

TAKING EXCEPTION

An audit of the policies and processes used by PCTs to determine exceptional funding requests

August 2008



● About the Rarer Cancers Forum

Although 'cancer' is a unifying word, there are as many differences between types of tumour as there are similarities. Between 30% and 50% of all cancers can be classified as 'rarer.' A cancer may be classed as 'rarer' either because it affects an unusual site in the body, or because the cancer itself is of an unusual type, is difficult to diagnose, or requires special treatment.

For patients affected by less common or rarer cancers there may be few survivors or people to compare experiences with. Support networks may not be available and GPs are unlikely to know a great deal about the condition. The Rarer Cancers Forum offers advice and information to people affected by rare and less common cancers, providing bespoke information and advice, raising awareness and putting people in touch with others affected by their condition.

● About this audit

This audit was undertaken using information provided by PCTs following requests by the Rarer Cancers Forum under the Freedom of Information Act 2000.

As PCTs are not duty-bound to respond in a set format, the information received was not directly comparable. The analysis used in this report is therefore based on the interpretation of the Rarer Cancers Forum and not that provided by PCTs themselves.

The research contributing to this audit was kindly supported by an unrestricted educational grant from Roche. Editorial control rests with the Rarer Cancers Forum alone.

Content

Summary of findings	4
Key recommendations	6
Chapter 1: Introduction.....	9
Chapter 2: Report methodology	11
Chapter 3: Analysis of how PCTs process exceptional case requests	14
Chapter 4: Analysis of the decisions PCTs make on exceptional cases.....	23
Chapter 5: Reducing the burden on exceptional case processes	32
ANNEX 1	36

Summary of findings

The system for determining exceptional cases is confusing for patients. For example, in one PCT a case will be processed through a 'priorities panel', whereas in another it will be a 'commissioning advisory group' and in another it may be an 'exceptional cases panel.'

The information held by the vast majority of PCTs is so poor that they are unable to answer simple requests for information on the process they use, the number of cases they have assessed and the outcome of these cases. This paucity of data will render it impossible for many PCTs to meet the spirit of the commitment in the draft NHS Constitution to transparency on funding decisions. It will also make it difficult for some PCTs to assess their adherence to the duties they have under the NHS Act.

There are widespread variations in the processes used by PCTs, resulting in different patients being subject to radically different processes. Not all PCTs even have written protocols to explain how their process works.

Many PCTs have processes dominated by administrators with seemingly little role for clinical expertise. This calls into the question the ability of PCTs to adequately consider clinical factors, alongside budget, when making decisions.

Some PCTs are failing to process exceptional cases in a timely fashion. For patients with advanced cancer, time can be of the essence and this delay could potentially compromise their health outcomes.

Many PCTs are applying social criteria, including personal circumstances, whilst many others are not.

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

There is a 'postcode lottery' in the proportion of applications for cancer treatment accepted and rejected. Of the 62 PCTs from which we received directly comparable information on this issue, 11 PCTs approved all the requests made to their exceptional cases panel, while 2 PCTs approved none of the requests.

Our audit exposes for the first time the underlying demand in England for cancer treatments which are not generally available on the NHS:

- There are approximately 3,000 applications for exceptional funding for cancer patients annually.
- Of these applications, approximately 2,200 cases are approved annually.
- Approximately 800 cancer patients are denied funding for treatment through the exceptional cases process every year.

The vast majority of exceptional case applications are for drugs for very advanced cancer, i.e. those normally used in the last few months of life. PCTs have received most requests for sunitinib (Sutent), erlotinib (Tarceva), cetuximab (Erbix) and bortezomib (Velcade).

There are a number of actions which could be taken to reduce the number of cancer patients forced to rely on the exceptional cases process, including addressing problems with NICE.



**Some PCTs are failing
to process exceptional
cases in a timely fashion**

Key recommendations

The Department of Health should reiterate to all PCTs their responsibilities under the Freedom of Information Act. In order to make real the commitment to transparency in the draft NHS Constitution, the Department of Health should also provide guidance to PCTs on the type, format and detail of information they should publish on exceptional case procedures.

National guidance should be published on how PCTs should make decisions on exceptional cases. PCTs should be assessed on how well they implement this guidance as part of the World Class Commissioning Performance Framework.

The national guidance should include instructions for PCTs that:

- They should publish a written protocol, including a clear definition of the grounds for exceptionality. Information on a PCT's policy should be given to all patients whose case will be considered under this process.
- Exceptional cases panels should include at least an equal number of clinicians as administrators, as well as lay representation. There should be an odd number of voting members so as to ensure a decision can be made.
- Patients or their representatives should be able to contribute their views and experience to a panel meeting and should also have the right to attend the meeting if they so choose.
- Social criteria may have a place when making exceptional case decisions but this is a difficult area which requires value judgements to be made. Clear advice should be given on what social criteria will be used and how they will be applied. These criteria should be debated in Parliament.
- A written explanation should be given to all patients about why their exceptional case has been accepted or rejected.

There should be a clear timetable against which exceptional case decisions should be made. We suggest a maximum waiting time of five working days for decisions on urgent cases, with a maximum of an additional five working days for an appeal to be heard. All cancer patients should be considered as urgent.

The Department of Health should conduct an investigation into why there is such a significant variation in both the number of exceptional case requests submitted to different PCTs and the outcomes of these requests.

The Department of Health should urgently assess the cost of funding all rejected exceptional cases. Given the small volume of exceptional case applications, the Department of Health should also consider the merits of the following two options:

- Establishing a regional or central exceptional cases panel to assess requests.
- Creating an exceptional cases fund to cover the cost of funding these treatments, which have already been recommended by clinicians.

This would have the benefits of bringing greater consistency to decision-making processes and reducing the administrative burden inherent in operating 152 separate processes to hear 3,000 cases a year.

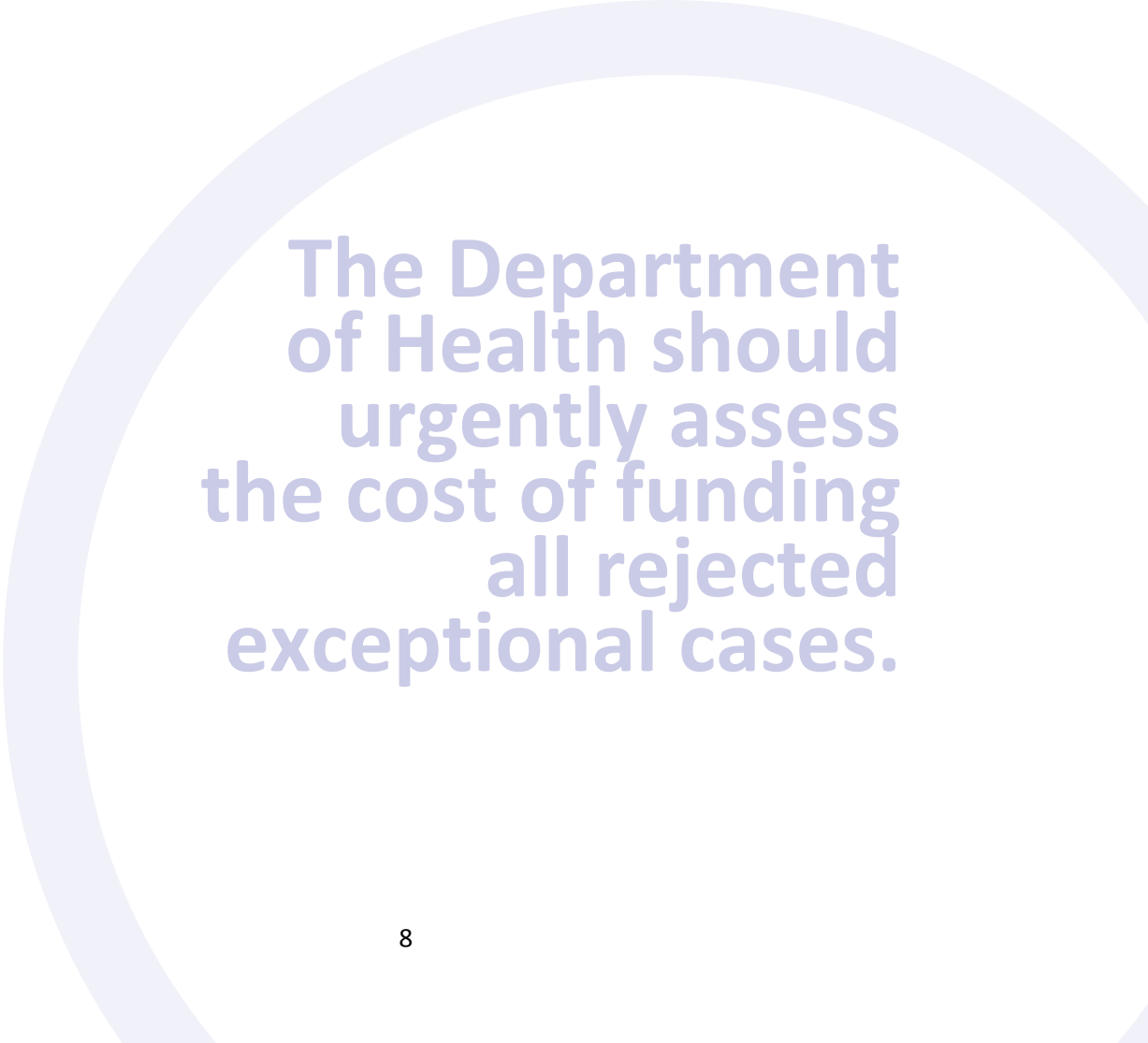
The Department of Health should prioritise improving the exceptional case processes used by PCTs. This will minimise occasions when patients will be forced to consider 'topping up' their NHS care with privately-purchased treatment. Unless this issue is addressed, efforts to reform the rules regarding when a patient may purchase a treatment privately will be doomed to fail and the founding principles of the NHS, soon to be enshrined in the NHS Constitution, will be fatally undermined.

In order to minimise the number of treatments which are being determined by exceptional case decisions, the NICE process for appraising new treatments needs to be speeded up. We welcome the commitment made on this in the NHS Next Stage Review and urge the Department of Health and NICE to publish details on how this will be achieved as soon as possible. Where for whatever reason NICE guidance has not been published within a certain number of months of licensing, we believe the Department of Health should stipulate to PCTs what other guidance should be used. This could be SMC guidance or perhaps a consensus opinion by clinical experts. Where NICE fails to deliver guidance within a set timeframe, performance sanctions should be introduced. These may include requiring NICE to report to Parliament on the reasons for the delay.

The increasing variance between the cancer medicines which are available in England and those available in Europe, resulting in 'passport prescribing,' must be addressed. Therefore the current NICE appraisal methodology for treatments for advanced cancer should be overhauled. There is a persuasive case for varying the cost-per-QALY threshold according to both severity of disease and the rarity of the condition in question.

Cancer networks should be encouraged to provide guidance to PCTs on treatments for which no national guidance is available. The role of cancer networks in doing this should be written into PCT guidelines on funding new treatments.

The legal obligation for PCTs to fund NICE approved treatments should be strengthened by including it within primary legislation and making clear the sanctions that will be used if a PCT fails to comply.



**The Department
of Health should
urgently assess
the cost of funding
all rejected
exceptional cases.**

Chapter 1: Introduction

Despite the welcome progress in improving cancer services since 2000, significant challenges remain. New treatments have offered hope to patients who would otherwise have run out of options, but they are invariably expensive.

The guidance that NICE produces has succeeded in reducing variations in prescribing for many treatments for more common cancers, but the story for rarer cancers is often less encouraging. For a variety of reasons NICE guidance is often not available for treatments for rarer cancers, leaving Primary Care Trusts (PCTs) to make their own decisions – the so called ‘exceptional cases’ decision-making process. This issue is increasingly affecting all types of cancer, but is particularly a problem for the rarer cancers.

The Department of Health has not published national guidance on how PCTs should make decisions on exceptional funding requests, stating that, “when NICE guidance is not yet available, it is for PCTs to determine whether or not to fund a drug locally should they receive a request from a doctor/patient for its use.”¹

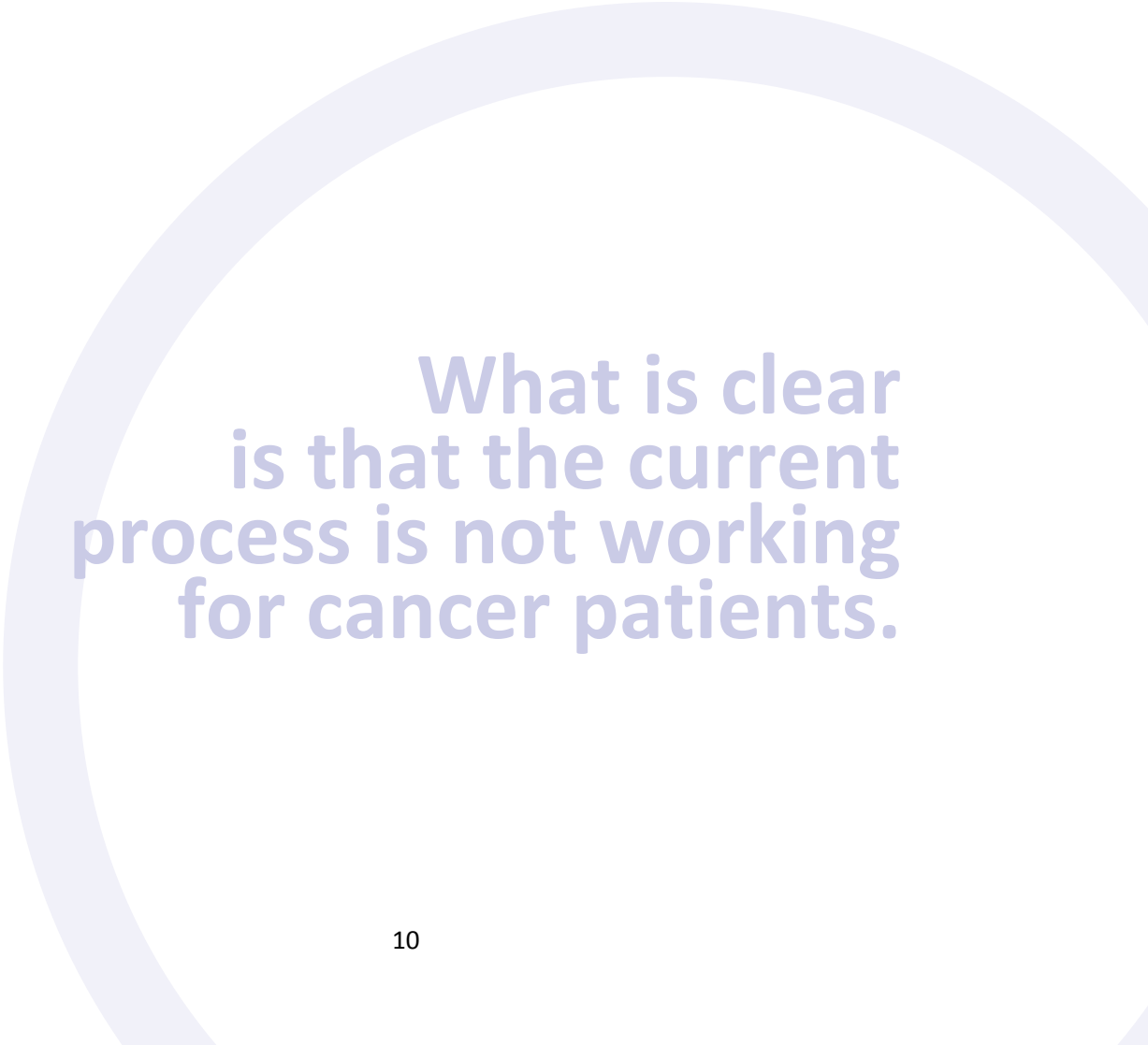
However the Department has also made clear that it is not acceptable for PCTs to use a lack of NICE guidance as a reason to reject an application for a drug and that there are sources of information and guidance that they should use as a minimum as part of their decision-making processes^{2 3}. It has also said that PCTs should have, “robust and fair processes in place for making decisions on drugs that have not yet been appraised by NICE”⁴.

The Rarer Cancers Forum offers advice and information to people affected by rare and less common cancers. Feedback from the people we support indicates that there are significant problems with the exceptional cases process. Decisions often take too long, are taken according to variable criteria, without the contribution of genuine experts and are not then fully explained to patients and their clinicians.

In order to uncover the true extent of the problem we decided to undertake an audit of the policies and processes used by PCTs to make exceptional funding decisions. This report is our analysis of the results. We have identified examples of good practice which should be spread, as well as what we consider to be glaring examples of the opposite.

What is clear is that the current process is not working for cancer patients. This is a major reason for the current political controversy about whether patients should be able to 'top up' their NHS care by purchasing additional treatment. Without radical change the Government's commitment to ensuring transparency in PCT funding decisions, as set out in the draft NHS Constitution, will not be realised. We have therefore made a number of recommendations which we believe will, if implemented, make a significant difference to patients.

We look forward to working with the Government, the NHS and other policymakers to turn these recommendations into reality. Unless this progress happens, people affected with rarer cancers will continue to take exception to the way PCTs make decisions about the treatments they need to live.



**What is clear
is that the current
process is not working
for cancer patients.**

Chapter 2: Report methodology

There is no common term for ‘exceptional case’ decision-making processes. Some PCTs refer to it as decisions on ‘unfunded treatments’, others as ‘individual patient’ decisions. Others still make reference to decisions on ‘non-normal’ or ‘case by case’ funding. However, all of these terms refer to the process of making decisions on whether to fund treatments for which there is no routine national or local guidance available. For the avoidance of doubt, this report refers to all such decisions as ‘exceptional cases.’

This report and the analysis it contains was compiled following a request under the Freedom of Information Act 2000 to every PCT in England. The information sought included:

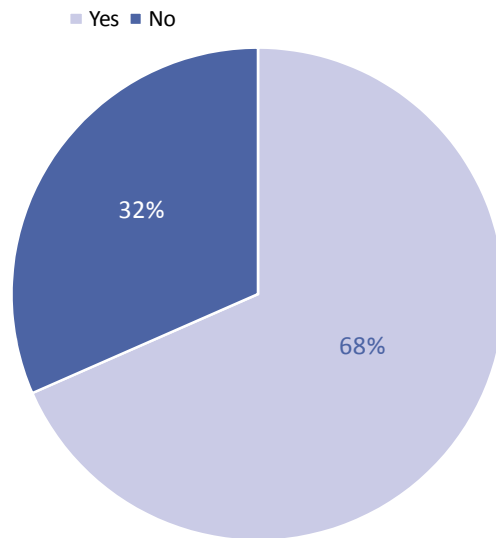
- Details of written protocols on exceptional requests for cancer treatment
- Details of the committees used to determine exceptional requests
- Whether social criteria are used in determining exceptional requests
- How many exceptional requests were accepted and rejected since October 2006
- Which cancer treatments were funded and not funded following exceptional requests since 1 October 2006
- The estimated total cost of cancer treatments funded and not funded following exceptional requests since 1 October 2006

As PCTs are not duty-bound to respond to Freedom of Information requests in a set format, the information we received was not directly comparable. The analysis used in this report is therefore based on the interpretation of the Rarer Cancers Forum and not that provided by PCTs themselves.

In total 104 out of 152 PCTs responded to the Freedom of Information requests, representing 68% of all PCTs in England. We are grateful to them for doing so. A list of those PCTs who responded is included in Annex 1.

60% of PCTs
responded

Percentage of PCTs responding to the FOI request

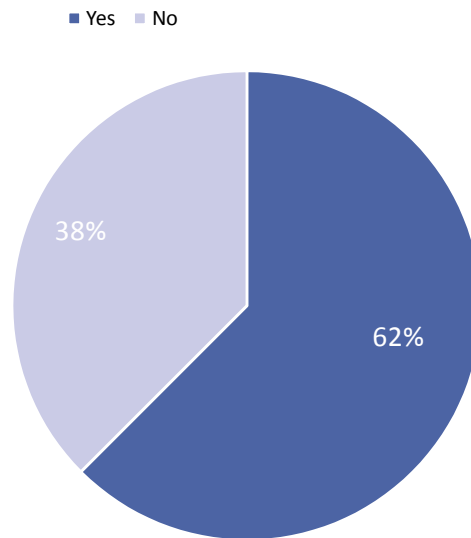


Some PCTs who did respond were unable to provide details on these issues. This calls into question their ability to effectively commission cancer services, in line with the obligations set out for them in the Cancer Reform Strategy and World Class Commissioning.

A small minority of PCTs obfuscated, resulting in a lack of transparency about their processes. For example Sunderland, South Tyneside and Gateshead PCTs said in their response, “Your request has been processed as one request in whole, and not as a series of 23 separate requests as you had requested in accordance with the Act...if you were to break down and resubmit your request at intermediate levels, the Trusts may be able to process these on an appropriately manageable level”.⁵

This clearly goes against the spirit of the commitment to transparency made in the draft NHS Constitution and is in contravention to the Freedom of Information Act itself.

Percentage of PCTs that responded to the request with substantive information



That a significant minority of PCTs did not respond at all is particularly concerning and calls into question their commitment to the principle of transparency to cancer patients and the communities they serve, as well as their ability to actually deliver any information about the quality of their commissioning processes for exceptional cases.

Recommendations on improving the transparency and accountability of PCTs

- 1 The Department of Health should reiterate to all PCTs their responsibilities under the Freedom of Information Act. In order to make real the commitment made to transparency in the draft NHS Constitution, the Department of Health should also provide guidance to PCTs on the type, format and detail of information they should publish on exceptional case procedures.

Chapter 3: Analysis of how PCTs process exceptional case requests

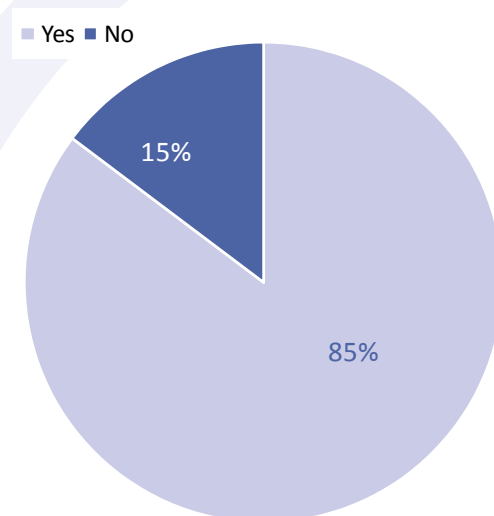
Beyond stipulating that PCTs should not deny funding for a treatment purely on the grounds of lack of NICE guidance and that there are certain sources of information that they should use to help inform their decisions, there are no national guidelines on how PCTs should process exceptional case requests.

The patients we support have told us that they want a consistent approach to making decisions on exceptional cases across England. Yet our analysis shows that the absence of clear national guidance has resulted in significant variations in how PCTs process exceptional case requests in practice.

Written protocols

Disturbingly, 15% of PCTs that gave a substantive response reported that they had no written protocols to govern how exceptional case requests are processed. Westminster PCT refused to supply their written protocol on the grounds that they were: "intended for future publication".⁶ This is in contrast to the transparency displayed by other PCTs who noted that they were in the process of revising their protocols but were still willing to supply details of their previous policies.

Percentage of responding PCTs that have written protocols for making exceptional case decisions



The Department of Health has made it clear in the draft NHS Constitution that it expects PCTs to make funding decisions in a rational and transparent manner, yet it is difficult to see how this can be achieved if a PCT does not even have a written policy on how it will make such decisions.

“When my mum's ‘exceptional case’ was taken to her PCT I asked on her behalf for the criteria on which the decision would be made, the framework of the panel meeting and the names of the panel members. I received no information at all. I found the whole process very confusing. In fact it was the worst nightmare anyone could be involved in.”

Angela Stevens – Carer

There were, however, several examples of good practice which we would encourage other PCTs to emulate:

- Shropshire County PCT sets a clear budget for managing individual funding requests and states that the process must be concise, transparent, explicable and defensible⁷.
- Cambridgeshire PCT sets out a series of questions to consider as part of the review process and states that the answers should be clearly minuted⁸.
- West Kent PCT anonymises all patient cases and seeks external expert opinion to assist medical colleagues on the panel⁹.

What is exceptionality?

PCTs also appear to have differing interpretations about what constitutes exceptionality:

- Hillingdon PCT asserts that, "the legality behind 'exceptionality' is not clear." If the PCT is not confident of the legal basis for exceptionality, it is not clear that its process for determining exceptional cases would be robust¹⁰.
- Mid Essex PCT defines one ground for exceptionality as, "where the whole health economy is affected." It is difficult to envisage how an individual funding request could justify this criteria¹¹.
- Bedfordshire PCT, in response to a request to "Please supply the written protocols used by your PCT to assess requests for cancer treatments which have not been approved by the National Institute for Clinical Excellence," stated that, "Bedfordshire PCT uses the NICE guidance on this"¹². This apparent confusion will be of little help to patients whose cases are being considered through the exceptional cases process.

Defining exceptionality

Norfolk PCT and Great Yarmouth and Waveny PCT offers the following guidance on exceptionality:

Above average benefit

... there may well be sub-groups of patients who get above-average benefit from the treatment. So, if the clinical circumstances of a patient suggest they:

- Are likely to derive above-average benefit or
- Have an above-average likelihood of benefit in treatments with a low probability of success,
... then this would lend support for exceptional funding.

Additional circumstances

- For patients who otherwise meet licensing criteria or other agreed eligibility criteria for a medicine which is not routinely available, we would look for additional clinical circumstances and take into account relevant personal / social circumstances – which might lend support for exceptional funding. This is assessed on a case by case basis.

Unexpected or unpredicted circumstances/Unusual combination of illnesses and treatment history

- An individual patient can have such a unique combination of illnesses, previous treatment history, side-effects, reactions etc – which may mean the patient no longer 'fits' a standard treatment pathway. We would assess the proposed alternative treatment on a case-by-case basis.

Rarity

- The rarity of a condition means that PCTs are less likely to have derived a policy to cover its treatment. Of necessity then, the PCTs will often need to consider requests for funding treatments for rare conditions on grounds of exceptionality. We would assess the proposed treatment on a case-by-case basis. This is because the rarity of a condition, by itself, does not confer grounds for the exceptional funding of its treatment.

“What is exceptional is considering using this medicine at all”

- When ‘usual’ treatments fail, clinicians seek out alternatives... these may be unlicensed indications... as for whether this merits exceptional funding or not depends on the strength of the case for the use of the drug, the implications for the patient, the patient’s clinical and personal and social circumstances etc – in other words it has to be assessed on a case-by-case basis.

New residents already on treatment which is not routinely available locally

- Patients... are already on treatment funded by the NHS elsewhere, but not covered by existing local policy, are likely to have their treatment continued...

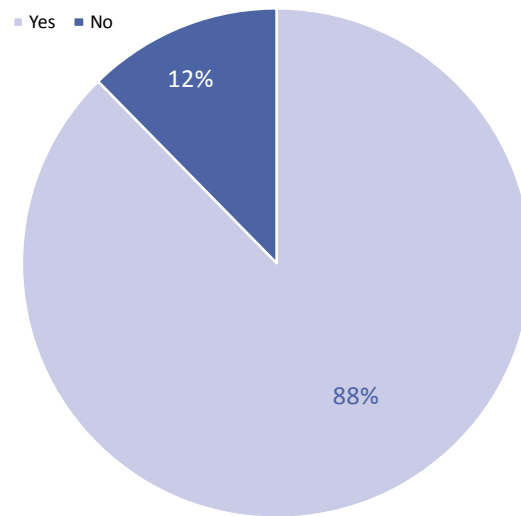
What is NOT exceptional

- When a proposed treatment is likely to be effective in that individual, this is not in itself exceptional.
- A patient who meets the criteria for the licensed indication, but one which is, nevertheless, not routinely available – is not exceptional.
- For many conditions, there is a predictable ‘end-stage’ for many of the patients. The fact that a patient has now reached the end-stage is not in itself grounds for exceptionality.
- ‘It’s the only treatment left... the restatement of the medicine’s unique potential in an individual case does not... by itself lend support for exceptionality’¹³.

The role of exceptional cases panels

Six percent of responding PCTs reported that they did not have a panel to consider exceptional case requests. Of those with a panel, 18% did not provide details of its membership.

Percentage of responding PCTs that have an exceptional cases panel and provided details of membership



For those that did provide membership details, 12% did not include clinical expertise within the panel membership. A lack of clinical representation on exceptional cases panels does call into question how the clinical merits of a case can adequately be taken into account when a decision is made.

Although the majority of panels did include clinical representation, there was widespread variation in the proportion of a panel's membership which has a clinical background. For example:

- Derbyshire County PCT includes two clinicians out of a total of 11 members¹⁴.
- Sheffield PCT has two administrators (a Cost per Case Manager and a Cost per Case Business Manager) and one medical advisor¹⁵.
- Calderdale PCT includes two administrators and one clinician¹⁶.

“I have had a number of requests for treatment funding considered by different exceptional case committees and, to put it bluntly, the process seems a bit of a lottery. Whilst my clinical opinion seems to be greatly valued by some committees others are more inclined to allow non-clinical factors to over-ride clinical considerations. We urgently need some standardisation and transparency in the decision making process.”

Dr Steve Schey, Consultant Haematologist

Yet other PCTs include much greater clinical and caring expertise:

- Hillingdon PCT includes five clinicians, a lay representative and only one administrator¹⁷.
- Central Lancashire PCT includes a representative from social services on its panel, ensuring that wider social impacts of decisions are taken into account¹⁸.
- Havering PCT's panel includes one administrator, six clinicians and a lay representative¹⁹.

We are also concerned that not all panels provide a mechanism for a patient viewpoint to be made. We are encouraged that Wakefield District PCT explicitly provides patients with an opportunity to write to the panel but are dismayed that they or their representative will not be able to attend the meeting considering their case.

Some PCTs also do not have clear criteria for how a panel should make its decision. For example, Wakefield District PCT states that, "a fully constituted panel would have four voting members"²⁰. Yet it does not appear to provide guidance on how a tie in the voting should be resolved.

Timetables for making exceptional case decisions

"My PCT turned down the application for Sutent made for me by my consultant, a decision I had to be informed of by making my own enquiries. This was devastating news and I felt that with a prognosis of only a year if left untreated, I was being left to die.

I then began a lengthy appeals process (four separate appeals boards). The PCT eventually agreed to fund my treatment, although the process had taken three months during which time I had not been receiving treatment of any kind. My condition worsened significantly during these three months. I have been taking the drug now for seven months and have had a massive positive response with all my tumours shrinking."

Steven Dallison - Patient

There are also significant variations in the timetables that apply to exceptional case decision-making processes in different PCTs. The North Yorkshire and York PCT has a panel that meets on a weekly basis, providing the potential for decisions to be made and communicated to patients and clinicians rapidly²¹.

East & North Herts PCT ranks requests by urgency, with the most urgent decisions being taken in a week. However, 'routine' decisions are required within 4-6 weeks²². Given that this response was given to a question specifically about cancer, we would be concerned if any cancer patient's case was to be considered as 'routine.'

Perhaps the most concerning response was given by Camden PCT which stated that its maximum waiting time for decisions is 12 weeks and that, in urgent cases (ie most cancers) treatment can be initiated earlier but that the provider must bear the financial risk pending a decision²³. We believe that this is unacceptable as it provides a clear disincentive for providers to recommend appropriate treatment if it is unlikely to be funded.

Ministers have made clear in Parliament that the time taken to make a decision on whether to fund treatment should be included in the new maximum waiting time of 31 days for all cancer treatments²⁴. Yet, under Camden PCT's approach, it would be impossible for this commitment to be met.

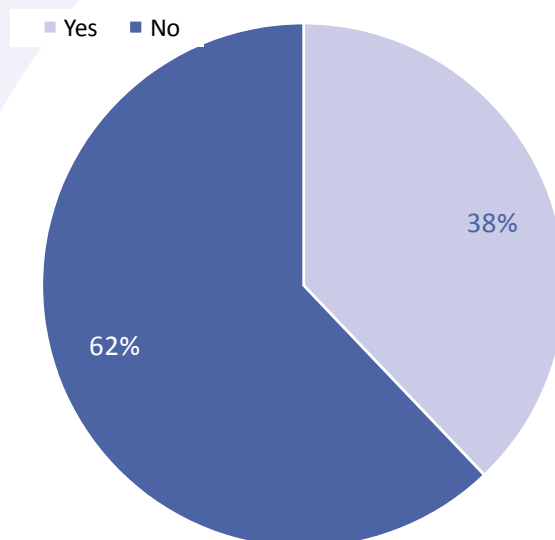
Use of social criteria

"The only social criteria used to date was a male, to live long enough to see his second child born."

A PCT's response to our audit

When it comes to taking social criteria into account when making decisions on exceptional cases, there is a profound split amongst PCTs. It is concerning that over two thirds of PCTs state that they have not taken any social criteria into account, meaning that the personal circumstances of patients are not considered as part of funding decisions.

Percentage of responding PCTs who report using social criteria in making decisions on exceptional cases



Many PCTs appear confused about what can constitute social criteria. For example:

- Barking and Dagenham PCT states, in guidance to clinicians on completing exceptional cases requests, that, "You should also not give social circumstances unless clinically relevant (e.g. marital status, dependent children etc)"²⁵. It is unclear when a social circumstance would be clinically relevant and elsewhere the PCT states that the issue of dependent family members will be taken into account.
- Herefordshire PCT states that it would be relevant when an, "individual's clinical or personal circumstances are significantly different to the wider group of patients"²⁶. Without a definition of personal circumstances and what would constitute these being "significantly different," this guidance would appear hard to apply in practice.
- Salford PCT states that, despite taking social criteria into account, "we do not have a list of social criteria"²⁷. Such an approach is difficult for patients to understand, could give rise to suspicions of unfair treatment and does not foster confidence in the rationality of the process. Solihull PCT refused to release the social criteria it has used.

However, other PCTs do provide clearer principles against which to judge social criteria. A number of PCTs use the principles of medical ethics (autonomy, beneficence, non-maleficence and justice). East & North Herts PCT explicitly asks about implications on carers of any decision²⁸.

Recommendations to improve the process used by PCTs

2 National guidance should be published on how PCTs should make decisions on exceptional cases. PCTs should be assessed on how well they implement this guidance as part of the World Class Commissioning Performance Framework. The national guidance should include instructions for PCTs that:

- They should publish a written protocol, including a clear definition of the grounds for exceptionality. Information on a PCT's policy should be given to all patients whose case will be considered under this process.
- Exceptional cases panels should include at least an equal number of clinicians as administrators, as well as lay representation. There should be an odd number of voting members so as to ensure a decision can be made.
- Patients or their representatives should be able to contribute their views and experience to a panel meeting and should also have the right to attend the meeting if they so choose.
- Social criteria may have a place when making exceptional case decisions but this is a difficult area which requires value judgements to be made. Clear advice should be given on what social criteria will be used and how they will be applied. These criteria should be debated in Parliament.
- A written explanation should be given to all patients about why their exceptional case has been accepted or rejected.

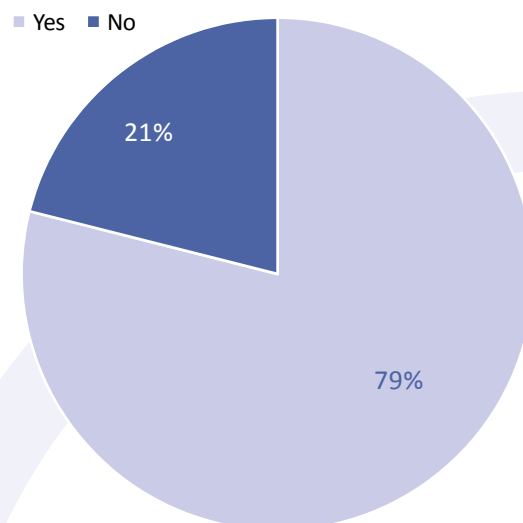
3 There should be a clear timetable against which exceptional case decisions should be made. We suggest a maximum waiting time of five working days for decisions on urgent cases, with a maximum of an additional five working days for an appeal to be heard. All cancer patients should be considered as urgent.

Chapter 4: Analysis of the decisions PCTs make on exceptional cases

We asked for data from PCTs on the number of exceptional cases they had received, the number of patients and the treatments that had had funding requests approved and rejected, and the value of the treatments funded and rejected.

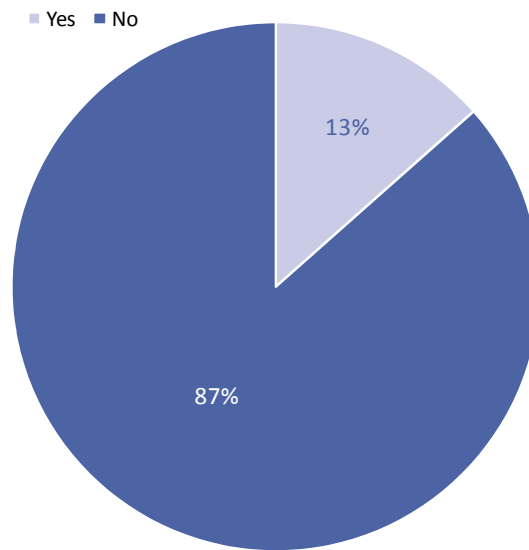
The data we received from PCTs was variable, but we were most concerned by those PCTs which could not offer us any information at all on patient volumes and treatments, more than one in five PCTs were unable to provide any information at all, summarised in the chart below:

Percentage of responding PCTs which were able to provide some information on exceptional cases decisions



Those PCTs which could provide what we would describe as ‘complete’ information – insofar as they provided information on all treatments where funding had been approved and rejected, on how many occasions, and what the value of these treatments was – were in a significant minority. Of 104 PCTs, just 14 (13%) were able to provide this information.

Percentage of responding PCTs able to provide complete information on exceptional case decisions



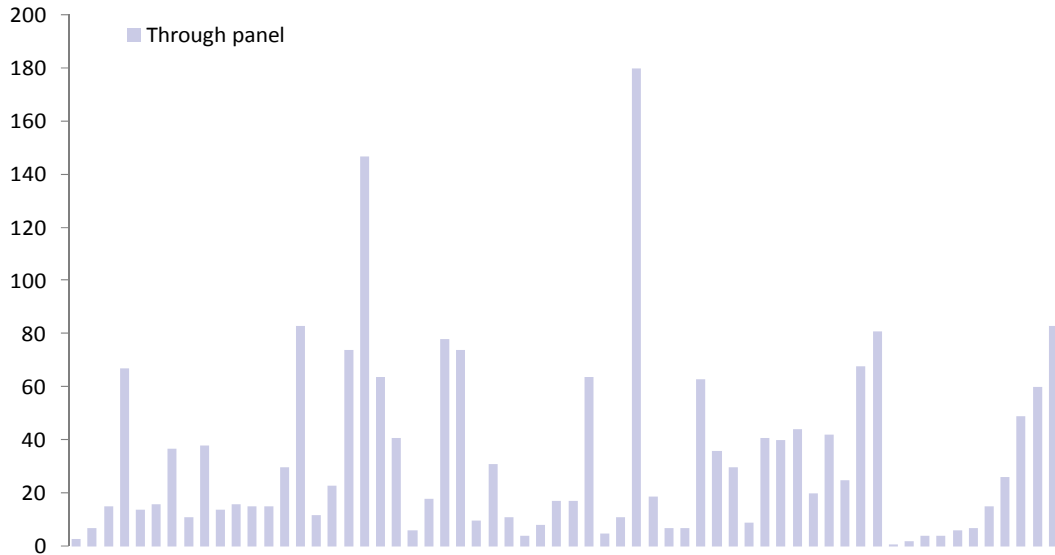
These findings are worrying. The absence of accurate information on these issues makes it difficult to see how PCTs can:

- Meet the obligations on them contained in the draft NHS constitution to put in place “clear and transparent” arrangements for considering exceptional funding requests, and to publish information on them²⁹.
- Audit and performance-manage their exceptional case procedures to ensure they are working .
- Understand how much they are paying in funding as a result of approvals of exceptional cases.
- Understand how drugs not routinely funded impact on the health outcomes of patients.

The latter point is particularly crucial, because many drugs which have not been approved by NICE have not been approved due to the lack of clear evidence that they would be of sufficient benefit. The use of the drug in more patients may help to build this evidence base to the point where NICE is able to issue a positive recommendation, but without its collection by PCTs this valuable data is lost.

Funding decisions

Our audit uncovered large divergences in the number of applications for funding for cancer treatment processed by different PCTs' exceptional cases panels. This spread is illustrated in the chart below.



As the chart shows, the number of applications received by exceptional cases panels varies hugely – with one PCT receiving one application in the period requested, while another PCT processed 180. The mean number of applications received is 33.

Variation in exceptional funding requests received

Leeds PCT reported that it received 180 exceptional funding requests over the audit period³⁰ whereas Halton and St Helens PCT reported that it received only one request³¹.

This huge discrepancy cannot be explained by differences in population size: the largest PCT in the country is, for example, only 13 times the size of the smallest³² – not 180. We would offer two possible explanations for the discrepancy, with the actual reason for it likely to be a combination of both:

- Some PCTs are particularly generous in their funding of cancer medicines which have not been approved by NICE, meaning that very few treatments ever get to the stage where exceptional requests for funding are made
- Some PCTs tell clinicians that a treatment is extremely unlikely to be funded through the exceptional cases route and, as a result, no applications to the exceptional case panel are ever made

Without further information, it is difficult to ascertain where the balance lies, and we would ask the Department of Health to investigate this matter further.

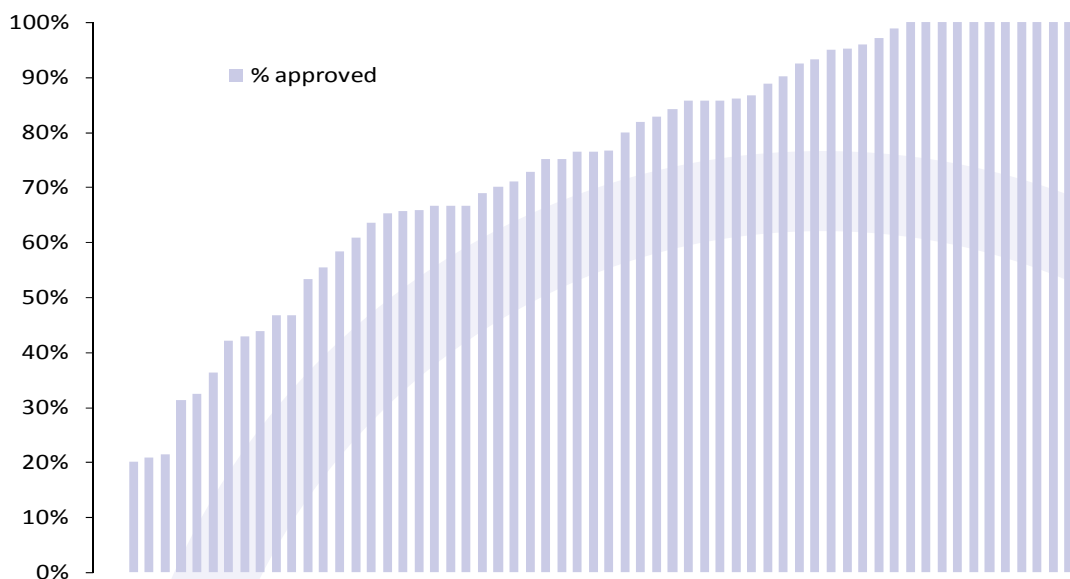
We also found evidence of a 'postcode lottery' in the proportion of applications for cancer treatment accepted and rejected. Of the 62 PCTs from which we received directly comparable information on this issue, 11 PCTs approved all the requests made to their exceptional cases panel, while 2 PCTs approved none of the requests.

Geographically close areas also exhibit huge variation

Mid Essex PCT approved 96% of requests made to its exceptional cases panel³³. This compares to 0% in neighbouring South West Essex PCT³⁴.

The spread is illustrated in the chart below:

Percentage of exceptional cases approved by PCT



Such wide variations are deeply concerning. We would urge the Department of Health to investigate why – in some areas of the country – patients have virtually no chance of getting their treatments approved whilst in others patients are virtually guaranteed to receive their treatment.

It is nevertheless encouraging that the average approval rate across all PCTs is 74%, meaning that almost three in every four patients receive funding through the NHS for the treatments that they and their clinicians have requested. This has implications for policymakers:

- The additional resource implications to the NHS for funding all cancer treatments recommended by clinicians does not appear to be large given that only one in four of the small number of patients who go through the exceptional cases process have their case rejected.
- Given the high number of approvals for some drugs, PCTs may wish to consider adopting a blanket policy of funding these interventions instead of using the exceptional cases procedure, so preventing patients from having to experience the anxiety of having a case heard and PCTs having to devote administrative resources to running the process.

Analysis of the treatments requested

The number of cancer treatments being processed through exceptional cases panels is shown in the table below. As can be seen from the figures, and unsurprisingly, the list is dominated by those high-cost treatments reported heavily in the media, including:

- [Sunitinib \(Sutent\)](#). A treatment primarily used for kidney cancer.
- [Erlotinib \(Tarceva\)](#). A treatment primarily used for lung cancer.
- [Cetuximab \(Erbix\)](#). A treatment primarily used for bowel cancer.
- [Bortezomib \(Velcade\)](#). A treatment for blood cancer.

The vast majority of treatments requested are used to treat less common or rarer cancers, either in a licensed indication or off-licence.

Treatment	Number of applications processed
Sunitinib (Sutent)	218
Erlotinib (Tarceva)	175
Cetuximab (Erbix)	106
Trastuzumab (Herceptin)	100
Bortezomib (Velcade)	94
Dasatinib (Sprycel)	78
Pemetrexed (Alimta)	75
Docetaxel (Taxotere)	69
Sorafenib (Nexavar)	62
Temozolomide (Temodar)	61
Bevacizumab (Avastin)	56
Lenalidomide (Revlimid)	40
Rituximab (MabThera)	39
Capecitabine (Xeloda)	38
Gemcitabine (Gemzar)	31
Alemtuzumab (Campath)	23
Gemtuzumab (Mylotarg)	16
Irinotecan (Campto)	13
Fulvestrant (Flaslodex)	13
Paclitaxel (Taxol)	12
Ibritumomab (Zevalin)	11
Azacytidine (Vidaza)	11
Imatinib (Glivec)	11
Clorfaribine (Clolar)	10
Cisplatin (CDDP)	9
Trabectedin (Yondelis)	7
Nilotinib (Tasigna)	6
Infliximab (Remicade)	3
Topotecan (Hycamtin)	3
Yttrium-90	3
Letrazole (Femara)	2
Other cancer	826
TOTAL	2221

At first glance, it may appear surprising that trastuzumab (Herceptin) is so high in the list given that it has been NICE-approved since August 2006³⁵.

This may be due to the fact that our audit covered a period from 1 October 2006, and between 1 October and 23 November 2006 PCTs were not under a statutory obligation to fund it for eligible patients. In addition, the requests for funding for Herceptin received an extremely high acceptance rate, as the following table shows:

Treatment	% acceptance by exceptional cases panels
Gemtuzumab (Mylotarg)	94%
Trastuzumab (Herceptin)	93%
Imatinib (Glivec)	91%
Alemtuzumab (Campath)	87%
Capecitabine (Xeloda)	84%
Paclitaxel (Taxol)	83%
Rituximab (MabThera)	82%
Azacytidine (Vidaza)	82%
Docetaxel (Taxotere)	81%
Temozolomide (Temodar)	80%
Clorfaribine (Clolar)	80%
Dasatinib (Sprycel)	78%
Fulvestrant (Flaslodex)	77%
Trabectedin (Yondelis)	71%
Bortezomib (Velcade)	70%
Irinotecan (Campto)	69%
Gemcitabine (Gemzar)	68%
Nilotinib (Tasigna)	67%
Infliximab (Remicade)	67%
Topotecan (Hycamtin)	67%
Yttrium-90	67%
Pemetrexed (Alimta)	64%
Erlotinib (Tarceva)	63%
Lenalidomide (Revlimid)	60%
Letrazole (Femara)	50%
Sorafenib (Nexavar)	47%
Sunitinib (Sutent)	46%
Bevacizumab (Avastin)	41%
Cetuximab (Erbix)	41%
Ibritumomab (Zevalin)	36%
Cisplatin (CDDP)	22%

It is important to highlight that many of these treatments are used in the treatment of cancer patients in the final, advanced stages of their condition (with the notable exception of Herceptin) – usually to prolong life by a few extra months. The volume of applications to exceptional cases panels for these treatments implies that NICE is either not appraising, or appraising and rejecting, treatments for which there is a high demand, and treatments which both clinicians and patients believe should be made available on the NHS (indeed, draft guidance recently issued by NICE recommends that three of the drugs in the list above – Avastin, Sutent and Nexavar – should not be made available to patients with kidney cancer on the NHS³⁶). We discuss the implications which this finding has on the NICE approvals process in the next chapter.

Our audit exposes for the first time the underlying demand in England for cancer treatments which are not generally available on the NHS. For example, over the audit period (October 2006 – July 2008 (20 months)):

- 2,065 applications to exceptional cases panels were made in 62 PCTs. If this figure is extrapolated to include all PCTs, it suggests that around 5,000 applications were made in this 20-month period, or 3,000 cases annually.
- Of these applications, 1,529 were approved. If this figure is again extrapolated to all PCTs, it suggests that around 3,750 applications for funding were approved in the 20-month period, or 2,200 cases annually.
- Of the applications, 536 were rejected – suggesting that 1,300 patients' requests for funding were denied across the country in the 20-month period, or 800 annually.

It is these 800 patients a year, whose applications for funding are not deemed 'exceptional' enough, who form the cohort of cancer patients who may be forced to 'top-up' their care. This is a crucial finding, and will be of relevance to the Department of Health's own review of the rules relating to so-called 'top-up' payments. It demonstrates both the relatively small number of cancer patients who are forced into a position where they may have to top-up their care, and also that the issue of 'top-ups' can be partly addressed by putting in place a fair and responsive system for processing exceptional cases.

Recommendations on reducing the variations in PCT decisions

4 The Department of Health should conduct an investigation into why there is such a significant variation in both the number of exceptional case requests submitted to different PCTs and the outcomes of these requests.

5 The Department of Health should urgently assess the cost of funding all rejected exceptional cases. Given the small volume of exceptional case applications, the Department of Health should also consider the merits of the following two options:

- Establishing a regional or central exceptional cases panel to assess requests.
- Creating an exceptional cases fund to cover the cost of funding these treatments, which have already been recommended by clinicians.

6 This would have the benefits of bringing greater consistency to decision-making processes and reducing the administrative burden inherent in operating 152 separate processes to hear 3,000 cases a year.

The Department of Health should prioritise improving the exceptional case processes used by PCTs. This will minimise occasions when patients will be forced to consider ‘topping up’ their NHS care with privately-purchased treatment. Unless this issue is addressed, efforts to reform the rules regarding when a patient may purchase a treatment privately will be doomed to fail and the founding principles of the NHS, soon to be enshrined in the NHS Constitution, will be fatally undermined.

Chapter 5: Reducing the burden on exceptional case processes

From our analysis of the responses to our Freedom of Information requests, we estimate that there have been approximately 5,000 exceptional funding requests involving cancer patients over the past two years. Feedback from clinicians and PCTs suggests that this number is set to grow.

Decisions on exceptional cases have become increasingly important for patients with rarer cancers because of a number of reasons:

- **Not all treatments for rarer cancers are licensed** – because of the extreme rarity of their cancer, some patients are forced to rely on treatments which do not have a licence for their exact condition. Often their condition is so rare that it is extremely difficult to conduct clinical trials. This means that national guidance is invariably unavailable.
- **Not all treatments for rarer cancers are submitted to NICE** – NICE has traditionally not appraised many treatments for rare conditions. The Cancer Reform Strategy states that “as a default position all new cancer drugs and significant new licensed indications will be referred to NICE, providing that NICE agrees that there is a sufficient patient population and evidence base on which to carry out an appraisal”³⁷. However in reality this means that many treatments for rarer cancers will continue not to be appraised by NICE. Indeed, the latest edition of NICE’s Social Value Judgements states that: “NICE does not expect to receive referrals from the Secretary of State for Health to evaluate ‘ultra-orphan drugs’ (drugs used to treat very rare diseases or conditions)”³⁸
- **The NICE process takes too long** – despite the welcome introduction of the Single Technology Appraisal process, there is still usually a significant lag between when a drug is licensed and when a NICE appraisal is available.

Restricting funding for drugs which have yet to be appraised by NICE

Warwickshire PCT has a list of 'Code 6' drugs which consists of cancer drugs that have not been assessed by NICE and are not in the Arden Cancer Network formulary. The PCT's policy is that these drugs will not be funded until the Network's Drugs and Therapeutics Committee has prioritised the drug, or NICE has approved it. This includes treatments for lung cancer, leukaemia, kidney cancer, gastric cancer and head and neck cancer³⁹.

The result is that patients requiring these treatments will invariably have to be assessed through the exceptional cases process.

- **The NICE process is inappropriate for treatments for advanced forms of rarer cancer** – there is a growing inequality between the treatments for cancer available in Europe and those available in England. This has resulted in the phenomenon of 'passport prescribing.' We believe that the methodology NICE uses for appraising treatments for advanced cancer, and particularly advanced rarer cancers, is increasingly inappropriate. Unless it is reformed, more and more treatments will be rejected leaving clinicians and patients with no other option but to pursue the exceptional case process.

Patients in England have to be 'exceptional' to gain access to many cancer drugs but patients elsewhere in Europe get them as standard

The process used by NICE is putting England increasingly out of step with the rest of Europe. One such example is that NICE published draft guidelines in August 2008 which would deny patients with kidney cancer access to Avastin (bevacizumab), Sutent (sunitinib) and Nexavar (sorafenib).

As a result, the only chance that the 1,700 patients who develop advanced kidney cancer every year will have to gain access to these drugs is through the exceptional cases process. By contrast, these drugs are already available as standard in every other country in western Europe, with the exception of Belgium. A number of organisations have indicated that they will appeal against NICE's recommendation.

- **NICE guidance is not always implemented** – there is a tension between the statutory duty on a PCT to achieve financial balance and the obligation to fund NICE-approved treatments within three months of guidance being issued. We are concerned that the duty on PCTs to break even is on a stronger statutory footing than the duty to fund NICE guidance.

Failing to implement NICE guidance – In Vitro Fertilisation (IVF)

Our audit revealed that both Portsmouth City Teaching PCT and Sheffield PCT have policies of only funding one cycle of IVF, despite NICE guidance stating that PCTs should fund three cycles. This issue is important to cancer patients as many will be left infertile by their cancer or the treatment they received to tackle it.

Limiting the use of NICE-approved medicines

Our audit found that Peterborough PCT has adopted a policy of requiring clinicians to agree an indicative number of cases to be treated per year, based on the NICE estimates for the PCT population. Funding is only given up to the NICE estimated costs. The PCT rejected an option of funding all NICE-approved treatments for every eligible patient, irrespective of whether costs might be higher than those recommended by NICE.

By imposing a review mechanism if the agreed treatment thresholds are exceeded, the PCT could place itself in the position of refusing to fund further treatment which has been approved by NICE. We believe this would be in contravention of the principle set out in the draft NHS Constitution⁴⁰.

The greater the number of requests being processed through the exceptional cases route, the harder it will be for PCTs to adequately consider the evidence in individual cases. Therefore we consider it vital that everything possible is done to provide guidance on the availability of different treatments, reducing the pressure on the exceptional cases process.

Our audit shows that some cancer networks are already playing an important role in providing advice to PCTs on treatments which do not yet have NICE guidance. For example, a number of cancer networks (e.g. Mount Vernon, Derby-Burton and Peninsula) now have advisory committees on the availability of new treatments. However it is concerning that not all PCTs served by these cancer networks make reference to the advice provided by these committees.

Recommendations to reduce the burden on exceptional case process

7 In order to minimise the number of treatments which are being determined by exceptional case decisions, the NICE process for appraising new treatments needs to be speeded up. We welcome the commitment made on this in the NHS Next Stage Review and urge the Department of Health and NICE to publish details on how this will be achieved as soon as possible. Where for whatever reason NICE guidance has not been published within a certain number of months of licensing, we believe the Department of Health should stipulate to PCTs what other guidance should be used. This could be SMC guidance or perhaps a consensus opinion by clinical experts. Where NICE fails to deliver guidance within a set timeframe, performance sanctions should be introduced. These may include requiring NICE to report to Parliament the reasons for the delay.

8 The increasing variance between the cancer medicines which are available in England and those available in Europe, resulting in 'passport prescribing,' must be addressed. Therefore the current NICE appraisal methodology for treatments for advanced cancer should be overhauled. There is a persuasive case for varying the cost-per-QALY threshold according to both severity of disease and the rarity of the condition in question.

9 Cancer networks should be encouraged to provide guidance to PCTs on treatments for which no national guidance is available. The role of cancer networks in doing this should be written into PCT guidelines on funding new treatments.

10 The legal obligation for PCTs to fund NICE-approved treatments should be strengthened by including it within primary legislation and making clear the sanctions that will be used if a PCT fails to comply.

ANNEX 1

List of PCTs which responded to the Freedom of Information request, correct as of 5th August 2008

Ashton, Leigh and Wigan PCT
Barking and Dagenham PCT
Barnsley PCT
Bassetlaw PCT
Bedfordshire PCT
Berkshire West PCT
Bexley Care Trust
Birmingham East and North PCT
Blackburn with Darwen Teaching PCT
Blackpool PCT
Bolton PCT
Bournemouth and Poole PCT
Bradford and Airedale Teaching PCT
Brighton and Hove City Teaching PCT
Bristol PCT
Bromley PCT
Bury PCT
Calderdale PCT
Cambridgeshire PCT
Camden PCT
Central Lancashire PCT
City and Hackney Teaching PCT
Cornwall and Isles of Scilly PCT
Coventry Teaching PCT
Darlington PCT
Derby City PCT
Derbyshire County PCT
Devon PCT
Doncaster PCT
Dorset PCT
Dudley PCT
East & North Herts PCT
East Riding of Yorkshire PCT
East Sussex Downs and Weald PCT
Eastern and Coastal Kent PCT
Gateshead PCT
Gloucestershire PCT
Great Yarmouth and Waveney PCT
Greenwich Teaching PCT
Halton and St Helens PCT
Hampshire PCT
Haringey Teaching PCT
Hartlepool PCT
Hastings and Rother PCT
Havering PCT
Heart of Birmingham Teaching PCT
Herefordshire PCT
Heywood, Middleton and Rochdale PCT
Hounslow PCT
Hull Teaching PCT
Islington PCT
Kirklees PCT
Knowsley PCT
Leeds PCT
Liverpool PCT
Manchester PCT
Mid Essex PCT
Milton Keynes PCT
Newcastle PCT
Norfolk PCT
North East Lincolnshire PCT
North Lincolnshire PCT
North Somerset PCT
North Tees PCT
North Yorkshire and York PCT
Northamptonshire Teaching PCT
Nottingham City PCT
Nottinghamshire County Teaching PCT
Oxfordshire PCT
Peterborough PCT
Plymouth Teaching PCT
Portsmouth City Teaching PCT
Redbridge PCT
Richmond and Twickenham PCT
Rotherham PCT
Salford Teaching PCT
Sheffield PCT
Shropshire County PCT
Solihull Care Trust
Somerset PCT
South Gloucestershire PCT
South Tyneside PCT
South West Essex PCT
Southwark PCT
Stockport PCT
Suffolk PCT
Sunderland Teaching PCT
Swindon PCT
Tameside and Glossop PCT
Telford and Wrekin PCT
Torbay Care Trust
Tower Hamlets PCT
Trafford PCT
Wakefield District PCT
Walsall Teaching PCT
Warrington PCT
Warwickshire PCT
West Essex PCT
West Kent PCT
West Sussex PCT
Western Cheshire PCT
Westminster PCT
Wiltshire PCT
Worcestershire PCT

-
- ¹ Department of Health, Cancer Reform Strategy, December 2007
 - ² Department of Health, Good Practice Guidance on Managing the introduction of New Healthcare Interventions and Links to NICE Technology Appraisal Guidance, December 2006
 - ³ Department of Health, Cancer Reform Strategy, December 2007
 - ⁴ Department of Health, Cancer Reform Strategy, December 2007
 - ⁵ Correspondence dated 11th July 2008
 - ⁶ Correspondence dated 14th July 2008
 - ⁷ Shropshire County PCT, Policy for Managing Individual Funding Requests, May 2007
 - ⁸ Correspondence dated 15th April 2008
 - ⁹ West Kent PCT, Individual Treatment Panel Policy, August 2007
 - ¹⁰ Hillingdon PCT, Drugs, Devices and Interventions Funding Policy, August 2007
 - ¹¹ Mid Essex PCT, Exceptional Cases Panel Terms of Reference, February 2007
 - ¹² Correspondence dated 15th April 2008
 - ¹³ A policy and procedure for introducing new medicines and indications across: Norfolk PCT and Great Yarmouth & Waveney PCT, September 2007, Section 13.4
 - ¹⁴ Correspondence dated 14th July 2008
 - ¹⁵ Correspondence dated 16th July 2008
 - ¹⁶ Correspondence dated 27th May 2008
 - ¹⁷ Hillingdon PCT, Exceptions Committee Terms of Reference, December 2006
 - ¹⁸ Correspondence dated 11th July 2008
 - ¹⁹ Correspondence dated 15th July 2008
 - ²⁰ Correspondence dated 14th July 2008
 - ²¹ North Yorkshire & York PCT, Terms of reference for individual case panel, November 2007
 - ²² East & North Herts PCT, Request Form for the Exceptional Cases Panel, undated
 - ²³ Camden PCT, Policy on managing individual treatment/care requests, undated
 - ²⁴ Hansard, Column 1504W, 8 July 2008
 - ²⁵ Barking and Dagenham PCT, Applying to the difficult decisions panel: notes of guidance and application form, December 2007
 - ²⁶ Herefordshire PCT, The named patient panel: roles and responsibilities, undated
 - ²⁷ Correspondence dated 30 June 2008
 - ²⁸ East and North Herts PCT, Request form for the exceptional cases panel, undated
 - ²⁹ Department of Health, Consultation on the draft NHS constitution, 30 June 2008
 - ³⁰ Correspondence dated 18 July 2008
 - ³¹ Correspondence dated 2 July 2008
 - ³² Department of Health, Attribution data set of GP registered populations, 24 January 2008
 - ³³ Correspondence dated 11 July 2008
 - ³⁴ Correspondence, undated
 - ³⁵ NICE, Breast cancer (early) – trastuzumab, 23 August 2006
 - ³⁶ National Institute for Health and Clinical Excellence, Renal cell carcinoma - bevacizumab, sorafenib, sunitinib and temsirolimus: appraisal consultation, 7 August 2008
 - ³⁷ Department of Health, Cancer Reform Strategy, 1997, page 64
 - ³⁸ NICE Social Value Judgements - principles for the development of NICE guidance, second edition, 2008, page 20
 - ³⁹ Correspondence dated 11 July 2008
 - ⁴⁰ Commissioning arrangements for drugs which have been recommended for NHS use by NICE, Peterborough PCT, December 2006