

Legal and ethical aspects of local decision-making about medicines and treatments

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This presentation considers the legal and ethical aspects of local decision-making about the funding of medicines and treatments. Part of this session considers aspects of judicial review and exceptional clinical circumstances. These are expanded and put into a practical legal context in a complementary presentation in this e-learning resource which focuses on Judicial Review.

Many of the ethical considerations in local decision-making represent sound management practice and, as such, are likely to be considered necessary by courts. This presentation is a bit like putting the pieces of a jigsaw together, composed of statutes, case-law, the NHS Constitution and other Department of Health guidance. The various bits form a picture of the legal and ethical considerations necessary for robust local decision-making.

All Primary Care Trusts are under a legal duty to balance their books and this means that PCTs need to promote care for all patients in their communities within finite resources. In order to do this, PCTs typically identify priorities in a number of different ways, and at a number of different levels for example, (1) making long-term strategic plans, for example to reduce health inequality in the community, (2) planning for developments in the annual spending round, (3) responding to new treatments arising during the year, and (4) responding to individual claims for exceptional access to care during the year. A key challenge for PCTs is ensuring these sometimes separate processes with different timescales are consistent with each other in order to achieve the PCT's overall aims and objectives.

In this session we look at the way in which PCTs set priorities at stages (2), (3) and (4), i.e. decisions taken in the annual commissioning round, requests for access to (or extended access to) treatments arising during the year and individual funding requests. And, we focus on the legal and ethical considerations necessary throughout these processes. So, in this presentation we will consider:

1. Is priority setting lawful?
2. The impact of NICE
3. Priority setting outside NICE
4. Exceptional clinical circumstances

1. Is priority setting lawful?

Priority setting in the NHS is lawful. The National Health Service Act 2006¹ provides that:

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The Secretary of State must continue the promotion in England of a comprehensive health service (s 1(1)).

And

It is the duty of every Primary Care Trust in respect of each financial year, to perform its functions so as to secure that the expenditure of the trust...does not exceed [its income] (s 229).

Note that since the first National Health Service Act of 1946, the duty has been to “promote”, but not to *provide* a comprehensive health service. Note too that the word “comprehensive” leaves room for discretion as to how finite resources should be allocated. It implies the need to prioritise commissioning decisions according to local need.

Reflecting this need for priority setting in the NHS, competency 6 of *World Class Commissioning* requires commissioners to “Prioritise investment according to local needs, service requirements and the values of the NHS².”

The courts too recognise the inevitability of hard choices in deciding how to allocate finite health service resources. As the Court of Appeal said in the case of *Girl B* in 1995³

...in a perfect world any treatment which a patient, or a patient’s family, sought would be provided if doctors were willing to give it, no matter how much it cost, particularly when a life was potentially at stake. It would however be shutting one’s eyes to the real world if the court were to proceed on the basis that we do live in such a world... Difficult and agonizing judgments have to be made as to how a limited budget is best allocated to the maximum advantage of the maximum number of patients.

And in 1999, the Court of Appeal said about the Secretary of State’s duty in *R v N and E Devon HA, ex p Coughlan*⁴

...the fact that the service will not be comprehensive does not mean that he is necessarily contravening [the Act]...a comprehensive health service may never, for human, financial and other resource reasons, be achievable....

The NHS Constitution also acknowledges the impact of finite resources in the NHS⁵. It says:

The NHS is committed to providing best value for taxpayers’ money and the most effective use of finite resources.

And guidance from the Department of Health and the National Prescribing Centre for local decision-making groups⁶ also says:

Like all public authorities, PCTs are required to operate within finite budgets and, therefore, have to prioritise some treatments over others according to the needs of local communities... [] Disinvestments should be considered along with investments.

Although priority setting is lawful, it is crucial that the process is conducted in a fair and reasonable way. The courts and the Department of Health insist that PCTs follow reasonable procedures to ensure patients are treated fairly, equally and consistently. PCTs that fail to do so may be subject to judicial review and their decisions referred back to the PCT to be reconsidered in the light of the courts' comments.

PCTs should also manage priority setting in a candid and transparent way, so that everyone knows how and why it is happening. Where possible, the public should be consulted about the process and engaged in making it work.

2. The impact of NICE

Before turning to the processes of priority setting, let us consider the impact of the National Institute for Health and Clinical Excellence or NICE.

Let's start by distinguishing the different guidance NICE publishes. In a minute, we will consider its *Technology Appraisal Guidance* (NICE TAGs). But NICE also publishes guidance that does not concern specific technologies in the form of *public health guidance*, guidance on *interventional procedures* and *clinical guidelines* on specific diseases and conditions. Only NICE TAGs are mandatory in law, as we will discuss in a moment. Its other guidelines are discretionary for PCTs.

This does not mean that *public health guidance*, *clinical guidelines* and guidance on *interventional procedures* can simply be ignored. They are discretionary in the sense that they should be understood and considered properly. Decision-makers are free not to follow them, but if they do so, they should be capable of explaining why and the factors that led them to do so.

Now let us return to NICE TAGs. PCTs must fund the treatment recommended by NICE in a Technology Appraisal Guidance. NICE TAGs have mandatory status. This means that, unless the Secretary of State directs otherwise in respect of a drug, or treatment, PCTs shall normally make funding available to support the costs associated with the guidance within three months of the guidance being published. The NHS Constitution⁵ says:

You have the right to drugs and treatments that have been recommended by NICE for use in the NHS if your doctor says they are clinically appropriate for you.

NICE TAGS, then, must normally be funded and may not be subject to PCT priority setting.

Note, however, that while NICE Technology Appraisal Guidance is *mandatory* for PCTs, it is *discretionary* with respect to clinicians. The reason is explained by NICE itself, which says:

NICE does not override [clinical] responsibility... even the best clinical guideline is unlikely to be able to accommodate more than 80% of patients for whom it has been developed⁷.

So, health professionals must still decide whether and how NICE guidance applies to a particular patient. As NICE says:

[NICE] guidance that does not recommend a treatment or procedure, or that recommends its use only in defined circumstances, is not the same as a ban on that treatment or procedure being provided by the NHS. If, having considered the guidance, a health professional considers that the treatment or procedure would be the appropriate option in a given case, there is no legal bar on the professional recommending the treatment or on the NHS funding it...

It is not the intention of NICE guidance to replace the clinician's knowledge and skill, rather to support it⁸.

However, this calls for two further comments. First, it would normally be unreasonable to ignore guidance from NICE. It is a well-respected body with an international reputation. If health professionals decide not to adhere to NICE guidance they should have good reasons which explain the clinical logic for doing so.

Second, if a clinician prescribes treatment that has not been recommended by NICE, PCTs are under no automatic duty to fund it. And this brings us to the question of "priority setting outside NICE" which we consider next.

3. Priority setting outside NICE

What about treatment that has not been considered in a NICE TAG? Priority setting systems are crucial here. We have said that priority setting is lawful. Now we'll expand on the reasonable processes that should surround priority setting decisions.

The NHS Constitution⁵ says:

You have the right to expect local decisions on funding of other drugs and treatments to be made rationally following a proper consideration of the evidence. If the local NHS decides not to fund a drug or treatment you and your doctor feel would be right for you, they will explain that decision to you. (*NHS Constitution, Principle 2a*).

And Directions to PCTs⁹ state:

...each PCT must have in place arrangements for making decisions and adopting policies on whether particular healthcare interventions are to be made available for patients for which the PCT is responsible... Each PCT must compile and publish clear written information outlining the arrangements specified....

So, what are these local arrangements and how should they be implemented? Clearly, PCTs need to have processes and systems in place to support all stages of decision making. The Department of Health and National Prescribing Centre has issued guidance for local decision making groups which outlines the context in which groups need to make decisions about medicines and gives some high level principles⁶.

PCTs also need to have a framework for making decisions which promotes consistent decision-making across all treatments and interventions. To assist understanding of these systems we consider the following:

- A. An overall framework for priority setting
- B. Priority setting criteria
- C. Incomplete or ambiguous clinical evidence
- D. Developing and disseminating coherent policies
- E. The need to give a statement of reasons

A. A Framework for Priority Setting

PCTs need to develop a coherent and consistent structure for making decisions about health care priorities. In outline, the concept of priority setting involves three things; (a) general *ethical principles* within which the process will take place (eg equality, fairness and consistency between patients), (b) the particular *criteria* which will be applied to give the principles practical effect (e.g., NICE guidance, cost-effectiveness), and (c) the procedures and operating instructions governing the decision-making process (eg, the composition of the committee, submission of requests and quorate decision-making).

The *ethical principles* and *practical criteria* may be explained in a single document, or separately. There are examples of different frameworks on NPC's sharing practice website. The case study which accompanies this e-learning resource illustrates how one 'ethical framework' has been used to describe how PCTs set priorities; it combines ethical principles and practical decision-making criteria.

Without a framework of this nature, the process of deciding whether to accommodate new treatments could be inconsistent and unfair between patients. However, each PCT is responsible for its own ethical principles, criteria and processes and there is room for differences as to how each system works.

For example, some may use a scoring system (sometimes called "balanced score cards") in which a specific number is put against selected criteria such as "strength of clinical evidence," or "extent of effectiveness for patients" with a final score generated by adding each score together. Bear in mind, however, that these

scores still contain a degree of subjectivity because opinions may differ as to the criteria to be used, the weight each one deserves the score appropriate to individual criteria and therefore, as to the final “score” awarded. Judgment is inherent in the scoring exercise. For this reason, it is preferable not to regard such a system as decisive, but rather as an important step in the exercise of well-informed judgment.

However, with over 100 PCTs if every one developed different criteria there would be unnecessary variation throughout the NHS and further concern for post-code differentials. Therefore, PCTs should collaborate together to harmonise decision-making wherever possible. It makes sense for local PCTs to collaborate in the process so that differences, if they exist, are reduced to a minimum.

B. Priority Setting Criteria

What criteria should be included in the framework and how should they be applied? There is no objective calculator to generate a right answer to this question. Well informed, fair-minded people have to make a judgment based on relevant considerations. PCTs will develop their own criteria, preferably in collaboration with their neighbours, however, a number of considerations are likely to be relevant for everyone, for example:

- Is the treatment supported by a NICE TAG?
- Is the treatment supported by NICE guidelines, or other government policy?
- What is the nature and seriousness of the illness?
- What benefit does the treatment offer? What are the associated risks?
- What is the clinical evidence that it will be effective?
- What is the cost of the treatment by comparison with other treatments for the same condition (if they are available)?
- Will the cost of the treatment have an impact on other care provided by the PCT?

C. Incomplete or ambiguous clinical evidence

What happens when the clinical evidence supporting use of a treatment is incomplete, say because a medicine is new and long-term clinical trials have yet to be completed, its potential cost is difficult to assess, or it is an off-label or unlicensed use? Say, a clinician requests use of a treatment which does not have the benefit of evidence from extensive clinical trials, but which he, or she believes will assist their patient?

The matter must be considered by the PCT according to the policy in its priority setting framework. As the Court of Appeal said in *R v N Lancashire HA, ex p A, D & G* (1999)¹⁰

...the mere fact that a body of medical opinion supports the procedure does not put the health authority under any legal obligation to provide the procedure... However, where such a body of opinion exists, it

is...not open to a rational health authority simply to determine that the procedure has no proven clinical benefit while giving no indication of why it considers that is so.

So responsible clinical opinion in favour of treatment is not decisive, but if the PCT decides that it cannot support it, it must be able to explain why.

Clearly, clinicians work within a “Hippocratic” perspective with duties to *individual* patients, while PCTs allocate finite resources to a *community* of patients. Each may have a different view of the clinical evidence that suggests a treatment will be effective. An accepted hierarchy of clinical evidence which may help local decision making groups is outlined as follows¹¹:

- Well-conducted meta-analysis of several, similar, large, well-designed randomised controlled trials (RCTs)
- Large well-designed RCT
- Meta-analysis of smaller RCTs
- Case-control and cohort studies
- Case reports and case series
- Consensus from expert panels
- Individual opinion

As treatments become more expensive, so does the need for evidence that they will be effective. This is necessary to ensure that proper sums are invested in treatments which are likely to work.

D. Developing and disseminating coherent commissioning policies

Good practice suggests that commissioning policies should be developed as rapidly as possible (bearing in mind that some decisions may have to be provisional pending the publication of new clinical evidence, or guidance from NICE). PCTs should have a process to revisit policies to ensure they are still valid in the light of current developments. This requires PCTs to develop the capacity to make well-informed decisions about the commissioning of new treatments. Again, PCTs will benefit from collaboration. There is little point in a number of PCTs all reviewing the evidence base for exactly the same treatments.

The development and application of a decision-making framework, should be supported and administered by a standing committee governed by procedural rules, for example with respect to membership, declarations of interest, voting rights, and quorate decision-making. It is crucial that its members include a representative cross-section of clinical and managerial interests

We should also be clear about the authority of the recommendations made by priorities or other advisory groups. Normally, these groups “recommend” policies for adoption by the PCT board because they have no statutory identity of their own. These committees should understand the limits of their authority and, especially, whether their role is *advisory* to the PCT.

Arrangements also need to be made to disseminate the policies to clinicians in primary and secondary care. This requires clear channels of communication between the PCT, GPs and hospitals. The system depends on trust and confidence between clinicians and PCT managers and reinforces the need to have good clinical representation on all local decision-making groups.

All these points are covered in more detail in the DH guiding principles⁶, and NPC's good practice Handbook¹¹.

E. Statement of Reasons

If the treatment is not commissioned, or an individual funding request is declined, the PCT must explain why this is so. This requirement has also been included in the 2009 Directions to PCT and NHS trusts⁹.

Where a PCT makes a decision to refuse a request for the funding of a healthcare intervention, where the PCT's general policy is not to fund that intervention, the PCT must provide that individual with a written statement of its reasons for that decision.

The statement should describe the PCT's *general* priority setting framework, the *particular* policy on the treatment in question and the basis on which it was made, and explain how this affects the *individual* circumstances of the patient's case.

4. Exceptional clinical circumstances for treatments not routinely commissioned

Priority setting is difficult because PCTs have a duty to balance the needs of the community with those of individuals. Procedures on exceptional circumstances recognise the interests of individuals in this difficult balancing exercise.

This means that where a PCT does not routinely commission a treatment because it is a low priority, it should allow for the possibility that an individual may have exceptional circumstances which justify access to the treatment which is not generally available. It must focus on the individual to test whether they should be given exceptional access. As the Court of Appeal said in *R v N Lancashire HA, ex p A, D & G* (1999)¹⁰

... the more important the interests of the citizen that the decision affects, the greater will be the degree of consideration that is required of the decision-maker. A decision that... seriously affects the citizen's health will require substantial consideration, and be subject to careful scrutiny by the court as to its rationality.

Similarly, the *NHS Constitution Handbook*¹² says:

...a PCT can have a policy not to fund a particular treatment (unless recommended by NICE in a TA), [but] it cannot have a blanket policy, ie it must consider exceptional individual cases where funding should be provided.

The usual purpose of this principle is to ask whether the treatment is likely to be significantly more effective in *this* particular patient than in other patients in similar circumstances. Their clinical circumstances are so unusual that they are likely to benefit much more from this treatment than other patients with the same condition.

This is also important for treatments to which patients are known to be intolerant. If patients are likely to develop intolerance to Treatment A so that it is no longer available to them, what is the PCT's policy with respect to the alternative Treatment B?

To establish this will normally require the advice of clinical specialists in the field, clinical evidence of the likely benefit (and risks) of the treatment and evidence of its cost (and cost-effectiveness). PCTs must still consider the cost of the treatment because there is no duty to accommodate exceptional treatment irrespective of its cost.

Evidence of "exceptionality" should be considered in the round, rather than in isolation, so that an accumulation of different factors may satisfy the test.

However, PCTs should be careful to explain that very few patients will be considered to be exceptional. It is impossible to be precise as to the magnitude of the number of patients required to justify the term 'exceptional'.

But another way of responding to the problem of "numbers" is to ask when a new PCT policy is needed. If more than a small handful of patients present with similar claims to being "exceptional," the PCT should quickly respond by introducing a new *policy* to deal with the new treatment, so that the group is dealt with consistently. To fail to have proper policies in place, and to respond as if larger numbers of patients were exceptional, and therefore, fell *outside* the normal priority setting framework, would be inconsistent and unfair.

Therefore, even when the numbers of patients likely to apply for the treatment is very small, and especially if the costs of the treatment are large, procedures should exist to enable PCT priority committees to respond to these circumstances rapidly and with appropriate policies.

Exceptional clinical circumstances are considered in more detail in our presentation on Judicial Review.

Are *personal* circumstances likely to constitute exceptional circumstances? For example, what if the mother of young children suffers terminal cancer for which usual treatment has not been effective. However, another

treatment is available for which there is incomplete evidence that it may prolong a patient's life by three months. There is a request for funding in the interests of the patient's young children. However, it is very expensive and the PCT does not normally fund it in the interests of the needs of other patients. What about a request for pinnaplasty for a child who is being bullied at school because of his prominent ears? Or an elderly carer who is looking after his frail wife and, without a treatment not routinely funded, both will have to go into care?

These patients are not exceptional in the sense that they would gain greater *clinical* benefit from care. The question is whether their social circumstances may ever be considered exceptional in the sense that they provide additional time to settle children, or integrate at school, or remain independent at home?

Some cases have suggested that personal circumstances, if the PCT included them the factors that may be considered, may make someone's circumstances exceptional. However, the point has never been fully argued and the argument on the other side is significant because such a policy may discriminate against patients who are not carers and who would be excluded from this category. If such a policy could include parents, could it also favour those in work over the unemployed? This is an important consideration, but the weight of this counter-argument has not yet been considered by the courts so the point is still unsettled in law.

This question is also considered in more detail in our Judicial Review presentation.

Guidance from the DH and NPC recommends that treatments for individual funding which are not approved may be brought back to the PCT as an appeal⁶

PCTs should establish an appeals process for decisions made on individual funding requests, including clearly defined grounds of appeal, independent of the original process and open to patients and the public

A comparable right of appeal is available against decisions made by NICE and these are that it:

- failed to act fairly and in accordance with its procedures;
- came to a decision which is perverse in light of the evidence submitted;
- exceeded its legal powers.

The grounds of appeal against a PCT decision to refuse an individual funding request could be set out in the same way. The process should be conducted in a way that is independent of the original process. Over time, the appeal process is likely to evolve in the light of advice and guidance of the courts.

Conclusions

The NHS Constitution clearly gives patients the right to expect PCTs to make rational local decisions about the funding of medicines and treatments. At the same time, PCTs have a legal duty to stay within budget and to commission care as effectively as possible for their populations.

PCTs can decide not to routinely commission treatments for their populations, however, they must have robust and transparent processes in place to enable this to happen. “Robust” means that the PCT must understand why the decision may not be universally popular, but can explain the reasons for it, and the fair and reasonable procedures used to come to its conclusion.

Where a decision is taken not to fund a treatment, PCTs must have a process which allows for the possibility that an individual may have exceptional circumstances which justify access to the treatment which is not available to the rest of the population.

Where practicable PCTs should collaborate to avoid duplication of effort and unnecessary differences in decision-making based on different processes.

[End]

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