONTEXT

The Department of Health accepted Professor Mike Richards’ report *Improving access to medicines for NHS patients: a report for the Secretary of State for Health* recommendations:

- There should be no withdrawal of NHS care because a patient chooses to purchase private care over and above their NHS care.
- Any private care should be provided separately from NHS care.

OUR RESPONSE

1. We welcome the requirement that patients retain their NHS entitlement when they choose to purchase private health care over and above their NHS care.

2. We also welcome the measures taken by NICE to assess what could be done to make available more drugs used near the end of life. We hope this will lead to a nationally consistent NHS entitlement where cancer drugs are available to patients based on clinical need and the approval of NICE rather than local budget constraints.

3. We believe the private insurance sector will have a significant role to play in enabling the majority of patients, not just the wealthy, to make meaningful choices and engage in decisions about their healthcare.

4. The industry anticipates continuing to engage with the Department of Health to enable patients to benefit from the proposed new guidance arrangements and to consider how the public and private systems could work more effectively together in the future.

December 2008

Memorandum by Roche (TF 23)

TOP UP FEES

EXECUTIVE SUMMARY

1. Roche welcomes the opportunity to submit written evidence to the Committee's inquiry into "top up fees".

In this submission we:

- Set out the situations in which a patient may have to purchase additional drugs.
- Assess the cost impact of making innovative drugs available.
- Comment on the recommendations made by Professor Richards in relation to improving access to medicines for NHS patients and the proposed NICE scheme.
- Examine how Professor Richards' recommendations on "separate care" might work in practice.

2. We also identify a number of potential recommendations that the Committee may wish to make, including that:

- An urgent investigation is undertaken into the extent of, and reasons for, international variations in access to medicines. Holding further evidence sessions later next year in relation to the investigation's progress and findings may prove fruitful.
- A comparison of variations in the pricing of drugs in different countries should be undertaken.
- NICE is adequately resourced to undertake timely appraisals of new medicines.
- The Department of Health should publish a report on progress in improving PCT decision making processes.
- A flexible approach is adopted to the 7,000 patient limit in NICE's proposed scheme for medicines used near the end of life, so long as the manufacturer demonstrates that the actual number of patients used in practice will not exceed 7,000. This ensures that the spirit of the scheme is implemented and that it does not unduly discriminate against any particular patient populations.
- NICE should undertake further work with the Department of Health, industry and patient representatives to refine the methodology used for calculating cost effectiveness data.
- Further consideration be given to how pricing mechanisms for innovative medicines can best reflect value and affordability, as well as unit cost, in line with previous Committee recommendations.
- The Department of Health should commission a follow-up investigation so as to ensure that the system of separate care is properly implemented.

INTRODUCTION

3. Roche is a leading manufacturer of innovative medicines for a number of disease areas, including oncology, rheumatology and virology. We are aware that a number of patients have been denied NHS funding for medicines we manufacture which their clinician has recommended and we therefore welcome the publication of Professor Richards' report, *Improving access to medicines for NHS patients*.

4. Given the fundamental issues raised by the debate about access to medicines for NHS patients, and the potential implications that this has for the future of comprehensive health services, based on need and not ability to pay, we welcome the decision by the Select Committee to stage an inquiry into this important issue.

5. In this submission we:

- Set out the situations in which a patient may have to purchase additional drugs.
- Assess the cost impact of making innovative drugs available.
- Comment on the recommendations made by Professor Richards in relation to improving access to medicines for NHS patients and the proposed NICE scheme.
- Examine how Professor Richards' recommendations on "separate care" might work in practice.

We would be happy to provide the Committee with further evidence if this would be helpful.

Are innovative medicines affordable?

6. During the course of recent debates about “top ups,” there has been some comment that new medicines are inherently unaffordable in a tax-based system where access is universal and based on need and not ability to pay. A number of inaccurate and, in the context of this debate, unhelpful estimates have been cited in the media. Roche does not accept that innovative medicines are unaffordable for NHS patients. It is worth noting that a number of European countries with comparable economies are able to make available new medicines.⁵⁵ Further examination of why other countries are able to make available new medicines is warranted and we therefore welcome the Secretary of State’s acceptance of Professor Richards’ recommendation that an urgent investigation is undertaken into the extent of, and reasons for, variations in access to medicines. The Committee may wish to consider supporting this recommendation and holding further evidence sessions later next year in relation to the investigation’s progress and findings.

7. There is legitimate discussion about how the price of innovative medicines in England compares to that in Europe and some commentators have called for an independent comparison into this.⁵⁶ In the interests of transparency, we would support a comparison of pricing. The Committee may wish to consider recommending that such a comparison takes place.

When might patients need to purchase additional drugs?

8. From our experience we agree with Professor Richards’ findings that situations where patients may seek to purchase additional drugs can arise in three main scenarios:

- NICE has yet to issue guidance to the NHS and a PCT has declined to fund the treatment.
- Due to the rarity of a condition, NICE will not appraise a treatment and a PCT has declined to provide funding.
- NICE has declined to recommend the treatment for use in the NHS and a PCT has refused an exceptional funding application.⁵⁷

In each of these situations we believe that there are steps which can be taken to improve access for patients on the NHS, so removing the need for them to “top up.”

TAKING STEPS TO IMPROVE ACCESS TO NEW MEDICINES

9. Professor Richards made a number of recommendations relating to improving access to new medicines for NHS patients. These broadly relate to the following issues:

- Making NICE appraisals more timely to ensure the prompt availability of guidance for the NHS.
- Improving the quality of decision making in the absence of NICE guidance.
- Ensuring that NICE methodology is fit for purpose.
- Introducing greater flexibility into pricing mechanisms.

Making NICE appraisals more timely to ensure the prompt availability of guidance for the NHS

10. We fully support measures to improve the timeliness of NICE guidance. Roche manufactures erlotinib (Tarceva), a medicine for second line metastatic non-small cell lung cancer. Despite being conducted through the streamlined Single Technology Appraisal process, the erlotinib appraisal took nearly three years from initiation to the publication of final guidance.⁵⁸

11. It can be very frustrating for patients, clinicians, NHS managers and industry when appraisals are unnecessarily delayed, so preventing the publication of appropriate guidance. We therefore welcome the Secretary of State’s announcement that further measures will be taken to expedite NICE technology appraisals and we look forward to the publication of more details in the New Year. It is important that NICE is provided with appropriate resources to facilitate the production of timely guidance whilst maintaining high standards of appraisal. The Committee may wish to make recommendations about ensuring that NICE is adequately resourced.

⁵⁵ ABPI, *Media briefing: top-ups*, October 2008. Page 1.

⁵⁶ Rarer Cancers Forum, *Exceptional England—An investigation of the role of Primary Care Trusts in making cancer medicines available through exceptional cases processes*, October 2008. Page 28.

⁵⁷ Professor Mike Richards, *Improving access to medicines for NHS patients* November 2008. Page 18.

⁵⁸ *Hansard*, 4 November 2008, Column 371W; available here: <http://www.publications.parliament.uk/pa/cm200708/cmhansrd/cm081104/text/81104w0031.htm#08110485001654>

Improving the quality of decision making in the absence of NICE guidance

12. PCT processes for determining whether to fund treatment for which there is no NICE guidance or, in exceptional circumstances, there is negative NICE guidance, have been the subject of much controversy and could be improved significantly. For example, an audit conducted by the Rarer Cancers Forum found that:⁵⁹

- The system for determining exceptional cases is confusing for patients, with different terms being used by different PCTs.
- Many PCTs do not hold sufficient information to meet the spirit of the commitment in the draft NHS Constitution to transparency on funding decisions.
- Many PCTs have processes dominated by administrators with seemingly little role for clinical expertise.
- Some PCTs are failing to process exceptional cases in a timely fashion. For patients with advanced cancer, time can be of the essence and this delay could potentially compromise health outcomes.
- The policy on applying social criteria is inconsistent.

13. Professor Richards' report rightly pointed out that problems with PCT decision making processes affect many more conditions than cancer. They will also impact upon non-drug treatments, which still form a significant minority of exceptional case applications.⁶⁰

14. Such variations in process inevitably undermine the confidence of clinicians, patients and the public in the NHS and we therefore wholeheartedly support Professor Richards' recommendations. In particular, we support the recommendation that PCTs should make greater use of regional expertise through collective decision-making processes. This will be particularly important for treatments for rarer conditions where appropriate expertise is unlikely to exist within 152 PCTs, building on the recommendations made in the Carter Review of commissioning specialised services.⁶¹

15. In order to restore public trust in PCT commissioning processes for treatments not routinely funded, we believe that the Department of Health should commit to publishing a progress report in April 2010. The Committee may wish to recommend that the Department of Health publishes a report on progress in improving PCT decision making processes.

Ensuring that NICE methodology is fit for purpose

16. England has pioneered health technology assessments which has played a significant role in reducing variations in access to medicines. However, it is important to note that health technology assessment processes are still relatively new and will need to continue to evolve to reflect advances in science.

17. In recent years NICE has successfully adapted and evolved to a number of challenges including:

- Improving uptake of guidance, ensuring that the NHS acts upon advice from NICE.
- Speeding up appraisals, introducing the Single Technology Appraisal process to expedite the availability of guidance.
- Taking greater account of measures such as progression free survival, reflecting the challenges presented by treatments for advanced disease.

18. However, it has become increasingly apparent that NICE's processes have led to a growing disconnect between clinical practice elsewhere in the world and the availability of innovative treatments in England. We therefore warmly welcome Professor Richards' fifth recommendation and the related announcement by NICE of a new scheme for treatments used in end of life settings.⁶²

19. The recognition within NICE's consultation of the principle that society places a higher value on the last few months of life, and is therefore willing to pay additional sums for end of life medicines, is a significant and positive development for health technology assessment within England and Wales. It reflects recent feedback from NICE's Citizen's Council and is to be welcomed.⁶³

20. However, clarification would be helpful as to whether this figure relates to the maximum eligible population according to the strict definition of the licensed indication or whether further refinements to this population are permitted in order to potentially improve its accuracy in the context of anticipated UK clinical practice. For example, adequately accounting for alternative/competitor medicines, identified sub-groups, exclusions due to co-morbidity and patient preference may generate alternative population

⁵⁹ Rarer Cancers Forum, *Taking Exception: An audit of the policies and processes used by PCTs to determine exceptional funding requests*, August 2008. Page 4.

⁶⁰ Professor Mike Richards, *Improving access to medicines for NHS patients*, November 2008. Page 62.

⁶¹ Department of Health, *Review of commissioning arrangements for specialised services*, June 2006.

⁶² Professor Mike Richards, *Improving access to medicines for NHS patients*, November 2008. Page 43.

⁶³ NICE, *Report of the NICE Citizens Council meeting—Quality Adjusted Life Years and the severity of illness*, July 2008. Page 4.

estimates compared to the strict application of epidemiological data. We would suggest such refinements are necessary to avoid overestimations of the population likely to be eligible for the new medicine/indication under question. We consider that adopting a flexible approach to the identified patient population for a drug is both consistent with the principles underpinning the proposed NICE scheme and the imperative of encouraging greater collaborative working between industry and NICE to ensure affordable access to medicines.

21. Adopting a rigid approach to interpreting the 7,000 patient limit would appear to be irrational. Providing a manufacturer can demonstrate that a drug will not in practice be used in a population exceeding 7,000 patients per annum, and that it satisfies the other criteria set out in Section 2, then the objectives of the scheme would be met. It would therefore appear to be in the interests of the NHS (in terms of value for money, cost effectiveness and innovation), patients (in terms of access to appropriate treatments), clinicians (in terms of ability to use appropriate treatments and apply clinical judgement) and industry (in terms of uptake of new products) to apply a flexible approach to the 7,000 patient limit. The Committee may wish to consider recommending that a flexible approach is adopted to the 7,000 limit, so ensuring that the spirit of the proposed scheme is implemented and that it does not unduly discriminate against any particular patient populations.

22. The proposed NICE scheme is a welcome move towards ensuring more appropriate interpretation of cost effectiveness data for end of life medicines. We consider that similar developments are also required in the methodology for calculating cost effectiveness data, including better reflecting the complexities of clinical trials, taking account that many new treatments for end of life conditions are additive and ensuring that quality of life measure are appropriate for the group of patients in question. The Committee may wish to consider recommending that NICE undertake further work with the Department of Health, industry and patient representatives to refine the methodology used for calculating cost effectiveness.

Introducing greater flexibility into pricing mechanisms

23. We welcome the recognition in Professor Richards' review of the role that industry play in partnership with the Department of Health in improving the availability of innovative medicines.⁶⁴ Measures such as patient access packages and flexibility in pricing both have a role to play in this respect.

24. It is important such pricing schemes genuinely deliver value for money to the NHS and reflect the value that a new medicine can bring to patients. Consideration needs to be given to the cost of making available a new medicine as well as the price at which a unit of new medicine is made available. For example, many end of life medicines have a high unit cost but a relatively low budget impact due to the size of the patient population in which they are used, as well as the duration of therapy. Therefore, although their price may be superficially high, they are also affordable.

25. Work by the Rarer Cancers Forum demonstrates that, if all patients were to have access to appropriate treatment, the additional cost would be approximately £175 million per year⁶⁵—a relatively small sum in the context of overall health expenditure. Conversely the total cost associated with an individual treatment is high, amounting to an average of £20,821⁶⁶—a considerable amount for any individual.

26. The Committee may wish to consider how pricing mechanisms for innovative medicines can best reflect value and affordability, as well as unit cost, in line with previous Committee recommendations.

HANDLING SITUATIONS WHERE PATIENTS HAVE TO PURCHASE ADDITIONAL DRUGS

27. If the measures outlined earlier in this document are implemented, then we believe that the number of patients who are forced into a position where they need to purchase additional drugs should be limited. However, we support the recommendation that there should be clear guidance to cover when and how patients should be able to “top up” in this way.

28. It is important to stress that there is no easy mechanism for determining this. First and foremost, patients should be able to gain access to the treatments which their clinicians believe they need on the NHS. However, given the difficulties inherent in any guidelines relating to the issue, we do believe that the ‘separate care’ approach is the least problematic.

29. It is important that safeguards are put in place ensuring that:

- the spirit of separate care is maintained and safeguarding against any inadvertent NHS subsidy for private treatment;

⁶⁴ Professor Mike Richards, *Improving access to medicines for NHS patients*, November 2008. Page 43.

⁶⁵ Rarer Cancers Forum, *Exceptional England—An investigation of the role of Primary Care Trusts in making cancer medicines available through exceptional cases processes*, October 2008. Page 5.

⁶⁶ Rarer Cancers Forum, *Exceptional England—An investigation of the role of Primary Care Trusts in making cancer medicines available through exceptional cases processes*, October 2008. Page 15.

- patients are made aware of appropriate unfunded treatment options and that no patient has the NHS care that they would have otherwise received withdrawn simply because they opt to purchase additional treatment; and
- the highest standards of clinical governance are maintained, including ongoing management of all aspects of a patients care by their multi-disciplinary team.

30. The clinical accountability steps outlined in the draft guidance issued by the Department of Health, together with Recommendation 11 of the Richards Review (“The Department of Health should take a lead on commissioning a national audit of demand for unfunded drugs and on the outcome of treatments, working closely with professional organisations and NHS managers.”) should ensure that the guidance is followed and that any unintended consequences are addressed. Nonetheless we are concerned by recent findings from a survey conducted by the JCCO that not all clinicians are informing patients of all appropriate treatment options.⁶⁷ We hope that Professor Richards’ recommendations on communications skills training and improving patient information should help address this issue. However, given the potential impact that a failure to inform a patient of appropriate treatment options could have on choice, clinical quality and safety, we believe that the Department of Health should commission a follow-up investigation to assess the extent to which these measures have improved clinical practice. The Committee may wish to consider recommending that the Department of Health commission a follow-up investigation so as to ensure that the system of separate care is properly implemented.

December 2008

Memorandum by Cancer Research UK (TF 24)

THE PURCHASE OF ADDITIONAL DRUGS BY NHS PATIENTS (“TOP-UP FEES”)

INTRODUCTION AND SUMMARY

Cancer Research UK welcomes the broad package of measures announced by Secretary of State Alan Johnson in response to Professor Mike Richards’ report on improving access to medicines for NHS patients. It strikes the right balance between improving access to effective medicines for cancer patients and not giving a blank cheque to pharmaceutical companies.

We particularly welcome recommendations on changes to the NICE process and increased flexibility in drug pricing. However, this report prompts a number of questions in the following areas to which we urge the Committee to find answers:

- How the changes to NICE will work in practice, and whether these will ensure that the right drugs are being approved.
- What changes will be made to exceptions panels to ensure that these are uniform, streamlined and transparent.
- How the establishment of arrangements to enable separate provision of NHS and private care will be managed and funded.
- The route through which patients will be able to access independent unbiased information on treatment options, including those not made available on the NHS.
- What system of support and independent advice will be available for patients making difficult decisions about whether to pay for their care.

The “top-up fees” issue is particularly pertinent for cancer. Increasing numbers of potential new anti-cancer treatments coming through the research pipeline, combined with rising public pressure for their provision by the NHS, means that the framework within which patients are getting access to these new treatments needs to be better defined.

As the major non-commercial cancer research funder in the UK, as a charity that has been involved for decades in high quality drug discovery and development, and a charity directly supported by one in ten people in the UK, we believe we have a valuable perspective in this debate. Our focus is, as always, on achieving the best outcomes for patients.

Cancer Research UK shares the view that we need to safeguard the fundamental principle of equal access to NHS care, free at the point of use, regardless of ability to pay. However, we also understand the challenge that this presents in respecting patients’ rights to use whatever resources they have to try to extend their lives, provided they get proper clinical advice.

⁶⁷ Professor Mike Richards, *Improving access to medicines for NHS patients*, November 2008. Page 18.

 COMMENTS AND QUESTIONS

These comments and questions are set out in sections according to the different elements of the recent package of announcements.

1. *NICE's proposals for changes to its appraisal system*

There are certainly specific cancer drugs that we believe NICE should be recommending for use that are falling foul of inflexibility within NICE's current appraisal processes. To this end, we welcome the proposals by NICE for changes to its current processes. However, there are a number of currently unanswered questions about NICE's proposals, which we urge the Committee to put to NICE.

We welcome NICE's commitments to audit the impact of these decisions in terms of number of patients who are subsequently prescribed these treatments and to track effectiveness. This would most appropriately be done in conjunction with other commitments to audit within the Richards review and, for cancer at least, actioned through the National Cancer Intelligence Network.

These are:

- How will the NICE's patient population "cut off" of 7,000 be applied in practice—which groups of patients will qualify?
- How does NICE plan to communicate this advice to its Committees? There are particular concerns about the interpretation of wording in this advice, such as the requirement that, to qualify for the higher cost-effectiveness threshold, medicines must offer a "substantial" extension to life.
- How does NICE intend to manage its audit commitments? The current proposals contain very little detail on what this will look like in practice. What plans do NICE have to link this with the work of the National Cancer Intelligence Network? How do NICE plan to avoid duplicating this effort?

2. *Drug pricing, the PPRS and the role of the pharmaceutical industry*

Drug pricing has been an important limiting factor in access to new medicines, particularly for cancer. When NICE come to appraise an expensive new cancer treatment, relative to the price set by manufacturers, they often find it too expensive in the often smaller population of end-stage disease patients with low life expectancy. The result is that treatments in areas of highest unmet medical need are increasingly unlikely to receive NICE approval.

Crucially, in the recent announcements, the Government and the pharmaceutical industry have together recognised the need for a fair pricing scheme/one that assesses a drug's value based on its effects on patients. Importantly, this deal adds flexibility to drug pricing, so NICE will be able to recommend more new cancer drugs for use in the NHS. We believe that pharmaceutical companies with confidence in the new treatments they are developing shouldn't be frightened of schemes aimed at pricing treatments according to their benefit.

In this area, we urge the Committee to ask the following questions of the Department of Health:

- How does the Department of Health consider that new negotiations for risk sharing or patient access schemes might fit into the system of NICE appraisal, to ensure that these appraisals are not unnecessarily delayed?
- What is the Department able to do in situations where pharmaceutical companies decide not to submit their drugs to NICE—as they predict a negative outcome and are not prepared to negotiate on price?
- What does the Department consider will be the impact of increasing NICE's threshold in some circumstances on the overall NHS drugs budget, and what extra funding will they be making available to ensure that local health providers can afford to make these expensive drugs available at the local level, without having to redirect money away from other services?
- What plans does the Department have for the future total drugs budget? The UK is directing too little financial resource towards cancer drugs, particularly those that have been licensed within the last 5–10 years. The medicines budget currently accounts for only four per cent of the NHS budget, suggesting that there should be flexibility within the UK health budget to increase our overall medicines spend.

3. *Support for local-decision making on funding and reform of exceptional case committees*

We welcome recommendations on the process of local decision-making about which drugs will be made available on the NHS. There are good models where joint decision-making, especially in cancer—such as the London Cancer New Drugs Group, are already benefiting both patients and local healthcare providers.

We believe that local providers should be supported through clear guidance on best practice, especially in the difficult area of the management and organisation of exceptional case committees. The system by which individual patients are judged exceptional or not needs to be standardised and made more transparent.

We look forward to the findings of the National Prescribing Centre inquiry into this subject, and urge the committee to ask the following questions of the NPC:

- What progress has the NPC made in developing guidance on local decision making and arrangements for exceptional case committees?
- What form does the NPC expect its recommendations will take, and what measures will they be introducing to ensure that PCTs are following this guidance?
- What recommendations will the NPC be making for improving the system of application to exceptional case committees?

We also welcome commitments to a national audit of demand for unfunded drugs and on the outcome of treatments. There is little accurate information on the actual level of demand for unfunded drugs. This will provide an important source of information for the NHS. For cancer we believe this would be most appropriately managed through the National Cancer Intelligence Network. This same route could also be used to capture information about medicines being approved through the new scheme proposed by NICE relating to treatment of rare and poor prognosis conditions.

4. *Developing a consistent approach for those patients who may still wish to purchase additional drugs*

We welcome recommendations in the Richards report that every Trust should clarify the arrangements under which NHS patients can pay for additional drugs. It is clear that currently different systems exist in different areas of the country.

We also welcome the commitment that the number of patients who may wish to purchase additional drugs should be kept to a minimum. However, despite this, arrangements will need to be in place to ensure that patients have this choice. Specific recommendations about how this should be implemented in practice will inevitably carry with them logistical difficulties. Our clinicians tell us that there are specific concerns around the practical implications of the recommendation that “private care should be carried out at a different time and a different place”. There is likely to be an up-front cost in establishing these arrangements and complications with finding an appropriate place to make these services available. There are also questions about what impact this separation will have on both the quality and safety of care being delivered to patients, some of whom will be very sick or have limited mobility.

There are also outstanding questions facing PCTs around how the cost of drugs will be established, and whether this is something that will happen at a national level. In addition, what the cost of these drugs should include, who will be responsible for administering these drugs, and what happens when a patient is showing response to treatment but can no longer afford to pay for their drugs, all need to be addressed. National guidance is also needed on whether patients will be expected to pay for genetic tests or scans associated with any treatment they are purchasing privately.

We hope the Committee will be able to gather evidence from individual Trusts on the extent of these problems and ask the Department of Health what additional support and funding will be available to address them.

5. *Informing patients about the implications of purchasing additional drugs*

The provision for patients of clear, relevant information, and accompanying support to help them interpret and understand the choices they are being given will be crucial.

We welcome commitments to provide additional training for clinicians in how they approach conversations with patients about treatments that are not routinely funded on the NHS. These conversations will also be difficult not only for patients but also for their doctors.

The last few years have seen vast improvements in the way we provide information to patients about their conditions, the treatment and care they are receiving and the choices they face. We agree that the existing patient information pathway initiative provides a good structure within which to deliver this information.

We also believe that this information and communication should be supported by the provision of advisors for patients to talk to when faced with difficult decisions about whether they should be purchasing drugs that the NHS is not making available. This would provide a safeguard for patients against undue pressure to pay for drugs, and will help them in reaching informed decisions in a protected environment with appropriate emotional support.

We look forward to participating in this important inquiry as it progresses and are keen to provide witnesses to give oral evidence, if requested.

December 2008

Memorandum by the American Pharmaceutical Group (TF 25)

TOP-UP FEES

INTRODUCTION

1. The APG supports the NHS and its founding principles of a comprehensive service, free at the point of use and based on need, not ability to pay. We also support the right of patients to make real choices about their care. The group therefore welcomed Professor Mike Richards' review of the consequences of additional private drugs for NHS care.

2. At the time of the review, it was increasingly apparent that the choices patients wished to exercise often went beyond the willingness of the NHS to pay. There was a clear need to assess why this was the case. However, it was also an important opportunity to address the low and slow access to new medicines for patients in the UK, and the changes needed to NICE's appraisals process to minimise the number of patients needing to top-up their care. With medicines being rejected by NICE which are deemed to be clinically effective but not considered "cost-effective", this issue continues to grow in significance.

3. Although the review has made some progress in terms of addressing these issues—for example, by recommending that NICE should take a more flexible approach to evaluating life-extending treatments for the terminally ill which exceed the current cost effectiveness limits—the APG remains fundamentally concerned about the assessment methodology used by NICE. It is this methodology that continues to cause inconsistencies and injustices in the healthcare system, and has given rise to the emergence of the debate around top-ups.

THE OUTCOMES OF THE RICHARDS REVIEW

4. The APG broadly welcomes the recommendation of the Richards' review that ensures that patients who choose to top-up their treatment privately are no longer denied NHS care as a result. The APG believes that patients should not be penalised for paying for medicines where a clinician has recommended a course of treatment not funded by the NHS, and is against charging a patient for the entire cost of their care in such an instance. We welcome the Government's decision to end this practice.

5. The findings of Professor Richards' review are also more in-keeping with the findings of Lord Darzi's NHS Next Stage Review published earlier this year. Lord Darzi's review emphasised the importance of a clinically-led NHS. The previous policy on top-ups seemed to undermine the principle of clinical judgement.

6. In response to the Professor Richards' review, NICE recognised that the current range of incremental cost per Quality Adjusted Life Year (QALY) considered acceptable within the standard criteria is not acceptable, particularly for end of life medicines. NICE is now conducting a consultation into guidance for appraising such medicines. The APG calls on NICE not to restrict or reserve this approach for end of life medicines. It may also be applicable in cases where there is a degree of uncertainty associated with the economic model.

7. In addition to the findings of the review, the Department of Health should seek to increase its efforts to communicate to the NHS and to patients that lack of NICE guidance, or pending NICE guidance, should not be a reason for withholding a treatment. There has long been NHS guidance instructing PCTs to take account of the views of other recognised bodies, such as the Scottish Medicines Consortium when making their own assessments of new medicines ahead of a NICE review decision. Furthermore, the draft NHS Constitution supports existing NHS guidance stating patients have the right to NHS treatment approved by NICE, whilst PCTs will also have to make clear their reasons for not funding medicines.

8. Under the terms of NICE's review of end of life treatments, top-ups should become the exception rather than the rule for patients. On the occasions that patients do choose to top-up their care, action must be taken to ensure that all the costs associated with doing so are transparent for the patients and consistent across all PCTs. We have heard anecdotally that some patients are incurring large dispensing costs on top of their medicine costs, which would seem to go against the spirit of Professor Richards' proposals.

 BEYOND THE REVIEW: IMPROVING NICE PROCESSES

9. The APG continues to have serious concerns about the assessment methodology used by NICE. We would advocate that the cost per QALY should not be used as a rule for decision making, but rather as a tool. It should also pay due consideration to issues affecting the patient such as severity of disease.

10. While we see the appeal in earlier appraisals as a way to reduce the postcode lottery we have long advocated that using the current NICE processes to evaluate medicines too early in their lifecycle is fraught with methodological difficulties. This is because the evidence base is relatively immature and the modelling leads to too much uncertainty.

11. In addition, a broader definition of “value” needs to be developed, and the cost per QALY should not be used as the only criteria for decision-making. The formal assessment should also include a broad range of clinical and societal factors that have a real impact on patients and carers. One example is the potential to alleviate the stress, anxiety and burden of care imposed on relatives and carers in Alzheimer’s disease. This is a significant benefit not routinely included in decision-making.

12. NICE’s methodology has proved particularly controversial for patients suffering terminal illnesses, where NICE’s system does not reflect the fact that relatively small extensions to life are highly prized by patients and their families. The current cost-effectiveness threshold should be reviewed and consideration should be given to how it could be applied with more flexibility within the current system.

13. In succession to the findings of Professor Richards’ review, the APG would like to see a review of the health technology assessment process to allow access to more innovative medicines. Moreover, NICE processes need to take into account that as you move the appraisal nearer to launch there is less data available for analysis.

AFFORDABILITY OF MEDICINES

14. Much has been made in recent months of the affordability of new medicines in the UK, with some suggestions that this has led to the need for patients to resort to top-ups. In reality, this issue needs to be considered in the correct context. While NHS expenditure overall continues to grow, the UK NHS medicines bill as a percentage of total NHS expenditure, has declined with medicines accounting for only 11% of total NHS expenditure and branded pharmaceuticals for 8% of the total budget. This is a lower share of the NHS budget than 10 years ago despite a year-on-year growth in the number of prescriptions issued. There is clear evidence to show that overall spend of medicines is one of the most stable components of NHS costs.

15. The pharmaceutical sectors’ contract with society means that once our medicines lose patent protection, Government can expect to derive savings through lower prices resulting from generic competition. The saving achieved from off-patent medicines is an essential part of the medicines lifecycle, releasing budget for newer medicines; whilst the patent period rewards and incentivises innovation. The UK already has a high level (c 80%) of generic prescribing and industry research shows that some of the most widely used medicines in the NHS will come off patent by 2012, generating an estimated £3.4 billion worth of savings. These savings should be taken into account when discussing the affordability of new medicines.

CONCLUSION

16. Overall, the APG welcomes the findings of Professor Richards’ review into the consequences of additional private drugs for NHS care. In particular, we welcome the fact that NICE is now seeking to adopt a more flexible approach to end of life treatment, thereby limiting the number of drugs that have to be purchased privately by patients, and calls on the Institute use this opportunity to reconsider its wider appraisals process. We would like to see a review of the way NICE assesses new medicines, with less reliance on the cost per QALY and greater attention given to the wider benefits of a medicine and the value patients and their carers place on extension of life.

17. The APG welcomes the decision to end the practice of penalising patients for paying for medicines where a clinician has recommended a course of treatment not paid for by the NHS by charging them for the entire cost of their care in such an instance.

18. If Professor Richards’ review is implemented in full, and as intended, the issue of top-ups should be the exception rather than the rule. On the occasions where patients choose to top-up, all costs associated with doing so must be consistent and transparent.

19. In view of NICE’s review on appraising end of life medicines, moves to improve patient access to new medicines in the new PPRS agreement, and following the publication of Professor Richards’ review, the APG believes this is an important opportunity to tackle the inconsistencies and injustices inherent in the healthcare system. However, the success of this opportunity depends on the progress of these three issues over the next 12 months.

20. As part of PPRS negotiations and the Darzi review, the Department of Health ran a series of focus groups to look at additional factors that could be included in a wider perspective for NICE, including: productivity benefits in terms of value to economy of individuals returning to the workplace; wider public sector impacts of treatment; and both health and non health benefits to carers. We believe this work should now be extended and the feasibility of new approaches tested.

21. We suggest that the affordability of new medicines is not currently the key issue and would draw attention to the fact that the proportion of NHS budget spent on medicines has declined (Source: ABPI analysis of Department of Health data; see Appendix 2) over the last 10 years. It is not the case that the NHS “cannot afford” new medicines. The NHS has received unprecedented funding in recent years, and is currently in surplus. The APG is hopeful that the outcome of Professor Richards’ review, along with the changes proposed by NICE in appraising end of life medicines, and the improvements to patient access included within the new PPRS deal, can lead to substantial improvements in delivering fair and consistent healthcare to all.

December 2008

APPENDIX 1

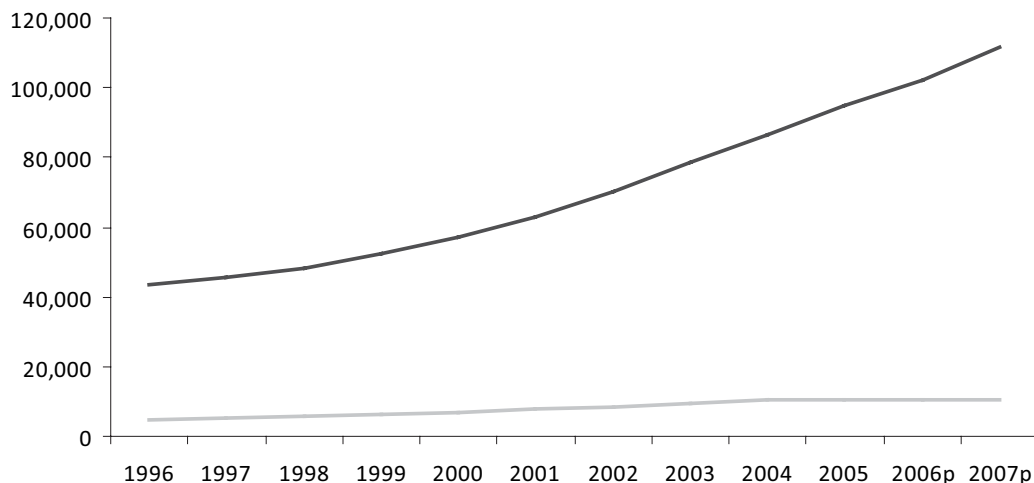
ABOUT THE APG

The American Pharmaceutical Group (APG) member companies comprise some of the largest and most innovative research companies in the UK. As a group, our time and investment (£1.5 billion each year) is spent on the discovery and development of new and innovative medicines. The product portfolios of member companies include some of the world’s most important medicines, combating life-threatening illnesses such as heart disease, cancer, mental health and diabetes. The contribution of the industry to the UK’s science base was highlighted in the Department for Innovation, Universities and Skills’ 2007 R&D Scoreboard, which identified the pharmaceutical sector as the biggest contributor to R&D growth, ahead of the aerospace, defence and IT sectors.

The 10 member companies are Abbott; Amgen; Bristol-Myers Squibb; Janssen-Cilag Ltd; Lilly; Merck Sharp & Dohme (MSD); Pfizer; Procter & Gamble Pharmaceuticals; Schering-Plough; and Wyeth.

APPENDIX 2

ABPI analysis of DH figures on how loss of exclusivity savings that pay for new products have ensured the NHS spend on medicines has remained stable for a number of years.



Source: PPA, ONS, DH, HAW, ISD, IMS dataview.

Memorandum by the British Medical Association (TF 26)

THE PURCHASE OF ADDITIONAL DRUGS BY NHS PATIENTS—“TOP-UP FEES”

The British Medical Association (BMA) is an independent trade union and voluntary professional association which represents doctors from all branches of medicine all over the UK. It has a total membership of over 140,000.

EXECUTIVE SUMMARY

- The BMA is committed to the continuation of a tax-funded NHS which provides care based on clinical need, not the ability to pay. The principle of equity remains central to our vision of the NHS and the core values that underpin it. Whilst we still maintain the view that upholding the equity principle should be strongly supported, the ultimate effect of past policy—to deny those patients who purchase additional drugs to access to all other NHS-funded care associated with that condition—appeared to be unduly punitive.
- The BMA has cautiously welcomed the report of the Richards' Review and its recommendations. The BMA had been hopeful that the review would deliver, as a priority, an interpretation of policy that was practical in terms of both managing the equity dilemma and avoiding the issue of conflict of interest in respect of NHS clinicians inducing payments for care.
- A much wider debate is required to allow for the examination of the larger tension that exists between the principles of equity and autonomy in a modern, tax-funded health service where patient choice plays an increasing role.
- The BMA does not advocate a permissive view on the introduction of further “co-payments” into the NHS nor a common acceptance of “top-ups”.
- The BMA supports the view, promoted by the report of the Richards' Review, that privately and publicly-funded elements of care should remain as separate as is practicable with patients normally expected to engage a private provider and/or clinician distinct from that directly administering the associated NHS care, or ensuring a clear separation in time where appropriate.
- It is crucially important that there is close monitoring of the implementation of the draft Department of Health guidance on NHS patients who wish to pay for additional private care.
- The BMA fully supports moves to improve the scope and sensitivity of advice used to inform decision making and the making of recommendations. Therefore, the proposed changes to NICE's technology appraisal process which will result in supplementary advice being made available to NICE's Appraisal Committee initially appear to be welcome.
- The BMA strongly believes that any consideration being given to establishing new means to meet the small but growing demand to mix elements of privately and publicly-funded care should be founded on a precautionary approach.
- The BMA would welcome a further examination of the function of the National Institute for Health and Clinical Excellence (NICE) including a review of its appraisal processes—in particular the speed and transparency of decision-making in this area—and how the availability of, and access to, all NICE-approved treatments in the NHS can be safeguarded with a view to ensuring that the number of drugs not available on the NHS is kept to an absolute minimum.

OVERVIEW

1. The BMA is committed to the continuation of a tax-funded NHS which provides care based on clinical need, not the ability to pay. The principle of equity remains central to our vision of the NHS and the core values that underpin it. The guidance and policy recently subjected to scrutiny by the Richards' Review was often interpreted as providing a safeguard against the possible erosion of this principle. Under this interpretation, past policy aimed to ensure that all NHS patients could expect to receive the same level of care for similar conditions in the NHS—a patient could not be both a private and a NHS patient for the treatment and thus secure additional benefit above that provided routinely by the NHS due to their ability to pay.

2. However, whilst the BMA remains of the view that upholding the equity principle should be strongly supported, it was our view that the ultimate effect of past policy—to deny those patients who purchase additional drugs access to all other NHS-funded care associated with that condition—appeared to be unduly punitive. Our members, in large part, expressed disquiet at the prospect of those patients choosing to exercise their right to purchase additional drugs having their access to free, NHS care denied as a result. This appeared contrary to principles of social justice and may well fail under legal challenge.

3. Consequently, the BMA has cautiously welcomed the report of the Richards' Review and its recommendations. The BMA had been hopeful that the review would deliver, as a priority, an interpretation of policy that was practical in terms of both managing the equity dilemma and avoiding the issue of conflict of interest in respect of NHS clinicians inducing payments for care. Though one might consider that the recommendations (in combination with the associated draft guidance issued by the Department of Health) address these issues we have real concerns that the framework outlined suffers from a number of inconsistencies and contradictions and that there remains an opportunity for misinterpretation and misunderstanding such that NHS managers and NHS doctors will remain confused, and, most importantly, NHS patients might find the continuity and standard of their care compromised.

4. The Health Select Committee's own interest in the “top-ups” dilemma reflects a growing awareness of the pressures currently being experienced in the NHS as a result of ever-rising public and patient expectations, the increasing costs of new medicines and technologies and the realities of an NHS operating

in the context of finite resources. However, it is noteworthy that the Richards' Review and the Committee's inquiry have limited its focus to the issue of additional non-NHS drugs, rather than treatment more broadly. It is our belief that a much wider debate is required to allow for the examination of the larger tension that exists between the principles of equity and autonomy in a modern, tax-funded health service where patient choice plays an increasingly central role. The necessary debate must address how best to reconcile the role and operation of an NHS wedded to the habitual exercise of rationing healthcare with the growth of a consumer-orientated, information-rich society.

5. Given the complexity of these issues—social, economic, legal and ethical—we have been disappointed by both the limited scope of the recent review and related policy discussions (eg the Department of Health draft guidance and NICE consultation) in respect of their rather narrow focus, compounded by the often challenging time-tables to which they have been conducted. It is not certain that, despite the contemporary acute interest in the “top-ups” issue, the relevant wider debate on funding pressures in the NHS, and with it matters such as “rationing”, the “postcode lottery”, patient choice and the implications of devolving much greater responsibility for determining the scope and nature of services to the local level, will be encouraged. The BMA believes that initiating a mature and open dialogue with the public, patients, health professionals and others on these broader issues is imperative in order to maintain confidence in the NHS and safeguard its future. It would be a concern if further policy development in this area and changes in practice were to take place without a dialogue involved considered scrutiny of the tension between the NHS's stated aims of comprehensiveness and universality and the evident reality of rationing that confronts clinicians, managers and patients on a daily basis.

POSITION VIS-À-VIS “TOP-UPS”

6. As noted above, the BMA supports the direction of travel outlined in the report of the Richards' Review. It is our favoured position that where—following the advice of clinicians on the potential efficacy of a drug in the category described above—patients wish to undertake drug treatment on a private basis, these patients should still receive their NHS care free at the point of use. In other words, patients should be granted the right to access privately-funded drugs in combination with NHS care and to do so without penalty—they should not have associated NHS care denied to them. This approach safeguards the freedom of individuals to move between NHS and private provision without disadvantage and also ensures NHS patients can choose to have access to clinically effective drugs that have not been approved for use in the NHS.

7. The change to guidance arising from the Richards' Review may, of course, be regarded as subtle or significant dependent on one's personal views. The issue often provokes a polarised response with arguments driven by strong beliefs concerning the principles of liberty on one hand and equity on the other. The BMA has not shied away from the complexities of the debate nor from the fact that managing a system as currently proposed will prove challenging, in particular upholding the principle of “separateness”, maintaining continuity of care and financial administration. However, we recognise that the changes are a necessary and pragmatic step to address the consequences of shifting societal trends which make policy in its current guise unsustainable.

8. Nevertheless, as noted above, such a change should not be undertaken in the absence of much wider consideration. It carries with it the potential to add momentum to the growing demands and expectations it is attempting to address and that continue to put strain on the values that underpin the NHS. We do not advocate a permissive view on the introduction of further “co-payments” into the NHS nor a common acceptance of “top-ups”.⁶⁸ Whilst the BMA supports patient choice and personal autonomy we also are clear that the wish to promote liberty in this manner must be balanced with our own commitment to ensure that the NHS maintains its principles of equity and being free at the point of use.

VIEWS ON DH GUIDANCE ON NHS PATIENTS WHO WISH TO PAY FOR ADDITIONAL PRIVATE CARE

9. The BMA supports the view, promoted by the report of the Richards' Review, that privately and publicly-funded elements of care should remain as separate as is practicable with patients normally expected to engage a private provider and/or clinician distinct from that directly administering the associated NHS care, or ensuring a clear separation in time where appropriate. The draft DH guidance on NHS patients who wish to pay for additional private care attempts to put forward a practical framework in which this approach can be taken forward.

10. A significant concern remains that the guidance does not sufficiently address what is likely to be the increasing incidence of patients who express the wish to be able to “top up” their care within the NHS itself. Here then, rather than a patient augmenting NHS care by privately accessing drugs outside the bounds of the NHS and then returning to the NHS for further care, the patient seeks to pay the NHS directly to administer treatment not currently available for free on the NHS (as a result of a NICE assessment).

⁶⁸ We define “co-payments” as a charge on patients for NHS services and define “top-ups” as charges on patients levied by organisations for excluded or non-NHS treatments and services.

11. We readily accept that in certain instances clearly separating elements of private and NHS care in time or space or by provider will not be practicable, either for clinical reasons or due to capacity issues. The guidance does attempt to accommodate such circumstances. However, we believe this position remains at a significant remove from the development of a system wherein patients expect, or are in fact enabled to, make additional payment or “top up” to the NHS directly in order to enhance a single episode of NHS care. The acceptance of this practice would inevitably raise the possibility of the NHS routinely permitting NHS patients with similar needs being treated differently as a result of an individual’s ability to pay. This would be to the detriment of the equity principle that is at the heart of the NHS. We would therefore wish to see the common acceptance of such a situation avoided.

12. It is therefore crucial that as the guidance is formally introduced its implementation is closely monitored. In allowing for the concurrent provision of NHS care and non-NHS funded drug treatments (where this is clinically appropriate) it must be ensured that such episodes are suitably regulated so as not to diminish the levels and standard of care available to all other NHS patients, nor cause detriment to the NHS financially eg alongside the cost of the additional drugs, patients choosing this route would be accountable for any extra expense incurred as a result of the drugs’ use and its related administration costs.

13. Of great concern to the BMA are the potentially very wide-ranging implications that the draft guidance, and the new principles it is set to support, will have for doctors, in particular, and for other health professionals. The guidance apportions significant extra responsibilities to clinicians in respect of their role as patients’ advocates and in terms of providing support to help patients navigate through treatment and funding pathways. We are concerned that the doctor-patient relationship will suffer if this bond is strained through a requirement to address financial considerations as part of the consultation process.

14. Returning to the principle of “separateness”, this has particular resonance for consultants who are already subject to a code of conduct on private practice which addresses this issue. The BMA is concerned that the new duty of informing patients about “all” available care options, including those administered privately alternatives, may generate irreconcilable conflict with existing code of conduct. Moreover, there is in our view some internal inconsistency within the Department of Health guidance. Section 5.1 states:

“NHS clinicians should make all care options available to patients, including those not offered by themselves or their NHS organisation, in line with GMC guidance”.

And goes on to note that:

“In the course of their NHS duties and responsibilities consultants should not initiate discussions about providing private services for NHS patients, nor should they ask other NHS staff to initiate such discussions on their behalf”.

15. In so doing, the guidance creates an environment in which consultants will be required to make known to patients all care options available, including those where the patient may be required to pay for access, yet ignores the fact that in many instances the very same consultant is likely to be the only person thereabouts who qualified to talk about the evidence base for such a therapy, and more importantly with regard to the inherent conflicts, is perhaps the only person qualified to deliver it in the private sector. The BMA is continuing to address this dilemma as we prepare to respond formally to the Department of Health consultation.

VIEWS ON NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE CONSULTATION ON “APPRAISING END OF LIFE MEDICINES”

16. The BMA fully supports moves to improve the scope and sensitivity of advice used to inform decision making and the making of recommendations. Therefore, the proposed changes to NICE’s technology appraisal process which will result in supplementary advice being made available to NICE’s Appraisal Committee initially appear to be welcome. However, the supplementary advice, in this particular case, will be employed in such a manner as to alter significantly the *raison d’être* of the Appraisal Committee as originally conceived. Ultimately, in enabling access to medicines licensed for terminal illnesses which, although offering demonstrable and substantial survival benefits over current NHS practice, are deemed not to represent a good use of NHS resources, the proposed changes require NICE to alter the fundamental basis upon which it has historically made its recommendations. It is therefore a concern that what is potentially a very noteworthy change has been the subject of such a short consultation.

17. Notwithstanding this concern, the BMA can see certain benefits arising from asking the Appraisal Committee to place additional weight on proven survival benefits in patients with terminal illness and short life expectancy, not the least that this is likely to have the effect of reducing the incidence of “top-up” payments by increasing the range of drugs available to those sections of the population most vulnerable to the “top-up” dilemma.

18. There remains some unease with regard to the nature of the justification which will allow the standard £30,000 per QALY ceiling to be set aside. Whilst the consultation outlines a set of criteria defining the circumstances in which the supplementary advice would be appropriately provided it is not clear how these thresholds were agreed. The BMA would therefore welcome further information on the decision to limit the population to 7,000 patients a year. Whilst this criteria necessarily means the proposals will impact on only

the less common conditions it is unclear how this decision can be justified to patients comprising a further intermediate group of conditions (more than 7,000 but still uncommon) that will not benefit from the revised guidance.

19. Similarly, clear justification is also lacking with regard to the condition that for a medicine to be considered under this supplementary advice it would need to be indicated for the treatment of patients with a diagnosis of a terminal illness and who are not, on average, expected to live for more than 24 months. This is likely to be problematical. For example, there is an ongoing debate around Alzheimer disease and the benefits of starting treatment early to delay or arrest the progress of the disease before it has become debilitating. The consensus appears to be forming such that health professionals advocate treatment sooner than later to gain maximum benefit. The 24 month condition would not be sensitive to episodes of this kind.

20. On a more general note, the BMA would welcome a further examination of the function of NICE including a review of its appraisal processes—in particular the speed and transparency of decision-making in this area—and how the availability of, and access to, all NICE-approved treatments in the NHS can be safeguarded with a view to ensuring that the number of drugs not available on the NHS is kept to an absolute minimum.

CONCLUSION

20. The BMA strongly believes that any consideration being given to establishing new means to meet the small but growing demand to mix elements of privately and publicly-funded care should be founded on a precautionary approach. Discussion concerning an extension of the capacity for NHS patients to mix these elements of care must involve all stakeholders and be undertaken on the basis of an agreed understanding that any changes should be extremely limited given that the NHS would always be expected to strive to provide the highest levels of care available. No policy change must allow the NHS offering to be reduced now or in the future.

December 2008

Memorandum by Bupa (TF 27)

THE PURCHASE OF ADDITIONAL DRUGS BY NHS PATIENTS—“TOP-UP FEES”

EXECUTIVE SUMMARY

Bupa welcomed the Richards' Review for two reasons:

- DH/NHS policy guidance on this matter was unclear and unevenly interpreted;
- Bupa expects funding pressures on the NHS in England to increase in the coming five years as rates in real annual funding increases reduce from 7%+ (2002–08) to 3%–4% (2008–10) to 1.5%–2% from 2010. Bupa's prima facie assumption is that this may increase demand for drugs that are not funded on the NHS.

In 2006 Bupa commissioned a report⁶⁹ (*Mind the Gap*) on how to sustain improvements in the NHS beyond 2008. Bupa believes that the underpinning assumptions and concerns expressed in that report remain valid. The report summarised modelling of national healthcare needs up to 2015 undertaken for Bupa by NERA and reviewed by Frontier Economics. The modelling pointed clearly to the need for annual budget increases in the order of 4.9% (real) through the period 2008–15 if gains made in the period 2002–08 are to be sustained. The modelling assumed that the NHS makes real productivity gains of 1% a year through the latter period. The conclusion of that report was that the Government may need to use a combination of instruments (including NHS productivity gains, NHS co-payments, and private supplementary (top-up) up funding) in order to achieve the overall levels of funding necessary to meet the “needs” of patients through the period 2008–15.

The Richards Review was a therefore a timely and useful vehicle for examining: the future demand for and supply of a key (but not the only) medical technology; and the optimal role of additional (non NHS) funding in the English health system.

Bupa welcomed Professor Richards' key Recommendations 7 and 8. Bupa was pleased to note the Secretary of State's immediate and unequivocal acceptance and endorsement of these recommendations in his statement to the House on 4 November.

Bupa also welcomes the Recommendations 1 and 2 in Professor Richard's Report.

⁶⁹ http://www.bupa.co.uk/about/html/pr/health_finance_project.html

Bupa however has one important reservation about Professor Richards' report which it would wish to bring to the Select Committee's attention. The Professor's recommendations flow logically from his selection of "Option 3—Separate Care"—as his preferred option. Option 3 would have been Bupa's second choice.

Bupa's first choice would have been "Option 2—Voucher Scheme". Only this option (amongst those identified) creates a situation where the role of additional financing would have become wholly "supplementary", according to the terms of the OECD taxonomy of health insurance. In lay terms, only Option 2 is a true and full "top up" option.

The potential impact of such a change in role was illustrated in Bupa's submission to the Richards' Review by reference to Israel and The Netherlands. The Select Committee may wish to consider whether such a change in the role of additional financing is now (or may at a future date be) desirable and/or necessary in England.

Bupa also wish to draw to the Select Committee's attention their analysis of the draft EU Directive on Cross Border Healthcare, published on 2 July 2008.⁷⁰ Bupa believe that if the draft Directive completes to EU co-decision process as published it will, in simple terms, lead to a situation where NHS patients will be able to travel abroad for treatment and be reimbursed up to a maxima of the nearest equivalent NHS treatment. In that case, if patients travel abroad, they will, in effect, have the freedom to "top up" found in "Option 2—Voucher Scheme". This may lead to a challenge through the European Court of Justice from an English patient who paid the greater expense of receiving additional care in England under Option 3.

INTRODUCTION

1. Bupa recognises that the NHS is a highly valued national institution that commands support across the mainstream political parties. Bupa also recognises both the unprecedented financial investment that has been made in the NHS over the past five years and the considerable political, policy making and managerial efforts that have accompanied that investment in order to modernise the NHS. Bupa has actively supported these reforms and participated in them. Bupa continues to support the NHS modernisation agenda, principally through its involvement in the NHS FESC programme and other support for World Class Commissioning. Bupa is also a major customer of the NHS.

2. As a customer focused organisation delivering diverse high quality health and care services to 10 million people across a number of geographic markets (including UK, Spain, Australia, New Zealand, Saudi Arabia, Hong Kong and USA) Bupa has considerable experience of delivering services that meet the needs of patients and consumers of healthcare which operate within a variety of funding systems alongside a significant state health system. Accordingly, Bupa hopes it can contribute real experience and practical examples to this important Inquiry.

3. Bupa welcomed the Secretary of State's announcement on 17 June 2008 that he had asked Professor Mike Richards to under a Review to examine policy relating to patients who choose to pay privately for drugs that are not funded on the NHS.

4. Bupa made a single submission to the Richards' Review, and the parallel Conservative Party Consultation, on 21 August.

5. Bupa welcomed the Richards' Review for two reasons: one, DH/NHS policy guidance on this matter was unclear and unevenly interpreted; and two Bupa expects funding pressures on the NHS in England to increase in the coming five years as rates in real annual funding increases reduce from 7%+ (2002–08) to 3%–4% (2008–10) to 1.5%–2% from 2010. Bupa's prima facie assumption is that this may increase demand for drugs that are not funded on the NHS.

6. In 2006 Bupa commissioned a report⁷¹ (*Mind the Gap*) on how to sustain improvements in the NHS beyond 2008. Bupa believes that the underpinning assumptions and concerns expressed in that report remain valid. The report summarised modelling of national healthcare needs up to 2015, undertaken for Bupa by NERA and reviewed by Frontier Economics. The modelling pointed clearly to the need for annual budget increases in the order of 4.9% (real) through the period 2008–15 if gains made in the period 2002–08 are to be sustained. The modelling assumed that the NHS makes real productivity gains of 1% a year through the latter period. The conclusion of that report was that the Government may need to use a combination of instruments (including NHS productivity gains, NHS co-payments, and private supplementary (top-up) up funding) in order to achieve the overall levels of funding necessary to meet the "needs" of patients through the period 2008–15.

7. Bupa therefore welcomed the Richards Review as a vehicle for examining: the future demand for and supply of a key (but not the only) medical technology; and the optimal role of additional (non NHS) funding in the English health system.

⁷⁰ http://ec.europa.eu/health/ph_overview/co_operation/healthcare/cross-border_healthcare_en.htm

⁷¹ http://www.bupa.co.uk/about/html/pr/health_finance_project.html

BUPA'S REACTION TO THE RICHARDS' REVIEW REPORT AND THE GOVERNMENT'S RESPONSE AND THE GOVERNMENT'S DRAFT REVISED GUIDANCE ON NHS PATIENTS WHO WISH TO PAY FOR ADDITIONAL PRIVATE CARE

8. Professor Richards' Report was published on 4 November. On the same day the Secretary of State for Health made a statement in the House of Commons, outlining the Government's response. Simultaneously the Government issued draft revised "Guidance on NHS patients who wish to pay for additional private care". This Guidance was introduced with immediate effect—and is being consulted on in parallel by the Government (deadline for consultation 27 January 2008).

9. Bupa welcomed Professor Richards' key Recommendations (7) and (8) that:

"The Department of Health should clarify the policy on how the NHS should handle situations where a patient wishes to purchase additional treatment. The objective should be to ensure consistency of practice across the NHS" and

"The Department of Health should make it clear that no patient should lose their entitlement to NHS care they would otherwise have received, simply because they opt to purchase additional treatment for their condition."

10. Bupa was pleased to note the Secretary of State's immediate and unequivocal acceptance and endorsement of these recommendations in his statement to the House.

11. Bupa also welcomes the following Recommendations in Professor Richard's Report:

Recommendation 1—re improving the timeliness of the NICE decision making process; and

Recommendation 2—re promoting collaborative decision making by PCTs.

12. Bupa does not disagree with any of Professor Richards numbered recommendations—though it questions whether some of them should be a priority in the present economic climate.

13. Bupa however has one important reservation about Professor Richards' report which it would wish to bring to the Select Committee's attention. The Professor's recommendations flow logically from his selection of "Option 3—Separate Care"—as the preferred option in his Option Appraisal set out in Chapter 5 of his Report. "Option 3—Separate Care"—would have been Bupa's second choice.

14. Bupa's first choice would have been "Option 2—Voucher Scheme". Only this option (amongst those identified) creates a situation where the role of additional financing would have become wholly "supplementary", according to the terms of the OECD taxonomy of health insurance⁷²—which Bupa commends for policy making purposes. Only Option 2 would wholly change the role of additional financing in the UK (excluding NHS co-payments or associated "complementary insurance") from "duplicative" to the NHS to "supplementary" to the NHS. In lay terms, only Option 2 is a true and full "top up" option—in which patients pay only the marginal cost of the marginal benefit received from "topping up" their NHS entitlements.

15. The potential impact of such a change in role was illustrated in Bupa's submission to the Richards' Review (page 16ff) by reference to Israel and The Netherlands. The Select Committee may wish to consider whether such a change in the role of additional financing is now (or may at a future date be) desirable and/or necessary in England.

16. Bupa also wish to draw to the Select Committees attention their analysis of the draft EU Directive on Cross Border Healthcare, published on 2 July 2008.⁷³ Bupa believe that if the draft Directive completes to EU co-decision process as published it will, in simple terms, lead to a situation where NHS patients will be able to travel abroad for treatment and be reimbursed up to a maxima of the nearest equivalent NHS treatment. In that case, if patients travel abroad, they will, in effect, have the freedom to "top up" found in "Option 2—Voucher Scheme". This may lead to a challenge through the European Court of Justice from an English patient who paid the greater expense of receiving additional care in England under Option 3.

17. In Bupa's view "Option 1—Either NHS or Private Care" would have been high undesirable. Professor Richards' Report (paragraph 4.16) describes a "strong consensus from stakeholders that withdrawing NHS care was wrong". He further amplifies "words used by patients and the public to describe this approach include "despicable" . . . "cruel" . . . "perverse" and "unjust".

18. In Bupa's view "Option 5—NHS tops ups" would have lead to the further conflation between the distinct roles of the NHS as a "universal and comprehensive" primary health services financing vehicle (its dominant statutory role) and its subordinate and partial role as the publicly owned provider of health services, primarily to the NHS. A clear inference can be drawn from the reported consultations in the Richards' Review Report that the views and interests of citizens and patients are not always identical to those of "NHS staff".

19. In Bupa's view "Option 4—Simultaneous care" would have been an acceptable option whether in an NHS or independent sector provider context. Indeed this situation already pertains re NHS funded social

⁷² <http://www.oecd.org/dataoecd/24/52/31916207.pdf>

⁷³ http://ec.europa.eu/health/ph_overview/co_operation/healthcare/cross-border_healthcare_en.htm

care, NHS amenity beds and any NHS hospitals that still have “private bed allocations” as opposed to separate “Private Patient Units”.

December 2008

Memorandum by the Royal College of Physicians (TF 28)

TOP-UP FEES

The Royal College of Physicians (RCP) plays a leading role in the delivery of high quality patient care by setting standards of medical practice and promoting clinical excellence. We provide physicians in the United Kingdom and overseas with education, training and support throughout their careers. As an independent body representing over 20,000 Fellows and Members worldwide, we advise and work with government, the public, patients and other professions to improve health and healthcare.

1. SUMMARY

1.1 When he announced that patients were now free to buy additional secondary care services, without risking their publicly funded entitlements, the Secretary of State for Health published draft guidance for staff in the NHS on how to proceed in such situations.⁷⁴ For the purposes of this exercise we have chosen to comment exclusively on the proposed mechanics of the new system laid out in that document.

1.2 The view of members whose opinion we canvassed was that while the guidance provided a clear statement of separateness, it did not explore in sufficient depth the kinds of steps that may be required to preserve the space between public and privately funded services, while ensuring high quality continuity of care.

2. SEPARATE CARE—ITS MEANING AND EXPRESSION IN CLINICAL SETTINGS

2.1 To protect against the risk that NHS monies are used to subsidise private care, it has been recommended that clinicians adhere to the following principles when commissioning additional private services on behalf of a patient:

- The question of whether a procedure or treatment is to be privately or publicly funded must be established clearly beforehand by all parties.
- Patients are not permitted a “pick and mix” approach (whereby they pay to upgrade individual elements of their existing NHS care).
- Private care and publicly funded care should be kept as separate as possible, and ideally be carried out at a different time and place (with the obvious caveat that patient safety is of overriding importance).

Keeping care separate while ensuring continuity

2.2 The guidance mandates that patients receiving top ups should do so during a separate episode of care, either in a separate environment, or at different time to NHS care. In many cases, units are fully occupied and so would not be able to deliver top up care at a separate time without disadvantaging other NHS patients. When this situation arises clinical governance requirements will mean patients must be moved to a separate environment specifically set up for the task at hand. While some hospitals have private units (such as for the delivery of systemic anti-cancer therapies) not all trusts do, and would be unable to provide patients who desired them with immediate access to top ups. We expect therefore that some patients may need to be referred to a new Consultant at a different unit. This may cause problems for continuity of care and additional protocols will need to be developed to minimise the risks inherent in patients switching between units to receive different treatment regimens.

2.3 Much of this may hinge on putting in place appropriate data protection and data management policies. In order to facilitate continuity we recommend that there should be a formal agreement in place between any private provider and the NHS clearly setting out the standards for information flows in both directions so that the clinical governance arrangements of both institutions are satisfied. While potentially controversial, the hypothetical case studies in below explain why this might become necessary, though it also acknowledges the inherent practical difficulties of changing the present recording system.

⁷⁴ Department of Health (2008) *Guidance on NHS patients who wish to pay for additional private care—A consultation*. HM Government: London.

Case Study A

If a patient has part of their chemotherapy treatment given privately and suffers toxicity as a result, the expectation within the guidance is that the NHS will manage the complications. To do so safely, NHS staff will need access to the full and complete treatment details, ideally entered into the NHS record by private staff in real time. One way to do this might be to grant specific personnel within private institutions VPN access through the NHS firewall in order to keep the NHS record entirely up to date. This may require the lowering of the data protection barriers between NHS and private providers.

Case Study B

Equally, in cases where the private drugs are administered within an NHS unit, the drug will need to go through the usual customary clinical governance systems. Specifically, the drug will have to be entered into a clinical care algorithm, either using proformas, or preferably, a computerised prescribing system. This poses problems for combination regimens as it would be unsafe to have the NHS drug date stored on one computer, and the private on another. However to include them both on the same record makes billing difficult and will pose additional challenges for the nursing and pharmacy staff involved in the administration of the drug.

3. MANAGING EXPECTATIONS

3.1 We also believe that the guidance is presently inadequate when it comes to how clinicians discuss the potentially sensitive topic of top up drugs with patients. The *Code of Conduct of Private Practice* prohibits clinicians from initiating conversations about private treatment, though we are obliged to provide the full range of advice. Clearer guidance is needed for doctors on how to help patients weigh up the benefits of a new drug versus an older one.

3.2 Moreover, where patients do initiate this conversation, they will need to recognise that will be liable for not just the price of the drug itself, but also the associated administration costs (eg nursing and pharmacy time) that come with a private episode of care. This point has not been spelt out clearly enough either in the draft guidance, or in the media's reportage of the new policy. Therefore there may be cases where a patient initiates the conversation, but then realises that they are unable to afford private care. This may cause some patients to feel that they are getting "second best" treatment. The potential emotional difficulties entailed in this lead us to recommend that conversations with the doctor about the clinical effectiveness of one drug versus another are decoupled from conversations about cost and methods of payment, which should happen with a trained professional with experience of handling this sensitively.

December 2008

Memorandum by Professor Karol Sikora and Professor Nicholas James (TF 29)

TOP-UP PAYMENTS IN CANCER CARE

SUMMARY

Each year 250,000 people in Britain will develop cancer. It affects one in three of us and there are currently 1.3 million people living with the disease. By 2028 this number will rise to 3.3 million. This is good news as it means we are making cancer a controllable illness. But medical advances cost money and cancer care is no exception. Innovation in drug and radiotherapy technology is amazing. Driven by a better understanding of the molecular defects that result in abnormal growth and precision, pin-point accuracy in radiotherapy, we can now target the specific differences between normal and malignant cells better than ever before.

Cancer care costs are spiralling out of control in every healthcare environment. Aging populations with a wide range of medical problems are consuming vastly increasing amounts of care. New technology—drugs, devices and procedures—are powerful inflationary drivers in an information rich, consumer world. In the era of Google, the old paternalistic medical monopoly of health knowledge is gone forever. Recent research in Birmingham shows that over 50% of newly diagnosed cancer patients in three inner city hospitals had accessed internet information, mostly via their relatives.⁷⁵

There is now evidence of a growing use of top-up payments to break access barriers. This applies to areas as diverse as implanted hearing aid devices, access to diagnostics such as scans and even home nursing care services. In some areas, for example fertility treatment this is enshrined in the NHS by NICE guidance, whereby the NHS pays for three assisted fertilisation attempts but further ones must be self-funded. If

⁷⁵ James N, Daniels H, Rahman R, McConkey C, Derry J, Young A. A study of information seeking by cancer patients and their carers. *Clin Oncol* 2007;19:356–62.

successful when self funded the mother to be reverts back to the NHS for her ante-natal care. Many politicians, whatever their persuasion, have until now been in denial. Meanwhile cancer patients have learned to become very sophisticated consumers of extra clinical services outside the NHS.

Healthcare in Britain has never been totally tax based. Private practice has developed significantly throughout the last 60 years and most of us top up NHS dentistry and optical services. An ethically driven top-up system is the only sustainable solution for the current challenge of cancer care and carries the best chance of sustaining a high quality core service for all. Furthermore it will drive a new, patient-led, competitive marketplace which will create greater efficiency throughout cancer care.

BACKGROUND

Over the last decade an increasing number of cancer drugs have been licensed. Some are expensive. In the UK those responsible for commissioning care in the NHS have been increasingly challenged by patients and their relatives to provide new cancer drugs. Over 40 are now in the final stages of the global development pipeline.⁷⁶ These are the products of the molecular revolution triggered by the discovery of the structure of DNA in 1953 and the development of remarkable techniques to examine the function of genes, gene expression and proteins. Most of them will receive their market authorisation within the next three years. They work by targeting the molecular cogs of signal transduction between the cell surface and the nucleus. This is an exciting time for those involved in cancer research and care. But the cost of getting a single drug to market now exceeds \$1 billion per compound.⁷⁷ More sophisticated molecular diagnostics are being developed which will shortly be able to personalise care so increasing its cost-effectiveness. Giving the right medicine to the right patient will eventually drastically reduce the overall costs of care but we're not there yet.⁷⁸

Drugs are not the only problem. Precision based radiotherapy techniques which considerably reduce the late side effects of radiotherapy given with radical intent are now possible. Intensity modulated radiotherapy (IMRT) and image guided radiotherapy (IGRT) are techniques to ensure a more precise deposition of radiotherapy to the tumour.⁷⁸ They allow an increased dose to be delivered to the tumour without side effects so increasing local control in prostate, lung, breast and head and neck cancers. Delivery costs are higher as they require more sophisticated planning and longer linear accelerator time. Currently 30 of Britain's 61 radiotherapy centres are equipped to provide both modalities but fewer than 10 are doing so regularly. Staff shortages, equipment capacity and sheer volume of routine work are given as reasons. Although fewer patients are aware of the advantages of these techniques than new drugs, the more sophisticated are now travelling considerable distances to receive optimal radiotherapy and opting for private care if necessary.

NICE AND ASSESSING COST-EFFECTIVENESS

Denying the existence of innovative drugs is no longer acceptable in a democracy where patients can access all the information. The internet is a great equaliser—it tells it as it is. Ageing populations leading to a soaring cancer incidence, new but expensive technology and an informed and consumerist society is a perfect recipe for financial meltdown.⁷⁹ The NHS is no exception. The National Institute of Health and Clinical Excellence (NICE) produces superb health technology assessments—thorough, evidence based and extremely well respected internationally. But its decision process is painfully slow often taking up to three years after a drug has been licensed. During this time, the drug is available to privately insured patients in the UK and to patients in most EU countries. In many recent cancer drugs the NICE decision has been negative as the costs exceed the unwritten threshold of £30,000 per quality adjusted life year saved (QALY).⁸⁰

DECISION MAKING BY PRIMARY CARE TRUSTS (PCT's)

PCT's are responsible for commissioning healthcare for their populations. A person's PCT is determined by their postcode of residence. Although most will provide drugs and treatments approved by NICE, sometimes even these are subtly restricted for example by telling clinicians only a fixed number can receive a particular drug. Those that have not been approved are considered in different ways by the 149 PCT's. Some determine a general policy in consultation with the relevant specialist and the Cancer Network. In some areas, for example Greater Birmingham, this process is pooled with economies of scale and improved decision making through shared expertise. Others have no blanket policy and convene a sub-committee to consider each patient on their own merit, a very wasteful and capricious process. Social, domestic and other factors may be taken into account with such individual funding requests but mostly are not. The names and

⁷⁶ Sikora K. Cancer drugs—the economic challenge, *Scrip News*, 1–3 May 2008.

⁷⁷ Jonsson B and Wilking N. A global comparison of access to cancer drugs *Ann Oncol* 18 supp3: 1–80, 2007.

⁷⁸ Sarker D, Pacey S and Workman P. Use of pharmacokinetic/pharmacodynamic biomarkers to support rational cancer drug development. *Biomarkers Med* 1:399–417: 2007.

⁷⁹ Guckenberger M, Baier K, Richter A, Vordermark D and Flentje M. Does IMRT prevent toxicity of treating the pelvic lymph nodes? *Radiation Oncology* 3: 1–12: 2008.

⁸⁰ Appleby J, Devlin N, Parkin D. NICE's cost effectiveness threshold. *British Medical Journal* 2007;335:358–359.

composition of these groups varies around the country and none have any oncology specialist representation. This diverse approach to rationing is the underlying cause of post code prescribing. Some of the differences in availability may be for sound public health reasons—a PCT may choose to prioritise a non-cancer health area over cancer for reasons of local patterns of disease for example.⁸¹ At the individual level this causes resentment—why should my cancer care be compromised to pay for better adolescent mental health which will not help me—for example?

DETERMINING EXCEPTIONALITY

The Primary Care Trust (PCT) decision-making for a drug not approved by NICE depends wholly on the exceptionality of an individual patient. This creates huge variability and inequity—the composition of the individual funding request panels, the procedures and the costs of the process are all inappropriate and cause huge stress for patients, their families, their doctors and indeed the staff of the PCT itself. Justifying to a patient and their family that they are not “exceptional” is almost impossible and engenders much anger in the clinic. This process is not engaged with by the PCT and is wholly delegated to the treating consultants. There are problems in the procedures by which PCTs decide which patient should receive the right treatment. The PCT procedures mean:

- lack of information about the application and appeal process;
- lack of clarity and openness about how they make their decision;
- long delays in decisions;
- poor analysis of the evidence and poor decision making;
- the decisions are not communicated properly; and
- leads to patients seeking costly judicial reviews of PCT decisions.

The costs in both financial and emotional terms are enormous. There is too much variation in outcome based on the persuasion powers of the treatment consultant and the persistence of motivated and educated patients to convince the panel in writing that an individual is truly exceptional. A recent document from the NHS Confederation⁸² indicates that to make a case for special consideration requires the demonstration that:

- the patient is significantly different to the general population of patients with the condition in question; and
- the patient is likely to gain significantly more benefit from the intervention than might be normally expected for patients with that condition.

The fact that a treatment is likely to be efficacious for a patient is not, in itself, a basis for exceptionality. But according to leading lawyers this definition is fatally flawed.⁸³ There is no legal definition of exceptional in an NHS setting and exceptional in law only means what is not normal. The word *significant* has a meaning that is different to a health economist, a statistician, an oncologist, a layperson and to a lawyer. Most PCTs are struggling to define what it means.

Another problem is defining the patient group. Is it all those who present with a certain cancer at the date of diagnosis of the patient or just those that survive up to an equivalent time point as the patient? In principle, why should the definition restrict and confine what is set by the dictionary term for the word exceptional? And why are there two tests instead of one when using the word *significant*? Even if there was consensus on what it meant, it sets the bar too high for most patients. How can they establish that they will benefit more *significantly* than trial data would suggest? PCTs currently have very wide discretion but this definition is unnecessarily restrictive. The composition of most panels is unsuitable in that there are rarely any doctors that actually treat patients present and certainly no oncologists. In private, PCT representatives will say that very few patients should be considered as exceptional and that rejection of requests should thus be the norm, making the whole process a charade.

The Appeals process at PCT level is unfair—PCT employees are essentially reviewing their own decision making and the patient has no right to representation. The system has been judged unlawful by senior human rights lawyers. The process is too slow often lasting months. Sadly, patients do not have the luxury of time. The emotional havoc wreaked by the current system on patients and their families is unprecedented. Many, with only months to live, now spend their time totally preoccupied in battling with what they perceive a faceless bureaucracy.

If the Appeal process fails then a patient must resort to Judicial Review in the High Court; a slow and potentially expensive process. Judicial review is also unfair as so far PCTs, with the exception of *Mrs Otley v Barking and Dagenham PCT*, have just caved in literally at the courtroom door. Making a noise clearly gets results but cannot be an equitable way forward (Figure 1). The cost of using the legal system is enormous and again fraught with emotion. It's using a sledgehammer to crack a nut. In reality, there can be no winners.

⁸¹ National Radiotherapy Advisory Group Report, February 2007. www.dh.gov.uk

⁸² Cancer Reform Strategy, December 2007. www.dh.gov.uk

⁸³ Bosanquet N and Sikora K. The economics of cancer care. Cambridge University Press, Cambridge 2007.

 TOP—UP PAYMENTS FOR CANCER CARE

If the PCT cannot be persuaded to pay for a drug or for precision radiotherapy then the only way to proceed is to either go completely to the private sector or to take what the NHS provides and buy the required agent as a private patient. The same process has not yet occurred for IMRT/IGRT but is likely soon. There is complete confusion on the NHS top-up care rules with no central direction. There are three key questions.

1. *Are patients allowed to combine private and NHS care?*

Veiled and overt threats have been used by PCT officials to withdraw all NHS care if patients persist in trying to obtain top-up drugs. Most are not made in writing but in some cases patients have been issued with bills for NHS care after paying for additional private drugs. Such harassment of very vulnerable people is appalling and goes against natural justice and the principle of a caring health service. The legal opinion obtained by Western Provident Association's Mycancerdrugs top-up policy concludes that this is unlawful and this finding has never been challenged in Court.⁸⁴ Several other top-up insurance policies are now becoming available. Despite the unease of managers there seems to be no bar in law to a patient topping up NHS with diagnostics, drugs and radiotherapy bought privately from either the NHS or the independent sector.

Although a patient may not be both public and private during the same episode of care, there is no accepted definition of what an episode is. This has been interpreted differently across the country. Some Trusts interpret the episode as the entire course of treatment for the cancer, which may be many months. Others view the episode as finished as soon as the patient leaves the consulting room or ward. Even this is not clear cut—in dentistry patients can have one tooth treated on the NHS and another with a more expensive private technique at the same sitting. There is nothing in law to say that two episodes/consultations cannot take place sequentially. So far topping up for precision radiotherapy has not been attempted but will almost certainly occur in the next two years as services become available. Clearly agreeing that patients can continue to use NHS services whilst they pay for extra services has to be the first step in moving forward.

2. *What happens if patients procure a top-up without telling the NHS?*

Telling people they will lose the right to all NHS care if they top-up, as has been implemented by some PCTs and Trusts could accelerate the evolution of a dangerous market of underground cancer care through internet pharmacies. There already exist a number of organisations willing to work with the NHS to provide a true partnership for care delivery to avoid this situation. Urgent clarification is required. Natural justice would suggest that all taxpayers and their dependents are entitled to the NHS component of their care whatever else they do outside it. Otherwise the purchase of an over the counter drug or a session with a complementary therapist would constitute a top-up so logically leading to cessation of NHS care. Getting a workable arrangement for top-ups in place is therefore a priority to avoid secret top-up payments.

3. *How can services to NHS patients not topping up be protected?*

There is considerable under-capacity in the NHS system to deal with the physical delivery of top-up drugs in some areas. It is not clear who will pay for extra health professional time, investigations and the cost of treating side effects for giving a top-up drug safely. And who will pay for other drugs given by the NHS but that would not normally be given in a particular patient's circumstances without the top-up drug eg irinotecan with cetuximab for colorectal cancer? With radiotherapy the situation is even more complex as there is an estimated 90% under capacity in NHS provision currently. Will future patients be able to convert to precision planning by paying a fee just for this component but have their treatment delivered on an NHS machine? A new ethical framework is needed so that those not topping up suffer no disadvantage. In practice, patients topping up may require less "regular" NHS care as effective treatment of the core problem may reduce the frequency of visits for cancer related complications.

POTENTIAL SOLUTIONS

NICE—PCT decisions

A provisional NICE decision within a month of EU Market Authorisation for a drug is essential. It is likely to be negative in most cases but if positive, then funding must to be provided for all PCTs immediately. This would require that pharmaceutical companies submit data on their product simultaneously to the MHRA and to NICE. At present many drugs are withdrawn from the NICE process by the companies presumably if they think a negative decision likely, as they fear it will harm sales elsewhere in the private sector or overseas. Companies are not required to disclose this information to NICE so this may need a

⁸⁴ Priority setting: managing individual funding requests. NHS Confederation, London, 2008.

change in the law. If the provisional decision is negative then no exceptionality can be allowed—we simply have to admit that we just can't afford the drug. However, English common law will not allow a policy that has no exceptions so this is not a complete solution. NICE must also decide at which point in the care pathway the NHS stops providing active treatment. But if a consultant believes there is a clinical indication and a fully informed patient wishes to pay, then a system should be developed to permit this without adversely affecting the service as a whole. The patient should still remain under the care of the same NHS consultant.

It is possible that a consortium of pharmaceutical companies could create a Patient Support Programme for exceptional use. This has been developed successfully in the US and China. This enables physicians to directly request drugs for their patients on compassionate grounds. This needs to be explored urgently.

Top-up administration

We need to develop a system for top up administration which evaluates not only the cost of the top-up drug or procedure but also:

- The cost of its administration—infusion time, complexity and cost of accompanying symptom control medication. This applies to oral as well as intravenous infusions and to planning and other procedures in radiotherapy.
- The average cost of dealing with side effects.
- The average cost of extra doctor, nursing and pharmacist time.
- The impact on the total chemotherapy capacity of an NHS unit—all NHS patients including those paying top ups to be treated equally for their NHS component of care.
- If there is no capacity to deliver top-up drugs or precision radiotherapy without adversely affecting routine NHS patients then outsourced delivery is the only option.
- A fixed total tariff including all the costs above is calculated in advance of drug delivery and given as an option to the patient in writing. A supplement of 30% to the actual drug cost is likely for most intravenous and 15% for oral administration.

Constructing a package from the above which also factors in the average duration of care would enable patients to be offered a fixed price for the provision of a single top-up drug over a set period of time. This could be delivered by either the NHS or an independent contractor. The box below shows the steps required to develop a fair and equitable system to manage the forthcoming developments in cancer. Clearly there is much complexity around the issue of how to pay. Do we make patients pay in advance? If not paying in advance, what happens with those who default on payments? How do we factor in the extra administrative costs to the NHS, which struggles already to capture activity data on its current activities?

BOX 1: STEPS FOR THE FUTURE

- NICE decisions on new drugs within 30 days of market authorisation;
- CancerNICE created as an advisory group consisting of oncology professionals and patient representatives similar to Oncology Drug Advisory Group (ODAC) for the FDA in the US;
- PCTs to accept consultant and MDT decisions on drugs and procedures licensed but not yet evaluated by NICE as default position;
- CancerNICE to create national exceptional circumstances panel with 14 day turn around to avoid inevitable post code lottery with devolved decisions from 149 PCTs;
- continued provision of NHS care to those choosing to top-up cancer treatments;
- price of top-up calculated to include costs of delivery, extra investigations and management of side effects—transparent package prices to be offered;
- independent sector providers to compete for delivery of top-up care to encourage choice, competition and efficiency;
- patient to remain under the care of their existing NHS consultant throughout pathway;
- efficiency savings in NHS cancer services—including inpatient management programme highlighted in Cancer Reform Strategy—to bring NHS services into line with those of Western Europe in availability of both chemotherapy and radiotherapy to avoid the frequency of top-up; and
- no detriment whatsoever to NHS patients through the provision of top-up services.

CONCLUSION

It is essential that NHS patients not paying for extra care are not adversely affected by any solution to the right of patients to top-up. This can be achieved by the NHS charging the true total costs of extra care including its management together with a supplement to ensure improvement in NHS chemotherapy and radiotherapy facilities for all. A flow chart showing the current complex path to obtaining a cancer drug is shown in Figure 1.

Independent providers, both new and existing, could be sought to bid for contracts to supply a top-up service to cancer centres to drive efficiency, competition, value for money, convenience and quality for patients. This could be in partnership with the NHS either on the same site or in separate centres at a different location. Such centres could offer a menu of top-up drugs to patients also receiving NHS care. In all cases the relevant drug would always need to be licensed for the particular clinical situation and prescribed by the patient's NHS consultant who will retain responsibility for their care. The NHS consultant should not receive additional income just for prescribing the top-up.

Whatever is implemented it is essential that efficiency savings throughout cancer care delivery are ploughed back into service improvements. It is clear that our spending on new cancer drugs and high quality radical radiotherapy are falling behind other countries of comparative wealth. Yet the per capita total amount we spend on cancer is similar. Only by efficiency savings can funding be made available for innovation without the need for top-up so bringing us into line with our European neighbours. A suitable infrastructure for the ethical delivery of top-up services is urgently required. This could drive choice and competition throughout cancer care leading to real reform and value, whoever pays.

ACKNOWLEDGEMENTS

We are grateful to Peter Telford QC⁸⁵ of Exeter for his contribution to the legal aspects, Kate Spall of the Pamela Northcott Fund—a patient led, voluntary organisation with charitable aims, providing unprecedented advocacy and support to cancer patients denied access to new drug therapies and Charles Macewan of Western Provident Association for Figure 1.⁸⁶

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December 2008

Table 1

10 CURRENT PROBLEM CANCER DRUGS

<i>Generic name</i>	<i>Trade name</i>	<i>Maker</i>	<i>Indication</i>	<i>Delivery</i>	<i>Cost²</i>	<i>Estimated number per year³</i>	<i>Cost for 3m:50%⁴</i>
Bevacizumab	Avastin	Roche	colon, breast	iv	£60K	40,000	300m
Cetuximab	Erbitux	Merck	colon, lung	iv	£50K	30,000	187m
Lapatinib	Tykerb	GSK	breast	oral	£40K	4,000	20m
Sorafenib	Nexavar	Bayer	renal, liver	oral	£30K	6,000	22m
Sunitinib	Sutent	Pfizer	renal, GIST	oral	£40K	5,000	25m
Erlotinib ¹	Tarceva	Roche	lung, pancreas	oral	£40K	30,000	150m
Bortezomib	Velcade	J&J	myeloma	iv	£30K	3,000	11m
Pemetrexed	Alimta	Lilly	lung, mesothelioma	iv	£60K	4,000	30m
Panitumomab	Vectibix	Amgen	colon	iv	£45K	20,000	112m
Lenalidomide	Revlimid	Cellgene	myeloma	oral	£70K	1,000	9m

¹ Erlotinib is approved for non small cell lung cancer in Scotland

² Cost for a one year supply including iv infusion costs and patient management. In practice many patients will not receive a full year of drug because of failure to respond or the development of side effects. Therefore the package price could be considerably less.

³ Estimated total number of patients for current European Medicines Evaluation Agency approved indications. All the above drugs are currently available without top-up fees in most western EU countries even though the total per capita spend on cancer in the UK is now comparable.

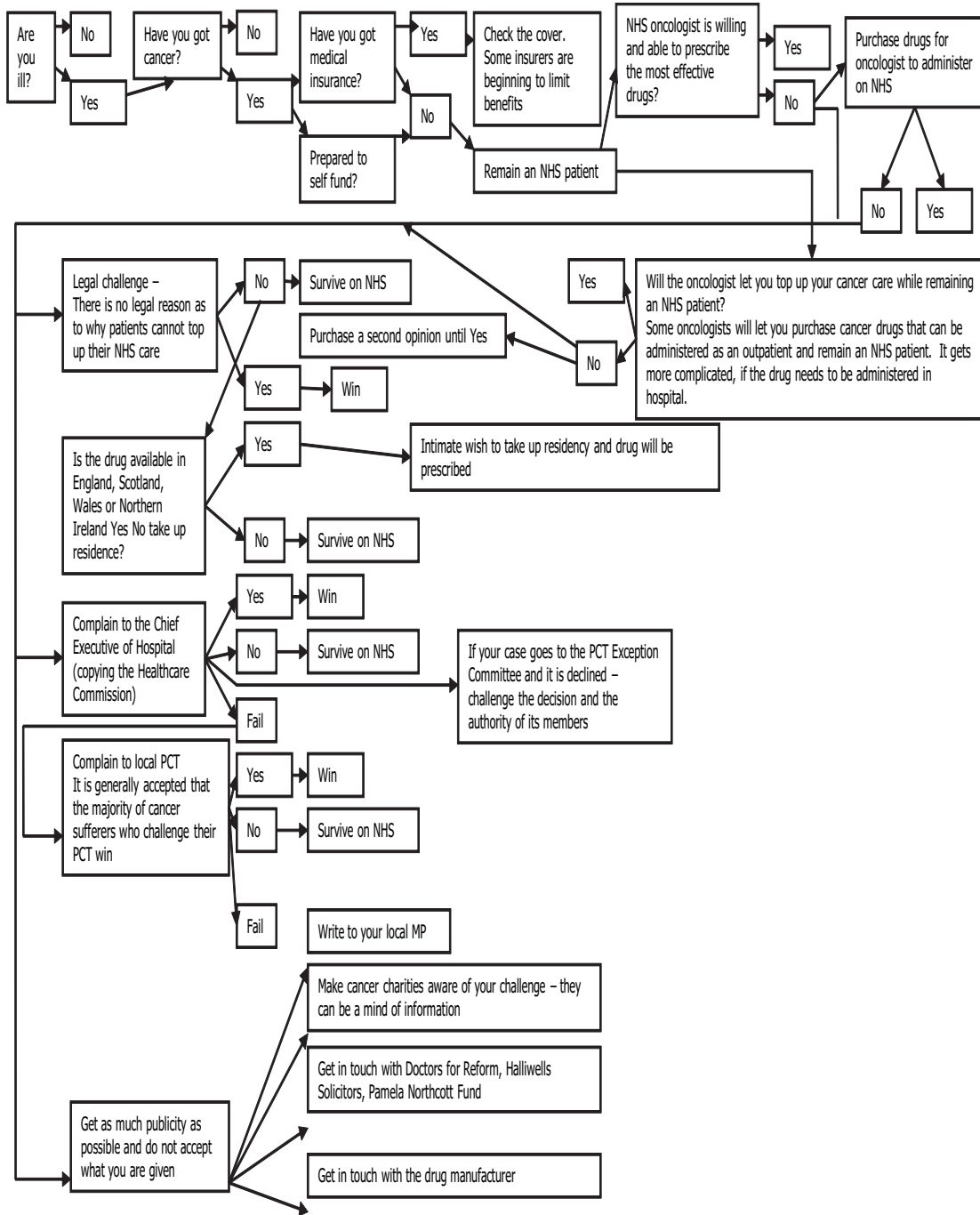
⁴ Assuming average of three months drug supply as some patients will fail to respond or develop serious side effects and so the drug will be stopped and only 50% of patients are eligible for the drug. Total numbers estimated from CancerResearchUK statistics and labelled drug indication from the EMEA registration file.

⁸⁵ Personal communication, Peter Telford.

⁸⁶ www.mycancerdrugs.org

Figure 1

HOW TO OBTAIN A CANCER DRUG NOT APPROVED BY NICE
(COURTESY CHARLES MACEWAN, WESTERN PROVIDENT ASSOCIATION)



Memorandum by the Specialised Healthcare Alliance (TF 30)

THE PURCHASE OF ADDITIONAL DRUGS BY NHS PATIENTS—“TOP-UP FEES”

The Specialised Healthcare Alliance is a broad coalition of 43 patient groups supported by nine corporate members⁸⁷ which campaigns on behalf of people requiring specialised medical care. These conditions tend to be rarer and both complex and expensive to treat. Examples are numerous but include certain cancers, cystic fibrosis, haemophilia, HIV and neurological conditions. Accidents or complications of more common conditions such as diabetes can also trigger the need for specialised services. Cumulatively, the number of patients affected by such conditions number many tens of thousands.

We welcome this opportunity to submit evidence to the Health Select Committee’s inquiry into the purchase of additional drugs by NHS patients (“top-up fees”).

1. EXECUTIVE SUMMARY

The Specialised Healthcare Alliance broadly welcomes and supports the recommendations of Professor Richards’ review but questions whether speedier appraisals along with NICE’s proposed arrangements for end of life medicines will be sufficient to deliver his overwhelming priority “that patients get access to drugs that could potentially benefit them on the NHS”. In particular, the Alliance notes that a minority of exceptional funding cases relate to cancer. The Department of Health is unable to provide further detail but this suggests that a significant proportion do not fall in the end of life category though they may relate to severe clinical need with ramifications for life expectancy.

The Alliance questions the ethical consequences of a hard and fast definition of end of life medicines and suggests a guideline that includes life-threatening conditions where treatment, or other technologies could extend life indefinitely, might be more appropriate.

The Alliance believes NICE’s evaluation system should be more transparent, follow all of the Secretary of State’s directions and include wider cost implications with PCT funding decisions being subject to similar considerations. Parliament has a role to play as guardian of the social values underpinning decisions on orphan or ultra-orphan treatments.

The Alliance would like to emphasise that while this dilemma has arisen most conspicuously in relation to drugs, it is by no means restricted to them and any adjustment in policy should apply equally to other forms of treatment.

2. RECOMMENDATION 1

2.1 Insofar as exceptional funding cases arise for PCTs pending appraisals by NICE, a speedier appraisals process can only be helpful. There is, however, a significant proportion of treatments which will fall foul of NICE’s threshold for clinical and cost effectiveness or which will not be appraised given the very small patient numbers involved ie ultra-orphan products. As things stand, this body of treatments is growing as the NICE threshold fails to keep pace with inflation.

2.2 The Health Select Committee’s own report on NICE observed that the threshold has remained unchanged while NHS funding has risen nearly threefold. The Alliance recommends a substantial increase in the threshold to ensure that this welcome increase in spending is reflected in all aspects of patient care.

2.3 In addition, it is important that NICE follows the Secretary of State’s directions in their entirety. The Next Stage Review recognises the importance of innovation to the future of the NHS but, in the SHCA’s view, NICE pays relatively scant regard to the link between orphan drugs and innovation. Similarly, clinical need should be weighed alongside cost effectiveness in the context of a compassionate NHS; a view supported by the recent NICE Citizen’s Council on Quality Adjusted Life Years and the Severity of Disease.

2.4 The Alliance also believes consideration should be given to reforming the evaluation process to allow for consideration of broader economic costs when assessing the cost-effectiveness of particular treatments. The SHCA is concerned that in the current financial climate, with a tendency to silo budgeting, decisions about whether to fund treatment are often made without weighing the wider cost implications, such as palliative care, social service costs and lost productivity. NICE is well placed to broaden understanding of costs and benefits and these considerations should be reflected in its evaluation process.

2.5 The SHCA understands the balance between the needs of society and individual in allocating limited tax resources. The Alliance supports the adoption of innovative and effective medicines by the NHS and the

⁸⁷ A full list of members is available on our website at www.shca.info

use of generic alternatives when they become available. The savings arising from several major categories of treatment moving off patent over the next few years should help the NHS maintain treatment based on clinical need rather than ability to pay, as endorsed by the Richards' Report.

3. RECOMMENDATIONS 2, 3 AND 4

3.1 Where, despite such improvements to NICE procedures, it still falls to PCTs to decide on funding of drugs and other treatments, the Alliance welcomes the recommendation that such decisions should be made rationally following proper consideration of the evidence and with a proper explanation where funding is withheld. The Alliance considers that much greater transparency and consistency is essential but has doubts about the capacity of individual PCTs to undertake such work. Given their responsibility for orphan conditions, Specialised Commissioning Group might be better placed to do so, in the same way as the National Commissioning Group decides on funding for some, but not all, ultra-orphan treatments. Similar considerations apply to off-label drugs.

4. RECOMMENDATION 5

4.1 The Richards' Review recommends NICE "assess urgently what affordable measures could be taken to make available drugs used near the end of life that do not meet the cost-effectiveness criteria currently applied to all drugs".

4.2 A survey conducted alongside the Richards' Review found that of 14,000 exceptional funding applications, 8,000 related to drug treatments and 26% of those to cancer. Unfortunately, it was not established how many applications related to end of life medicines and how many related to severely debilitating conditions which were not immediately life-threatening. It is however clear that any approach predicated purely on the end of life is inadequate. NICE's own Citizen's Council recently concluded appraisals should take more explicit heed of clinical severity in reaching decisions.

4.3 The Alliance therefore questions the ethical consequences of limiting the NICE review to end of life medicine. It would be more appropriate for the definition to cover drugs used near the end of life and where severe clinical need is involved. In particular, poor treatment may not only affect morbidity and quality of life in the short term but ultimate life expectancy as well.

5. RECOMMENDATION 6

5.1 The Alliance supports an urgent investigation of the extent and causes of international variations in drug usage.

6. RECOMMENDATION 9

5.1 The SHCA understands the motivation behind separating privately funded treatments from NHS care. It would, however, be unfortunate if this denied the NHS the opportunity to accumulate data of potential public benefit about the effectiveness of such treatments. There might be merit in making the continued provision of NHS care contingent on sharing information about the privately funded element of treatment.

7. RECOMMENDATION 14

7.1 The Alliance would like to emphasise that while this dilemma has arisen most conspicuously in relation to drugs, it is by no means restricted to them and any adjustment in policy should apply equally to other forms of treatment.

8. CONCLUSION

8.1 The Specialised Healthcare Alliance supports Professor Richards' view that the problem of top-ups is best addressed by improving access to medicines for NHS patients making the need for private payment the exception that proves the rule. The Alliance is, however, concerned that the current mix of proposals will prove inadequate and, by seeking to draw a distinction between end of life medicines and medicines addressing severe clinical need, invidious.

December 2008

Memorandum by Beating Bowel Cancer (TF 31)

TOP-UP FEES

EXECUTIVE SUMMARY

Beating Bowel Cancer believes that every patient, regardless of ability to pay, should have equal access on the NHS to a licensed drug that their clinician has judged to be clinically appropriate.

Bowel cancer patients should not be put in a position where they feel they have to pay for treatment recommended by their clinician. Top-ups are a measure of last resort for the minority of people. Whilst we are pleased that the unfair policy of withdrawing NHS treatment for those who choose to top up has ended, we believe that top-ups should be unnecessary.

All licensed drugs should be available for clinicians to offer to their patients, as they feel appropriate, regardless of a patient's ability to pay. Until such time as these drugs receive NHS funding, we would like to see greater clarity around the exceptional funding appeals process and how top-ups will be administered. NICE should review its criteria for judging cost-effectiveness, and there needs to be consistency across the country in decision-making about funding.

INTRODUCTION

1.1 Beating Bowel Cancer is a leading UK charity for bowel cancer patients, working to raise awareness of symptoms, promote early diagnosis and encourage open access to treatment choice for those affected by bowel cancer. Through our work we aim to save lives from this common cancer.

1.2 We welcome the Health Select Committee's inquiry and hope that the scope of the inquiry will extend beyond the narrow issue of top-up fees, taking into consideration wider funding concerns for existing and emerging treatments.

1.3 As a patient-centred organisation, we frequently come into contact with patients with advanced bowel cancer, whose clinicians have recommended non NICE-approved drugs. For bowel cancer, these include Avastin (bevacizumab), Erbitux (cetuximab) and Vectibix (panitumumab) which can halt disease progression, potentially prolonging life, as well as providing a quality of life that will allow patients to continue working or enjoying precious time with their family.

1.4 We strongly believe that policy-makers' and the media's focus on the narrow issue of top-ups is a distraction from the wider issue of NHS funding for vital drugs, available as a matter of course in most other EU countries. The UK remains the poor relation in terms of access to cancer treatments.

1.5 Whilst the change in the Government's policy on top-up fees is very welcome for the small minority of patients who can afford to top up their treatment, the vast majority of patients simply cannot afford to do so. Most of these patients will continue to be denied access to licensed, clinically effective drugs as recommended by their clinician.

SECURING NHS FUNDING FOR NON NICE-APPROVED TREATMENTS

2.1 In making his statement to the House of Commons, the Secretary of State for Health accepted Prof Mike Richards' recommendation "to improve access to certain drugs on the NHS, reducing the need for such patients to resort to private care" (4 Nov 2008 : Column 131), which Beating Bowel Cancer very much welcomes.

2.2 Currently, patients recommended for a particular non NICE-approved treatment are forced to go through the complicated and traumatic process of applying for exceptional funding, at a time when they are already struggling to cope with their illness and their mortality.

2.3 For those patients who are turned down, they then must undergo further stress, trauma and delay whilst appealing the PCT's decision. Not only does the final outcome vary across the country, and from patient to patient, but we also know that some clinicians do not pursue exceptional funding applications because their PCTs have told them that no such application will be funded any under circumstances.

2.4 Therefore, whilst we welcome the first part of Prof Mike Richards' recommendation number 9: "clinicians should exhaust all reasonable avenues for securing NHS funding before a patient considers whether to purchase additional drugs" (6.20, p 59), we have a number of concerns and questions:

- (a) Will "exhaust[ing] all avenues" simply cause further delay, confusion and stress to patients?
- (b) We are concerned that clinicians under immense time pressure already may not be able to fulfil this recommendation.
- (c) This recommendation needs clarification, as it is not clear what "exhaust[ing] all reasonable avenues" entails beyond the exceptional funding process.
- (d) Definitive, nationally set timeframes need to be put in place around this recommendation so that patients do not have treatment unnecessarily delayed whilst they explore all avenues.

2.5 We also welcome the Secretary of State's desire for more transparency and consistency in the appeals process, and his announcement that the Department of Health will "publish detailed good practice guidance for primary care trusts about the processes that they should have in place to make decisions about funding new drugs and handling exceptional cases" (4 Nov 2008: Column 133). We look forward to the details of this being issued as a matter of urgency.

2.6 Beating Bowel Cancer believes that the appeals process for exceptional funding needs to be streamlined, more transparent and patient-focussed, with clear timelines. Patients should have a right to expect PCTs to adopt uniform processes for making funding decisions and for these decisions to be taken quickly. They should also be given the right to attend any appeal panels to make their case for funding.

2.7 We are delighted that the Government has reached an agreement with the pharmaceutical industry on extending the systematic use of access schemes, as well as flexible pricing schemes. Beating Bowel Cancer has been calling for greater co-operation on pricing for many years, as outlined in our 2012 Challenge document. We would like to see patients given clear support and guidance on how access schemes will operate and benefit them.

PATIENTS CHOOSING TO TOP UP THEIR TREATMENT

3.1 For those who receive an unfavourable decision from the exceptional funding panel, a small minority are in a financial position to fund their treatment. We welcome that they will no longer face a further lottery as to whether their remaining NHS care is to be withdrawn, but a large number of issues need to be addressed and clarified in terms of how top-ups will operate.

3.2 The regulation and guidance around how top-ups will be administered will necessarily be complex and confusing for patients. Patients need clear, step by step advice on how to access the treatment they have been recommended. At such a difficult time in the patient journey, a simple and straightforward patient guide to top-up treatment is required. We welcome recommendation 13 in this regard and would like to see the DH act on this urgently. This must be national and not devised on a PCT by PCT basis.

3.3 As well as written guidance, patients have told us that they and their families would want appropriate support, advice and counselling when making the difficult decision on whether to access treatment through topping up.

3.4 Patients have also told us that continuity of care is vital—they want their care to be joined up. However, the second part of recommendation 9 in Prof Richards' review states that "patients should only be able to receive additional private drugs if delivered separately from the NHS elements of their care" (6.20, p 59). Although the next part of this recommendation emphasises good continuity of care, we remain to be convinced that this will happen in practice. In numerous cases, a different clinician will be administering their top-up care to their NHS care, which will necessarily cause disruption. Furthermore, patients being treated in NHS hospitals with no private areas could be forced to travel to another hospital, potentially a significant distance away.

3.5 Healthcare professionals who are already pushed for time and under pressure will not want to be overburdened by a complex bureaucratic system. How is time going to be found in clinic when patients already express that they wish they could have more time with their consultant and colorectal nurses? The need for clear guidance and training has been identified in the review (recommendations 10, 12 and 14) but in the meantime what guidance is being given to clinicians?

3.6 A number of questions need to be addressed on pricing to prevent inequality of cost arising where some patients have access to cheaper options than others:

- (a) Will tariffs be set nationally or locally or by each individual provider?
- (b) Will patients be given a clear and comprehensive menu of options on how they could receive their additional care and the associated costs?
- (c) What advice will be provided around treatment at home which is VAT exempt and likely to be significantly cheaper than the cost of treatment in a local private hospital?

NICE QALY THRESHOLD

4.1 We are pleased that NICE has recognised the value that society places on treatments that prolong life and is reviewing its QALY threshold for end of life treatments benefitting patient populations of 7,000 or less.

4.2 Beating Bowel Cancer is seeking clarification on the definition of "patient population". In our response to the NICE consultation we have requested that a more flexible interpretation of the "7,000 patient population" is taken by the Institute to focus upon the number of patients per year who would benefit from a positive appraisal of the treatment, as opposed to the size of the patient population affected by the condition.

4.3 We are also concerned that the cost-per-QALY “threshold” of £30,000 requires urgent review. The range NICE uses now to assess “cost effectiveness” is £20,000 to £30,000—the same as it was in 1999. This figure has not kept pace with NHS-specific inflation and is subsequently no longer an appropriate figure to use when calculating healthcare costs.

December 2008

Memorandum by Wyeth Pharmaceuticals (TF 32)

TOP-UP FEES

NICE CONSULTATION, APPRAISING END OF LIFE MEDICINES: ULTRA ORPHAN CONDITIONS

SUMMARY

- Wyeth believes the end of life medicines consultation will not deliver necessary reform for patient access to cancer medicines as ultra orphan conditions will be excluded from the outcome of this exercise.
- Wyeth believes that without appropriate reform to the NICE appraisal process, ultra orphan drugs will not be subject to appropriate cost effectiveness appraisal criteria, and patients with ultra orphan conditions will continue to face difficulties in gaining access to these medicines.
- Wyeth recommends that the NICE end of life medicines supplemental guidance should contain a separate set of appraisal criteria for ultra orphan drugs.
- Wyeth believes that the exclusion of ultra orphan conditions from the review arises from the unhelpful inconsistency within and between the Department of Health (DoH) and NICE policy regarding the assessment of ultra orphan drugs.

BACKGROUND

Wyeth welcomes Professor Mike Richards’ report, *Improving Access to Medicines for NHS Patients*, and the proposed changes to the NICE technology appraisal process in relation to medicines used to treat rarer conditions where their function is to extend survival at the end of life.

Wyeth believes that this is an important step in the process of reviewing the decision rules currently recommended by NICE, and that it will improve patients’ access to new beneficial treatments. Wyeth also believes that innovative new medicines used to treat rarer conditions should be recognised as such, and should be made available to NHS patients. In addition, the impact on a patient’s quality of life should also be taken into consideration.

APPRAISAL OF MEDICINES FOR ULTRA ORPHAN CONDITIONS

Wyeth has however noted that the NICE consultation, *Appraising End of Life Medicines*, clearly states that:

“This supplemental advice is not intended to cover medicines developed for what are commonly described as ultra orphan conditions involving very small groups of patients. Treatments for these conditions are not referred to NICE for appraisal and are generally dealt by the National Specialist Commissioning Advisory Group”. (Paragraph 1.3)

The definition of an ultra orphan condition used by NICE is a UK prevalence of less than 1 in 50,000. NICE advises the adoption of this definition for two reasons: first, it matches the prevalence criteria (less than 1,000 persons in the UK) used by the National Specialist Commissioning Advisory Group in determining those conditions that should fall within its programmes; and, second, it encompasses all products that appear, both now and in the foreseeable future, to be particularly problematic.

NICE has identified:

“There would [however] be problems in the appraisal of drugs for very rare diseases—“ultra-orphan drugs”—largely because of their high costs”.

“... estimates of the ICERs for ultra-orphan products will invariably give rise to values that would be considered cost ineffective under NICE’s conventional criteria. Under its current decision rules, therefore, the Institute would be most unlikely to ever recommend their use in the NHS”.

“If the Institute is to appraise ultra-orphan drugs, and be prepared to accept substantially higher ICERs than those hitherto considered to be cost effective, then separate decision rules (ie the range of ICERs considered ‘cost effective’) will need to be developed and adopted for these products. The Institute proposes that these ultra-orphan drug decision rules are based on the ICERs of those ultra-orphan drugs currently on the UK market”.

“The further work would determine a rationale for a new IOD threshold. However, it appears that at current prices indicative ICERs for ultra-orphan products are in the range of £200,000 to £300,000 per QALY (ie a 10-fold increase on the decision rules currently applied in conventional appraisals)”.⁸⁸

Wyeth believes that without appropriate reform to the NICE appraisal process, ultra orphan drugs will not be subject to appropriate cost effectiveness appraisal criteria, and this group of patients will continue to face difficulties in gaining access to these medicines. Drugs for very small populations are, by their nature, more expensive than other drugs and thus need to be assessed with different criteria, a point NICE itself has raised in the past, as referenced above.⁸⁹

As it stands, the end of life medicines consultation will not deliver necessary reform as ultra orphan conditions will be excluded from the outcome of this exercise.

Wyeth believes that this supplemental guidance should not discriminate against ultra orphan drugs in the absence of a clear policy as to how such drugs should be appraised.

Wyeth recommends that the NICE end of life medicines supplemental guidance should contain a separate set of appraisal criteria for ultra orphan drugs.

DEPARTMENT OF HEALTH AND NICE POLICY

Wyeth believes that the exclusion of ultra orphan conditions from the review arises from the unhelpful inconsistency within and between the Department of Health (DoH) and NICE policy regarding the assessment of ultra orphan drugs.

In contrast to the statement in the end of life medicines consultation, currently NICE states that it should review all oncology products, which includes ultra orphan drugs:

“Joanna Richardson informed the panel that NICE had received feedback at recent scoping workshops relating to concerns about NICE appraising cancer drugs for very small populations. The Chair was clear that appraisal is required in the case of these technologies, because postcode prescribing will result in the absence of guidance, irrespective of the size of the population in question”.⁹⁰

In addition, the Minutes of the Cancer Topic Selection Panel (27th February 2008) meeting also refer to the “default position” “that new cancer drugs would be appraised, unless there was a compelling reason not to do so”.⁹¹

On the one hand the DoH has indicated that ultra orphan drugs could be reviewed by NICE:

“In future the default position for all new cancer drugs and significant new licensed indications will be that they will be referred to NICE, providing that NICE agrees that there is a sufficient patient population and evidence base on which to carry out an appraisal and that there is not a more appropriate alternative mechanism for appraisal”. (Cancer Reform Strategy p 9)

Yet at the same time, the DoH’s own current topic selection criteria states that ultra orphan drugs should not be referred to NICE:

- “1. Is it appropriate for NICE to provide guidance on the topic? In particular,
 - (a) is the proposed topic within NICE’s remit?
 - (b) has NICE already provided guidance or is NICE developing guidance on the proposed topic?
 - (c) is the proposed topic emerging as a future public health issue?
 - (d) is the proposed topic an ultra-orphan disease?”⁹²

Wyeth’s experience in the current appraisal of kidney cancer medicines⁹³ is a case in point. Temsirolimus (Torisel), with an ultra orphan indication, is included in the current NICE HTA appraisal, despite the DoH criteria. Wyeth wrote to NICE at the beginning of the appraisal process (22 August 2007) to state that we did not believe that Torisel should be included in this appraisal process, however no change was made by NICE, and it was included.

⁸⁸ National Institute for Health and Clinical Excellence—Appraising Orphan Drugs, March 2006.

⁸⁹ *Ibid.*

⁹⁰ Minutes of NICE Cancer Topic Selection Consideration Panel Meeting 7 July 2007.

⁹¹ <http://www.nice.org.uk/media/8FB/83/TSMminutes27Feb08Cancer.pdf>

⁹² Department of Health, Selection Criteria for Referral of Topics to NICE, 19 September 2006.

⁹³ National Institute For Health And Clinical Excellence Health Technology Appraisal, bevacizumab, sorafenib, sunitinib and temsirolimus for renal cell carcinoma.

Torisel has also been referred for appraisal of its Mantle Cell Lymphoma (MCL) indication which is for an even smaller population than Renal Cell Carcinoma. Torisel, with both its indications together, is still an ultra orphan drug and yet has been referred twice by the DoH.

Wyeth believes that NICE should have a clear view on the appraisal criteria of ultra orphan drugs to inform the current appraisal, and any future appraisals of ultra orphan drugs, referred to NICE as a consequence of The Cancer Reform Strategy.

We further note that NICE has been invited to appraise Vorinostat for cutaneous T-cell lymphoma (0.4 patients/100,000), Arsenic trioxide for acute promyelocytic leukaemia (190 cases in 2002) and Nilotinib for Chronic myeloid leukaemia (one or two patients/100,000).

We believe that patients who suffer from very rare cancers should have the same right to access to medicines as those who suffer from rare cancers. We also trust that the current appraisal on kidney cancer medicines will fully recognise the special end of life circumstances affecting patients with poor prognosis renal cell carcinoma as well as the larger group with good intermediate prognosis.

December 2008

Memorandum by Merck Sharp & Dohme Limited (TF 33)

APPRAISING END OF LIFE MEDICINES CONSULTATION

Merck Sharp & Dohme Limited would like to use the occasion of the Health Select Committee's (HSC's) inquiry into "top up fees" to share with the committee our views on NICE's recent proposals for appraising end of life medicines.

Merck Sharp & Dohme Limited (MSD) is the UK subsidiary of Merck & Co., Inc., of Whitehouse Station, New Jersey, USA, a leading research-based pharmaceutical company that discovers, develops, manufactures and markets a wide range of innovative pharmaceutical products to improve human health.

It is important to acknowledge NICE's proposals for appraising end of life medicines as a constructive step towards improving access to medicines for patients in England and Wales. MSD welcomes the fact that NICE now recognises the need to be more flexible in how it weights different attributes during the appraisal process and congratulates NICE on responding to public opinion in this way.

However, we do anticipate that the apparent arbitrary nature of the proposed criteria determining eligibility for the new process will draw criticism, particularly if a rigid approach to their implementation is adopted. MSD strongly advocates flexibility in their application in order to avoid further inequalities in access to care.

For example a narrow focus on terminal illness in small numbers of patients is problematic; it is not clear to us why a patient with a relatively common disease should not have the same entitlement to life saving therapy as someone with a relatively rare disease. The same consideration applies to the potential discrimination between patients with terminal illness based on how long they are expected to live.

PROCESS ISSUES

MSD would ask the Health Select Committee to seek some further clarification on the proposed new process. In particular:

- When and how it will commence? The consultation states that "a medicine will need to have **been through** a single or multiple technology appraisal to be eligible". MSD believes that in the interests of equity, the new process should be applied retrospectively ie to those medicines which have been subject to a NICE appraisal in the past.
- Will it be the responsibility of companies to trigger the new process as part of their NICE submissions?
- What will happen on occasions where companies and NICE disagree on whether or not the entry criteria are met? For example if they differ on estimates of cost per QALY.
- Examples of what NICE would deem "sufficient" in terms of acceptable evidence would be useful eg Phase III RCTs, MTC, registry data etc. To require the level of evidence specified in the consultation may result in delays in patient access due to extended trial programmes, or agents which meet all other criteria being excluded from consideration.

 CRITERIA FOR ELIGIBILITY

MSD welcomes the transparency that is delivered by applying published criteria to determine the medicines that would qualify for the additional process. However, we disagree with the proposal that medicines should have to meet all the criteria listed in section 2 of the NICE consultation document. In our opinion this would create a hurdle so high that few medicines would qualify. As before, MSD believes NICE should be asked to introduce flexibility in how it applies the criteria. MSD has specific comments on a number of the proposed criteria. These are as follows:

- *“Threshold of 7,000 new patients per year”*:

This appears to be an arbitrary number. As well as therapies for more common cancers, this benchmark would exclude medicines for heart failure and certain inherited paediatric disorders, where life expectancy may be low, but patient populations are large.

MSD would ask the Committee to seek clarification as to whether this figure relates to the maximum eligible population for the licensed indication, or the number of patients which are anticipated to receive treatment in UK clinical practice. MSD believes the latter approach is preferable in order to avoid overestimations of the population likely to be eligible for the new medicine/indication under question, and therefore unnecessarily disadvantaging patients.

- *“Second and subsequent licences for the same product will . . . take into account the cumulative population for each product”*.

MSD would welcome further clarity on how this provision should be interpreted. If a cumulative population is considered alongside a 7000 patient threshold, this runs the risk of excluding many new indications particularly in oncology, where multiple indications are common. MSD believes that in order to avoid arbitrary discrimination, the treatment population for different indications should be considered independently.

- *“Twenty four months life expectancy”*.

This also appears arbitrary. MSD would welcome clarification as to whether this is life expectancy of 24 months with or without existing pharmacological treatment.

- We disagree with the assertion in the consultation document, that it is not sufficient to infer a survival gain from morbidity benefit. This is important because biomarkers can very accurately predict long term survival; eg cardiovascular events have a known association with lipid and blood pressure levels. Given the known associations and causative relationships between many biomarkers and outcomes, this would seem highly restrictive.
- For patients during the end stages of life, the quality of life can be as important as an extension of life. These are intrinsically linked. To disregard the benefits of quality improvements would seem inappropriate and MSD believes the criteria should address this in some way.
- *“No alternative treatment with comparable benefits is available through the NHS”*

In MSD’s opinion this criterion effectively restricts access to this process to medicines which are first in class. In so doing it:

- has the potential to restrict physician choice and the benefits to patients of having a range of therapies for treatment;
- fails to acknowledge that no two patients are the same and, due to factors such as individual patient response to treatment and tolerability, the importance of having more than one agent in the same therapeutic area;
- is anti-competitive, creating a winner takes all market, and;
- fails to recognise that the first-to-market product will often be superseded by safer and more effective treatments in the class.

OTHER COMMENTS

- MSD welcomes the proposal to support the new process with a programme of evidence development. However, we believe it essential that manufacturers are involved, ideally as partners, in these programmes. We also note that a budget of around £100,000 is anticipated for each data collection exercise. While this might be adequate in the field of oncology which is already supported by established cancer registries, some disease areas (particularly those with small populations) do not have a similar infrastructure. In these areas costs could be considerably higher.
- Finally, MSD believes that NICE should set a date for reviewing this process in order to ensure further improvements can be made based on real-life experience.

Memorandum by Sanofi Pasteur MSD (TF 34)**THE PURCHASE OF ADDITIONAL DRUGS BY NHS PATIENTS****EXECUTIVE SUMMARY**

Professor Mike Richards' report "*Improving access to medicines for NHS patients*" and the announcement of NICE's consultation on appraising end of life medicines, have resulted in the Health Committee inquiry into the purchase of additional drugs by NHS patients ("top-up fees"). The first prophylactic vaccine against cervical cancer, Gardasil[®], received European Marketing Authorisation on 20 September 2006 and is available on the NHS and privately in the UK. Cancer vaccines represent an area of increasing clinical interest and such vaccines may eventually even replace the costly chemotherapy and radiation treatments currently being used to treat cancer. Lord Darzi's report from June 2008 placed choice and patient empowerment firmly on the agenda for the NHS. If patients now have the choice to pay top up fees to gain access to newer medicines, an inquiry into patient top-up fees must consider vaccines and drugs alongside one another.

Sanofi Pasteur MSD is the only company in the UK totally dedicated to vaccines and our purpose is to bring innovative vaccines to the UK, protecting public health and preserving quality of life for all ages.

There are two vaccines licensed in the UK for the prevention of cervical cancer, a quadrivalent vaccine (Gardasil[®]) and a bivalent vaccine (Cervarix[™]). Whereas the bivalent vaccine is indicated for premalignant cervical lesions and cervical cancer causally related to Human Papillomavirus (HPV) types 16 and 18, Gardasil[®] is licensed for the prevention of cervical cancer as well as for the prevention of a range of premalignant genital lesions (cervical, vulvar and vaginal) and external genital warts (condyloma acuminata) causally related to Human Papillomavirus (HPV) types 6, 11, 16 and 18.

The Department of Health has selected the bivalent vaccine for the national schools immunisation programme against cervical cancer which covers 12 to 13-year-old girls with a three year catch up campaign which will make the HPV vaccine available for 13 to 18-year-olds.

There is evidence from clinicians to suggest that there are considerable opportunities to reduce healthcare inequalities, preserve patient choice and encourage rational prescribing outside of the national schools immunisation programme for cervical cancer and we would urge the Health Select Committee to consider the issue of top-up fees for HPV vaccination alongside its inquiry into the purchase of additional drugs by NHS patients ("top-up fees") and make appropriate recommendations to the Department of Health.

INTRODUCTION

1. Professor Mike Richards' report "*Improving access to medicines for NHS patients*" and the announcement of NICE's consultation on appraising end of life medicines, have resulted in the Health Committee inquiry into the purchase of additional drugs by NHS patients ("top-up fees"). However we are moving into an era where vaccines must also be considered alongside traditional drugs. The first prophylactic vaccine against cervical cancer, Gardasil[®], received European Marketing Authorisation on 20 September 2006 and is available on the NHS and privately in the UK. Cancer vaccine pipelines represent an area of increasing clinical interest, which can only continue in the future. There are a plethora of other vaccines in development with differentiated profiles that include further prophylactic vaccines which will compete with and hopefully eventually even replace the costly chemotherapy and radiation treatments currently being used to treat cancer. As a consequence of these developments and the shifting therapeutic, and now (increasingly) prophylactic landscape, patients are faced with choices about the right prophylactic or therapeutic approach in discussion with their clinicians. Lord Darzi's report from June 2008 placed choice and patient empowerment firmly on the agenda for the NHS and if patients now have the choice to pay top up fees to gain access to newer medicines that are not available freely on the NHS, an inquiry into patient top-up fees must consider vaccines and drugs alongside one another as the examples below for Gardasil[®] clearly demonstrate.

About Sanofi Pasteur MSD

2. Sanofi Pasteur MSD is the only company in the UK totally dedicated to vaccines. With a heritage that includes pioneers in vaccination, such as Louis Pasteur (rabies vaccine) and Maurice Hilleman (measles vaccine), we have grown to become a leading supplier of vaccines. Using a combination of research and manufacturing expertise, our purpose is to bring innovative vaccines to the UK, protecting health and preserving quality of life for all ages.

3. Our main way of achieving this goal is to develop and make available innovative vaccines against a wide spectrum of diseases. We are also dedicated to increasing the understanding of the value of vaccines and vaccination by providing relevant and accurate information on their quality, safety and efficacy.

Vaccination Against Cervical Cancer and Genital Warts

4. Cervical cancer and a number of other genital diseases are known to be caused by HPV, which has been isolated in more than 99% of cervical cancers.⁹⁴ HPV is very common within the population, and up to 80% of sexually active women will be infected during their lifetime. There are four HPV types responsible for the majority of female genital disease.^{95, 96, 97} HPV types 16 and 18 are considered “high risk”, and are responsible for 75% of cervical cancers, and HPV types 6 and 11 are considered lower risk, but are responsible for 90% of genital warts and 10% of low grade smear abnormalities (cervical intraepithelial neoplasia (CIN)).

5. There are two vaccines licensed in the UK for the prevention of cervical cancer, a quadrivalent vaccine (Gardasil®) and a bivalent vaccine (Cervarix™). Whereas the bivalent vaccine is indicated for premalignant cervical lesions and cervical cancer causally related to Human Papillomavirus (HPV) types 16 and 18, Gardasil® is licensed for the prevention of cervical cancer as well as for the prevention of a range of premalignant genital lesions (cervical, vulvar and vaginal) and external genital warts (condyloma acuminata) causally related to Human Papillomavirus (HPV) types 6, 11, 16 and 18.

National Immunisation Programme against Cervical Cancer

6. From September 2008, the national immunisation programme has offered the bivalent vaccine (Cervarix™) for schoolgirls in year 8 (aged 12 to 13 years), with a catch up programme to be implemented so that other years can also receive the bivalent vaccine. A three year catch up campaign also commenced in September 2008 and will make the bivalent vaccine available for 13 to 18-year-olds. Girls aged 17–18 will be offered the vaccine in the 2008–09 school year, girls aged 16–18 in the 2009–10 school year and girls aged 15–17 in the 2010–11 school year.

SUPPORTING EVIDENCE

7. SPMSD has received feedback from clinicians which is pertinent to the Health Select Committee inquiry on top-up drug payments. Most recently a UK clinician, Dr Phil Hammond, expressed his view in the *British Medical Journal* (BMJ 2008;337:a2186) regarding to the possibility to have a choice between cervical cancer vaccines for his daughter because he wanted to top-up the difference between the cost of Cervarix™ (which is used in the national immunisation programme) so that his daughter could have Gardasil®, within the NHS programme. This request was denied.

8. The Department of Health has estimated that there are approximately 300,000 girls in each age cohort in England and primary care trusts should be offering the vaccine to all of these girls. Dr Hammond’s view highlights that there are some important additional considerations when considering vaccinating against HPV.

9. SPMSD is supportive of the Department of Health’s national schools immunisation programme for cervical cancer and does not wish to question the choice of vaccine being used in the programme. However there are patient groups which will fall clearly outside the scope of the programme and provision should be made so that they may also have the opportunity to be vaccinated.

10. It’s interesting to note that women over the age of 18 are currently not eligible for the HPV vaccine under the national programme. Whilst routine vaccination in this age group is not considered cost effective, the expert Joint Committee on Vaccination and Immunisation (JCVI) that advises ministers on vaccine policy acknowledges that certain groups of individuals could well derive significant individual benefit from vaccination. The Department of Health has so far issued no guidance in this area. Dawn Primarolo has recently been quoted as saying that “Doctors may prescribe the HPV vaccine to women over the age of 18 in exceptional clinical circumstances”. In addition to apparently extremely limited availability on the NHS outside the routine programme, HPV vaccines are also available privately. Further complicating matters, a letter from Ann Keen MP, Parliamentary Under Secretary of State (22 August 2008), states that NHS patients who wish to be vaccinated with Gardasil® instead of Cervarix™ cannot be prescribed this vaccine privately by their own GP. These patients can ask their GP to refer them to another GP practice, or to a private clinic where the vaccine can be prescribed privately.

11. Compounding this, a survey published in “GP” on 5 December 2008 showed that 52% of responding GPs stated that their primary care organisation had stated not to give HPV vaccine outside of the programme. 67% would vaccinate outside of the programme anyway if a suitable patient requested it and 70% believe the programme should be extended to include women over 25 years. From this it would seem that outside of the schools programme, there will be significant inconsistency/inequality and uncertainty

⁹⁴ Walboomers Jan M M et al. Human papillomavirus is a necessary cause of invasive cervical cancer worldwide. *J. Pathol* 1999; 189:12–19.

⁹⁵ Smith J S, Lindsay L, Hoots B et al. Human papilloma virus type distribution in invasive cervical cancer and high-grade cervical lesions: A meta-analysis update. *Int J Cancer* 2007; 121:621–632.

⁹⁶ Wieland U and Pfister H. Papillomaviruses in human pathology: Epidemiology, pathogenesis and oncogenic role. In: Gross, Barasso Eds. *Human papilloma virus: A clinical atlas*. Ullstein Mosby; 1997. p1–18.

⁹⁷ von Krogh G. Management of anogenital warts (condylomata acuminata). *Eur J Dermatol* 2001 11:598–604.

across regions regarding who will be able to receive HPV vaccination without paying privately and which (if any) vaccine will be made available with NHS funding. If the bivalent vaccine is offered on the NHS, will there be the option to make a top-up payment in order to receive Gardasil® which is also licensed and had clearly demonstrated additional benefits? For example, there are opportunities for Genito-Urinary Medicine (GUM) clinics to tackle the sizable challenge of genital warts (89,838 new cases in 2007 in UK GUM clinics)⁹⁸ by offering HPV vaccination with Gardasil®. They are however, prevented from doing so because many PCTs have taken a stance not to prescribe, with the only other option being private prescribing which is beyond the affordability of many, including vulnerable individuals that may need the vaccine the most. This is an example of where top-up payment which could cover the cost of vaccination with Gardasil® (but not the cost of the GUM clinic visit) which would help to target vulnerable groups of the population. Immunocompromised individuals such as those who are HIV positive are at particularly increased risk from the consequences of HPV infection, such as cervical cancer and genital warts.

12. Similarly, those outside the national programme should be allowed to ask their own GP for Gardasil® without paying an inflated private price. In some areas, for example, further education students who fall outside the national programme are paying to receive HPV vaccination where the PCT will not provide funding.

SUMMARY AND RECOMMENDATIONS

13. Cervical cancer and genital warts present a sizeable economic and societal burden. There are two licensed vaccines against HPV related diseases in the UK, a bivalent vaccine and a quadrivalent vaccine. The former has been chosen for the national immunisation campaign which was introduced in schools in the autumn of 2008. However outside of this programme there is considerable scope to reduce healthcare inequalities, preserve patient choice and encourage rational prescribing.

14. SPMSD believes that the Health Select Committee should consider the issues surrounding the changing therapeutic landscape for cancer where vaccinations are increasingly available as an option to patients alongside the evidence it reviews for drug top-up fees for anti-cancer medicines and recommend to the Department of Health that guidance needs to be issued on the following so that healthcare standards can be raised, inequalities removed and a patient's right to choose be protected.

15. Being able to make a top up payment for a vaccine that is not being centrally funded so that patients only need to pay the difference in cost between vaccines rather than the whole cost of the vaccine. In order to preserve the commercially sensitive price of the centrally procured bivalent vaccine, an arbitrary "top up cost" could be agreed based upon various data that are in the public domain.

16. Those falling outside the national programme, but for whom the vaccine is licensed and could provide benefit, should be allowed to ask their GP for Gardasil® and cover its cost to the NHS rather than the greater private price.

December 2008

Memorandum by Pfizer Limited (TF 35)

TOP-UP FEES

EXECUTIVE SUMMARY

1. The issue of access to appropriate medicines and to NHS care for those paying for treatment privately is of critical and increasing importance to UK patients and as such Pfizer is pleased to have the opportunity to contribute to the Health Select Committee's "Top-up Inquiry". We welcome Mike Richard's report and recommendations for "Improving access to medicines for NHS patients", but whilst Pfizer believes this report offers the best and most pragmatic approach for improving access to medicines for patients, the issue of "top-ups" is far from ideal and should, as Mike Richards sets out, be kept to an absolute minimum.

2. We welcome the Government's commitment to improve patient choice and access to medicines in the UK and a broader debate about how the NHS should evolve, including how different funding models including the role of a different public/private mix might be developed. When private funding of treatment on the NHS is necessary—however infrequent—we feel it is important to ensure transparency, fairness and consistency of the price patients pay for such medicines at the pharmacy. Anecdotal feedback that we have received suggests the size of dispensing fees placed by pharmacies over and above the list price of the medicine that are purchased privately varies significantly across the country.

⁹⁸ HPA 2007, website link: hpa.org.uk/web/HPAweb&HPAwebStandard/HPAweb_C/1195733775264org

3. We recognise that the reality of keeping “top-up” to a minimum will be very much dependent on the numerous recommendations in the report being implemented as set out, largely by the DH and NICE but also with implications for the NHS and the pharmaceutical industry. Pfizer is committed to working constructively with Government, NICE and other stakeholders to ensure the recommendations are implemented and to ensure the medicines we produce for the NHS offer value for money.

ENSURING THE DEBATE ON ACCESS TO MEDICINES IS BASED ON EVIDENCE

4. Pfizer is optimistic that achieving a vision of world-class access to the best medicines for UK patients in a way that neither bankrupts the NHS nor undermines the legitimate return on innovation for the pharmaceutical industry is within our reach. To achieve this though, we need to ensure that the debate is based on a common and realistic understanding of the evidence.

5. We believe that much of the current debate is based on a false premise—namely, that the NHS is faced with an unmanageable financial burden, driven by new high-cost medicines and that, as a result, a shift in the NHS funding model is necessary, requiring individual patients to pay directly for part of their care.

6. In reality, there is clear evidence to show that overall spend on medicines is one of the most stable components of NHS cost and, across primary and secondary care, is far from out of control. In addition, factors including:

- the £1.7 billion NHS surplus—however we understand this is now due to be spent elsewhere as a result of the current economic issues;
- the additional savings that will be achieved through the pharmaceutical industry delivering on the recently agreed PPRS price cut; and
- the historical poor uptake performance in the UK—acknowledged by Government;

all suggest to us that we ought to challenge the notion that patients need to be put in a position where they have to fund medicines privately in the first place.

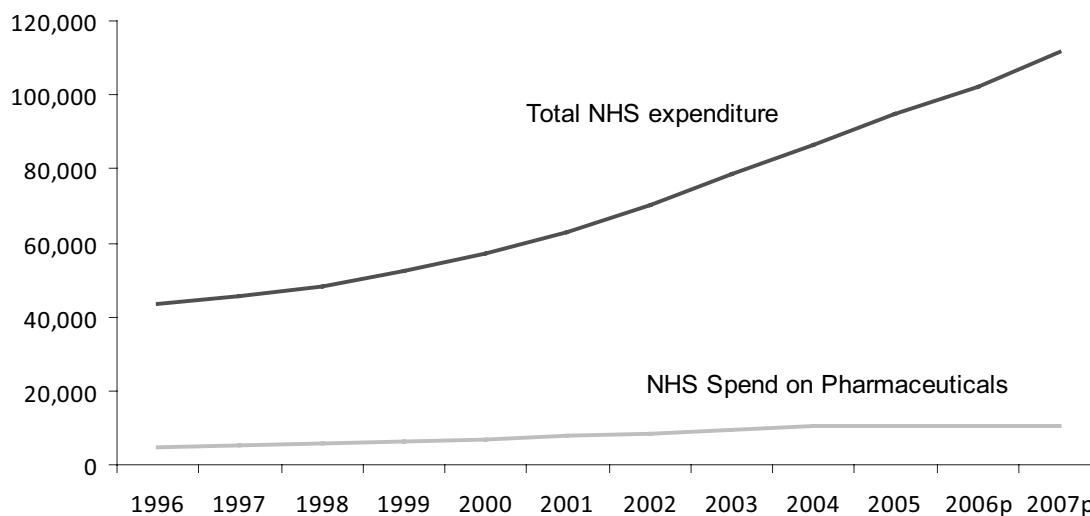
7. We would recommend Government’s efforts are geared towards developing a more effective way of reallocating resources across the NHS to ensure that available funds are used to treat patients in need.

MEDICINES SPEND IN CONTEXT

8. The last 10 years have seen a substantial expansion of NHS resources. However, within overall Government health expenditure, the proportion spent on medicines has declined.

Figure 1

NHS EXPENDITURE—TOTAL AND PHARMACEUTICAL 1996–2007



Sources: Prescription Pricing Authority, Office of National Statistics, Department of Health, NAW, ISD, IMS dataview.

9. Figure 1 illustrates quite simply the point that spend on medicines has not driven expansion of NHS budget. Despite the stability of medicines spend, more patients have been treated. In other words, not only has medicines spend been under control, it has become more productive. The NHS’ ability to treat more patients for less money has in part come from the efficiencies the Government has achieved in driving down the price of generic medicines through, for instance, the creation of Category M of the Drug Tariff. We support such measures. As an industry, we are aware that once our medicines lose patent protection Government can and should expect to reap the benefits of lower prices through competitive markets.

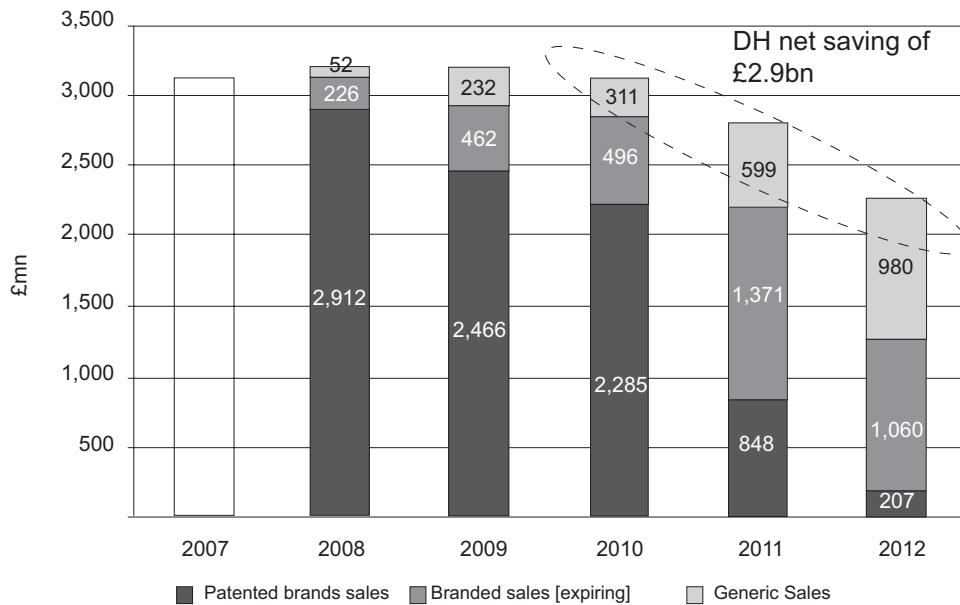
10. Government savings derived from out of patent competition are an essential part of the medicines life-cycle that should help pay for newer medicines coming onto the market. But the existence of such savings is often overlooked by policymakers faced with decisions on whether to fund individual new treatments and by the media which comment on their availability and on their, often high, unit cost.

11. The current debate has been dominated by a small number of cancer medicines which, although high cost, treat far fewer patients than the sort of medicines going off patent over the next few years. In the context of the recent PPRS negotiations, ABPI has undertaken a major forecasting exercise⁹⁹ looking at the entire medicines bill, across both primary and secondary care. The analysis shows that, in reality, some of the most widely prescribed medicines on the NHS will be losing exclusivity between now and 2012 generating an estimated £2.9 billion worth of savings—illustrated by Figure 2.

Figure 2

NHS SAVINGS FROM BRANDED MEDICINES LOSING EXCLUSIVITY BETWEEN 2007–12

Growth in medicines bill can largely be paid for by savings the medicines bill post-patient



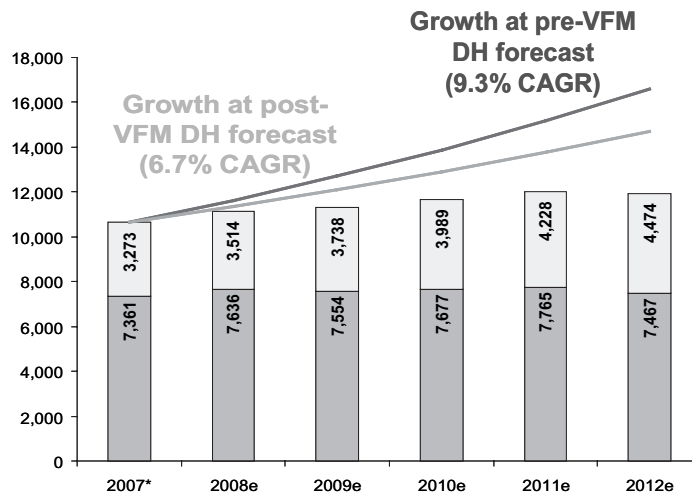
Source: IMS data; ABPI analysis.

12. The analysis also shows that if Government increased its spend on new cancer medicines to the average levels of major European countries it would cost in the region of £300 million to £500 million annually. Although these sums of money seem large, they should not be seen in isolation from the rest of the medicines bill. A holistic view shows that growth due to new products is largely off-set by savings driven from LoE. Figure 3 shows the ABPI forecast of net growth across primary and secondary care medicines, indicating a compound growth rate of 2.4% until 2012—well below the growth rate of overall health spend (even accounting for agreed DH efficiency commitments) of 6.7% growth.

⁹⁹ Details of the ABPI forecast have been shared fully with HM Government during the course of the recent PPRS negotiation. The forecast is, at the time of drafting this document, unpublished.

Figure 3

FORECAST EVOLUTION OF BRANDED PRIMARY CARE AND HOSPITAL MEDICINES BILL (2007–12) INCLUDING GENERICS (IN £000s)



Source: ABPI analysis.

INTERNATIONAL COMPARISONS OF PHARMACEUTICAL PRICES

13. Pfizer believes that not only is the overall growth of the medicines bill under control, the UK gets a comparatively good deal on the price of its medicines relative to similar countries.

14. ABPI and Government have—over a number of years—examined international pharmaceutical prices and the Department of Health has periodically included a ranking of comparable countries, following an agreed methodology, in the annual PPRS report to Parliament. The last figures published by the DH, from 2006, show that UK ranked 5th in order of medicine cost in a basket of 11 countries. ABPI has recently re-calculated the ranking—using the same data and methodology for 2008—which is shown in Figure 4 and which shows how the UK has dropped to 9th most expensive out of the same basket of 11 countries—see figure 4.

15. Whilst Pfizer recognises that international price comparisons are not without their problems, we feel there is overwhelming evidence supporting the view that, at least, UK prices are not out of line with other countries.

Figure 4

INTERNATIONAL PHARMACEUTICAL PRICE COMPARISONS

Country	1992	1993	1994	1990	2000	2001	2002	2003	2004	2005	2006 DH	Rank	est Q1 2008	Rank
France	91	109	100	84	80	81	81	91	84	96	89	10	99	10
Germany	148	125	120	97	91	94	94	102	106	108	105	3	121	2
Italy	96	78	98	73	79	82	86	90	78	84	79	12	88	12
Netherlands	135	132	133	81	81	84	88	93	92	95	95	9	107	5
Spain	84	74	82	64	64	67	75	81	80	84	86	11	91	11
UK	100	100	100	100	100	100	100	100	100	100	100	5	100	9
USA	188	194	190	184	209	217	201	190	176	198	192	1	211	1
Austria				83	77	81	86	94	94	96	95	8	101	8
Belgium				84	78	81	86	91	90	95	97	6	105	6
Finland				85	83	84	88	98	96	101	96	7	103	7
Ireland				88	83	88	93		99	103	106	2	116	3
Sweden											103	4	111	4

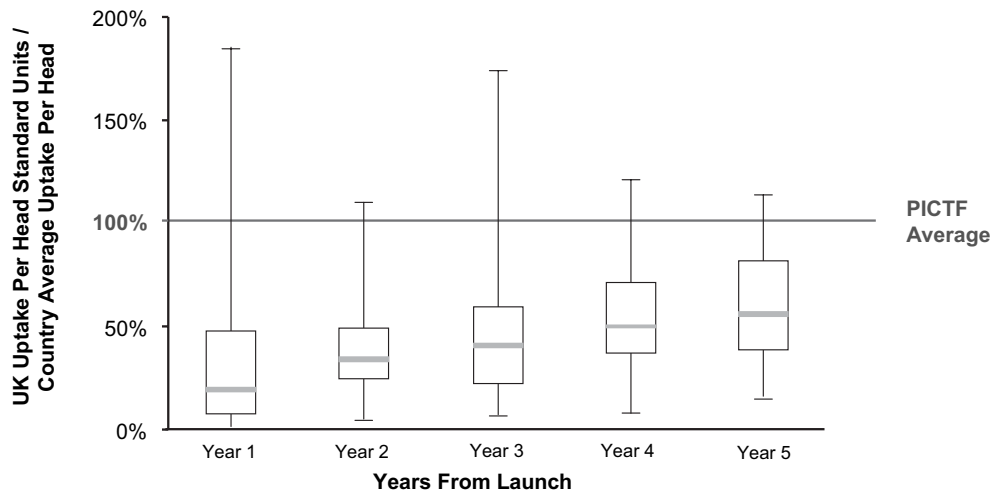
*Prevailing exchange rates. Q1 2008 estimate is based on Q4 2007 prices at Q1 2008 exchange rates

UPTAKE OF NEW MEDICINES IN THE UK

16. As noted in Mike Richards’ recent Cancer Strategy¹⁰⁰ the UK uses approximately 60% of the volume of modern cancer medicines compared to other major European Countries. The picture in cancer, however, reflects a wider trend, acknowledged in the Ministerial Industry and Strategy Group’s Long Term Leadership Strategy¹⁰¹ report that the uptake of new medicines in the UK has been below average and, although some progress has been made in recent years, is far from reaching levels that compare with similar countries.

Figure 5

UK UPTAKE OF MEDICINES COMPARED TO PICTF AVERAGE (2005)

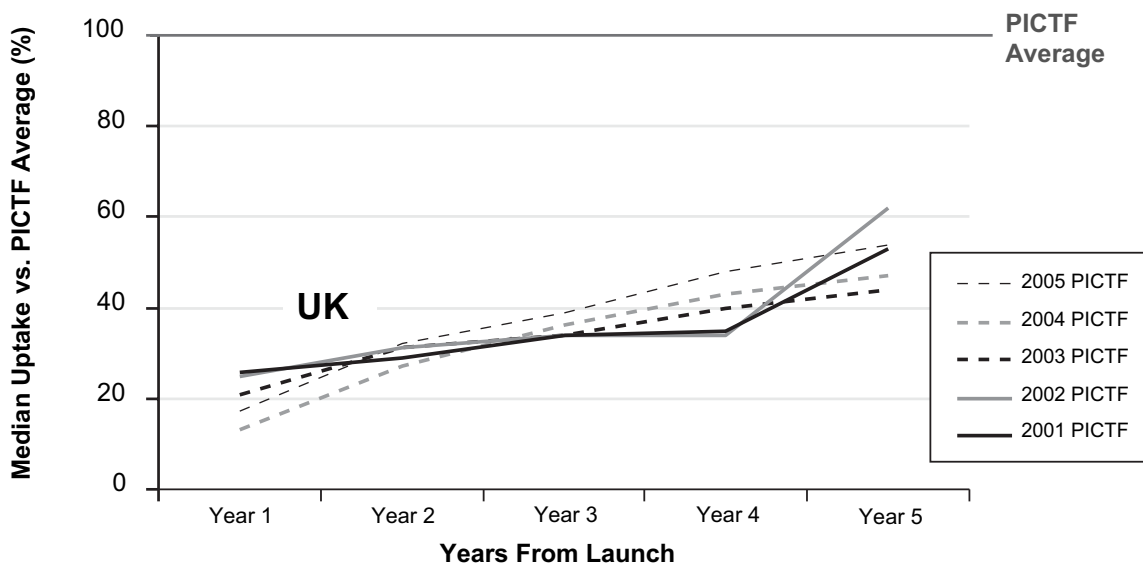


* PICTF Countries include Australia, Canada, France, Germany, Italy, Japan, Netherlands, New Zealand, Spain, Sweden, Switzerland, UK and US

17. Figure 5 shows how, for each year after launch of a given medicine, the average rate of uptake in the UK lagged the average rate across the agreed list of comparator countries in 2005. Figure 6 shows how this pattern changed between 2001 and 2005. The trend is positive, but there is still some way to go.

Figure 6

UK UPTAKE OF MEDICINES COMPARED TO PICTF AVERAGE (2001–05)



¹⁰⁰ Department of Health, December 2007 “Cancer Reform Strategy”, page 65.

¹⁰¹ Department of Health and The Association of the British Pharmaceutical Industry, February 2007, “Ministerial Industry Strategy Group: Long Term Leadership Strategy”.

IMPLICATIONS OF THE RECENT PPRS DEAL FOR THE UPTAKE OF MEDICINES

18. Reflecting our shared understanding of the problem of slow uptake of new medicines in the UK the pharmaceutical industry recently agreed with Government a range of positive measures to support innovation as part of the new Pharmaceutical Pricing and Regulations Scheme (PPRS). Measures include:

- *Single* Horizon Scanning process to enable NHS planning.
- NICE appraisal guidance to be implemented locally *without qualification*.
- Pilot incentive schemes to promote uptake of innovative products in PCTs and PbR levers.
- Agreed review of economic and social parameters to be included in NICE assessment.
- Commitment to publication of local metrics for uptake of clinically and cost- effective medicines AND annual publication of local and international uptake comparisons.
- For products not assessed—development of best practice guidance for local evaluation.
- For products not initially assessed as cost effective by NICE—flexible measures to facilitate earlier patient access.

19. Alongside the committed measures on uptake, industry has agreed to deliver a price cut on medicines. Whilst we do not feel the Government provided an adequate rationale for a price cut, as an industry, we have agreed to play our part by cutting prices given the context of the Department of Health’s recent comprehensive spending review settlement which required it to deliver savings.

20. The flexible measures, (or patient access schemes), which form a part of the PPRS agreement—and highlighted in Mike Richards’ top-up review—are another good opportunity for those responsible for the provision of healthcare in the UK to ensure patients get access to medicines and that government feel they are getting value for money. Hopefully the points agreed within the PPRS will mean that “top-ups” are kept to an absolute minimum, and even then, only in the short-term whilst more, fundamental developments are agreed for the longer-term.

POSSIBLE CHANGES TO NICE PROCESSES

21. Pfizer welcomes the recent announcement of the “Appraising end of life medicines” consultation by NICE, which demonstrates a common understanding that improving patients’ access to important, innovative new medicines, particularly for patients nearing end of life, is of critical importance. We commend NICE for recognising that the willingness to pay for end of life medicines may be higher than that for other types of medicines, and therefore may require a more tailored approach to appraisal of these important medicines through the use of significantly improved tools and methodologies.

22. However, while we welcome the additional flexibility that these proposed rules will offer the NICE appraisal committee, they do not address the underlying problems, widely recognised, on how to measure and value incremental health gain. We contend that the measurement and valuation of health gain remains at best a very crude exercise and left unresolved has the potential to continue to distort NHS resource allocation decisions. We therefore recommend urgent research into more sophisticated methods for measuring and valuing health gain.

23. We also believe that the problem of over-reliance on these crude measures of economic efficiency extends beyond “end of life medicines” and is common to all disease areas. While value for money is clearly an important consideration, other factors such as unmet need, equity and social preferences on how we allocate tax payers money within the NHS are equally important considerations. We recommend urgent research to agree a framework for systematically considering this more complete range of considerations within the appraisal decision making process for all appraisals, not just those medicines that constitute end of life care.

24. Pfizer believes that medicines assessment is one of the fundamental access issues in the UK, and a review of tools and methodologies used for all types of treatments is critical to ensuring the value of medicines are assessed appropriately for patients to receive the right treatments for the best possible health outcomes. In order to meet the aspirations set out in the Mike Richards report, of “top-ups” being kept to a minimum, NICE will undoubtedly need to introduce a degree of flexibility in the application of existing HTA criteria and remove the requirement to meet them all.

IMPLICATIONS FOR THE TOP-UP DEBATE

25. The implication for the top-up debate of the issues raised above is straightforward. The NHS, as a whole, has sufficient resource to pay for those medicines valued by patients. Where NICE has suggested a particular medicine is unlikely to be cost effective for widespread use on the NHS it is surely the case that, where an individual patient has a particular clinical need, funding ought to be found if the NHS is to provide a comprehensive and equitable service.

26. We recognise that it is not always easy—in an organisation the size of the NHS—to allocate resources efficiently. But it is surely questionable that individual patients should be expected to pay for treatments simply because available funds are sitting in the wrong part of the NHS.

27. We believe that significant progress has been made in the last year through constructive joint working between Government, NICE and industry. Mike Richards report offers a pragmatic solution to a perverse and unnecessary problem; whilst the package of measures agreed as part of the PPRS agreement, alongside NICE's willingness to improve the way it appraises treatments for end of life hopefully mean that patients needing to fund treatments privately will be kept to a minimum. We need to continue to monitor whether the above measures deliver our jointly-held objectives of improving access to medicines in the UK in a financially sustainable way for the NHS. We are keen to continue to work with Government and other stakeholders to further improve the service UK patients receive through our medicines.

December 2008

Memorandum by the Association of the British Pharmaceutical Industry (ABPI) (TF 36)

TOP-UP FEES

INTRODUCTION

1. The Association of the British Pharmaceutical Industry represents more than 70 companies in the United Kingdom producing prescription medicines. Its member companies are involved in all aspects of research, development and manufacture, supplying more than 80% of the medicines prescribed through the National Health Service. The ABPI also represents companies engaged solely in the research and/or development of medicines for human use. In addition, there is general affiliate membership for all other organisations with an interest in the pharmaceutical industry in the United Kingdom.

2. The ABPI welcomes this inquiry into top-up fees.

OVERVIEW

3. The ABPI submitted responses to both Mike Richards and the Conservative party on 21 August and 4 September respectively.

4. The review of the "top-up fees" carried out by Mike Richards was a sensitive task and the document "*Improving access to medicines for NHS patients*" is well balanced with sensible recommendations. In our view, Mike Richards understood well the sensitivities associated with top-up payments. We agree that patients confronting the difficult choice of having to pay for innovative medicines that are not funded by the NHS should be a very small group. Indeed, Lord Darzi's review and the draft Constitution both stipulate that patients should be entitled to NICE-approved medicines. Patients will benefit if the NHS moves to a more innovation-embracing culture, providing them with access to modern medicines that can improve quality and quantity of life and enable many to continue to work thereby helping the economy.

BACKGROUND

5. UK patients do not enjoy the full benefit of the innovations coming from the R&D investments made by pharmaceutical companies. Healthcare spending on medicines has been low and the uptake of innovative treatment slow compared to other European countries. The discontinuation of the 2005 PPRS has destabilized the market and further price cuts have undermined confidence in the UK within the industry. The ABPI could see no reasons for these further cuts given that UK medicines prices are lower than in other comparable European countries. There should not be any reason, therefore, why patients should be refused treatments on the ground of cost if their clinicians deem such treatments appropriate.

6. The NHS tends to look at the budget impact of a medicine rather than the value it brings to the NHS and to society. A broader definition of value should be adopted both in Health Technology Assessment processes and by local NHS organisations when decisions are being made on whether patients should receive treatment.

OUR REVIEW OF MIKE RICHARDS' RECOMMENDATIONS

Recommendation 1: *The measures the government is already taking to improve the timeliness of the NICE decision making process are extremely welcome and should be strongly supported. The Department of Health and NICE should publish an update on the timelines for delivering these important commitments.*

7. The ABPI agrees with this recommendation. It is important to remember, however, that at the time of launch clinical and economic data derived from use in a "normal" clinical setting are by definition limited and that such limitations should be recognised via a pragmatic approach to decision-making with decisions open to review once additional information becomes available.

Recommendation 2: The Department of Health should urgently consider how PCTs can be encouraged to work together to make proactive commissioning decisions. Consideration should be given to whether collaborative processes already developed, such as in the North East for cancer drugs, could be used as a model.

8. Whilst collaboration by PCTs with similar populations makes sense to avoid unnecessary bureaucracy and duplication of work, the processes they use to evaluate innovative medicines and make funding decisions for populations or individual patients should be transparent, robust and according to accepted best practice. Such collaborations should ensure that the companies that have developed the medicine are able to provide up-to-date information and critique factual information on their products.

Recommendation 3: The commitment made in the draft NHS Constitution to ensure transparency in PCT decision making, and the resulting work being undertaken by the Department of Health to support PCTs in delivering this, is extremely welcome. The government should set out as soon as possible more detailed plans for how it will achieve the commitment in the NHS Constitution, including the timescale for this work.

9. The Department should ensure that best practice principles and processes are adopted by local NHS organisations when making decisions on the funding of medicines; transparency on how those decisions have been made is essential for patients, clinicians and other stakeholders in order to limit unnecessary postcode prescribing and engender confidence in the system.

Recommendation 4: In developing collaborative arrangements for decision making, the government should also consider how PCTs can be better supported to make decisions on funding off-label drugs, whether as a matter of policy or on an exceptions basis.

10. Again, consistency in decision making will be welcomed.

Recommendation 5: The Department of Health should work:

- *with NICE to assess urgently what affordable measures could be taken to make available drugs used near the end of life that do not meet the cost-effectiveness criteria currently applied to all drugs; and*
- *with the pharmaceutical industry in the context of the current Pharmaceutical Price Regulation Scheme (PPRS) negotiations to promote more flexible approaches to the pricing and availability of new drugs. This will require partnership working with the pharmaceutical industry and greater flexibility in approach from all parties.*

11. The ABPI welcomes the consultation from NICE on the appraisal of end-of-life treatments. Our response is attached and brief commentary given below.

12. We also welcome the new flexible arrangements, agreed as part of the PPRS, which will enable the industry to re-evaluate the price of their medicines when new information or data become available.

Recommendation 6: The Department of Health should urgently undertake further work to investigate the extent and causes of international variations in drug usage.

13. The DH and ABPI are working together to make robust comparisons of uptake in the UK vs other countries; these are in the planning stage. Industry values this dialogue.

Recommendation 7: The Department of Health should clarify the policy on how the NHS should handle situations where a patient wishes to purchase additional treatment. The objective should be to ensure consistency in practice across the NHS.

14. Consistent adoption of DH policy will be essential to ensure equity of decision-making and confidence in the system of top-up payments. We indeed welcome the consultation issued by the Department of Health on their Guidance on NHS patients who wish to pay for additional private care. The ABPI will respond to this and gives a brief commentary below.

15. We believe clarity should be given by the DH on dispensing fees for these medicines; it is our understanding that currently dispensing fees for private prescriptions vary widely.

Recommendation 8: *The Department of Health should make clear that no patient should lose their entitlement to NHS care they would have otherwise received, simply because they opt to purchase additional treatment for their condition.*

16. The ABPI agrees with the decision not to withdraw patients' right to NHS treatment when purchasing additional treatment. If NHS funding is regrettably refused because of a negative NICE recommendation or a local PCT decision, patients should have the option to top up their NHS care by paying for the medicine—without losing the remainder of that NHS care. The clinician should additionally ensure that the top-up payment will not seriously damage the lifestyle of the patient and/or their family, for example through sale of the family home.

17. The reasons for any decision to refuse funding should be made clear to the patient and be subject to public scrutiny. Top-up payments for medicines should be a rare event and the industry does not anticipate that they would represent a major source of revenue.

Recommendation 9: *The government should make clear that:*

- *clinicians should exhaust all reasonable avenues for securing NHS funding before a patient considers whether to purchase additional drugs;*
- *patients should be able to receive additional private drugs as long as these are delivered separately from the NHS elements of their care; and*
- *providers should establish clear clinical governance arrangements to ensure that patients who do elect to purchase additional private treatment receive good continuity of care.*

18. These recommendations represent a pragmatic and practical route to enabling patients to choose additional private care. The dialogue between the clinician and patient will be critical in establishing clearly to the patient what their options are. As said above, it is important that decisions not to fund a medicine are made under transparent and robust processes that follow best practice principles and operating procedures. For rare disease treatments, funding could possibly be derived from a central fund, using consistent criteria to avoid unfair decisions.

Recommendation 10: *Strategic Health Authorities, working where appropriate through cancer networks, should ensure that local policies are developed to ensure that any revised guidance issued by the government is implemented properly. This might include using a designated hospital with private facilities for all patients wishing to purchase additional drugs, making use of homecare provision or designating an area of an NHS hospital for the delivery of privately funded treatments.*

19. Government guidance will contribute towards a consistent approach across England. Consistent adoption of the guidance will be essential to ensure equity of decision-making and confidence in the system of top-up payments.

Recommendation 11: *The Department of Health should take a lead on commissioning a national audit of demand for unfunded drugs and on the outcome of treatments, working closely with professional organisations and NHS managers.*

20. As we are moving into unknown territory, data collection will be essential to evaluate the new policy and inform future decisions. It would be sensible to work with companies on data collection on treatment outcomes, to share conclusions and avoid unnecessary duplication.

Recommendation 12:

- *Doctors who are likely to have conversations with patients about treatments that are not routinely funded on the NHS should ensure that they have the necessary knowledge and skills to communicate complex information effectively and in a balanced way. This will help patients to make informed assessments about the balance of risk, cost and benefit involved in any potential treatment.*
- *The Department of Health should commission a training programme for clinicians to enhance the quality of discussion about these difficult issues.*
- *Relevant Royal Colleges should consider how assessment of communication skills could best be incorporated into recertification processes.*

21. Providing information to patients is an extremely important task, the more so when patients are required to make important decisions about whether to pay for their treatment. Ideally, decisions should be made by the patient and doctor together and doctors need excellent communications skills to convey complex information at a very difficult time in the patient's life.

22. The pharmaceutical industry, as an important source of information on the medicines that it has discovered and developed, can help through the provision of factual, evidence-based information for patients. The ABPI welcomes the recent European Commission's proposals as part of the pharmaceutical

package that industry should be allowed to provide limited information to the public on websites and in response to enquiries. Companies will also play their part in supporting health professionals to communicate effectively with their patients.

Recommendation 13: The Department of Health should consider how patients could best be given access to balanced written information on the benefits, toxicities and, where appropriate, costs of novel treatments, especially those given to patients near the end of life.

23. As said above, the pharmaceutical industry is an important source of information and would be happy to engage in discussions with the Department of Health to support implementation of this recommendation.

Recommendation 14: In responding to this Review, the government should confirm how situations where patients wish to purchase additional non-drug interventions should be handled.

NICE CONSULTATION ON APPRAISING END OF LIFE MEDICINES

24. The ABPI welcomes NICE's consultation on the appraisal of end of life medicines. The consultation is an important recognition that society places different values on different health interventions and whether patients should have access to treatment. The use by NICE of a one-size-fits-all application of a set cost per QALY threshold fails to reflect the complexity of evaluating the vast array of interventions that NICE has to assess, and whilst we understand that the NICE Social Value Judgements have some part in decision-making, we do not believe that factors other than the cost per QALY are sufficiently taken into account by the Appraisal Committee. The methods used to derive a cost per QALY are generally prejudicial to patients at the end of life, the elderly and those with long term conditions where small incremental improvements in treatment can make a big difference to daily life.

25. Overall comments are as follows:

26. It is not clear why the guidance to the Appraisal Committee is limited to end of life medicines only and not to other rare and debilitating conditions. This seems to discriminate unnecessarily against patients with life-threatening conditions for whom there is no alternative treatment.

27. The additional guidance focuses on extension to life only and appears to give no consideration to patient quality of life. This is unlikely to be consistent with the consideration taken by the patient and clinician in actual clinical practice of options and trade-offs for the patient.

28. Whilst we have no issue with data collection by NICE following appraisal, we would caution against overlap with existing studies and against under-estimation of costs where there is no existing disease registry. We believe that the focus of the data collection should include appropriate use of the medicine by the NHS as well as survival gains and that the manufacturer should be given the opportunity to comment on the protocol to ensure that their expertise relating to the product and therapy area is maximised.

29. It is not clear from the consultation document how NICE will report on how the additional guidance has been applied by the Appraisal Committee. Clarity in ACDs, FADs and final guidance will be essential to stakeholders' understanding of the decision making process.

EARLY THOUGHTS ON THE DH CONSULTATION "GUIDANCE ON NHS PATIENTS WHO WISH TO PAY ADDITIONAL PRIVATE CARE"

30. The DH guidance appears to be clear, useful and pragmatic. The challenge will be in local implementation.

31. The consultation makes the point that provision of information to patients on their options will be an extremely important aspect of making the new scheme work. As we say above, the pharmaceutical industry has an important role to play in the provision of information on its medicines.

32. The guidance states that "clinicians should exhaust all reasonable avenues for securing NHS funding before suggesting a patient's only option is to pay for care privately". In the case of end-of-life treatments, timely decisions are essential and delays should be avoided to enable patients for whom days count to receive urgent treatment, should it be funded by the NHS or paid for privately.

33. We believe additional guidance should be given to the NHS on the dispensing fees for these medicines, as we understand that these can vary widely.

December 2008