YOUR PATIENT'S RIGHT TO TREATMENT

Applying for NHS Exceptional Funding for Treatment

www.rarercancers.org.uk

Supported by an equal unrestricted grant from
Bayer and Bristol-Myers Squibb

Bayer HealthCare
Bayer Schering Pharma

Bristol-Myers Squibb

THE NATIONAL HEALTH SERVICE ACT 2006
(A consolidating act)

Part 1 Promotion and Provision of the Health Service in England

The Secretary of State and the health service in England

1 Secretary of State's duty to promote health service

(1) The Secretary of State must continue the promotion in England of a comprehensive health service designed to secure improvement—
  (a) in the physical and mental health of the people of England, and
  (b) in the prevention, diagnosis and treatment of illness.

(2) The Secretary of State must for that purpose provide or secure the provision of services in accordance with this Act.

(3) The services so provided must be free of charge except in so far as the making and recovery of charges is expressly provided for by or under any enactment, whenever passed.

YOUR PATIENT'S RIGHT TO TREATMENT

Applying for NHS Exceptional Funding for Treatment

INTRODUCTION

The principles that underpin the NHS – equality of access to treatment to all irrespective of personal wealth or social standing, "free" at the point of access, quality of treatment – apply as much today as they did 60 years ago.

Rationing of these treatments is established and here to stay. The NHS cannot or will not afford all treatments to all patients. However, we believe it is important for the rationing of those treatments to be a fair and rational process.

This guidance booklet is intended to help ensure that these principles apply now and continue in the future. It is not designed to 'knock' the NHS, but to support it. The aim is to explain and hopefully improve the existing means by which the right treatment is provided to the right patient at the right time.

Funding for exceptional treatment

The booklet is designed to guide consultants whose National Health Service (NHS) patients need Primary Care Trust (PCT) approval for funding for rarer cancers. Where funding is sought it is usually referred to as funding for exceptional treatment.

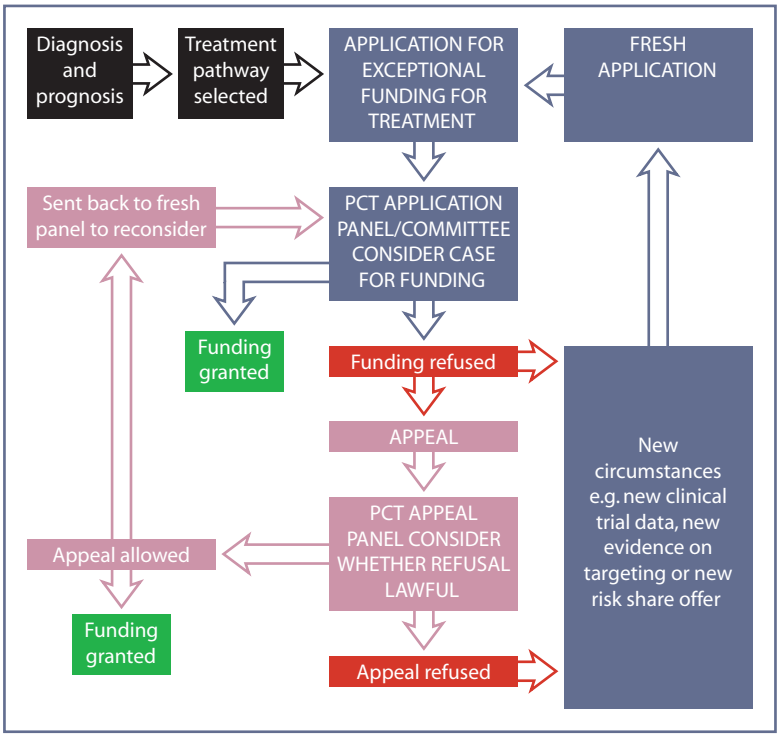
The treatment might have no guidance from the National Institute for Health and Clinical Excellence (NICE) or may even have negative NICE guidance. It can still be applied for. The consultant usually makes the initial application on behalf of the patient and the patient, if refused funding, can appeal to the PCT with the consultant's help.

This booklet provides support in the application and appeal process for the beleaguered consultant and the needy patient. We explain the procedural steps of how to apply, the information and evidence required, the considerations a PCT must and may take into account, and how to go about appealing. Although the booklet focuses on funding for rarer cancers, the procedures described could apply equally to other more common cancers and types of treatment not routinely funded by NHS PCTs.

All applications should be made in partnership with your patient, or where relevant, the patient's guardian or carer. The patient must be the driving force but together you will be more effective.

Rarer cancers not routinely funded: overview of pathways to treatment

The flowchart gives an overview of the processes by which PCTs make decisions on exceptional treatments. We'll consider the different processes in more detail in the rest of this booklet.



INTRODUCTION

HOW TO APPLY FOR NHS EXCEPTIONAL FUNDING FOR TREATMENT

Application process

There is no common standard national application process. Each of the 131 different PCTs have their own procedure. Some require lengthy application forms, some only a letter. Some insist the application is made by a designated consultant in a particular hospital.

There is no common name given to each PCT procedure. Some are called Exceptional Funding; some are called Special Circumstances or Special Funding. Some are termed Patient Individual Needs (PIN) Panels. The PCTs all make reference to the concept of exceptionality yet each PCT adopts a different meaning to the word exceptionality. The process is not transparent and there are few guidelines. In 2006, the Department of Health reissued *Good practice guidance on managing the introduction of new healthcare interventions and links to NICE technology appraisal guidance*. However, this does not provide guidance on the exceptionality process and procedure for a PCT or consultant.

You need to obtain information about the process followed by your particular PCT. The PCT should have written procedures for their application process. You can obtain these from the PCT.

Person making the application

Usually the PCT treat it as the consultant's application. However, because the duty of primary care is to the patient, it is in law the patient's application with the consultant applying on behalf of the patient. The PCT can choose to manage the application in their way. What they cannot do is refuse to consider the application, whoever makes it.

PCT application panel/committee

The application should be made to the relevant panel or committee of the PCT. Some are permanent committees whilst others are only formed for individual cases. In the smaller PCTs some are formed *ad hoc*. Often PCT panels and committees have no consultant oncologist members. Some PCTs have only one panel member and little or no consultation. Some make the decision without waiting for all the evidence or notifying the patient or consultant. This is wrong. PCTs should be open and transparent and accountable

in their decisions. The PCT should take account of the best information and that might mean wider informed consultation. After all, the matter might be challenged in the Courts.

Circumstances in which an application can be made
An application can be made in exceptional circumstances where:

- there is no NICE or PCT guidance; or
- there is negative NICE and/or local PCT guidance.

When the application should be made
The application should be made well before the patient needs the treatment. In the case of some rarer cancers and aggressive cancers, this will be from the time that you decide that the treatment will be needed even if months away. There is nothing wrong in obtaining a decision in principle even if the patient does not yet need that treatment. This is important if the patient seeks to take the matter further by having the treatment privately or by seeking judicial review. The target of a maximum of 28 days for consideration of new treatments is not always met. There are inevitable time delays in the whole process, especially if there is an appeal.

When the condition means the patient will not live long without the treatment, it is imperative that the application process is begun as soon as possible.

Consultant's role in the application

The consultant needs to ensure all relevant information (documents, letters etc) is sent to the PCT well ahead of the panel or committee meeting.

The application will rarely succeed without your help. It is part of your duty to the patient to provide to the PCT timely and relevant information so that a fully informed decision can be made.

You can refer the patient for an independent second opinion. Some PCTs like a second opinion when the case is for them 'borderline'.

Writing the letter in support

It is essential that your letter of support for the application should address the PCT's own criteria. As the consultant you should state the preferred treatment. You should give full written reasons for this treatment. You should refer to relevant clinical data and research as well as any experience of the drug in practical

settings and the effect on this type of patient. You should set out the exceptional factors.

Have a critical look at two hypothetical but typical examples of letters written by consultants in support of patients' applications.

"This patient of mine, Mr. X, has MCRC. There's nothing to be done for him in terms of survivability. I think that X should get treatment Y because in my view it may prolong his life by a few months".

"Unfortunately, as you are aware, drug Z is not funded on the NHS, although it has been proven an effective treatment. My patient is now in the unenviable position of having to decide whether to spend some of her savings on funding this drug privately. In view of the fact that trials have shown the drug to be effective I would be grateful if you would consider allowing my patient to have this recommended treatment on the NHS".

Whilst the content of each letter may be true it is far from the whole story. These letters would not convince you. There is no reason for them to convince the PCT. The letters highlight some important considerations when writing letters of support.

- The patient is a patient of the PCT so it is important for them to recognise the patient is ultimately their responsibility.
- Some empathy might be helpful in establishing this is a patient worth bothering with.
- Prolonging life is not the only criterion. It is often not determinative unless the period is beyond six months to a year.
- The word "may" is a terrible word in legal terms. It might as well be "may not". There is no force in it.
- The effectiveness has to be spelt out i.e. effective in terms of when, where and how.
- If patients have their own money, they are not such a problem for the PCT.
- What trials have shown the treatment to be effective? How effective is it?

Here's an example of what might be contained in a fuller letter in support. Comments on the letter are included in italics.

Heading – name, address, mobile telephone number, qualifications, experience in this field

I have a patient X who comes within your PCT. X has [name of condition]. The history of the matter is as follows [set out chronology, condition and diagnosis]. Dr [Y] has previously treated the patient/been involved and come to the same conclusion that I have in regard to the need for this application. ***(Give evidence of additional support, not just one person's word or opinion)***.

X is currently being treated with [Z] which is the usual line of treatment. X has had [previous treatment including any question or issue over delay or misdiagnosis]. X currently presents with [symptoms]. These symptoms are non-responsive to the current treatment Z. The prognosis on treatment Z is poor.

There is another line of treatment available. (This should be a treatment that isn't just funded automatically or by arrangement). It is [A**]. It is licensed/not licensed for use [in the particular setting]. As a licensed treatment it is clinically effective/even though unlicensed, it is nevertheless clinically effective because [B+]. ***(Even unlicensed is valid as ultimately the professional duty of the consultant is to consider whether it is worth prescribing)***.

There is evidence that use of A** will be clinically effective in the case of X. This evidence is found at [list and explain C, D, E trials, studies etc].

Furthermore, in actual use, at [F hospital] since [200?] it has proved its worth. Consultant and patient groups have advised that the results are [set out results in summary G]. Although not a clinical trial/study this is supportive of use and clinical effectiveness.

The use of A** in similar patients at H Area PCT has improved their response rate for the purposes of the government initiative [I] target for this condition.

The condition is exceptionally rare. The percentage of the population who contract it is [J%].

The summary of the evidence C, D, E supported by G is that the median survival/improvement in function is [K/L].

Furthermore, patient X has an unusual history of [M] in the family. This means that X starts at a lower level than the average patient presenting with cancer.

When taken together as a whole, the combination of late diagnosis/missed diagnosis, late treatment once diagnosed, lack of readily available easy to source treatment and X's pre-existing condition mean that his case is wholly exceptional.

When taken together with the availability of A** and the absence of other drug treatments on the NHS which are likely to be of benefit to X, there is a clinical case made out in support of the use of A** in this particular case of X.

As exceptional, X should not be unduly penalised under consideration of cost.

If cost is a factor, however, then it is not the only or deciding factor.

HOW TO APPLY FOR NHS EXCEPTIONAL FUNDING FOR TREATMENT

THE PCT EXCEPTIONAL CASES PANEL

PCTs begin from an assumption that the treatment is not cost effective. You must provide reason why this treatment fits outside the normal rules of the PCT. PCTs are entrusted with public money and have competing pressures for the use of that money. They have to justify the use of that money for your patient on rational grounds.

PCTs are required to conduct a proper assessment of the available evidence to see if the patient can be funded outside the normal rules. The panel carry this assessment out on behalf of the PCT.

Exceptional circumstances

Each PCT has its own way of interpreting what amounts to exceptional circumstances.

Some PCTs describe something as exceptional treatment when it is "a treatment or diagnostic procedure that falls outside the usual commissioning arrangements, contracts and Service Level Agreements …" (Bromley Primary Care Trust, 2008).

Barking and Dagenham Primary Care Trust adopted another approach – to refine further or define exceptional circumstances by comparison with other similar patients at that stage of treatment (England and Wales High Court Decisions, 2007).

As noted in Australian case law, every case is different, so there are always some aspects of a case that may be regarded as exceptional. The question inevitably arises: exceptional compared with what?

In the English Courts, exceptional has not been defined. Exceptional has been construed as an ordinary, familiar English adjective. To be exceptional, a circumstance did not need to be unique, or unprecedented, or very rare; but it could not be one that was regularly, routinely, or normally encountered.

It is lawful for a PCT to leave it at that and not try to further define or refine the meaning of exceptional.

THE PCT EXCEPTIONAL CASES PANEL

Examples of exceptionality

Please note that none of the following cases set any precedent for the PCT.

A patient unable to tolerate a full dose of usual chemotherapy for health reasons was able to receive a NICE negative guidance biological agent in combination to bring her up to the level of chance of success that the usual full dose alone would have provided.

A patient was a single parent and looked after a severely disabled teenage child who would otherwise be hospitalised. Their case was considered in a joint cost effectiveness model and was deemed exceptional.

A patient who had been misdiagnosed was given the wrong surgery. Delay in treatment exacerbated the condition. He was exceptional.

A patient who could not risk blood transfusion obtained exceptional treatment of combination chemotherapy which managed the risk.

THE PCT EXCEPTIONAL CASES PANEL

FACTORS INFLUENCING THE APPLICATION PANEL'S DECISION

Consultant's letter
The PCT must take into account clinical advice from a consultant.

Medical research and information
This will include source material on the clinical condition, research evidence and information from the drug manufacturer, including licensed indications.

Local PCT criteria
Criteria should not make it impossible to qualify for treatment. There must not be either explicit rule or implicit assumption within the criteria such that when they are applied in practice, they rule out all or nearly all applicants. An example of that would be to administer the drug only to those on clinical trials of the proposed treatment. The PCT must not place the decision making process in the hands of non-PCT bodies.

NICE guidance

All guidance is just that – guidance and not directions. Guidance does not always apply to all circumstances and the responsibility for proper treatment is always with a consultant and PCT.

Guidance from NICE must be taken into account. Whether it is followed depends on the particular and personal circumstances of the patient. It is not to be automatically or blindly followed by a PCT as that would make it a mandatory direction, which it is not. NICE guidance and local and other guidance when taken into account specifically do not override the clinical opinion of the consultant.

NICE states of its guidance: "Once NICE publishes clinical guidance, health professionals and the organisations that employ them are expected to take it fully into account when deciding what treatments to give people. However, NICE guidance does not replace the knowledge and skills of individual health professionals who treat patients; it is still up to them to make decisions about a particular patient in consultation with the patient and/or their guardian or carer when appropriate" (National Institute for Health and Clinical Excellence, 2005).

Lack of NICE guidance is no reason to refuse treatment (Department of Health, 2006).

Sometimes, in the fast changing world of cancer treatment, some aspects of clinical evidence or assumptions made in the historical process of formulating NICE guidance can become out of date. It is important to note, however, that unless withdrawn or amended by NICE, NICE guidance remains in force for a PCT. It follows that the final date of admission of evidence to the NICE Final Technology Appraisal Guidance becomes increasingly important the longer time goes on. The PCT is able to take into account changes in the evidence base.

FACTORS INFLUENCING THE APPLICATION PANEL'S DECISION

Other sources of information

The Cancer Reform Strategy (Department of Health, 2007) set out the minimum sources of information that PCTs should take into account:

- London Cancer New Drugs Group
  www.druginfozone.nhs.uk
- National Prescribing Centre (NPC)
  www.npc.nhs.uk
- Scottish Medicines Consortium (SMC)
  www.scottishmedicines.org.uk

Other sources could include the Centre for Evidence-based Purchasing (CEP)
www.pasa.nhs.uk/PASAWeb/NHSprocurement/CEP/

Non-clinical circumstances

Case law rules out a PCT taking into account non-health factors e.g. potentially emotive issues, such as the age of patients, or whether they have children. However, the important issue here is that the PCT has a wide ambit of discretion and may take into account those factors that are considered relevant. For example,

age might be relevant if only a small percentage of the population have the condition at that age and there is a good success rate in treatment.

Cost of treatment and risk share

Cost cannot be the sole or determinative factor. It is only one aspect of the decision. Where a person lives and the level of funding allocated by a particular PCT should not in theory affect the decision unless there is a provable lack of funding. Where the NHS has found itself in surplus, cost restrictions should not be used as a reason to refuse, especially where there is positive NICE guidance. If there is positive NICE guidance the treatment should be in place within a maximum of three months.

Risk share schemes proposed by drug companies may be available nationally or offered locally on a case by case basis to the PCT. Some PCTs refuse all such schemes and others adopt a more pragmatic approach. Some areas allow co-payments for exceptional treatment and others do not.

Some key points about guidance are summarised opposite.

FACTORS INFLUENCING THE APPLICATION PANEL'S DECISION

DIFFERENT SCENARIOS

No NICE guidance
Absence of any guidance does not prevent funding. Failure to fund purely due to lack of NICE guidance would be wholly wrong and potentially constitute a matter to be reported. It should be reported first to the Department of Health for it then to be investigated by the relevant Strategic Health Authority (SHA). The SHA may reiterate the message contained in Health Service Circular 1999/176 as updated in December 2006. It would be grounds for the decision being challenged as irrational and unfair.

NICE guidance must be applied correctly
Sometimes, guidance does not strictly apply. Where it is 'negative', it might be negative for only a particular type or form of the condition. It might not cover the exact condition your patient has. Guidance might only apply to a specific combination of drugs or line of treatment. It might therefore be silent on a different combination of drugs or a different setting of treatment. It may be negative only due to cost. It may support clinical effectiveness.

The guidance does not list exceptions
The fact that NICE guidance does not list exceptions is no indication that they do not exist or that the PCT can opt not to treat purely because NICE has remained silent on this. It all depends on the wording of the guidance and the setting in which the guidance operates. It is correct that some NICE guidance sets out some occasion on which there may be an exception or a condition attached to its guidance. It would be illogical to apply this occurrence of guidance written 'with exceptions' to other unrelated NICE guidance. Even where NICE guidance is written with some exceptions, those exceptions cannot form an exhaustive list of situations in which an exception might occur.

PCT allows no exceptions
This would be unlawful. There must always be exceptions.

FACTORS INFLUENCING THE APPLICATION PANEL'S DECISION

Meeting of the Exceptional Cases panel
The PCT can ask that the consultant be available by telephone for questions from the meeting. It is usual for the case to be dealt with on the available papers.

The patient may or may not be invited to join the meeting. It is unusual for the PCT to allow access to the meeting by the patient, their lawyer or their friend. However, that said, these meetings are for exceptional criteria considerations and it is not unknown for the PCT to make a specific request for a patient, lawyer, friend, relative or consultant to attend, or to allow them to attend. Again, it all depends on circumstances. If the PCT panel need to have matters explained to them or need to see and make their own assessment of the patient, then they can get that organised.

If the patient's case is refused, he or she can ask for the PCT's full minutes of the meeting and the names of those on the committee (to ensure the appeal is not heard by the same persons) and copies of all the information the PCT panel had before them at their meeting. The Freedom of Information Act helps here and, in any event, patients are entitled to copies of all their medical notes and records.

FACTORS INFLUENCING THE APPLICATION PANEL'S DECISION

APPEAL

Grounds for appeal

Appeals can be made on grounds of irrationality, procedural unfairness and unlawfulness of the decision. In addition PCTs usually allow new evidence in an appeal. In some circumstances an appeal may be simple e.g. when the panel or committee minutes are disclosed it can be seen that the PCT did not have a crucial letter or technical information. The patient is entitled to have written reasons for refusal.

Irrationality

Sometimes the PCT have become misguided, or have omitted or misapplied factual information.

Procedure

Sometimes the PCT acted unfairly e.g. by moving the meeting date without informing the patient who was thereby denied a chance to put in all the information he or she wanted to be considered.

Unlawfulness

Sometimes the PCT has refused NICE approved treatment.

New evidence
Sometimes there is new information or evidence that changes the way the PCT view the case.

New evidence includes new clinical trial data or actual practice data, finalised results of existing trials in relevant settings, new genetic or coding or indicative markers for treatment groups and an almost endless list of other information, sometimes about the patients or their circumstances e.g. the patient having received the treatment privately and successfully.

Appeal panel/committee
An appeal can be made to a second panel or committee within the PCT who will decide the issue. These will not be the people who were on the first application panel or committee.

If an appeal is successful, the appeal panel may remit the issue back to a fresh application panel.

Occasionally, the appeal panel reverse the decision of the application panel and allow funding. There is also senior executive power in the PCT to step in and make the decision if the panel becomes bogged down.

APPEAL

FRESH APPLICATION

A new application can always be made in new circumstances. These will have to be relevant and material to any decision.

SUMMARY: CHECKLISTS FOR CONSULTANTS

APPLICATION

- **Obtain the PCT procedure information and decision criteria.**
- **Take account of any sources of information and guidance the PCT have to consider such as NICE, Drugs Groups.**
- **Provide any relevant additional technical research information and information on actual use.**
- **Fill out the application form and complete the letter in support.**
- **Provide the clinical evidence to support the assertions of exceptionality.**
- **Provide mobile contact telephone number and be available to the application meeting for questions.**

APPEAL

- **Obtain the written reason for refusal.**
- **Correct factual errors in the decision by informing the PCT and patient.**
- **If the patient appeals, assist by highlighting those areas where the PCT may have gone into error.**
- **Provide any new evidence that may be relevant.**

SUMMARY: CHECKLISTS

REFERENCES

Bromley Primary Care Trust (2008) *Policy: requests for exceptional treatments*. Available from: www.bromley.nhs.uk/papers/249/3104/ETPolBoard0308.doc

Department of Health (2006) *Good practice guidance on managing the introduction of new healthcare interventions and links to NICE technology appraisal guidance*. Available from: www.dh.gov.uk/en/Publicationsandstatistics/Publications/PublicationsPolicyAndGuidance/DH_064983

Department of Health (2007) *Cancer reform strategy*. Available from: www.dh.gov.uk/en/Publicationsandstatistics/Publications/PublicationsPolicyAndGuidance/DH_081006

England and Wales High Court Decisions (2007) *Otley, R. v Barking & Dagenham NHS Primary Care Trust [2007] EWHC 1927 (Admin) (18 July 2007)*. Available from: www.bailii.org/ew/cases/EWHC/Admin/2007/1927.html

National Institute for Health and Clinical Excellence (2005) *A guide to NICE.* Available from: www.nice.org.uk/media/EE5/AF/A_Guide_to_NICE_April2005.pdf

FURTHER INFORMATION

Rarer Cancers Forum
Rarer Cancers Forum website provides contact details of PCTs and information about their policies on funding for exceptional treatments:
www.rarercancers.org.uk/information/trust_us

Rarer Cancers Forum is also building a database of rare and less common cancers.

ACKNOWLEDGEMENTS

This booklet was written by Peter Telford, Barrister, and edited by Stella Pendleton, Education Advisor, Rarer Cancers Forum.

Design: Webstar Design

Caring about people with rarer cancers

Rarer Cancers Forum 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