EXCEPTIONAL FUNDING

Recommendations for improving access to cancer medicines and delivering better value for money for the NHS

August 2010
About the Rarer Cancers Foundation

The Rarer Cancers Foundation (RCF), which incorporates the Rarer Cancers Forum, 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. Between 30% and 50% of all cancers can be classified as ‘rarer’. A cancer may be classed as rare either because it affects an unusual site in the body, or because the cancer itself is of an unusual type, or requires special treatment.

Previous reports by the RCF include:

- *Taking Exception* – An audit of the policies and processes used by PCTs to determine exceptional funding requests, published in August 2008
- *Exceptional England?* – An investigation of the role of Primary Care Trusts in making cancer medicines available through exceptional cases processes, published in October 2008
- *Exceptional Scotland?* – An audit of the policies and processes used by NHS Boards to determine exceptional prescribing requests for cancer treatment, published in March 2009
- *Off limits* – An investigation into how NHS organisations determine requests for the use of off-label treatments for cancer patients, published in August 2009
- *Exceptional Progress?* – Assessing the progress made in improving access to treatment for people with rarer cancers, published in March 2010

This report draws upon information collected through requests made under the Freedom of Information Act, parliamentary questions and a survey of our industry reference group. We are also grateful to IMS Health for supplying data on the cost implications of increasing the usage of new cancer treatments to levels comparable with the international average, and for allowing us to publish the information in this report.

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Summary of key findings

• The current system of pricing and reimbursement for cancer medicines is not working and needs to be changed to reflect the complexities of modern cancer treatment. The Cancer Drugs Fund provides an opportunity to test new approaches.

• NICE has rejected, or announced that it is minded to reject, 10 cancer treatments since the General Election, reaffirming the need for the Cancer Drugs Fund. The Government’s decision to allocate in-year funding in 2010-11 of £50 million for cancer treatments is likely to benefit over 2,000 patients.

• However there are high levels of uncertainty about the true level of demand – and therefore cost – of cancer treatments which are currently not routinely funded by the NHS. The exact level of demands on the Cancer Drugs Fund may range from £175 million to £330 million, although we consider the lower figure to be a more realistic estimate.

• If the Government is to deliver on its pledge to enable patients to access the cancer treatments their clinicians wish to prescribe, then it is imperative that the £200 million per year which was initially earmarked for the Cancer Drugs Fund is indeed allocated. Otherwise there is a high probability that the spirit of the Government’s commitment will not be met and thousands of cancer patients will continue to be denied access to life extending treatments. If funding continues at the annual level of £100 million per year, our best estimate is that 3,600 patients will be denied treatment due to lack of funding.

• Even with the full funding of £200 million per year, there is a risk that over 6,300 patients every year will continue to be denied access to the cancer treatments they need if the higher end of predicted demand occurs, unless the effective safeguards are put in place to manage the cost exposure to the NHS.

• Local processes for determining access to cancer treatments have often been unfair, illogical and inconsistent. Furthermore, they have resulted in unnecessary and costly bureaucratic duplication.

• Patient access schemes – where manufacturers propose a package of measures to the reduce the overall cost of making a treatment routinely available on the NHS – have resulted in reductions in costs, ranging from just under 6% to nearly 38%.

• Both the NHS and manufacturers have reported significant problems with patient access schemes, including a lack of clarity about what is appropriate, administrative complexity and difficulties in securing approval for proposed schemes.
• If England is to achieve cancer outcomes which are comparable with the best in Europe then action will be required on a range of service areas, including encouraging earlier diagnosis of cancer, improving clinical nurse specialist support, improving information on cancer services, expanding support for cancer survivors and increasing research, particularly into rarer cancers.

• Expenditure on cancer services in England increased at a lower rate than the average for all NHS services in the last year for which national figures are available. Spending per capita on cancer in England remains substantially lower than in other countries, which devote a larger proportion of their health budgets to cancer services.
Summary of recommendations

In total, we make 25 recommendations in this report:

1. The NHS should respond to the spirit of the Department of Health’s announcement of in-year funding by commencing reimbursement for currently unfunded cancer treatments before 1 October 2010, when the in-year scheme is scheduled to begin operation. Manufacturers should work with the NHS to offer sufficient financial flexibility to enable patients to receive the treatments their clinicians wish to prescribe immediately.

2. The Department of Health should publish the guiding principles which it intends to underpin the implementation of the Cancer Drugs Fund from April 2011 as part of its consultation on the issue.

3. Given the complexities that occur in cancer treatment, cancer medicines represent an ideal pilot to test approaches to better reflecting value in pricing. Therefore the Cancer Drugs Fund should be used to test a new approach to ensuring that pricing and reimbursement fully reflects the value delivered by modern medicines. The learning from this should then be applied to treatments for other conditions.

4. The Government should allocate the £200 million per year earmarked for the Cancer Drugs Fund when the policy was initially announced.

5. Mechanisms should be put in place to limit the cost exposure to the NHS resulting from the Cancer Drugs Fund. The maximum exposure should be predicated on the initial proposed allocation of £200 million per year.

6. Manufacturers must match the flexibility shown by the Department of Health and play a role in making treatments available to patients at a price which is affordable within the constraints of the Cancer Drugs Fund.

7. Although patient access schemes have delivered additional cost savings to the NHS, they do not provide a sustainable mechanism for enabling every cancer patient to access the treatments they need. For this reason, patient access schemes should only be used in exceptional circumstances rather than as a routine part of appraisals. Where patient access schemes are in place and have proven to be successful, they should of course be continued.

8. For those situations where patient access schemes are being considered in future, the Department of Health should issue clearer guidance about what forms of scheme are acceptable. As part of this it should be made clear that, where a patient access scheme is offered, the manufacturer should be required to reimburse the administrative costs of the scheme.
9. Eligibility to the Cancer Drugs Fund should be restricted to treatments licensed by the European Medicines Agency since 1 January 2005 which have either been rejected by the National Institute for Health and Clinical Excellence (NICE) or have yet to receive a Final Appraisal Determination and situations where a clinician wishes to use a treatment in a near-label setting.

10. In order to ensure their treatments are eligible for the Cancer Drugs Fund, manufacturers should be required to commit to fully cooperating with all relevant NICE technology appraisal processes.

11. Where a treatment is approved by NICE, it should be funded in the usual manner from NHS baselines.

12. A national approach should be taken to the administration of the Cancer Drugs Fund, thereby minimising bureaucracy, ensuring consistency, providing clear accountability and reducing the risk of the Fund being disrupted by the impact of the implementation of reforms outlined in *Equity and excellence: Liberating the NHS*.

13. For licensed drugs included in the Fund, a commercially confidential total NHS cost cap should be calculated. Where this cap is exceeded, manufacturers should be required to reimburse the NHS, thereby ensuring that the £200 million is not exceeded. The negotiations on the cap calculation should be based on:
   - The potential size of the eligible patient population, adjusted for the impact of patient preference, contraindications and the availability of any clinical alternatives.
   - The listed unit price of the treatment.
   - Clinical effectiveness (based on feedback provided by expert advisory groups of cancer clinicians).
   - The extent to which the treatment addresses an unmet need (again, based on feedback provided by expert advisory groups of clinicians).

14. For near-label treatments it will be impossible to calculate a cap in the same way due to the paucity of available data. Instead we suggest that near-label treatments should be reimbursed at list price with manufacturers required to pay a contribution in the form of a rebate to cover the costs of clinical audit. A clear process should be set out for clinicians wishing to prescribe treatments funded by the Cancer Drugs Fund. This should ensure multidisciplinary input to treatment decisions, informed and documented consent from patients and robust clinical governance.

15. A clear process should be set out for clinicians wishing to prescribe treatments funded by the Cancer Drugs Fund. This should ensure multi-disciplinary input to treatment decisions, informed and documented consent from patients and robust clinical governance.

16. The requirement to use the exceptional cases process for treatments which are not routinely funded by commissioners but which are nonetheless included in the
Cancer Drugs Fund should be ended and this should be made explicit in guidance to support the NHS in implementing the Cancer Drugs Fund.

17. Safeguards should be established to encourage appropriate near-label prescribing, including ensuring clinical accountability for treatment decisions. Expert groups should be asked to develop guidance on near-label treatments where there is clinical support for the role that this treatment can play. The prospective clinical audit should be used to identify and prioritise scenarios for which guidance is required. Guidance on near-label treatments for haematological cancers may be a suitable candidate for prioritisation given the high level of demand uncovered in Off limits.

18. Prospective clinical audit should play a fundamental part in the Cancer Drugs Fund and participation in the audit should be a precondition of access to the Fund. The audit should be used to:
   - Enable a more accurate assessment of the true demand for cancer treatments.
   - Facilitate the collection of data on the benefits and toxicities of new cancer treatments in routine NHS practice.
   - Provide a mechanism for assuring the appropriateness of clinical practice.
   - Prevent abuses to the system, such as the parallel exporting of medicines procured through the Cancer Drugs Fund.
   - Develop further evidence on the benefits and toxicities of near-label treatment.

19. The opportunity created by the clinical audit should be used to further establish England’s position as a world leader on clinical research, particularly into rare cancers.

20. The National Cancer Director should be required to submit an annual report to the Secretary of State on the operation of the Cancer Drugs Fund which should then be published.

21. The National Audit Office should undertake a value for money study on the Cancer Drugs Fund in 2013. The results of this should be used to inform the development of value based pricing.

22. Balanced information on the benefits and toxicities of all treatments reimbursed by the Cancer Drugs Fund should be developed as a matter of urgency and included within cancer information prescriptions. Appropriate charities should be involved in the development of this information.

23. The policy attention devoted to improving access to treatments should not come at the expense of improving other aspects of cancer care.

24. As part of the Comprehensive Spending Review, the Department of Health should critically reappraise the balance of NHS expenditure and consider the case for savings. 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.

25. The review and refresh of the Cancer Reform Strategy should identify areas where efficiencies can be achieved in cancer services without compromising patient care or outcomes. Examples of good practice in this respect should be applauded and widely implemented.

Our recommended approach to implementing the Cancer Drugs Fund is summarised in Figure 1.

**Figure 1 – summary of recommended process for implementing the Cancer Drugs Fund**
Chapter 1: introduction

A diagnosis of cancer is one of the public’s greatest fears. When a person has been diagnosed with cancer, they need reassurance that they will receive the treatment that they need, when they need it, from a skilled and caring specialist. One component of effective care is being seen by a specialist quickly, and cancer waiting time targets have proven crucial in raising the standards of care up to the point that the public would expect. We welcome the Government’s recognition that cancer waiting time targets are clinically justified, and their retention in the latest revision to the *NHS Operating Framework*, published in June.

A further component of high-quality care is equitable access to effective treatment. This has been an issue which has rightly provoked public concern. A series of reports and investigations by the Rarer Cancers Foundation (RCF), which incorporates the Rarer Cancers Forum, exposed for the first time the level of demand for unfunded treatments led to the review in 2008 of so-called ‘top-up’ payments in the NHS. These investigations included:

- *Taking Exception*, which identified the number of exceptional case requests made to commissioners every year, and which identified perversities in the process for assessing them. Our findings were identical to a subsequent Department of Health investigation.
- *Exceptional England?*, which made a number of recommendations for changes to the system of assessing cancer therapies in order to address the problems identified in *Taking Exception*.
- *Off limits?*, which found similar levels of demand for what the NHS calls ‘near-label’ treatments (i.e., treatments which are not licensed for a certain indication, and therefore cannot be appraised by NICE, but which are licensed for other conditions which have a similar biology).

Many of the recommendations made in *Exceptional England?* were adopted by the Government following its review of ‘top-up’ payments, *Improving access to medicines for NHS patients*. A number of changes to the ways in which effective therapies should be assessed for use on the NHS have now been made, which include:

- The introduction of new ‘end-of-life’ flexibilities for use by the National Institute for Health and Clinical Excellence (NICE) in assessing whether drugs which extend life at the end of life are cost-effective.
• The implementation of directions to commissioners setting out a more structured approach they should use in order to assess so-called ‘exceptional case’ requests for treatment.  

    7

• The encouragement of greater flexibility for manufacturers in setting prices for their medicines.  

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Following our report Exceptional Scotland?, similar improvements were adopted in Scotland.  

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The improvements have already significantly improved the lives of people affected by cancer in the last two years. However, in our report of March 2010, Exceptional Progress?, we identified a residual level of unfunded demand for cancer therapies, which continues to cause the public concern. We found that unfunded demand for cancer therapies arises in three circumstances:  

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• Where a treatment has not been recommended by NICE, even after application of the end-of-life flexibilities.

• Where a treatment has not been assessed by NICE, rendering an increased chance that it must be assessed by commissioners’ ‘exceptional cases committees’ which often use inconsistent processes and lack the specialist expertise to take a decision.

• Where a treatment is intended for use in a ‘near-label’ indication, meaning that NICE cannot appraise it and that commissioners lack the expertise to determine whether it should be made available on the NHS. One such case achieved notable coverage in April 2010.

Given these residual areas of concern, we were understandably delighted by the Coalition’s Programme for Government when it was announced that:  

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“We will create a Cancer Drugs Fund to enable patients to access the cancer drugs their doctors think will help them, paid for using money saved by the NHS through our pledge to stop the rise in Employer National Insurance contributions from April 2011."  

    12

The importance of action to improve access to cancer treatments was reaffirmed by the findings of Extent and causes of international variations in drug usage, published in July 2010.  

    13 The report identified that the UK uses less than half the volume per capita of the latest cancer medicines than the average observed in fourteen comparable countries.  

    14

Despite our strong support for the commitment in the Coalition’s Programme for Government, we were, however, concerned that the potential benefits the proposed Cancer Drugs Fund would bring to patients would be unnecessarily delayed by a year to
April 2011, resulting in thousands more patients being denied the treatments they need. To put the pressing nature of the issue in context, to date NICE has rejected, or announced that it is minded to reject, 10 cancer treatments since the 6 May 2010 alone. A full list of the NICE appraisals published since the General Election is included in Annex 1.

The RCF therefore wrote to the Prime Minister on 19 July 2010 to ask him to bring forward the introduction of the fund. A copy of this letter is included in Annex 2 and a copy of the reply, from Earl Howe, is included in Annex 3.

In-year funding for 2010-11 of £50 million was subsequently identified by the Department of Health and announced on 27 July 2010. Announcing the fund, the Secretary of State for Health stated that:

“We...need to act to improve access to [cancer] drugs...the report [on international variations in drugs usage] underlines the need for action now to help NHS patients access the cancer drugs their doctors think will benefit them. I am therefore announcing today additional funding of £50 million for this financial year to support improved access to cancer drugs. This funding, which has been found from a review of Department of Health central budgets, will be made available through clinically-led regional panels from October 2010.”

We warmly welcome the in-year funding which has now been identified. However, we also recognise the need for further work to inform the development of the full system which will operate from April 2011. We will work constructively with the Department of Health, as we have done over the last two years, to ensure that all patients with cancer get access to the treatments they need.

This report sets out our analysis of the costs involved in enabling clinicians to prescribe the treatments which they think could most benefit their patients, as well as our recommendations for how the commitment to establish a Cancer Drugs Fund should be delivered. It is our contribution to the development of the consultation paper on the full system, expected later this year.

**Recommendation**

1. **The NHS should respond to the spirit of the Department of Health’s announcement of in-year funding by commencing reimbursement for currently unfunded cancer treatments before 1 October 2010, when the in-year scheme is scheduled to begin operation. Manufacturers should work with the NHS to offer sufficient financial flexibility to enable patients to receive the treatments their clinicians wish to prescribe immediately.**
Chapter 2: the current situation and suggested principles for the Cancer Drugs Fund

The Government has made clear that its long term objective is to introduce a system of value based pricing for new treatments. We strongly support this objective. The objective of value based pricing is to ensure that the price the NHS pays for medicines reflects the therapeutic benefits they bring to patients. However this objective, although simple in design will be challenging to implement. We hope to work with the Department of Health in translating this aspiration into reality for cancer treatments.

To date, problems with the pricing and reimbursement system for medicines have disproportionately affected cancer patients. This chapter sets out both the problems that have occurred and, in order to address these, the principles which we think should govern the implementation of the Cancer Drugs Fund, as well as how they can support the longer term policy objective of value based pricing. It also assesses the potential impact of the in-year funding announced for 2010-11.

Challenges in the current system

In terms of pricing, the current Pharmaceutical Pricing Regulation Scheme (PPRS) has a number of features which have made it more difficult to gain approval for cancer treatments to be routinely used in the NHS. The PPRS:

- Includes only limited scope for price variation during the lifecycle of a product, meaning that prices have been set at the initial launch of a medicine when data on the effectiveness of cancer medicines is often immature, so inhibiting an accurate assessment of the value – and therefore appropriate price – delivered by a treatment.

- Encourages price cuts to be focused on treatments in therapy areas where there is intense competition rather than those in areas of high unmet need. Given the rapid developments that have occurred in cancer care, many new cancer medicines have been licensed in areas where no previous effective treatments existed and therefore competition has been minimal. There has therefore been less incentive for price reductions in cancer.

- Ensures transparent pricing and the early launch of new medicines in the UK. Although superficially desirable, this has resulted in the UK being used as a reference
point by other countries considering the appropriate level of pricing for new medicines, so introducing a strong commercial incentive for manufacturers to avoid reducing list prices because of the potential adverse impact that this could have on international pricing.

In terms of reimbursement, the NICE technology appraisal process has:

- Resulted in significant delays in the availability of guidance to the NHS on cancer treatments. Primary Care Trusts often do not allocate any formal funding to allow the medicine to be prescribed routinely ahead of the publication of NICE guidance, particularly given that it is very difficult to take a medicine off the ‘available list’ once it has already been routinely prescribed. This in turn can make clinicians less likely to prescribe a medicine given the uncertainties about funding. The Health Select Committee has concluded that such delays are “harmful to patients who are waiting for treatment.”

- Failed to fully take into account the benefits that can be delivered by new cancer treatments, resulting in the overall clinical and quality of life impact of new treatments being understated, thereby damaging cost effectiveness calculations.

- Neglected to fully reflect the importance that society places on caring for people with severe disease who may be in the last few months of their life, although the supplementary advice on appraising life-extending, end of life treatments medicines has partially addressed this.

Cancer has also occupied a disproportionately large number of NICE technology appraisals when compared to its overall impact on health expenditure, in part due to the high level of unmet need and number of new treatments which have been developed in recent years. Overall, just under 30% of all technology appraisals have been for cancer treatments, despite the fact that expenditure on cancer has accounted for under 6% of NHS expenditure. Although this has had the benefit of encouraging greater consistency in clinical practice, it has also exacerbated the negative impact on cancer patients of the issues in pricing and reimbursement outlined above.

**Impact of in-year funding**

The announcement of in-year funding should enable significant numbers of cancer patients to benefit from NHS treatment they would otherwise have been denied. Assuming that the average cost of an unfunded cancer treatment is £20,821 as we estimated in *Exceptional England?*, the in-year funding will provide treatment 2,401 patients in England. Figure 2 sets out how many patients we estimate could benefit from funding in each Strategic
Health Authority area, using the same weighted capitation formula adopted by the Department of Health in allocating the in-year funding.

**Figure 2 – number of patients who could benefit from in-year funding, by Strategic Health Authority**

The in-year funding is very welcome news for patients. However, we believe that a more refined process for the longer term administration of the Cancer Drugs Fund should be adopted. Below we set out some proposed principles which should guide the implementation of the Fund.

**Suggested principles for the Cancer Drugs Fund**

In the longer term, the Cancer Drugs Fund is intended to act as an interim measure to enable patients to gain access to the treatments their clinicians wish to prescribe ahead of the introduction of value based pricing. Given this, we believe that the Fund should seek to address the challenges in both the pricing and reimbursement system which have worked in combination to limit access to cancer treatments.

We therefore propose that the following principles should govern the implementation of the Fund:
• Approaches to better reflecting the value delivered by a medicine in its pricing should be tested. Cancer treatments represent an appropriate pilot because of the level of therapeutic innovation which has occurred in treatment, the immaturity of data on clinical effectiveness of medicines that often exists at launch and the fact that the existing pricing and reimbursement system has failed cancer patients.

• The role of NICE in providing guidance on what constitutes good clinical practice should be supported. The Fund should not remove the incentive on manufacturers to participate in NICE technology appraisals, nor on NICE to continue to improve its processes for evaluating cancer treatments.

• Clinicians should be enabled to prescribe the treatments which they think will benefit their patients but they should be held accountable for their clinical practice and decisions.

• The collection of evidence both on the extent of demand for cancer treatments and the clinical benefit should be a primary objective of the Fund. This evidence should be used to inform the development of value based pricing, clinical guidelines and quality standards.

• The use of clinically effective near-label treatments for rare cancers should be enabled and encouraged. Equally, the wasteful use of clinically ineffective treatments should be prevented.

• Bureaucracy should be minimised and value to the taxpayer should be maximised.

Recommendation

2. The Department of Health should publish the guiding principles which it intends to underpin the implementation of the Cancer Drugs Fund from April 2011 as part of its consultation on the issue.

Implications for other conditions

We are aware that treatments for other conditions have also been rejected by NICE and understand that there is a need to improve access to all treatments, not just those for cancer. It is beyond our remit to comment on non-cancer treatments. Nonetheless, given the level of unmet need and the changes in clinical practice that have occurred in oncology, we do believe that there is a strong case for using cancer as a pilot for new approaches to enabling access to treatments. However we hope that the lessons learned
from the Cancer Drugs Fund will be applied to other conditions as a system of value based pricing is implemented.

**Recommendation**

3. Given the complexities that occur in cancer treatment, cancer medicines represent an ideal pilot to test approaches to better reflecting value in pricing. Therefore the Cancer Drugs Fund should be used to test a new approach to ensuring that pricing and reimbursement fully reflects the value delivered by modern medicines. The learning from this should then be applied to treatments for other conditions.
Chapter 3: assessing the benefits and costs of improving access to cancer medicines

When the commitment to establish the Cancer Drugs Fund was announced, the intention to allocate £200 million per year was made clear. This funding was to be found as a result of savings to the NHS resulting from halting the rise in Employer National Insurance contributions from April 2011. We were therefore concerned that the announcement of the in-year funding stated that the longer term resourcing of the Fund would be, “subject to the Spending Review outcome.” Commenting at the press conference held to announce the in-year funding, Earl Howe, the Minister responsible for administering the Cancer Drugs Fund, was reported as stating that £200 million could not be guaranteed and should be treated as, “an aspirational figure.”

This chapter sets out the potential costs of improving access to cancer medicines, as well as why we consider it vital that the Department of Health allocates the level of resources earmarked for the Cancer Drugs Fund when the policy was initially announced.

We estimated in our report, Exceptional Progress?, that 16,000 patients every year are potentially being denied access to cancer treatments. On the basis of these figures – and assuming that the cost of an unfunded cancer treatment is £20,821 – the level of resources required to support the Cancer Drugs Fund would need to be in excess of £330 million. Based on these figures:

- The in-year funding of £50 million will still mean that almost 5,600 cancer patients are denied access to the treatments they need during the remainder of 2010-11.

- Even at maximum levels of funding of £200 million per annum, over 6,300 patients every year will continue to be denied access to the treatments they need.

However, the figure we estimated in Exceptional Progress? must be treated with caution. It remains highly theoretical, and is based on the assumption that clinicians will wish to treat every patient who may be eligible for a medicine. In practice, the actual level of demand for these treatments is likely to be lower, for the following four reasons:

- **Patient choice.** Some patients – particularly those at the end of their lives – may decide that they are unwilling to undergo further courses of cancer treatment, which can have serious side effects. A proportion of the 16,000 patients estimated in Exceptional Progress? is therefore likely to refuse consent to treatment.
• **Contraindications.** All treatments have contraindications, i.e., constraints on when they can be used in certain patients. For example, patients – after a long and debilitating illness – may not be physically fit enough for treatment. A proportion of the hypothetical 16,000 patients considered in *Exceptional Progress?* can be expected to be contraindicated for treatment.

• **Clinician preference.** There is significant evidence, not least from the Department of Health’s report on the extent and causes of international variations in drug uptake, that clinicians in the UK are more conservative in prescribing chemotherapy than those in other countries. 36

• **Some of the drugs which are being demanded are substitutes for other treatments.** *Exceptional Progress?* estimated the figure of 16,000 patients by combining the eligible patient populations for each of the unfunded treatments we assessed. However, in some cases these unfunded treatments may be substitutes or alternatives to other forms of treatment. As multiple treatments would be unlikely to be used at once in patients, this is likely to significantly reduce the figure of 16,000.

NICE’s own costing analyses, produced to support the NHS in implementing its guidance, accepts that actual demand for treatment is significantly lower than the theoretical maximum level of demand. For example, the costing statement for one approved treated estimates that uptake will only reach 50% of the theoretical eligible patient population. 37

A different approach to assessing the costs and number of patients who would benefit from the Cancer Drugs Fund would be to examine the levels of cancer drug usage in European countries, and estimate the level of expenditure it would require to raise our levels of cancer treatment usage to those seen in other European countries. The recently published report on international variations in drug usage exposes for the first time comprehensive information on variations in drug uptake, and data kindly provided to us by health information provider IMS Health gives us an idea of the levels of expenditure required to raise our levels of prescribing up to the European and specific-country averages. 38 These are set out in Table 1.
Table 1 – estimating the costs associated with achieving drug usage in the UK which is similar to other countries and estimating the number of patients who could benefit

<table>
<thead>
<tr>
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<th>Five largest European countries in study</th>
<th>Ten European countries in study</th>
<th>All countries in study</th>
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<tbody>
<tr>
<td>UK uptake as a percentage of the country average</td>
<td>41%</td>
<td>45%</td>
<td>45%</td>
</tr>
<tr>
<td>Additional expenditure required to raise levels of UK uptake to the country uptake (figures are rounded to the nearest £5 million)</td>
<td>£260,000,000</td>
<td>£225,000,000</td>
<td>£205,000,000</td>
</tr>
<tr>
<td>Number of patients who would benefit each year (based on an average cost of £20,821 per year)</td>
<td>12,487</td>
<td>10,806</td>
<td>9,846</td>
</tr>
</tbody>
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On the basis of these figures, neither the £50 million announced as in-year funding in 2010-11 nor the £200 million size of the Fund will be enough to meet the demand for currently unfunded cancer treatments. However, once again, the costs which need to be met by the Cancer Drugs Fund are likely to be lower, because:

- The estimated costs are based on UK-wide figures, whereas the costs which need to be met through the Cancer Drugs Fund are for England alone. Therefore the overall figures on additional expenditure can probably be reduced by 8%.

- Some of the drugs assessed through the international variations study are NICE-approved, and would be funded by commissioners routinely – rather than through the Cancer Drugs Fund.

- The estimated costs do not take account of the impact of the introduction of patient access schemes or discounts, since the estimates of expenditure are based on list price – rather than actual cost to the NHS.

- It is not clear whether any of the levels of cancer drug use amongst the EU-5, EU-10 or EU-all country regions are in anyway optimal. Given that there is no consensus on the correct level of usage, it is likely that levels of usage (and costs) will be less than those given – particularly given the conservatism of clinicians referred to above.
Having said this, some of these downward pressures are likely to be partially offset by the upward pressure arising from new cancer treatments which have been launched since the study into international variations was concluded (data capture for the study concluded in March 2009), or will be launched during the period when the Cancer Drugs Fund is operational.45

In preparing this report, the RCF conducted an anonymous survey of member of its Healthcare Industry Reference Group. The survey found that46:

- Manufacturers of cancer treatments hope to secure 20 new marketing authorizations (licenses) for cancer treatments by the end of 2013.
- Although it is difficult to estimate the cumulative patient population for these treatments, it is expected to be significant.

Again, these responses need treating with caution:

- Not all treatments in the advanced stages of development will go on to secure marketing authorisation.
- Some treatments will secure a positive NICE recommendation, meaning they will not be reimbursed through the Cancer Drugs Fund.
- Many of the treatments will be substitutes for existing medicines.

For these reasons, we anticipate that the expenditure required to meet the currently unfunded demand for cancer treatment is likely to be inside the £200 million which the Government has set aside for the Fund when fully operational. We believe that our estimate of the maximum level of unfunded demand for cancer treatment, first published in Exceptional England? remains robust.

In Exceptional England? we modeled the expected increase in demand that would occur for cancer treatment if restrictions on access were lifted. Although, little evidence exists on the suppressed level of demand for cancer treatments, it is reasonable to assume that the uptake of those treatments currently subject to exceptional cases applications would increase in line with the increase in uptake for a treatment which follows a positive NICE recommendation. Based on observed increases in usage of medicines once NICE has approved a treatment, we assumed that:

- The increase in demand for a cancer treatment in the first year after a positive NICE recommendation is 120%.47
- The increase in demand for a cancer treatment in the second and third years after a positive NICE recommendation is 48%.48
Given this, we calculated that – rather than the existing 2,200 patients who receive funding for cancer treatments through the exceptional cases process at present – a less restrictive approach would see 4,840 patients receive such cancer treatments after one year, 7,163 patients after two years, and 10,602 patients after three years.\textsuperscript{49}

Based on the estimated average treatment cost of £20,821 this would result in overall costs to the NHS of just under £175 million per year by the end of year three, as set out in Table 2.

**Table 2 – estimated costs of a less restrictive approach**

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<tr>
<th></th>
<th>Number of patients</th>
<th>Total expenditure</th>
<th>Additional expenditure over present usage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Present usage</td>
<td>2,200</td>
<td>£45,806,200</td>
<td>£0</td>
</tr>
<tr>
<td>Usage in year 1</td>
<td>4,840</td>
<td>£100,773,640</td>
<td>£54,967,440</td>
</tr>
<tr>
<td>Usage in year 2</td>
<td>7,163</td>
<td>£149,144,987</td>
<td>£103,338,787</td>
</tr>
<tr>
<td>Usage in year 3</td>
<td>10,602</td>
<td>£220,734,581</td>
<td>£174,928,381</td>
</tr>
</tbody>
</table>

We believe that this figure is robust because:

- It is underpinned by actual levels of demand for unfunded cancer treatments, as observed in clinical practice, rather than theoretical demand.

- It recognises that there will be some level of increase in demand based on the relative greater access to cancer treatments the Cancer Drugs Fund brings about, and bases this rate of increase on actual, observed, levels of increases in uptake following a positive NICE appraisal.

- It includes estimates of the level of demand for the near-label use of drugs, which was not captured by the estimate in *Exceptional Progress*?

The £50 million allocated to the NHS to fund additional cancer treatments from 1 October 2010 to 31 March 2011 is also likely to be sufficient as clinical demand for treatment will take some time to grow to its true level, as set out above.

Nevertheless, it is clear that there is some degree of uncertainty about the exact demands on the Fund, which might range from £175 million to £330 million on an annual basis. Given this high level of uncertainty, it is imperative that safeguards are put in place to prevent the Fund being exhausted in-year, with patients with justifiable needs for cancer treatment continuing to be forced to ‘top-up’ their care or go without. It is also vital that
the Department of Health honours the initial commitment to allocate £200 million per year to the Cancer Drugs Fund. Otherwise, based on our best estimate, 3,600 cancer patients will continue to be denied treatment due to an absence of funding.

We set out our thoughts on how the Cancer Drugs Fund should be implemented in Chapter 5. However, at this stage we are clear that both the previous and current governments have worked hard to ensure patients with cancer are given access to the treatments they need, and that manufacturers must now play their part.

**Recommendations**

4. **The Government should allocate the £200 million per year earmarked for the Cancer Drugs Fund when the policy was initially announced.**

5. **Mechanisms should be put in place to limit the cost exposure to the NHS resulting from the Cancer Drugs Fund. The maximum exposure should be predicated on the initial proposed allocation of £200 million per year.**

6. **Manufacturers must match the flexibility shown by the Department of Health and play a role in making treatments available to patients at a price which is affordable within the constraints of the Cancer Drugs Fund.**
Chapter 4: delivering better value from cancer medicines

The purchase and administration of cancer medicines represents a relatively small but nonetheless significant proportion of wider expenditure on NHS services. Given the current financial context for the NHS, it is vital that good value for money should be secured in the procurement of cancer medicines, not least because expenditure on any intervention reduces the available resources for other services. The value imperative for treatments reimbursed from the Cancer Drugs Fund is particularly important, as many of these medicines have already been deemed by NICE not to represent a cost-effective use of NHS resources, albeit using a methodology and threshold which we consider to be flawed.

This chapter sets out the importance of securing additional value from the Cancer Drugs Fund, particularly given the uncertainties outlined in Chapter 3 that exist about the overall costs associated with delivering on the commitment to enable clinicians to prescribe any cancer treatment which they feel could benefit their patients.

It is important to recognise that the Department of Health, industry and NICE have already taken some welcome steps to reduce the overall costs of making cancer treatments available. Measures have included enabling manufacturers to vary prices post-licensing to reflect changes in the available evidence on efficacy and enabling manufacturers to propose patient access schemes.

The impact of patient access schemes

The purpose of patient access schemes is to enable manufacturers to reduce the overall cost impact to the NHS of providing treatment to patients, thereby improving the cost effectiveness of a medicine without necessarily adjusting its list price or unit cost. In Exceptional progress?, we found that the proposal of patient access schemes now forms an important part of the NICE appraisal process for the majority of cancer treatments.50

Patient access schemes vary significantly depending on the treatment in question. Our survey of manufacturers found that schemes which have been proposed include offering free initial cycles of treatment, capping the overall cost of treatment so as to provide overall certainty on costs, offering a rebate to the NHS and offering to reimburse ineffective treatment.51 Figure 3 summarises the details of the offers included within patient access schemes.
Given the varying nature of patient access schemes, it is difficult to assess the impact that they have had on the overall cost of treatment. However, by examining the costing templates produced by NICE to support the NHS in implementing its recommendations, it is possible to assess the impact that schemes have on overall NHS costs. For technology appraisals published since January 2009 where a patient access scheme was proposed and the treatment was subsequently recommended, cost reductions appear to range from just under 6% to nearly 38%.52

The principle underpinning patient access schemes is sound – that manufacturers and NICE should work together pragmatically to improve the cost effectiveness of making cancer treatments available – and it appears that they have made an important contribution towards reducing the overall NHS costs associated with making cancer medicines available. Nonetheless, clinicians, NHS administrators and manufacturers have all reported significant concerns with patient access schemes, including:
There is a lack of clarity about what forms of scheme will be acceptable and there has been an absence of consistency in the schemes that have been proposed. 73% of manufacturers who responded to our survey supported the need for clear guidance on patient access schemes, as set out in Figure 4. This view is also strongly supported by hospital pharmacists, who often have to administer schemes. 53

**Figure 4 – manufacturers’ attitudes to guidance on patient access schemes**

- The double sign-off process involving the Department of Health (which assesses the feasibility of a scheme and determines whether NICE should consider it) and NICE (which assesses its impact on cost-effectiveness) is further delaying the availability of guidance.

- The fact that the UK is used as a reference price and that a summary of the key parameters of patient access schemes has been included in NICE appraisal documents has limited the scope for flexibility within such schemes. 64% of manufacturers who responded to our survey reported difficulties in securing approval for patient access schemes from their global headquarters, as set out in Figure 5. The response of one company was indicative of the feedback we received, “Gaining agreement from our global colleagues can be a difficult process…NICE makes public all the details of a scheme…this has an effect on the global view as this
'deal' may affect pricing in other countries. If there was more confidentiality for the UK then that may result in more flexibility.\textsuperscript{54}

Figure 5 – manufacturers’ experience of gaining approval from global headquarters for patient access schemes

- The NHS has struggled with the complexity of schemes and the inconsistency between them. A lack of management resources and staff time has reduced the effectiveness of patient access schemes and, in some cases, has led to the NHS failing to realise some of the savings promised by such schemes.\textsuperscript{55}

The future of patient of patient access schemes

Despite the concerns that exist about patient access schemes, every manufacturer who responded to our survey indicated a willingness to consider proposing patient access schemes in the future.

However, given the issues that have been identified, we do not consider that patient access schemes offer a long term solution to the challenges in making available cancer treatments to patients at a cost to the NHS which reflects their value. Nonetheless, the additional savings that patient access schemes have generated for the NHS should be recognised and built upon in the Cancer Drugs Fund and, beyond that, in value based pricing.
It would be untenable to have a situation whereby the NHS incurred a higher cost for making available a treatment that had not been deemed cost effective by NICE through the Cancer Drugs Fund than it would have had the treatment been recommended by NICE as a result of a patient access scheme. Chapter 5 sets out our proposals for ensuring appropriate value is secured through the Cancer Drugs Fund.

**Recommendations**

7. Although patient access schemes have delivered additional cost savings to the NHS, they do not provide a sustainable mechanism for enabling every cancer patient to access the treatments they need. For this reason, patient access schemes should only be used in exceptional circumstances rather than as a routine part of appraisals. Where patient access schemes are in place and have proven to be successful, they should of course be continued.

8. For those situations where patient access schemes are being considered in future, the Department of Health should issue clearer guidance about what forms of scheme are acceptable. As part of this it should be made clear that, where a patient access scheme is offered, the manufacturer should be required to reimburse the administrative costs of the scheme.
Chapter 5 – implementing the fund

The way in which the Cancer Drugs Fund is implemented will determine its success in ensuring that cancer patients are able to benefit from the treatments that their clinicians wish to prescribe and that new approaches to reflecting the value of treatments in their reimbursement cost are tested. This chapter sets out our proposals for implementing the Fund from April 2011 onwards, based on the principles we proposed in Chapter 2. It outlines:

- A proposed scope for the Cancer Drugs Fund.
- A recommended approach to administering the Fund.
- A mechanism for determining reimbursement levels for treatments.
- A process by which clinicians could prescribe treatments which would be reimbursed by the Fund.
- Additional safeguards which should be established for near-label prescribing.
- The importance of clinical audit.
- Mechanisms for ensuring transparency.
- The need for patients to be provided with better information about the benefits and toxicities of treatments for advanced cancer.

Scope of the Cancer Drugs Fund

Our previous reports have exposed that unmet need in cancer treatment occurs primarily in relatively new treatments – which have either yet to be appraised by NICE or have been rejected by NICE on the grounds of cost-effectiveness – and in near-label treatments, where a clinician wishes to use a medicine to treat a very rare form of cancer which has a similar biology of disease to the cancer for which the medicine is licensed.

We therefore propose that eligibility for the Cancer Drugs Fund should be restricted to:

- Treatments licensed since 1 January 2005 which are not routinely funded by the NHS as they have either been rejected by NICE, are yet to receive a Final Appraisal Determination or fall outside the scope of a partial recommendation.

- Situations where a clinician wishes to use a treatment in a near-label setting.
Treatments reimbursed through specialist commissioning should continue to be commissioned in this manner.

Although we have been critical of some of NICE’s processes and the way in which it has interpreted its own guidance, the RCF believes that NICE plays an important role in supporting good clinical practice and developing standards against which the quality of services can be assessed. Any system which bypasses NICE entirely risks diminishing its role in a way which could be detrimental to the objective of improving clinical outcomes. In order to maintain the incentive for manufacturers to participate in NICE technology appraisals, we propose that treatments should only be eligible for reimbursement from the Cancer Drugs Fund if manufacturers commit to fully participating in relevant appraisal processes. In this scenario, NICE may of course wish to consider what steps it could take to expedite the appraisal process and minimise costs associated with it.

Where a treatment is recommended by NICE, it should be funded in the standard way from baseline NHS allocations. Negative NICE technology appraisals should also continue to be published as the information contained may be of use to clinicians and those administering the Cancer Drugs Fund.

Below we set out the safeguards which should be put in place to prevent abuse of the Fund and to maximise value to the taxpayer.

**Recommendations**

9. Eligibility to the Cancer Drugs Fund should be restricted to treatments licensed by the European Medicines Evaluation Agency (EMEA) since 1 January 2005 which have either been rejected by the National Institute for Health and Clinical Excellence (NICE) or have yet to receive a Final Appraisal Determination and situations where a clinician wishes to use a treatment in a near-label setting.

10. In order to ensure their treatments are eligible for the Cancer Drugs Fund, manufacturers should be required to commit to fully cooperating with all relevant NICE technology appraisal processes.

11. Where a treatment is approved by NICE, it should be funded in the usual manner from NHS baselines.
Administration of the Cancer Drugs Fund

There are a variety of different ways in which the Cancer Drugs Fund could be hosted and administered, including:

- At a local level by Primary Care Trusts (or by GP consortia once they are established).

- At a regional level by Strategic Health Authorities (or by cancer commissioning networks once they are established and hosted in this way by the NHS Commissioning Board).

- At a national level by the Department of Health (or by the NHS Commissioning Board once it is established).

The interim approach to making available more cancer treatments has adopted a regional approach. However, we do not consider this to be the most appropriate way to administer the Fund in the longer term. Strategic Health Authorities are to be abolished and will be scaling back their activities from April 2011, calling into question their organisational capacity to administer the Fund. Regional control could also involve decisions being made ten times over.

Similarly, local control over decisions about access to high cost cancer treatments has already proved to be ineffective and has undermined confidence in the NHS. Our investigation into exceptional case processes, first published in *Taking Exception*, revealed unacceptable inconsistencies in process, quality and outcomes. For these reasons we recommend that the Department of Health adopts a more consistent, national approach to administering the scheme.

We therefore believe that a national approach should be adopted. This would have a range of advantages, including:

- Minimising bureaucracy, ensuring that decisions are only taken once and are based on genuine expertise.

- Ensuring consistency, preventing a new form of postcode lottery from emerging.

- Providing clear accountability, enabling patients, policymakers and other stakeholders to hold the Department of Health to account on a key commitment in the Coalition Agreement.

It is also worth noting that, as a result of the reforms announced in *Equity and Excellence: Liberating the NHS*, the NHS will undergo significant changes in structure and process over the lifetime of the Cancer Drugs Fund. The most effective way to ensure that these
changes do not destabilise the implementation of the Fund would be to administer it on a national basis, given that the NHS Commissioning Board will be established in shadow form from April 2011.

**Recommendation**

**12.** A national approach should be taken to the administration of the Cancer Drugs Fund, thereby minimising bureaucracy, ensuring consistency, providing clear accountability and reducing the risk of the Fund being disrupted by the impact of the implementation of reforms outlined in Equity and Excellence: Liberating the NHS.

**Setting reimbursement levels from the Cancer Drugs Fund**

As set out earlier in this report, there are uncertainties about the exact level of costs associated with making available every cancer treatment which a clinician may wish to prescribe. Equally there is a need to ensure that value for money is secured from the Cancer Drugs Fund and that it acts as a mechanism for testing potential approaches to value based pricing. In order to create a system which minimises bureaucracy, ensures value for money and reflects the commercial realities faced by manufacturers, we propose that for medicines licensed since 1 January 2005:

- Treatments should be reimbursed at listed unit price.

- The Department of Health (or a designated agent, such as the NHS Purchasing and Supply Agency (PASA) and manufacturers should be required to negotiate a commercially confidential cap on the overall cost incurred by the NHS for use of a particular treatment.

- If the cap is exceeded, the manufacturer should be required to provide a rebate to the NHS up to a predefined level (for example, 125% of the cap). If the predefined level is exceeded, then the Department of Health and the manufacturer should be required to renegotiate the agreement, with all reimbursement ceasing until an arrangement is put in place.

A cap on the overall cost exposure to the NHS of making a treatment available would ensure that the savings generated for the NHS would grow as usage increases, resulting in cost certainty for NHS organisations and ensuring that, as volume sales to the NHS increase, manufacturers will return some of the benefits of commercial success to the health service.

The cap should be calculated using the following criteria:
• The potential size of the eligible patient population, adjusted for the impact of patient preference, contraindications and the availability of any clinical alternatives.

• The listed unit price of the treatment.

• The extent of clinical effectiveness (based on feedback provided by expert advisory groups of clinicians, with treatments which deliver a larger relative health gain for the form of cancer in question receiving a more favourable weighting).

• The extent to which the treatment addresses an unmet need (again, based on feedback provided by expert advisory groups of clinicians, with treatments which represent a particularly significant advance receiving a more favourable weighting).

Such a cap would not work for treatments used in a near-label setting given the paucity of evidence that can exist on effectiveness and demand. Therefore near-label treatments should be reimbursed at listed unit price, with manufacturers instead required to pay a percentage rebate to cover the costs of the clinical audit described below.

Recommendations

13. For licensed drugs included in the Fund, a commercially confidential total NHS cost cap should be calculated. Where this cap is exceeded, manufacturers should be required to reimburse the NHS, thereby ensuring that the £200 million is not exceeded. The negotiations on the cap calculation should be based on:

- The potential size of the eligible patient population, adjusted for the impact of patient preference, contraindications and the availability of any clinical alternatives.
- The listed unit price of the treatment.
- Clinical effectiveness (based on feedback provided by expert advisory groups of clinicians).
- The extent to which the treatment addresses an unmet need (again, based on feedback provided by expert advisory groups of clinicians).

14. For near-label treatments it will be impossible to calculate a cap in the same way due to the paucity of available data. Instead we suggest that near-label treatments should be reimbursed at list price with manufacturers required to pay a contribution in the form of a rebate to cover the costs of clinical audit.

The process for prescribing from the Cancer Drugs Fund

Once it has been determined which drugs will be available from the Cancer Drugs Fund, it will be important that patients are able to gain rapid access to the treatments their clinicians wish to prescribe. We reiterate our finding in Taking Exception that delays in treatment are upsetting for patients, undermine confidence in the NHS and can damage clinical outcomes.
We propose the following process should be adopted for gaining access to the Fund:

- The clinician should gain support for the proposed treatment from clinical colleagues. The usual mechanism for securing this would be through the multi disciplinary team (MDT), at which the treatment options for all patients should be considered.

- The proposed treatment should be discussed with the patient, alongside any alternatives (including, where appropriate, palliation), and the patient’s informed consent to proceed with treatment should be documented.

- A simple application should be submitted to the provider’s medical director for approval. This last step is to ensure that appropriate clinical governance procedures are followed and, providing they are, it should be a formality.

- The Cancer Drugs Fund is notified and treatment is authorised.

- Treatment should begin and the results should be recorded in the prospective national clinical audit (described below).

We are aware that Primary Care Trusts are under an obligation to use the exceptional cases process for those treatments where routine policies are not in place. However, given the establishment of the Cancer Drugs Fund, it is extremely unlikely that a Primary Care Trust would provide exceptional funding for a cancer treatment which would otherwise be reimbursed by the Cancer Drugs Fund. We therefore suggest that cancer treatments which are eligible for the Fund should be exempted from the exceptional cases process. This approach would have the benefit of minimising waiting times for cancer patients requiring treatments and reducing the administrative burden on exceptional cases committees, providing an additional benefit for patients with other conditions.

**Recommendations**

**15.** A clear process should be set out for clinicians wishing to prescribe treatments funded by the Cancer Drugs Fund. This should ensure multi-disciplinary input to treatment decisions, informed and documented consent from patients and robust clinical governance.

**16.** The requirement to use the exceptional cases process for treatments which are not routinely funded by commissioners but which are nonetheless included in the Cancer Drugs Fund should be ended and this should be made explicit in guidance to support the NHS in implementing the Cancer Drugs Fund.
Safeguards for near-label prescribing

In the case of near-label treatments, we reiterate the recommendations made in *Off limits*. In broad terms, there is a spectrum, of increasing evidence, along which prescribing can occur, as demonstrated in Figure 6.

**Figure 6 – the prescribing spectrum**

<table>
<thead>
<tr>
<th>Scenarios</th>
<th>UNLICENSED</th>
<th>USE IN UNLICENSED INDICATION</th>
<th>LICENSED</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Scenario</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>The medicine has not received licensing approval for any indication and therefore few data on safety or efficacy are available.</td>
<td>UNLICENSED</td>
<td>USE IN UNLICENSED INDICATION</td>
<td>LICENSED</td>
</tr>
<tr>
<td>The medicine has been licensed for an indication and therefore acceptable data on safety and efficacy exist for that particular disease. However the indication for which a clinician now wishes to use the medicine has a very different biology to that for which it is licensed.</td>
<td><code>Far-label</code></td>
<td><code>Near-label</code></td>
<td><code>Near-label</code></td>
</tr>
<tr>
<td>The medicine has been licensed for an indication and therefore acceptable data on safety and efficacy exist for that particular disease. The indication for which a clinician now wishes to use the medicine has a similar biology to that for which it is licensed.</td>
<td><code>Far-label</code></td>
<td><code>Near-label</code></td>
<td><code>Near-label</code></td>
</tr>
<tr>
<td>The medicine has been licensed for the indication for which the clinician wishes to use it. Therefore acceptable data on safety and efficacy exist for the indication.</td>
<td><code>Far-label</code></td>
<td><code>Near-label</code></td>
<td><code>Near-label</code></td>
</tr>
<tr>
<td><strong>Examples</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>An experimental cancer medicine used as part of a clinical trial.</td>
<td>UNLICENSED</td>
<td>USE IN UNLICENSED INDICATION</td>
<td>LICENSED</td>
</tr>
<tr>
<td>A bespoke medicine, or ‘special,’ which is prepared for a particular patient.</td>
<td>UNLICENSED</td>
<td>USE IN UNLICENSED INDICATION</td>
<td>LICENSED</td>
</tr>
<tr>
<td>Anti-psychotic medications, licensed to treat schizophrenia, are frequently used to control the behavioural symptoms of people with dementia.</td>
<td><code>Far-label</code></td>
<td><code>Near-label</code></td>
<td><code>Near-label</code></td>
</tr>
<tr>
<td>A treatment licensed for one form of haematological cancer is used to treat another, rarer, form of haematological cancer.</td>
<td><code>Far-label</code></td>
<td><code>Near-label</code></td>
<td><code>Near-label</code></td>
</tr>
<tr>
<td>A treatment for rheumatoid arthritis is used to treat another form of autoimmune disease such as vasculitis.</td>
<td><code>Far-label</code></td>
<td><code>Near-label</code></td>
<td><code>Near-label</code></td>
</tr>
<tr>
<td>A cancer treatment which has been subject to a NICE technology appraisal.</td>
<td><code>Far-label</code></td>
<td><code>Near-label</code></td>
<td><code>Near-label</code></td>
</tr>
</tbody>
</table>

We have no interest in encouraging inappropriate off-label prescribing. Our objective is to ensure that patients with rare cancers are able to access clinically effective treatments. We therefore see a critical role for experts in rare cancers in determining what forms of off-label treatment are genuinely near-label.
Expert advisory committees for different tumour types should be asked to produce guidance on what constitutes valid near-label usage of a medicine. We envisage that one such committee would be commissioned for each major group of tumours (for example upper-gastrointestinal or urological tumours). Guidance on treatments where there is known to be good clinical support should be prioritised and clinicians should be expected to follow the recommended approach unless there is good reason not to. An early candidate for prioritisation would be haematological cancers, given the relatively high occurrence of near-label treatment for this form of cancer that was identified in Off limits and the clinical support which exists for the role of near-label treatment in this area.  

In circumstances where no guidance exists, clinicians would be able to prescribe off-label subject to the criteria set out above, although they would be expected to account for their prescribing decisions. Those treatments which are requested relatively frequently through this process should then be prioritised for the development of guidance to govern future use.

**Recommendations**

17. Safeguards should be established to encourage appropriate near-label prescribing, including ensuring clinical accountability for treatment decisions. Expert clinical groups should be asked to develop guidance on what constitutes near-label treatment, with areas where there is good clinical support being prioritised. Clinicians should be expected to follow the recommended approach unless there is good reason not to. An early candidate for prioritisation would be haematological cancers, given the relatively high occurrence of near-label treatment for this form of cancer that was identified in Off limits.

**Prospective clinical audit**

We consider that prospective clinical audit must be a fundamental part of the Cancer Drugs Fund. Participation should be a mandatory obligation for clinicians wishing to prescribe treatments included in the fund.

Clinical audit would have a range of important benefits. It would:

- Enable a more accurate assessment of the true demand for cancer treatments.
- Facilitate the collection of data on the benefits and toxicities of new cancer treatments in routine NHS practice.
- Provide a mechanism for assuring the appropriateness of clinical practice, identifying and addressing outliers in terms of over or under usage.
• Prevent abuses to the system, such as the parallel exporting of medicines procured through the Cancer Drugs Fund.

Box 1 sets out some of the information which we believe should be recorded in the prospective clinical audit.

<table>
<thead>
<tr>
<th>Box 1 – suggested information for inclusion in prospective clinical audit</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Patient’s NHS number</td>
</tr>
<tr>
<td>• Patient’s cancer diagnosis and stage of disease</td>
</tr>
<tr>
<td>• Treatment prescribed, dosage and frequency</td>
</tr>
<tr>
<td>• Number of cycles of treatment given</td>
</tr>
<tr>
<td>• Side effects observed</td>
</tr>
<tr>
<td>• Reason for discontinuing treatment</td>
</tr>
</tbody>
</table>

We propose that the audit should be hosted by either the National Cancer Intelligence Network (NCIN) or the National Cancer Research Initiative (NCRI).

The clinical audit will be particularly important for near-label prescribing and will create an unprecedented opportunity to develop the evidence base for near-label treatment of cancer.

**Recommendations**

18. **Prospective clinical audit should play a fundamental part in the Cancer Drugs Fund and participation in the audit should be a precondition of access to the Fund.** The audit should be used to:
   - Enable a more accurate assessment of the true demand for cancer treatments.
   - Facilitate the collection of data on the benefits and toxicities of new cancer treatments in routine NHS practice.
   - Provide a mechanism for assuring the appropriateness of clinical practice.
   - Prevent abuses to the system, such as the parallel exporting of medicines procured through the Cancer Drugs Fund.
   - Develop further evidence on the benefits and toxicities of near-label treatment.

19. **The opportunity created by the clinical audit should be used to further establish England’s position as a world leader on clinical research, particularly into rare cancers.**

**Transparency**

In order to secure better value for money for the taxpayer, we have already recommended that agreements on the cost cap for each treatment should be treated as commercially confidential. However, this needs to be balanced with the importance of transparency in how money from the public purse is spent, as well as the legitimate public interest in the
results that the Cancer Drugs Fund achieves. We therefore propose that the National Cancer Director should provide a report annually on the number of patients treated as a result of the Cancer Drugs Fund, as well as the outcomes of the prospective clinical audit. We also suggest that the National Audit Office should be asked to conduct a value for money study on the operation of the Cancer Drugs Fund in 2013, which should be used to inform the development of value based pricing.

Recommendations

20. The National Cancer Director should be required to submit an annual report to the Secretary of State on the operation of the Cancer Drugs Fund which should then be published.

21. The National Audit Office should undertake a value for money study on the Cancer Drugs Fund in 2013. The results of this should be used to inform the development of value based pricing.

Ensuring patients receive appropriate information

Discussions between patients and clinicians about the benefits and toxicities of different treatment options can be very difficult, particularly for patients with advanced forms of cancer where the realistic outcome is often extended survival and / or symptom relief rather than complete remission. Although data will exist on the median expected health gain of such treatments, the response of every patient will be different, further complicating the decision-making process.

*Improving access to medicines for NHS patients* highlighted that these difficulties can be compounded if conversations also need to cover uncertainties over whether NHS funding will be available. It was therefore recommended that more should be done to ensure that clinicians have the necessary knowledge and skills to communicate complex information effectively and in a balanced way, and that high quality written information should be available on treatments which may not be funded by the NHS. This recommendation is as important today as it was in November 2008.

Effective communication can help improve the experience of patients and their families and what can be a very traumatic time. It can also ensure that patients do not feel misled if treatment proves not to be effective or if side effects are experienced, so potentially reducing the need for litigation. Furthermore, effective communication will help maximise the impact of the Cancer Drugs Fund, ensuring that patients are in a position to make an informed decision on the implications of treatment before proceeding, thereby reducing the likelihood of patients discontinuing treatment at a very early stage.
As a patient support charity, the RCF frequently assists patients and families who are facing difficult decisions about treatment options. These issues are particularly apparent if a patient has a rare form of cancer and a clinician wishes to prescribe a near-label treatment, where there is unlikely to be published information on the expected benefits and toxicities of the treatment in the type of cancer in question. It is – by definition – difficult to provide tailored information to support patients in this scenario. We have, however, published general advice for patients who may need near-label treatment. Box 2 sets out the issues that we suggest patients should discuss with their clinician in these circumstances.

**Box 2 – suggested issues for patients to discuss with clinicians when considering near-label treatment**

For some rare cancers, there may be no licensed treatments available, due to the difficulties in conducting large scale randomised trials in extremely rare conditions.

There may, however, be treatments which have been licensed for more common forms of cancer which develop in a similar way to the rare cancer in question. This is called near-label treatment.

For near-label treatments, there is likely to be less evidence about effectiveness and safety. However, it is important to stress that the medicine in question will have been tested extensively in patients with more common, but similar types of cancer.

Any use of near-label treatment should be done after full consultation between the doctor and patient. There are a number of issues which a patient may wish to discuss with the doctor, including:

- Are there any licensed alternatives to the near-label treatment? If so, why does the doctor suggest that this treatment would be better?
- What evidence is there to support the use of the medicine in this cancer / patient?
- What are the risks and benefits of near-label treatment with this drug?
- Are there any particular side effects associated with the treatment and are there steps that can be taken to minimise the risk of these?
- Is there a relevant charity or support group that the patient could speak to for further information?

There may be a lot of information to digest, so patients should feel free to ask for details in writing. Some patients also like to record the discussion with their doctor. Patients should also feel free to bring a family member, caregiver or friend into these meetings as well. Often important decisions about treatment are made with the input of the family or caregiver.

No patient should feel rushed into making a decision about whether to proceed with a particular course of treatment. They can take time to reflect on their options and can of course consult a nurse specialist if they require further support. A doctor should also be able to advise on the urgency of treatment.
Recommendation

22. Balanced information on the benefits and toxicities of all treatments reimbursed by the Cancer Drugs Fund should be developed as a matter of urgency and included within cancer information prescriptions. Appropriate charities should be involved in the development of this information.

Our recommended approach to implementing the Cancer Drugs Fund is summarised in Figure 7.

Figure 7 – summary of recommended process for implementing the Cancer Drugs Fund
Chapter 6 – the next steps for cancer services

The availability of treatment is only one – albeit important – aspect of ensuring high quality cancer services which delivered outcomes which are comparable with the best in Europe. Before the General Election, the RCF published *Towards the best in Europe: our 12 point plan*, which set out our priorities for improving rarer cancer care during this Parliament. Our 12 point plan is included in Annex 4.

Improving other aspects of cancer care

As well as treatment, there are many other areas of care which require further attention, including:

- Ensuring patients are diagnosed earlier, so improving their survival chances and giving them a greater range of treatment options.
- Enabling patients to benefit from cutting edge technologies such as proton therapy, for which there are currently no comprehensive facilities in the UK.
- Providing every patient with access to support from a clinical nurse specialist with expertise in their cancer, as well as appropriate support from allied healthcare professionals such as physiotherapists, nutritionists and psycho-oncologists.
- Rolling out cancer information prescriptions so that all patients have access to them.
- Expanding the support available to cancer survivors, enabling them to re-enter the labour market where this is feasible.
- Improving the collection of data on clinical quality and outcomes.
- Increasing research undertaken into rarer cancers.

We hope that the establishment of the Cancer Drugs Fund, combined with the recent improvements in national policy outlined in Chapter 1, will resolve the struggle that patients have faced in gaining access to the treatments they need. Although we will revisit the issue to ensure that the Fund is being implemented in a way which delivers on the spirit of the Government’s commitment, we now hope to be able to devote more attention to making constructive recommendations to improve other aspects of cancer care. We therefore warmly welcome the decision to review and update the Cancer Reform Strategy which we hope will provide an opportunity to address these important issues.

Recommendation

23. The policy attention devoted to improving access to treatments should not come at the expense of improving other aspects of cancer care.
**Funding for cancer services**

Ensuring appropriate funding for cancer services is vital to delivering high quality outcomes and we therefore welcome the Government’s commitment to deliver real terms funding increases to the NHS over the course of this Parliament. However, it is clear that cancer services will face multiple funding pressures, including:

- Incidence of cancer continues to rise due to the ageing population and the impact of lifestyle factors.
- New technologies are making cancer increasingly treatable and patients are often undergoing multiple lines of surgery, radiotherapy and drug treatment.
- The public’s and patients’ expectations are – rightly – increasing.
- As funding for local authorities reduces, so the NHS will be forced to provide a greater proportion of funding for social care services, thereby increasing the squeeze on other programme areas.

In recent years some commentators have argued that cancer has been over prioritised as an issue. Although there has been a great deal of public and political attention devoted to cancer services, we reject the argument that it has received a disproportionate amount of attention from the NHS. It is instructive that, for the last year which data are available, expenditure on cancer services rose at a slower rate than the NHS average – and a considerably lower rate than for some other conditions – as set out in Box 3.

<table>
<thead>
<tr>
<th>Box 3 – NHS spending on cancer in 2008-9</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Overall cancer accounted for 5.3% of expenditure in 2008-9</td>
</tr>
<tr>
<td>• Spending on cancer rose by 3.44% - compared to an average increase of 3.9%</td>
</tr>
<tr>
<td>• The highest increase in programme expenditure was 15.3% - over four times the increase observed in cancer</td>
</tr>
</tbody>
</table>

Other countries continue to spend considerably more per capita on cancer services than England. 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. It is likely that this disparity was even greater before the sustained increases in NHS funding of the past decade.

The difference in per capita funding cannot, however, be explained by differences in overall levels of expenditure on health in other countries. Cancer services in England receive a lower proportion of the health service budget than in other countries, such as France and Germany. Although we note the financial situation facing the country means that significant increases in spending on cancer are impossible, this is important context when considering the outcomes achieved by cancer services in England.
Recommendation

24. As part of the Comprehensive Spending Review, the Department of Health should critically reappraise the balance of NHS expenditure and consider the case for savings. 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.

Delivering efficiencies in cancer care

Therefore it will be important to realise savings in cancer services wherever possible. We urge the NHS to explore:

- Improving earlier diagnosis, thereby reducing the need for patients to receive expensive treatments for very advanced cancer.\(^6\)

- Reducing lengths of stay in hospital by adopting less invasive surgical techniques such as laparoscopic surgery\(^6\) and by rapidly spreading the Enhanced Recovery Programme which enables patients to leave hospital earlier.\(^7\)

- Ensuring the quality of basic care, such as nutrition for cancer, thereby reducing the risk of expensive complications for patients.\(^7,8\)

- Minimising emergency admissions for cancer-related complications by improving the safety and quality of chemotherapy services.\(^3\)

- Maximising the opportunities created by delivering care closer to home, for example through the use of oral chemotherapy instead of intravenous alternatives.\(^4\)

- Increasing the efficiency of cancer prescribing by ensuring that, where clinically equivalent, cheaper generic medicines are available, used instead of more expensive branded alternatives.

We will robustly defend from cuts those elements of services which we believe are valued by patients and improve their outcomes. However, we intend to work with the NHS to identify and champion savings opportunities which can also improve the quality of care for people with rarer cancers. Ensuring that high quality, efficient practice is spread to all NHS services will be vital if we are to realise our ambition of achieving the best outcomes in Europe.

Recommendation

25. The review and refresh of the Cancer Reform Strategy should identify areas where efficiencies can be achieved in cancer services without compromising patient care or outcomes. Examples of good practice in this respect should be applauded and spread.
# Annex 1: NICE appraisals published since 6 May 2010

<table>
<thead>
<tr>
<th>Name</th>
<th>Indication</th>
<th>NICE decision</th>
<th>Date</th>
</tr>
</thead>
<tbody>
<tr>
<td>Sorafenib</td>
<td>Hepatocellular carcinoma</td>
<td>Not recommended (TA 189)</td>
<td>26/05/10</td>
</tr>
<tr>
<td>Capecitabine</td>
<td>Advanced gastric cancer</td>
<td>Recommended (FAD)</td>
<td>27/05/10</td>
</tr>
<tr>
<td>Gefitinib</td>
<td>First-line treatment of locally advanced or metastatic non-small-cell lung cancer</td>
<td>Recommended (FAD)</td>
<td>27/05/10</td>
</tr>
<tr>
<td>Bortezomib</td>
<td>First-line treatment of multiple myeloma</td>
<td>Recommended (ACD)</td>
<td>2/06/10</td>
</tr>
<tr>
<td>Thalidomide</td>
<td>First-line treatment of multiple myeloma</td>
<td>Recommended (ACD)</td>
<td>2/06/10</td>
</tr>
<tr>
<td>Lapatanib</td>
<td>Advanced or metastatic breast cancer</td>
<td>Not recommended (FAD)</td>
<td>10/06/10</td>
</tr>
<tr>
<td>Trabectedin</td>
<td>Relapsed ovarian cancer</td>
<td>Not recommended (ACD)</td>
<td>14/06/10</td>
</tr>
<tr>
<td>Erlotinib</td>
<td>Maintenance treatment of non-small-cell lung cancer</td>
<td>Not recommended (ACD)</td>
<td>15/06/10</td>
</tr>
<tr>
<td>Pemetrexed</td>
<td>Maintenance treatment of non-small-cell lung cancer</td>
<td>Recommended (TA190)</td>
<td>23/06/10</td>
</tr>
<tr>
<td>Ofatumumab</td>
<td>Chronic lymphocytic leukaemia that is refractory (in other words, does not respond) to the drugs fludarabine and alemtuzumab</td>
<td>Not recommended (ACD)</td>
<td>24/06/10</td>
</tr>
<tr>
<td>Imatinib</td>
<td>Unresectable and/or metastatic gastrointestinal stromal tumours</td>
<td>Not recommended (ACD)</td>
<td>29/06/10</td>
</tr>
<tr>
<td>Everolimus</td>
<td>Second line treatment of advanced renal cell carcinoma</td>
<td>Not recommended (FAD)</td>
<td>2/07/10</td>
</tr>
<tr>
<td>Trastuzumab</td>
<td>Gastric cancer</td>
<td>Not recommended (ACD)</td>
<td>6/07/10</td>
</tr>
<tr>
<td>Bevacizumab</td>
<td>Breast cancer</td>
<td>Not recommended (ACD)</td>
<td>9/07/10</td>
</tr>
<tr>
<td>Mifamurtide</td>
<td>Non-metastatic, surgically treatable osteosarcoma</td>
<td>Not recommended (ACD)</td>
<td>9/07/10</td>
</tr>
</tbody>
</table>
Annex 2: Letter to the Prime Minister

The Great Barn  
Godmersham Park  
Canterbury, Kent CT4 7DT  
44 (0) 1227 738279  
info@rarercancers.org.uk  
www.rarercancers.org.uk

Rt Hon David Cameron MP  
Prime Minister  
10 Downing Street  
London  
SW1A 2AA

19 July 2010

Dear Prime Minister

Implementation of the Cancer Drugs Fund

We are writing to you to request your personal intervention to bring forward the planned introduction of the Cancer Drugs Fund from April 2011.

We were delighted by the Government’s commitment to enable cancer clinicians to prescribe all clinically-effective treatments to their patients. Our investigations have revealed that the Cancer Drugs Fund will be of benefit to thousands of patients who are currently denied life-extending treatments.

However, since the General Election, you may be aware that the National Institute for Health and Clinical Excellence (NICE) has rejected 10 treatments for cancer. These include trastuzumab for gastric cancer, sorafenib for liver cancer, and mitamustide for osteosarcoma. As a result of the latter decision, many children and teenagers will be denied access to life-saving treatment.

These decisions are already adversely affecting the lives of many patients who need access to these treatments, most of whom will not live until April 2011. Combined with previous rejections by NICE, they mean that thousands more patients could miss out on the treatment they need.

It seems illogical to allow bureaucratic barriers to prevent a common sense solution being put in place to help patients this year. We would urge you to do everything you can to ensure that patients with cancer get access to the treatments they need before then.

Yours sincerely

Andrew Wilson  
Chief Executive, Rarer Cancers Foundation

c: Rt Hon Andrew Lansley CBE MP; Professor Sir Mike Richards CBE
From the Earl Howe
Parliamentary Under Secretary of State for Health (London)

PMPO000525455
Andrew Wilson
Chief Executive
Rarer Cancers Foundation
The Great Barn
Godmersham Park
Canterbury
CT4 7DT

29 JUL 2010

Dear Andrew,

Thank you for your letter of 19 July to David Cameron about implementation of the Cancer Drugs Fund. As your letter raises health matters, it has been passed to me for reply as the Minister responsible for this policy area. A copy of my reply will be passed to the Prime Minister’s office.

I share your concern that cancer patients should not have to wait until 2011 to enjoy improved access to drugs that will benefit them. You will by now be aware that we have been working hard to identify additional funding for cancer drugs in the current financial year.

Despite the challenging fiscal climate, the Government’s commitment to protect spending on the NHS has enabled us to identify £50million from Department of Health budgets which we are allocating through Strategic Health Authorities to fund additional cancer drugs. Andrew Lansley announced this additional funding to Parliament on 27 July, alongside the publication of a report from Professor Sir Mike Richards on international variations in medicines use.

We want decisions on the use of cancer drugs to be taken by clinicians in consultation with patients, so that regional clinically-led panels will determine how these additional resources should be used for the benefit of patients.
The Department of Health will consult later this year on the arrangements for the Cancer Drugs Fund from April 2011. I am sure that the Rarer Cancers Foundation will have a very important contribution to make to that discussion, and know that you will make a thoughtful response to that consultation when it takes place.

I hope this reply is helpful.

Yours sincerely,

EARL HOWE
Annex 4: Towards the best in Europe: our 12 point plan

Earlier diagnosis

1. Reward GPs for identifying the signs and symptoms of cancer when they are at an early stage and for referring patients for investigation, paid for by reallocating points from within the Quality and Outcomes Framework which are currently focused on inefficient process measures

Better treatment

2. Develop a new system for determining access to near-label treatments for rarer cancers, building on the recommendations made in our report, Off-limits

3. Work with NICE to ensure greater flexibility in the way that cancer treatments are assessed, resulting in European standards of treatment for all patients

4. Translate new laws on exceptional cases into reality for every patient in every PCT in England

5. Enable patients in England to benefit from cutting-edge technologies, such as proton therapy, without having to travel abroad for care, in line with the 2009 commitment to build an NHS proton therapy centre

Improved care

6. Ensure every patient has support from a clinical nurse specialist who is expert in their condition, by creating a new virtual network of clinical nurse specialists for rarer cancers

7. Deliver on the existing commitment on cancer information prescriptions, ensuring that every person with a rarer cancer has access to personalised written information at every stage of their cancer journey

Greater transparency

8. Benchmark the outcomes achieved by the NHS in treating patients with rarer cancers against comparable countries

9. Develop new ‘service-level outcome measures’ so that the performance of every provider in the cancer care pathway can be assessed and made available to patients to assist them in making choices about where and how to be treated

10. Ask the Care Quality Commission to undertake the full service review of cancer services, first called for in the Government’s 2007 Cancer Reform Strategy
Increased research

11. Work in partnership with cancer charities and the pharmaceutical industry to establish a national prospective audit for the use of near-label treatments

12. Address the under prioritisation of research into rarer cancers by creating a dedicated research programme
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Caring about people with rarer cancers

Rarer Cancers Foundation offers advice and information to individuals with rare and less common cancers or 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.

Registered Charity No. 1109213
contact@rarercancers.org.uk
www.rarercancers.org.uk

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