

SOCIAL VALUE JUDGEMENTS: PRINCIPLES FOR THE DEVELOPMENT OF NICE GUIDANCE

Response from the Specialised Healthcare Alliance

The unboxed text briefly describes relevant parts of NICE's consultation document. The boxed text sets out the SHCA's comments in response.

Chapter One – Introduction

The document describes the principles NICE should follow in designing the processes it uses to develop its guidance. It is mainly about the judgements that NICE and advisory bodies should apply.

The Specialised Healthcare Alliance (SHCA) is a broad coalition of patient groups supported by a smaller number of corporate members. It was established in 2003 to campaign on behalf of people with conditions which require specialised medical care. These conditions tend to be rarer and both complex and expensive to treat. Examples are numerous but include certain cancers, cystic fibrosis, haemophilia, HIV and neurological conditions; Alliance members include organisations representing patients with these and many other conditions.

We welcome this chance to participate in an important consultation about the principles behind NICE's work. We recognise the complexity of the issues raised and hope our input can help NICE to develop its method for reaching decisions and supporting their implementation.

Chapter Two – Principles of bioethics

This chapter gives a very short description of the "four widely accepted principles" that apply when moral issues arise in healthcare: Respect for autonomy, non-maleficence, beneficence, and justice. 'Justice' is the least clear: the document defines it as "providing services in a fair and appropriate manner". In discussing distributive justice, the document distinguishes between utilitarianism and egalitarianism but seeing the two as irreconcilable sets out the main components of procedural justice as a basis for "reasonableness".

Procedural justice is a bureaucratic construct which should only be employed within parameters established by Parliament. The Alliance considers that striking the balance between the needs of society and the individual in allocating limited resources is a function of democratic accountability in a taxpayer funded system.

Chapter Three – Fundamental operating principles

This chapter refers to the legislation and regulation that define NICE's remit – the Establishment Order and Secretary of State's Directions. It lists the procedural principles NICE operates by, e.g. rigour, transparency, independence, challenge, support for implementation.

Cost effectiveness

The Secretary of State's Directions to NICE set out the various factors that NICE's appraisals should account for, covering costs and benefits, clinical need, the Secretary of State's guidance, and the benefits of innovation. Each of these is entirely appropriate. There is a legitimate concern, however, that appraisals have centred too heavily on cost effectiveness concerns, with other factors marginalised. This is perhaps shown by the primacy given to discussion of the QALY measure and ICER in the consultation document.

The range of factors

The Alliance believes each of the four factors listed are vitally important, and the Institute's approach should reflect this. The factor we believe receives least attention is the potential of innovation to provide extended benefits in the longer term. This was a point raised by the Citizens Council when discussing the Rule of Rescue: "If approving the technology aids further research into that disease, present costs might be partially offset against potential future benefits." At present there does not seem to be a clear way of capturing this potential in the appraisal process. One option would be to give greater weight to the opinion of clinical experts in the particular field, with NICE making a judgement partly based on how confident experts are about the potential for further innovation.

Few would argue that cost effectiveness continues to be the major concern during an appraisal. But the three other factors listed in the Directions should be incorporated in a systematic way. This could mean that the presumed ICER threshold is raised if the clinical need caused by a particular condition is severe, or an intervention will help achieve other goals drawn to the attention of the Institute by the Secretary of State (for instance, early intervention).

Broader benefits

The Directions are also clear that costs and benefits are to be determined broadly. The Alliance is concerned that appraisals have not accounted for the full range of benefits that a treatment may provide. The clearest example is the impact on a patient's carer(s); NICE does account for this, but perhaps without giving it appropriate weight in the appraisal process. Quite apart from costs, disabling conditions will affect the quality of life of carers and this needs to be taken into account. It would seem from the recent Health Select Committee inquiry that NICE is sympathetic to this view but considers that Parliamentary approval is required. If so, this should be sought as a matter of urgency.

Chapter Four – Evidence-based decision-making

This chapter describes how NICE reaches decisions on the evidence, setting out a set of five principles. It mentions that drugs for 'rare' conditions are treated in the same way as for other conditions; it does not give a position on 'very rare' conditions. The idea of a 'rule of rescue' is seen as unnecessary alongside the other principles enumerated.

Use of evidence

Clearly, NICE needs to base its decisions on the available evidence during an appraisal. However, in our view, there should be consideration of the difficulties of obtaining evidence in relation to some interventions. For rarer conditions, patient numbers may be small, while other conditions do not lend themselves easily to the use of randomised, controlled trials, often for ethical reasons. The Alliance is

aware that NICE has shown a degree of flexibility in the past but would see merit in a more consistent approach. The Alliance therefore sees merit in 'Principle 1' being recast to allow for more flexibility in approving treatments where there are significant barriers to research.

Assessing cost effectiveness

Principle 3 states that decisions should not be based on evidence of their relative costs and benefits alone but gives no details of other factors beyond the need to distribute health resources in the fairest way within society as a whole. In the Alliance's view, the need to take account of the severity of the condition and the potential value of the innovation should be reiterated at this point.

Rare and very rare conditions

The SHCA questions whether it is appropriate to assess drugs and other treatments for rare conditions in the same way as the generality of interventions. The EU's orphan drugs policy, to which the UK is a party, provides incentives for the development of such products to meet areas of unmet need. The costs of development relative to patient numbers are high. The application of normal cost per QALY thresholds therefore risks thwarting access to products whose development is being encouraged elsewhere. This is in addition to the generally severe nature of such conditions and the tendency of much innovation to spring from specialised areas.

Rule of rescue

The consultation document introduces the 'rule of rescue' but states that the Institute sees no need to adopt an associated rule. The Alliance is aware of the Citizens Council discussion on this matter but considers that the debate may have focused unduly on the 'life-saving' properties of treatment and insufficiently on broader 'quality of life' issues.

In the Alliance's view, these considerations cannot be addressed simply through the application of health economics but have an essentially political character. Pooling risk is at the heart of the NHS, and NICE's Citizens' Council on ultra orphan drugs suggested that most people favour treatments being made available to those unfortunate enough to suffer from very rare diseases drawing on this particular strength.

Chapter Five – Responding to comments and criticism

This chapter sets out the rationale for a sixth principle concerning the way in which NICE should respond to criticism but while giving primacy to its judgement in relation to cost effectiveness and the fairest distribution of resources.

It is important that NICE should avoid substituting procedural considerations for the exercise of genuine judgement in response to valid comments and criticisms. Such judgement should not be restricted to cost effectiveness but encompass the totality of the Secretary of State's directions, including the degree of clinical need and the impact on innovation.

Chapter Six – Avoiding discrimination and promoting equality

This chapter sets out the circumstances in which NICE might recommend the restriction of an intervention to a particular group of people and enshrines this in a seventh principle.

Discrimination may occur not only in circumstances where treatment is restricted to a particular group but also where it is denied a particular group. EU policy to which the UK is party is predicated on people with rare diseases having the right to equal diagnosis, prevention and treatment as other patients. In a cash-constrained environment this right cannot be absolute but should be reflected in NICE's understanding of discrimination and promotion of equality.