

Improving access to new drugs: a plan to renew The National Institute for Health and Clinical Excellence (NICE).

Preface by Mark Simmonds.

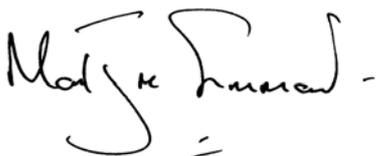
The National Health Service (NHS) is the Conservative Party's number one priority. We share the values of the NHS and are committed to the principle that access is based on need and not ability to pay. However, equity is not enough; we also aspire to excellence. We have committed ourselves to providing the NHS with the resources and the improvements necessary to deliver standards of healthcare among the best in the world. We believe that the best way to improve care is to put patients at the heart of the service and give professionals the freedom to focus on what they do best – making people better.

Conservatives are committed to ensuring that NHS funds are spent optimally. I have seen first hand a National Institute for Health and Clinical Excellence (NICE) Appraisal committee, and the rigorous scrutiny that is given to new drugs and treatments. We believe the Institute has an important long-term role in assessing the clinical efficacy and cost-effectiveness of new treatments, and safeguarding taxpayers' money. However, we recognise there are areas in which its configuration, operational structure and efficiency can be improved. I have set out below the key areas in which we have proposed these improvements.

In order to do this, we want to free the NHS from top-down targets and focus instead on patient outcomes. Conservatives aim to achieve outcomes for illnesses such as heart disease, cancer and stroke to match or exceed those of our European counterparts. Improving access to potentially life-saving treatments and medicines is a key element of achieving this aim, and is a priority for the Conservative Party.

We want to ensure that all patients receive the healthcare they need when it is needed. This means making the best of available NHS resources and ensuring that the care is both clinically-effective and cost-effective. NICE plays an essential role in advising NHS bodies (and the wider healthcare sector) on the effectiveness of treatments and healthcare pathways. In this document, we reaffirm our commitment to NICE and outline proposals for how the Institute can be strengthened and improved, within the context of our broader plan for securing improvements for the NHS.

I believe that this document sets out proposals for building on the achievements of NICE and developing the decision-making framework for improving patient access to potentially life-saving treatments. We believe the proposals contained within this document will bring clarity and improvements to the important work of NICE; enhance the allocation of NHS resources; enhance taxpayer value for money; and improve patient outcomes.



Mark Simmonds MP
Shadow Minister for Health

Executive Summary

Section 1 considers the role of NICE as it currently is and how we believe it can be clarified and strengthened. Conservatives have supported NICE since its inception and believe it is right that assessments of the relative benefits of treatments should be evidence-based and made by clinicians rather than politicians. However, we would like to see this role more clearly defined and strengthened, and have therefore proposed:

- To give NICE a statutory role;
- To introduce a “NICE Charter” to codify its roles and responsibilities;
- To simplify and clarify NICE terminology; and,
- To increase public involvement in the Institute’s work.

Together these proposals will make for a stronger NICE, with a more clearly defined role allowing for more easily communicable responsibilities and greater public involvement.

Section 2 considers the interaction between NICE and the pharmaceutical industry.

There have long been tensions between the two, particularly over expensive new treatments. The Conservative Party is supportive of the pharmaceutical industry, the benefits brought to patients by its research and development (R&D), and the investment and jobs it brings to the UK. However, we believe there needs to be greater cooperation and dialogue between the pharmaceutical industry and NICE. We have therefore proposed:

- To promote a policy of cooperation between NICE and the pharmaceutical industry;
- Ensure industry has a more active role in discussions pertaining to Health Technology Assessment (HTA) and NICE Appraisal; and,
- To set up a steering committee comprising the pharmaceutical industry and NICE representatives; and,
- To take responsibility for assessing vaccines and immunisation programmes.

Encouraging earlier dialogue between the industry and NICE should promote understanding, and help us move away from an adversarial relationship.

Section 3 considers the NICE process. This is the area for which NICE receives the most criticism for being slow and overly bureaucratic, with some evaluations having taken years. We have already pledged to speed up the evaluation and appraisal process by allowing NICE appraisal to start as the Medicines and Healthcare products Regulatory Agency (MHRA) licensing process begins, but there is more that can be done to enhance and improve the process. We have therefore proposed:

- To end the system of Ministerial referral and enable NICE, in conjunction with the NHS Board, to establish its own evaluative programme;
- To allow NICE appraisals to begin as Phase III clinical trials end as soon as a product is granted a licence. To shift the burden of proof from NICE to the manufacturer;
- To move appeals to a fully independent panel that is separate from NICE; and,
- To encourage post-appraisal benchmarking.

Together these proposals will reduce time delays and bureaucracy, while making the NICE process more transparent, inclusive and, most importantly, faster. This in turn should ensure that clinically efficacious and cost-effective treatments reach the patients more rapidly and reduce the “postcode lottery”.

Section 4 considers the affordability and cost effectiveness of new drugs and treatments. NICE’s Quality Adjusted Life Year (QALY) threshold is known to be between £20,000 and £30,000 per year. Yet until recently there has never been any Parliamentary or public discussion of whether this is an appropriate definition of cost-effectiveness. There is no need or defensible rationale for a single threshold. The measure of cost-effectiveness will differ relative to the nature of a drug (especially orphan drugs) and its associated efficacy. The job of NICE is to give advice and **not** make decisions about treatment. We have therefore proposed:

- To transfer responsibility for “recommending” or “not recommending” medicines to the NHS Board.
- To ensure that measures, and implied thresholds for cost-effectiveness, are periodically reviewed by the NHS Board.
- To consider the introduction of disease specific measures of cost-effectiveness; and
- To enable appraisal of the value or benefit of a treatment to include its wider social value.

Such proposals would put the QALY methodology in context and allow for greater public involvement and understanding in determining the challenging affordability and cost-effectiveness judgments that have to be made.

Section 5 considers the implementation of NICE guidance. There is often disparity in the speed and comprehensiveness of the implementation of NICE guidelines, which leads to concerns over the “postcode lottery”. We have no plans to make NICE guidelines mandatory, unlike NICE technology appraisals. However, we believe that our focus on outcomes will incentivise NHS bodies to implement guidance swiftly in order to achieve good outcomes for their patients. We have therefore proposed:

- To use our focus on outcomes to encourage the full, consistent and accurate implementation of NICE guidance across the NHS;
- To extend the requirement for the implementation of technology appraisals within three months to certain mandatory aspects of clinical guidelines, which relate specifically to the achievement of outcomes and clearly cost-effective care pathways;
- To extend “commissioning guidelines” building on the commissioning guides currently used by NICE; and,
- To treat implementation of NICE guidance as a key performance indicator for commissioning bodies.

These proposals will have a noticeable impact on ensuring guidance is swiftly adopted, which in turn will improve patient outcomes and reduce the “postcode lottery”.

Section 6 considers NHS drug availability. All health systems must confront the challenge of how best to allocate finite resources as innovative and expensive treatments are developed. We have therefore proposed:

- To remove ministerial and political interference in NICE decision making;
- To engage the public in decisions regarding NHS priority setting and resource allocation;
- To apply progressively the principles of value-based pricing (VBP) to new medicines; and,
- To support the use of risk-sharing schemes to enable early uptake of new medicines which lack cost-effectiveness data.

These proposals, particularly regarding VBP and risk-sharing, are innovative and will have a large impact on ensuring the efficient allocation of NHS resources while ensuring access to life-saving drugs and treatments to improve patient outcomes.

This paper demonstrates our commitment to NICE by enhancing and strengthening the Institute, while reaffirming its independence. We want to build on the achievements of NICE and this policy document provides a blueprint for improving the process for deciding which drugs and treatments should be provided by the NHS. Our proposals are designed to foster NHS priority setting; optimise decisions regarding resource allocation; improve patient outcomes; and eliminate the continued existence of the “postcode lottery”. Bringing about these changes is critical to the efficient and effective delivery of responsive healthcare, and enhancing the overall accountability of the NHS to patients.

Introduction

NICE is the NHS body responsible for making recommendations about the introduction of drug treatments and medical devices into the NHS. Originally founded in 1999 and then merged with the Health Development Agency (HDA) in 2005, the Institute's key functions are to appraise the clinical benefits and costs of healthcare interventions, and to develop clinical guidelines for providing advice on good healthcare practice. NICE plays a central role in formulating, maintaining and disseminating a strong evidence-base for effective public health action and the reduction of health inequalities. Its values and responsibilities are an essential component of the NHS, and are fully recognised and supported by the Conservative Party.

Spiralling healthcare costs, rising expectations, an ageing population and tighter budget constraints have impelled modern governments to place additional emphasis on allocating available resources efficiently and effectively.^{1,2} The purchasing and pricing of pharmaceutical drugs is one such important area. Approximately 10% of the entire NHS budget – roughly £11 billion per year – is spent on drugs and medicines. Of this expenditure, close to £8 billion is spent on branded products alone.^{3,4} It is from this premise that decisions regarding NHS drug availability are vital.

Health technology assessment (HTA) is increasingly employed by countries as a tool to more effectively control the diffusion and utilisation of health technologies.⁵ NICE is often seen as the most sophisticated national attempt to systematically review the value of different treatments.⁶ However, the environment in which NICE operates has changed considerably since the Institute was established in 1999. This is largely the result of a shifting healthcare landscape and a dramatic increase in NHS spending.

Under the broad rubric of developing guidance on health technologies and interventions, NICE should play a central role in:

- 1) identifying new treatments that offer the NHS the best value for money;
- 2) enabling evidence of clinical and cost-effectiveness to inform these value judgments;
- 3) supporting and advancing health care innovation;
- 4) promoting the most effective care pathways for the benefit of NHS commissioners; and,
- 5) advising Government and the NHS on the effectiveness of public health interventions.⁷

The key issues surrounding the use of economic evaluation in healthcare decision-making are some of the most important within the NHS. Supporting, improving and furthering the work of NICE is central to addressing concerns regarding NHS priority-setting; the allocation of resources; and swift and efficient patient access to drugs and medicines.

Despite often being cited as the pre-eminent body dedicated to HTA, NICE's processes and procedures have not been without controversy. NHS financial constraints and ongoing debates surrounding the prescribing of costly medications, such as Herceptin (breast cancer) and Lucentis (ophthalmology), have prompted the need to review the processes by which new drugs are made available to patients across the UK. The implications of balancing trade-offs between health care costs and patient benefits have rightly invoked much discussion, and formed the basis of the recent Health Select Committee (HSC) report.⁸

Founded to counter geographical variations in health care and to control costs, NICE is the watchdog responsible for recommending which drugs and treatments should be provided by the NHS. The Labour Government introduced the agency promising: “Its evidence-based guidelines will be used right across the country, so NICE will help end the unacceptable geographical variations in care that have grown up in recent years”.⁹

Ever since, NICE has attracted criticism and been a source of media controversy. Particular attention has been paid to the appraisal process for reaching decisions, raising key questions on NICE’s assessment of cost-effectiveness; the efficiency of current procedures; and the implementation of guidance. Addressing these concerns is essential for focused policy-making.

1. Defining the Role of NICE

Overview

We are supportive of NICE, as we believe it is right that decisions about the effectiveness of treatments are made by experts and clinicians rather than by politicians. However, there are ways in which NICE's role and responsibilities can be strengthened and their public engagement widened. We would therefore seek to clarify and enhance the role of NICE through the following proposals:

- Give NICE a statutory basis, working in collaboration with the NHS Board.
- Introduce a "NICE Charter" defining the Institute's exact roles and responsibilities.
- Clarify NICE terminology regarding different types of guidance to draw a clearer distinction between mandatory and non-mandatory decisions.
- Increase public involvement in the work of NICE by considering ways to improve and enhance the work of the Citizens Council.

NICE is the organisation within the NHS responsible for providing national guidance on the promotion of good health and the prevention and treatment of ill health. Acting as a special health authority, NICE's remit is to evaluate both clinical and cost-effectiveness as part of appraisal decisions. Its guidance essentially confers a quasi-legal function directing clinical practice, instituting national standards, and informing funding decisions on new treatments.¹⁰

In general, NICE guidance is advisory, meaning decisions regarding its adoption and implementation are left to the discretion of local healthcare bodies. As of January 2005, technology appraisals are mandatory.¹¹ NHS providers in England and Wales are now legally obliged to provide funding for medicines and treatment recommended by NICE within three months of the date of guidance being issued.

The range of NICE recommendations differs according to each type of guidance. Public health guidance applies solely to England. Health technological and interventional procedures guidance covers England, Wales and Scotland. Clinical guidelines concern England and Wales only.

As the recent HSC report noted, there is a worrying lack of public confidence in NICE. Although not helped by media-related opprobrium, recurring criticisms are arguably a consequence of poor communication regarding NICE's *raison d'être* and indistinct clarity over its appraisal methods. To address these concerns, it is important to clearly define the reasons for NICE's existence.¹² This essentially requires better communication of the Institute's roles and responsibilities.

1.1. Statutory Role

We believe that the work of NICE transcends that of a special health authority. As outlined in *'NHS Autonomy and Accountability'*, we pledge to put NICE on a statutory basis.¹³ It will be subject to legislative requirements relating to the level of patient, public and interest group engagement, which it must demonstrate when reaching decisions.

In line with our proposals to create an independent NHS Board responsible for the day-to-day running of the healthcare system¹⁴, the Institute will operate through service-level agreements with the NHS Board, the Department of Health, and national and international healthcare providers. NICE will specifically support the NHS Board by providing evidence-based commissioning guidelines to drive development of effective commissioning on a consistent and uniform basis across the NHS. NICE will continue to conduct appraisals of new drugs and technologies and to encourage the adoption of the most clinically and cost-effective treatments. It will also have a continued responsibility to produce public health guidelines and, as outlined in *NHS Autonomy and Accountability*, it will have a duty to conduct evaluations of public health services and interventions on behalf of the Chief Medical Officer and the Secretary of State.¹⁵

1.2. A “NICE Charter”

We believe it is important for NICE's role to be clearly and openly defined. It is also important that NICE improves communication with interested bodies, such as the pharmaceutical industry, the public and patient groups (see section 2). To this extent, we propose a “NICE Charter”. The Charter will outline the Institute's purpose, role and wider responsibilities, making it clear that NICE is an advisory body which provides expert evaluation to inform important judgements on NHS drug availability. This will provide a reference point and provide a platform for better communication.

1.3. Assessing Vaccines

At present, the remit of NICE does not extend to the assessment of vaccines as treatment options, or their evaluation as public health measures. This is reserved for the Joint Committee on Vaccination and Immunisation (JCVI). While there is undoubted expertise in JCVI and its committees, it is essential for the future that vaccines and immunisation programmes form part of a consistent process of evaluation and advice to Ministers and NHS commissioners. For this to be the case, the evaluation of vaccines and immunisation programmes must be added to the NICE remit, with a corresponding transfer from the Department of Health (DoH). This will also ensure greater transparency in the evaluation of vaccines.

1.4. Increasing Public Involvement

Public expectations in the NHS are rising, not least given the unprecedented commitment from the taxpayer. Ensuring adequate public and patient involvement in all aspects of decision-making is essential to increasing confidence in the NHS.

NICE has established a Citizens Council, which is designed to bring the views of the public to decision-making. Comprising a group of 30 people independently drawn from wider society, the Council is charged with tackling questions about value judgments such as equity and need, and providing input on NICE guidance.¹⁶

In some quarters, the Citizens Council has attracted criticism having been described as a “token gesture” and “toothless tiger”.¹⁷ It is important that this body is seen to be meaningful and substantive.

We will therefore examine how the work of the Citizens Council can be extended, particularly on issues such as valuing a QALY, alternative methodologies for ascribing value to clinical benefits – including for orphan drugs, life-extending medicines, or for medicines which have limited incremental effects but which cost very little – and how to include a calculation of the wider social value of a given treatment when undertaking NICE appraisals. More generally, we will look at ways to increase public involvement and consultation in the work of NICE. This is central to determining the valuation society places on health as well as increasing overall representation and improving patient outcomes.

2. Improving Dialogue with the Pharmaceutical Industry

Overview

The Conservative Party fully recognises and values the strategic importance of the pharmaceutical industry to the UK. One in ten of the leading medicines in use originate in British science and the pharmaceutical industry. It is responsible for a quarter of all R&D activity in the UK^{18, 19}. Furthermore, at over £4bn, pharmaceutical manufacturing is responsible for the single largest sectoral contribution to our current balance of payments.¹⁹

We recognise that in the past there have been difficulties in the relationship between NICE and the pharmaceutical industry, particularly when medicines are deemed not cost-effective for use on the NHS. It has been too easy for NICE and the industry to blame one another for delays in the appraisal process, and this must be prevented.

We would therefore seek to further enhance and improve the relationship and dialogue between the pharmaceutical industry and NICE through the following proposals:

- Promote a policy of cooperation between NICE and industry.**
- Ensure industry has a more active role in discussions pertaining to HTA and NICE appraisal.**
- Set up a steering committee comprising representatives from the pharmaceutical industry and NICE.**

As a key player in the wider NHS, we recognise the importance of directly involving pharmaceutical companies in the NICE process. This is not simply the case as the manufacturers of given medicines and treatments, but also as the purveyor of new ideas and strategic insight to aid ongoing consultations and discussion. The next Conservative government will work closely with industry to advance R&D, foster business opportunities, and create an economic environment of dynamic incentives to encourage pharmaceutical investment in the UK.

We recognise that drugs account for approximately 10% of overall NHS spending, but they must not be treated as a “soft target” when trying to reduce NHS expenditure; medicines are often life-saving and have a pivotal role to play in advancing preventative health care and thereby delivering significant cost-savings. To try to “control the drugs budget” falls prey to the fallacy that there is an autonomous drugs budget, when in reality there is a “health budget” of which expenditure on medicines will be a variable part depending on its relative clinical benefit. It is therefore vital that there is a consistent evaluation methodology extending across all treatments.

To this extent, we encourage a policy of cooperation between NICE and the pharmaceutical industry. It is our belief that there is room for improving dialogue in order to maximise outcomes for all concerned, but most importantly for patients.

2.1. DoH + Industry = Best Outcomes for Patients.

Critics have justifiably pointed to the “us versus them” nature of the NICE process and thereby the scope for defensive appraisal. This is most noteworthy for those controversial submissions attracting extensive media coverage. We believe it is imperative that HTA assessment and, in particular, “jumping the fourth hurdle” is seen less as a game and more as a cooperative process delivering evidence-based outcomes for both the NHS and industry.

We therefore recommend the creation of steering committee comprising representatives from the Association of the British Pharmaceutical Industry (ABPI), Department of Health (DoH) and NICE. The committee could meet on a periodic basis to discuss issues pertaining to HTA and facilitate a forum for dynamic and ongoing communication. This would provide opportunities for improved cooperation and an educating mechanism for the requirements associated with NICE appraisal.

2.2. Industrial Policy

We recognise the fundamental importance of industrial policy for providing dynamic incentives to the pharmaceutical industry. This is particularly the case for encouraging research and development and rewarding innovation. The Conservative Party is committed to developing an industrial policy to incentivise and foster the significant contribution of the pharmaceutical industry to the UK economy.

3. “Winning the Race Against Time”: The NICE Process

Overview

The evaluation and appraisal process of NICE has been regularly criticised for being slow and bureaucratic. Decisions as to whether drugs and treatments should be routinely prescribed on the NHS have taken many years causing frustration and distress for patients and their families, as well as confusion and inequality for GPs and Primary Care Trusts (PCTs).

Delayed and drawn-out appraisals of cancer drugs, most notably Herceptin, have proven to be particularly contentious. Some argue that NICE has become bogged down in administrative practices, leading to the so-called ‘NICE blight’.²⁰ Manufacturers, clinicians and patient groups have highlighted problems relating to the delay in access to treatment caused by the relatively long period between licensing and the publication of NICE guidance.

Speeding up the evaluation and appraisal process would play a large part in helping us fulfil our overall objective of ensuring clinically efficacious and cost-effective drugs reach patients faster. It could also assist in changing the public’s perception of NICE from an inhibitor to a facilitator.

We have already pledged to speed up the process by allowing the NICE Appraisal process to begin as the MHRA licensing process begins. We are pleased the Government has adopted this proposal, which will go some way towards shortening the time taken by NICE to produce guidance on new medicines and treatments.

However, there is more that can be done and we would therefore seek to further enhance and improve the evaluation and appraisal process through the following proposals:

- End the system of Ministerial referral for NICE appraisal and transfer the responsibility to NICE in conjunction with the NHS Board.
- Allow NICE appraisals to begin as soon as Phase III clinical trials end and when the licencing process commences by encouraging consultation on the design of trials and studies to better inform pharmaeconomic evaluation.
- Encourage the appraisal of all new active substances and technologies, but accept that new formulations or “me too” innovators do not routinely need NICE evaluation.
- Encourage greater patient, public and interested body involvement throughout the appraisal process.
- In line with other international authorities, shift the burden of proof from NICE to the manufacturer.
- Clearly outline the requirements for submission extending the use of subgroup analysis and restricted guidance where appropriate.

- **Use Single Technology Appraisal (STA) as an evaluative screen during the period between at licensing and product launch adjusting the measure of cost-effectiveness as appropriate.**
- **Use Multi Technology Appraisal (MTA) for more complete appraisal for those technologies where product value is most uncertain.**
- **Transfer appeals to a fully independent panel which is separate from NICE.**
- **Encourage post-appraisal benchmarking to track existing treatments and thereby foster targeted disinvestment.**

3.1. The Return of the “West Lothian Question”

Concerns over the length of time it takes NICE to undertake a review of a new drug or treatment have prompted inevitable comparisons with NICE’s Scottish counterpart, the Scottish Medicines Consortium (SMC), established in 2001. Despite doing broadly the same job, the channels for issuing guidance markedly differ between the two agencies. NICE performs an exhaustive, time-consuming and evidence-based review of each drug, while the SMC makes decisions faster but at the expense of detailed research. These differences in process mean the SMC typically issues guidance within 12 to 16 weeks compared with an average of 18 months for NICE depending on appeals.^{8,21} In the extreme case of the bone marrow cancer drug Velcade, the SMC issued guidance four years ahead of NICE.

In terms of potentially life-saving treatments, the “race against time” is crucial. In a cross-comparative study, the patient group Cancerbackup found that of 23 products pending NICE appraisal in late 2005, 16 had already been reviewed in Scotland. For the remaining seven products, guidance from the SMC was expected some 21 months before NICE.²²

The SMC has also evaluated its performance against NICE. Comparing 18 products reviewed by both agencies from 1999 up to late 2005, the SMC found that the decisions for 17 of these products were roughly the same. Despite this, the SMC’s guidance was issued some 10 months earlier than NICE.^{21, 23}

This efficiency differential has prompted accusations of “asymmetrical health care” and the emergence of a type of “West Lothian question” in the NHS as Scottish patients have quicker access to new medicines. In addition, some consider the SMC process to be more open and collaborative.^{23, 24}

In defence of NICE, the extra time stems from more rigorous and comprehensive appraisal – often multiple technology appraisals (MTAs) – that uses greater public and expert consultation. Indeed, some consider this to be a shortcoming of the SMC process, which is less systematic. Furthermore, the SMC only appraises new medicines or new applications of existing treatments, and is responsible for a different sub-population of the UK.

While direct comparison between NICE and the SMC is not entirely fair, similarities in guidance raise questions of whether the NICE approach is value for money, especially at a cost of roughly £80,000 per appraisal.²⁵ There is certainly an argument for a closer review of practices in Scotland to consider whether some elements of the SMC practice can be incorporated into the NICE process.

3.2. Topic Selection and Ministerial Referral

An important part of the NICE process is topic selection. This concerns the system for deciding which new treatments and indications are selected for NICE review.

Currently, for any new drug or treatment to be appraised by NICE it has to be referred by a Minister in the DoH. This can result in a delay, sometimes up to a year, in new drugs or treatment beginning the NICE process. We therefore propose to remove this delay by transferring the responsibility for referral to NICE from Ministers. Not all new drugs and treatments are required to undertake the NICE appraisals process. It will be up to NICE in consultation with the NHS Board to select its own priorities for appraisal through horizon-scanning and using the expertise of the Horizon Scanning Centre at the University of Birmingham.

Importantly, NICE does not evaluate every new medicine which enters the market or every new indication for a given medicine. Rather, technology appraisal is limited to a select number of new and often expensive products, meaning most medicines and technologies prescribed across the NHS have not been evaluated by NICE. This therefore means that NICE-appraised treatments only constitute a relatively small percentage of PCT work.²⁶ It has also prompted concerns about NHS spending being skewed towards new and expensive medicines for acute illness in secondary care.²⁷

We believe it important that topic selection is focused around these concerns to ensure a better mix of HTA. However, we do not consider it necessary for all new preparations (e.g. new generic products) for existing active formulations of indications that are already licensed, to undergo NICE evaluation. Consistent with current practice, decisions regarding local formularies should be taken by local Drugs and Therapeutics Committees.²⁸

3.3. Consultation and Transparency

The wide and extensive consultation that forms part of the development of NICE guidance is an integral component of the appraisal process. We acknowledge the efforts that NICE put into ensuring full and inclusive involvement by patient and public groups alongside the pharmaceutical industry. Nevertheless, we believe consultation could be improved. This is particularly the case in terms of giving stakeholders greater warning of forthcoming consultations and ensuring clear communication of the system of appraisal.

Consistent with the Court of Appeal's ruling over Alzheimer's drugs, we fully support the "opening up" of NICE decisions and the methodologies applied for reaching recommendations.²⁹ This is important for ensuring public confidence and instituting accountability for the decisions NICE takes.

We also believe it is imperative for NICE to clearly establish itself as a facilitator rather than an inhibitor of decisions relating to NHS drug availability. This is essential for creating a more amenable environment for open and effective appraisal, and should be enshrined in any NICE Charter (see section 1.2). Although NICE inevitably has tough choices and difficult trade-offs to make, better public communication of decisions and greater stakeholder involvement would help to improve the overall perception of the Institute.

3.4. Speed of Decisions: Shifting the “Burden of Proof”

In response to pressure for speedier decisions, NICE has introduced a fast-track system called Single Technology Appraisal (STA) for prioritised drugs with single indications.³⁰ There are two legitimate concerns this new process has tried to address:

- 1) technologies that are clearly and demonstrably **good value** for the NHS receive expeditious approval; and
- 2) technologies that are clearly and demonstrably **not good value** for the NHS are promptly rejected.

With all the time and resource required, more extensive appraisal such as MTAs should be targeted on those technologies where full review is really needed. This essentially requires more selective application.

In attempting to improve the speed and efficiency of the appraisal process, it is important to identify those aspects which consume substantial time and resource. A key reason concerns the “burden of proof”, which currently rests with NICE. This contrasts with other reimbursement authorities such as the SMC in Scotland and PBAC in Australia where proving cost-effectiveness lies firmly with the manufacturer. It is this burden which is often costly in terms of time and resource, e.g. NICE having to “prove” that implantable cardioverter defibrillators (ICDs), kidney cancer treatments and Alzheimer’s drugs are not cost-effective.

We believe the burden of proof should not be with NICE, but rather the manufacturer. It should not be the responsibility of NICE – acting on behalf of the NHS and taxpayer – to have to prove that a new technology is **not cost-effective**. Rather, it should be for manufacturers to prove that their technologies **are cost-effective** and thereby a good spend for the NHS. No other reimbursement authority in the world attempts to undertake timeline-driven evaluation while shouldering the burden of proof. Should a sponsor not be in a position to demonstrate cost-effectiveness, they would have the option to offer “risk-sharing” approaches, facilitating uptake while deferring full cost-effectiveness approval (see section 6.4 below).

This change would help to expedite the assessment of new treatments and speed up the appraisal process. It would also go some way to remove “defensive evaluation” and the associated media-related opprobrium. The onus would be on the manufacturer to demonstrate product value rather than NICE and indirectly the NHS and taxpayer.

The *status quo* offers the distinct possibility of increasing the acceptance of false claims and thus the sub-optimal allocation of available NHS resources. NICE must be allowed to operate in an environment where guidance is formulated free of media-driven pressures and patient group lobbying. Although NICE **must** always be **accountable** for its decisions and uphold absolute procedural **transparency**, this essentially requires a shifting of the “burden of proof” as is the case with other international reimbursement and advisory authorities.

3.5. Requirements for Submission

Another resource intensive aspect of NICE’s work concerns the evaluation of technologies for restricted use. The Institute rarely says “no” in absolute terms and often undertakes additional evaluation to identify subgroups and stop/start rules to consider the wider cost-effectiveness of a technology.

However, this type of evaluation is usually inhibited due to a lack of accessible evidence. Most sponsors do not provide subgroup analyses in their submissions instead naturally seeking the widest possible indication for their products. Currently, there is no mechanism within the STA process to ensure this evidence forms part of evaluation. Thus, there is the danger of NICE being forced into binary yes or no decisions.

It is essential that the appraisal process has scientific credibility and analytical teeth. Sponsors need to be positively incentivised to provide complete analysis and good quality submissions rather than use evidence selectively and exclude viable comparator treatments. This would provide NICE with a stronger evidence base to make more informed and far-reaching recommendations, increasing the use of restricted guidance for borderline treatments.

3.6. Joining Up STA and MTA

At present, there is no explicit bridge from STA to MTA. This necessarily precludes the optimal use of NICE resources and stultifies the scope for time-efficient appraisal.

In order to improve the use of, and link between, STA and MTA, we propose using STA as an **evaluative screen** during the period between when **licensing begins and product launch**. If required, MTA could then be used to undertake a more complete appraisal. Given the quicker and less comprehensive nature of any such “opening assessment”, increasing the measure of cost-effectiveness to ensure all potentially cost-effective treatments are approved at launch should be considered. Thereafter, the cost-effectiveness threshold could be reduced should a product undergo more complete appraisal.³¹

Using STA for *ex ante* assessment offers important advantages. First, STA helps to expeditiously get treatments into the NHS that are demonstrably good value, while inhibiting the diffusion of technologies that are clearly a bad spend. Second, STA acts as a screen for full appraisal. MTA would only be used for *ex post* assessment when really needed (i.e. where uncertainty surrounding a product’s value is greatest) thereby saving NICE resources, reducing time delays, and fostering value for money. Third, the threat of referral to complete appraisal would give STA real teeth. There would be little value in companies compiling incomplete and selective submissions at STA for these would be detected and referred back for full appraisal.

3.7. Appeals

NICE decisions can be appealed by a range of stakeholders, including manufacturers, patient groups and professional organisations. Appeals are reserved for when “the Institute has failed to act fairly, has exceeded its powers or has formulated guidance which cannot reasonably be justified”³², but not other grounds such as the interpretation of evidence. The appeals process does not permit the use or submission of new information, which is in accordance with English law. Appeals are held in public by a panel of three non-executive NICE directors or two non-executive directors plus an NHS clinician. An experienced industry delegate and a lay representative also form part of any appeals panel.

Even though the number of appeals against NICE decisions is moderate, there has been a dramatic increase in the number of recommendations being appealed each year mostly relating to technology appraisals. The process itself has attracted considerable criticism.

Although we understand the pressures for reforming the grounds for appeal, we acknowledge potential difficulties in allowing the use of additional information at the appeals stage. This would prolong the process significantly; increase the scope for “gaming” appeals; and potentially provide disincentives for manufacturers to produce high quality submissions for opening assessment.

However, we do recognise the need for the NICE appeals system to be independent and impartial. To this extent, we propose shifting appeals to a fully independent panel which would be entirely separate from NICE. It would assess appeals on substance as well as process. Where appropriate, this panel should be able to judge criteria other than the perversity of decisions for appeals, allowing for differences in the scientific or technical quality and accuracy of the evidence underpinning a decision to be considered.⁵ An independent panel should offer the additional benefit of reducing the number of litigations levelled against NICE.

We also understand the importance of improving dialogue with the pharmaceutical industry to enable an environment of cooperative appraisal (see section 2). This would help to attenuate the growing culture of appeal and facilitate a process of decision-making transparency.

3.8. Post-Appraisal Benchmarking

The work of NICE predominately focuses on the evaluation of new treatments as selected for referral by the DoH. Less attention has been paid to examining old technologies to encourage targeted disinvestment. For the use of NHS budgets to be optimised, we recognise that disinvestment is a key priority area and must therefore be an important aspect of NICE’s work.

Although NICE is already undertaking a range of activities to focus disinvestment in old technologies, we believe there is scope for this to be extended. In addition to NICE’s optimal practice programme and “recommendation reminders”^{8,10}, we believe post-appraisal benchmarking of introduced treatments should be undertaken, particularly in priority disease areas and where different treatments are available on the NHS. As part of topic selection, a category for such treatments could be created. More generally, we encourage NICE to ensure that recommendations directing the NHS away from less effective practice are given greater prominence when guidance is issued and publicly disseminated.

4. Affordability and Cost-Effectiveness

Overview

There is a large amount of discussion in the media surrounding the cost of new drugs and treatments, and the ‘value’ this therefore places on a human life. NICE currently uses an unofficial QALY (Quality Adjusted Life Year) threshold of £20,000-£30,000 per annum to assess the cost effectiveness of drugs and treatments.

This threshold has never been formally debated, in Parliament or more widely, and as such is open to criticism from all sides. Arguably, it is not a threshold at all and rather a guide, but is seldom understood as such. We would therefore seek more widespread understanding and support for the difficult affordability and cost-effectiveness judgements that have to be made through the following proposals:

- **Transfer responsibility for “recommending” medicines to the NHS Board.**
- **Ensure the methods of expressing and interpreting value are periodically reviewed by the NHS Board.**
- **Increasingly relate the valuation of clinical innovation and social value to the pricing processes agreed under the PPRS (see Chapter 6).**
- **Commission additional research and public consultation to establish society’s opinion of the valuation of clinical benefits.**
- **Further consider the feasibility, desirability and options for broadening NICE’s evaluation perspective to include societal costs.**

4.1. The Quality-Adjusted Life Year (QALY)

In order to compare the relative costs and benefits of different treatments a unit of measurement is required. NICE uses the quality-adjusted life year (QALY), which combines information about a product’s value in terms of mortality (length of life) and morbidity (quality of life). Utility or quality of life instruments, such as the European Quality of life 5 Dimensions (EQ5D), are used to derive QALY scores for these two components.

To evaluate cost-effectiveness, the QALY score is integrated with the cost of treatment to calculate the incremental cost-effectiveness ratio (ICER), which denotes the change in costs in relation to the change in health benefits. This results in a cost per QALY measure, which allows NICE to determine the cost-effectiveness of a given treatment.

Since its creation, NICE has adopted an unofficial cost-effectiveness threshold of £20,000–£30,000 per QALY.³³ The QALY has been widely criticised. Writing in the British Medical Journal, Professor Appleby from the King’s Fund said: “the uncomfortable truth is that NICE’s threshold has no basis in either theory or evidence.”³⁴

Most criticisms are not this severe, and it is generally accepted that the QALY is a necessary tool in judging cost-effectiveness. However, there is evidence that the QALY varies between disease groups. Evidence suggests that the average Primary Care Trust (PCT) spends around £12,000 and £19,000 to gain an extra QALY in circulatory disease and cancer, respectively.^{35,36}

An inappropriate and inconsistent cost-effectiveness threshold has serious implications. If too high/low, NHS efficiency is undermined as potentially cost-ineffective/cost-effective treatments are approved/rejected. Either way, resources are poorly distributed and can lead to “crowding out” or distortionary effects.^{23,31,37} It is therefore of paramount importance that the basis for determining cost-effectiveness is accurate, clear and evidence-based.

4.2. In Need of A “NICER” Threshold

We would strive to improve the basis for valuing clinical benefits and relative cost-effectiveness, including placing decisions not to use treatments in the context of commissioning decisions more generally, by transferring responsibility for setting the threshold to the NHS Board. Insofar as it is appropriate to indicate cost-effectiveness thresholds, this will fall under the remit of the NHS Board as part of its responsibility to reconcile outcomes to resources. It is clearly not beneficial to patients to have a uniform and universal threshold across all disease types, which fails to take account of dynamic factors, disease-specific requirements, opportunity costs, and budgetary implications. We therefore propose that the Board should have a responsibility to periodically consider the application of disease-specific measures according to the type of product and patient population.

We would also commission additional research and public consultation to establish society’s actual value of a QALY, and more broadly the value associated with clinical benefits.

This approach confers important advantages. By formalising the terms of reference governing cost-effectiveness, NICE decisions would be more accountable and therefore robust. This would help reduce media-related opprobrium and political interference, while ensuring regular review of threshold appropriateness.

4.3. Measuring social value: societal costs and benefits

A keystone of any economic evaluation, particularly cost-effectiveness studies, is the type of costs included. Different costing methodologies foster different outcomes, often resulting in dramatically divergent conclusions for a given economic trade-off or resource allocation decision. Getting costing right is therefore seminally important.^{23,31,38}

Conservative Party leader David Cameron has made it clear that he believes that good government involves not only taking the economic consequences or impact of a decision on individuals into account when making policy, but also accounting for the wider social value of any decision, namely the costs and benefits to society. However, it has been the policy of the DoH not to include estimates of social value in the evaluation of health care interventions. Critics have argued that excluding these costs – particularly costs relating to productivity losses and caregiver burden – undermines the validity of QALY-based recommendations.

In recent NICE appraisals, the cost issue has proven very controversial; most notably with the Alzheimer's drug Aricept.^{20,39} In this case, caregiver burden was overlooked given the Department's statutory limits.

However, evidence suggests that carers consume more in the way of health care costs than Alzheimer's patients themselves.⁴⁰ This also applies to cancer (palliative care) and psychiatric illnesses (community and social care).⁴¹

NICE remains one of the few evaluating bodies to use the payer perspective. This contrasts with other international authorities in Australia (PBAC)⁴², New Zealand (PHARMAC)⁴³, Sweden (LFN)⁴⁴ and Germany (IQWiG)⁴⁵ where the societal perspective is adopted. Although including calculations of wider social value is a highly complex task, we believe it is essential for NICE to foster a broader evaluative perspective where possible. The work of NICE must not be seen as simply *economic evaluation* but rather *evaluation*. If society agrees that the sacrifices of families and carers should be considered, then it follows that the DoH's policy should be revised. This is central to understanding the true and wider benefits of new treatments.

We will investigate further this issue and formulate recommendations for broadening the evaluative perspective. We wish to develop appropriate assessment guidelines for including societal costs; assess the advantages and disadvantages of different methodological approaches; and provide recommendations for circumventing potential evaluative conflicts which may skew decisions, i.e. avoiding bias against certain groups such as the elderly who may not achieve the same productivity gains from treatment as younger patients.

5. Implementation of Guidance

Overview

Given the central importance attached to NICE decisions – and the resources diverted in doing so – complete and consistent implementation of guidelines throughout the NHS is crucial. This is particularly pertinent if we are to achieve our objectives of ensuring new drugs and treatments reach the patients more swiftly; ending the “postcode lottery”; and overcoming health inequalities.

NHS bodies are already legally obliged to provide sufficient funding for NICE technology appraisals within three months of approval, although this is not the case for guidance, which is advisory. We support this distinction, although we would like to see greater encouragement for NHS bodies to adopt guidance swiftly. We believe that a focus on outcomes will incentivise this, as NHS bodies will be unable to achieve good outcomes without swift adoption of new treatments and medicines.

We would therefore seek faster and more consistent implementation of NICE guidance through the following proposals:

- Use our focus on outcomes to encourage the full, consistent and accurate implementation of NICE guidance across the NHS.**
- Ensure that commissioning bodies are required to take account of commissioning guidelines including mandatory aspects which relate specifically to the delivery of outcomes and relative cost-effectiveness.**
- Extend commissioning guidelines and build on those guidelines currently used by NICE.**
- Treat implementation of NICE guidance as a key performance indicator.**

5.1. The Missing Link

Implementation of guidance has proved to be highly variable across local hospitals and PCTs especially in the case of cancer medicines. This has recently been acknowledged by the government following announcements surrounding the introduction of an NHS Constitution.

The Audit Commission has shown that initiation of NICE guidance is not routinely part of financial planning. A 2005 report suggested only 26% of NHS bodies were actively “horizon scanning” in order to prepare for, and absorb, future guidance.⁴⁶ The impact in 2005 and 2006 NHS budget deficits was hardly helpful in this regard. This means implementation is often deferred, or sometimes completely shelved, resulting in uneven and disjointed national uptake.

There is also the issue of inaccurate and mistaken implementation. A recent study commissioned by NICE revealed that, out of 28 NICE appraisals, 12 were under-implemented and four over-implemented.⁴⁷ Since many NICE decisions are based on marginal and sometimes optimistic judgments of cost-utility, over-implementation is likely to be expensive and under-implementation clinically inadequate.

5.2. Stamping Out the “Postcode Lottery”

These problems of implementation do absolutely nothing to advance NICE’s stated aim. If anything, irregular implementation is likely to compound existing health inequalities, widening the “accessibility gap” in priority disease areas and across target populations.

Despite not being a failing of NICE per se, failure of this kind severely compromises its impact. It is impossible to stamp out postcode prescribing if recommendations are not carried through to the front-line. Guidance without proper implementation is self-defeating.

Although recent government announcements to address this failing are welcome, the reality is that proposed changes will do little to facilitate quicker access to approved treatments. Local NHS bodies are already legally obliged to provide approved technologies within three months of NICE issuing final guidance. It is evident that this “mandatory requirement” is being ignored. Furthermore, the government’s pledge of an additional £100 million to facilitate swifter take-up will only go a small way to overcoming this delay.⁴⁸

We believe that moving towards an NHS focused on outcomes, with bodies responsible for commissioning alone, will incentivise the swift uptake of NICE guidance since without making new drugs and treatments swiftly available they will be unable to achieve good outcomes for their patients.

5.3. Guiding Implementation

We pledge to uphold the current legal requirement for NHS bodies to fully and properly implement NICE technology appraisals within three months of guidance being published.

Building on the “commissioning guidelines” and costing templates introduced by NICE, we propose enhancing the role of “commissioning guidelines” to provide NHS providers with a focused framework for bringing NICE guidance to the front-line. This will help to foster better forward-planning and ensure implementation is “on the radar”. Access to NICE approved drugs will be supported by appropriate funding arrangements. Effectively measuring guidance implementation is fundamental to understanding the impact NICE’s work is having on patient care across the NHS. Thus, implementation will be viewed as a key performance indicator by the Healthcare Commission and should be suitably prioritised when deriving local budgets.

6. NHS Drug Availability –Telling It Like It Is

Overview

All health care systems confront a basic economic problem: how best to allocate finite resource to satisfy infinite healthcare demands. The candid truth is that the NHS cannot afford to buy and reimburse every new treatment, and therefore needs to carefully decide between competing alternatives.^{23,49} As more expensive new drugs are coming to market, the rate of growth in NHS funding is declining. This reality dictates that cost must be a factor along with clinical efficacy. Thus, the work of NICE is crucial to establishing a focused decision-making framework that facilitates the efficient and effective allocation of scarce NHS resources.

However, ensuring maximum benefit from finite drugs budgets can be facilitated by innovative drug pricing schemes. We are supportive of a move towards more value-based pricing (VBP) for new medicines, under which a treatment would be judged in accordance with its clinical benefits. Such a system would require the input and participation of NICE. We would therefore work to ensure best use of finite NHS drug resources and increased use of innovative drug pricing schemes through the following proposals:

- Ensure debates surrounding NICE and NHS drug availability are conducted through an understanding of the finite nature of NHS resources.
- Engage the public in decisions regarding NHS priority setting and resource allocation.
- Establish NICE in statute removing ministerial and political interference in NICE decision-making.
- Foster better communication and collaboration with industry.
- Develop an approach to drug pricing that better fosters fair and affordable prices to the NHS, while dynamically incentivising pharmaceutical R&D.
- Where appropriate, apply the principle of VBP.
- Further explore the potential role of NICE in helping to determine optimal drug prices for the NHS.
- Commission further consultation into the use of risk-sharing schemes for pricing and reimbursement.
- Encourage the wider use of risk-sharing schemes where appropriate to the product and disease area.

6.1. The Role of Politicians

Given the reality of budgetary pressures within the NHS, tough decisions regarding drug availability are inevitable. This necessarily means that NICE sometimes has to make judgments between different treatments and balance the relative costs and benefits of marginal trade-offs. It should be remembered that introducing a new intervention can mean displacing an existing treatment. Understanding the overall costs and benefits of approving a new treatment is therefore central to informed decision-making, and it is vital that all debates surrounding NICE and NHS drug availability are conducted in the context of the NHS budget constraint.

In the recent Health Select Committee (HSC) Report, concerns were expressed that Health Ministers had undermined the work of NICE. A notable example of such political interference is that of the breast cancer drug trastuzumab (Herceptin). In November 2005, the then Secretary of State for Health, Patricia Hewitt, publicly announced her concern about the refusal of a PCT to prescribe Herceptin to a patient prior to NICE assessment.⁵⁰ As the HSC Report noted, “This made it almost impossible for NICE not to approve the drug, once licensed, regardless of cost.”⁵¹

For the work of NICE to have meaning and integrity, it is essential the Institute has the autonomy and support to perform appraisals in isolation to political factors. We recognise that it is **not** the role for Ministers, directly or indirectly, to seek to influence the NICE decision-making process. We pledge to remove all political interference, including decisions relating to topic selection (see section 3.2), and allow NICE independently to reach recommendations based on clinical and cost-effectiveness.

6.2. Drug Pricing

The issue of drug pricing is an important dimension to NHS drug availability. The Pharmaceutical Price Regulation Scheme (PPRS) is the voluntary agreement between the DoH and the pharmaceutical industry in which companies negotiate profit rates from the sale of branded drugs every five years. Using a combination of price and profit controls, the PPRS seeks to achieve a balance between reasonable prices for the NHS and a fair return for industry to facilitate future R&D.^{2,52}

The PPRS has been the subject of criticism and review. A recent Office of Fair Trading (OFT) report proposed a shift to value-based pricing (VBP) whereby a new treatment would be reviewed in accordance with its clinical benefits against an appropriate comparator. According to this system, prices would be determined during the licensing phase following assessment of a new drug’s clinical and cost-effectiveness, with periodic review as new data become available. The OFT report recommended that NICE should play a key role in any such VBP system.⁴ Despite these recommendations, the government abrogated the PPRS in August 2008 (claiming the OFT report as its purpose), then largely discarded the proposals of the OFT.⁵³

6.3. Value-Based Pricing and the Role of NICE

We believe that better mechanisms are needed to ensure that the NHS pays fair and affordable prices for drugs and treatments, while rewarding the exhaustive R&D that pharmaceutical companies undertake. Any change to the current system would likely have profound implications for NICE and should therefore be phased in following suitable consultation with affected parties. Although the OFT report sets out some important principles, it is important to recognise its flaws: it fails to recognise the importance of free pricing at the launch of new medicines; it understates the benefit of incremental innovation; it overlooks the value of innovation in sub-groups of patient populations; and, the negative impact of *ex-ante* VBP where full data is unreliable.

We believe that the principle of VBP offers potential opportunities to foster dynamic and allocative efficiency for the NHS. Where appropriate, the application of VBP should be considered and the role of NICE in helping to determine optimal prices for the NHS be further explored. This could provide the prospect for a more integrated and incentivised approach to NHS drug pricing.

6.4. Increasing the Use of Risk-Sharing

In recent years, the use of risk-sharing schemes to encourage drug availability and facilitate appropriate reimbursement has been furthered. The OFT report highlighted the problems of price setting in the presence of limited data and associated uncertainties about treatments at product launch. It has been argued that risk-sharing arrangements are a practical way of mitigating this reality in order to allow NICE to more readily say “yes”.

Although risk-sharing schemes would need to be clearly defined and used with caution, we believe there is scope for its increased application where appropriate.⁵⁴ The bone marrow cancer drug Velcade and ophthalmology treatment Lucentis are compelling examples of successful risk-sharing, having allowed early access to treatment and improving patient outcomes.⁵⁵ However, we fully recognise that the key to these successes is the appropriate measurement of treatment effect following the introduction of a new drug to the NHS. We therefore encourage the increased use of “monitoring studies” to determine the degree of risk the NHS should undertake, and broader consultation with the pharmaceutical industry as to how risk-sharing schemes can be applied.

Essentially, risk-sharing schemes follow the principle of “payment by results”, meaning full reimbursement is only provided when product value has been reasonably demonstrated. This can, of course, be a bridge to a more systematic VBP assessment. We believe that this principle is most closely aligned with the need to efficiently allocate available NHS resources and safeguard value for money, while ensuring ready access to life-saving treatments and better patient outcomes.

References

- ¹ Woolf, S. & Henshall, C. (2000), 'Health technology assessment in the United Kingdom', *International Journal of Health Technology Assessment*, 16: 591-625.
- ² Carroll, S. (2008), 'Pricing of Pharmaceuticals in the UK: Implications of Reform to the Pharmaceutical Price Regulation Scheme (PPRS)', *UBC Evidence Matters*, February 2008; XIV (1):8-10.
- ³ UK Department of Health, www.dh.gov.uk, August 2008.
- ⁴ Office of Fair Trading (2007), 'The Pharmaceutical Price Regulation Scheme: An OFT Market Study, 2007', www.offt.gov.uk/NR/rdonlyres/7C7A7CC1-F320-4978-AC64-F67F9AB67B6C/0/oft885.pdf.
- ⁵ Sorenson, C. Drummond, M. Kavavos. P & McGuire A. (2008), 'National Institute for Health and Clinical Excellence: How Does it Work and What are the Implications for the US?', 2008, London School of Economics.
- ⁶ Smith, R. (2004) The triumph of NICE. *British Medical Journal*, 2004;329:7459.
- ⁷ Culyer, A.J. (2006), 'NICE's use of cost effectiveness as an exemplar of a deliberative process', *Health Economics, Policy and Law*, 1: 299-318.
- ⁸ House of Commons Health Committee, National Institute for Health and Clinical Excellence: First Report of Session 2007-08, Volume 1, 17th December 2007, http://www.parliament.uk/parliamentary_committees/health_committee/hcpn251007.cfm.
- ⁹ Department of Health Press Release, 3rd February 1999 http://www.dh.gov.uk/en/Publicationsandstatistics/Pressreleases/DH_4025127.
- ¹⁰ The National Institute for Health and Clinical Excellence (2005), 'A Guide to NICE', April 2005. Accessible at www.nice.org.uk
- ¹¹ The National Institute for Health and Clinical Excellence (2008), 'Social Value Judgements: Principles for the Development of NICE Guidance', 2nd Edition, 2008. Accessible at www.nice.org.uk
- ¹² Schulpher, M. (2008), 'NICE's 2008 Methods Guide: Sensible Consolidation or Opportunities Missed?', *Pharmaeconomics* 2008; 26 (9): 721-724.
- ¹³ Conservative Party, 'NHS Autonomy and Accountability: Proposals for Legislation', June 2007.
- ¹⁴ The Conservative Party pledges to establish an NHS Board, independent of day-to-day interference by Ministers, and responsible for the commissioning of NHS services; the allocation of NHS resources; and the delivery of objectives to improve outcomes for patients, as agreed with the Secretary of State.
- ¹⁵ Conservative Party, 'NHS Autonomy and Accountability', p18
- ¹⁶ The National Institute for Health and Clinical Excellence (2008), 'Citizens Council', http://www.nice.org.uk/getinvolved/patientandpublicinvolvement/opportunitiestogetinvolved/citizenscouncil/citizens_council.jsp
- ¹⁷ Gulland, A. 'NICE proposals for citizens council condemned by patients', *British Medical Journal*, 2002 August 24; 325(7361):406.
- ¹⁸ PricewaterhouseCoopers, "Pharma 2020: The Vision. Which Path Will You Take?", <http://www.pwc.com/extweb/pwcpublishings.nsf/docid/91BF330647FFA402852572F2005ECC22>.
- ¹⁹ Association of the British Pharmaceutical Industry (ABPI), ABPI Official Review 2006, http://www.abpi.org.uk/publications/publication_details/annualReview2006/ar2006_success.asp
- ²⁰ Collier, J. (2008) 'Parliamentary Review Asks NICE to Do Better Still', *British Medical Journal* 12 January 2008; 336:56-57.
- ²¹ Pharmafocus, 'Head-to-head: NICE and the SMC', August 16th 2006, http://www.pharmafocus.com/cda/focusH/1,2109,22-0-0-AUG_2006-focus_feature_detail-0-462408,00.html.

-
- ²² Cancerbackup, 'Cancerbackup nurse explains access to drugs across the UK', Issue 58 Spring 2007,
<http://www.cancerbackup.org.uk/News/Newsletter/Issue58/NewsfromCancerbackup/CancerbackupnurseexplainsaccesstodrugsacrosstheUK>
- ²³ Carroll, S (2008), 'NHS Drug Availability: An Evaluation of the National Institute of Health and Clinical Excellence', *Bow Group Official Policy Paper*, January 2008,
<http://www.bowgroup.org/harriercollectionitems/NICE%20BOW%20GROUP%20Article%2027%2001%2008%20final.doc>.
- ²⁴ House of Commons Health Committee, 'National Institute for Health and Clinical Excellence: First Report of Session 2007-08', Volume 1, 17th December 2007, p. 47, paragraph 157.
- ²⁵ Cairns, J. (2006), 'Providing guidance to the NHS: The Scottish Medicines Consortium and the National Institute for Clinical Excellence compared', *Health Policy*, 2006 Apr; 76(2): 134
- ²⁶ House of Commons Health Committee, 'National Institute for Health and Clinical Excellence: First Report of Session 2007-08', Volume 1, 17th December 2007, p. 30, paragraph 86.
- ²⁷ House of Commons Health Committee, 'National Institute for Health and Clinical Excellence: First Report of Session 2007-08', Volume 1, 17th December 2007, p. 32, paragraph 94.
- ²⁸ National Institute for Health and Clinical Excellence (2008), 'Response to the Health Select Committee's Report on the National Institute for Health and Clinical Excellence', 2008. Accessible at www.nice.org.uk
- ²⁹ Dyer, C. (2008), 'Appeal Court rules that NICE procedure was unfair', *British Medical Journal*, 2008; 336 (10 May), doi.10.1136/bmj.39574.351782.DB
- ³⁰ National Institute for Clinical Excellence (2006), 'Guide to the Single Technology (STA) Process', NICE: London.
- ³¹ Carroll, S & Horsfall, L. (2008), 'Are the Winds of Change Blowing Through NICE?', *UBC Evidence Matters*, October 2008, Volume XIV, Issue 3: 1-4.
- ³² National Health Service England and Wales (2005), 'Directions and Consolidating Directions to the National Institute for Health and Clinical Excellence 2005', Available at www.nice.org.uk/niceMedia/pdf/DirectionFromSecretaryOfState2005.pdf
- ³³ House of Commons Health Committee, 'National Institute for Health and Clinical Excellence: First Report of Session 2007-08', Volume 1, 17th December 2007, pp. 32-33, paragraphs 97-101.
- ³⁴ Appleby, J. (2007), Chief Economist, King's Fund, *British Medical Journal*, BMJ 2007;335:358-359 (25 August), doi:10.1136/bmj.39308.560069.BE
- ³⁵ House of Commons Health Committee, 'National Institute for Health and Clinical Excellence: First Report of Session 2007-08', Volume 1, 17th December 2007, p. 60, paragraph 214.
- ³⁶ Martin, S. Rice, N. & Smith, P. (2007), 'The Link Between Health Care Spending and Health Outcomes: Evidence from English Programme Budgeting Data', *Centre for Health Economics Research Paper 24*, University of York, March 2007.
- ³⁷ McCabe, C. Claxton, K. & Culyer, A. (2008), 'The NICE Cost-Effectiveness Threshold: What it is and What that Means', *Pharmacoeconomics* 2008; 26 (9): 733-744
- ³⁸ Miners, A. (2008), 'Estimating 'Costs' for Cost-Effectiveness Analysis', *Pharmacoeconomics*, 2008; 26 (9): 745-751
- ³⁹ BBC, 'Alzheimer's drugs appeal granted', <http://news.bbc.co.uk/1/hi/health/7129623.stm>, 5th December 2007.

-
- ⁴⁰ Getsios, D. Migliaccio-Walle, K. & Caro, J (2007), 'NICE Cost-Effectiveness Appraisal of Cholinesterase Inhibitors', *Pharmacoeconomics* 2007; 25(12):997-1006.
- ⁴¹ Awad. G & Voruganti, L. (2008), 'The Burden of Schizophrenia on Caregivers', *Pharmacoeconomics*, 2008: 26 (2): 149-162.
- ³⁹ PBAC = Pharmaceutical Benefits Advisory Committee, <http://health.gov.au/internet/main/publishing.nsf/Content/Pharmaceutical+Benefits+Advisory+Committee-1/>.
- ⁴³ PHARMAC = Pharmaceutical Management Agency of New Zealand, <http://www.pharmac.govt.nz>
- ⁴⁴ LFN = Swedish Pharmaceutical Benefits Board, <http://www.lfn.se/>.
- ⁴⁵ IQWiG = Institute for Quality and Efficiency in Healthcare, <http://www.iqwig.de/index.2.en.html/>.
- ⁴⁶ Audit Commission, 'Managing the financial implications of NICE guidance', <http://www.audit-commission.gov.uk/Products/NATIONAL-REPORT/CC53DDFE-42C8-49c7-BB53-9F6485262718/ManagingTheFinancialImplicationsOfNiceGuidance08Sep05REP.pdf>, September 2005.
- ⁴⁷ Abacus International, 'NICE Guidance Implementation Tracking Data Sources, Methodology & Results', http://www.nice.org.uk/niceMedia/pdf/Abacus_report.pdf, January 2005.
- ⁴⁸ <http://news.bbc.co.uk/1/hi/health/7478173.stm>
- ⁴⁹ Birch, S. & Gafni, A. (2006), 'Information Created to Evade Reality (ICER): Things We Should Not Look to for Answers', *Pharmacoeconomics* 2006; 24 (11); 1121-1131.
- ⁵⁰ The Times, 8 November 2005, www.timesonline.co.uk/tol/news/uk/article588013.ece, BMJ, 2005.
- ⁵¹ House of Commons Health Committee, 'National Institute for Health and Clinical Excellence: First Report of Session 2007-08', Volume 1, 17th December 2007, p. 9, paragraph 3.
- ⁵² Claxton, K. (2007), 'OFT, VBP: QED?', *Health Economics*, 16: 545-558, 2007.
- ⁵³ Carroll, S. & O'Donohoe, P. (2008), 'Update on Changes to the Pricing of UK Pharmaceuticals', *UBC Evidence Matters*, October 2008, Volume XIV, Issue 3: 6-7.
- ⁵⁴ Andrew Lansley, The Telegraph, "Nice Doesn't have to be Nasty" 10th September 2008
- ⁵⁵ In the case of Velcade, the DoH and manufacturer, Johnson and Johnson (JNJ), agreed to make the drug available to patients for whom it is clinically appropriate. JNJ get paid when the product works, but forfeit reimbursement for patients where Velcade proves ineffective. As for Lucentis, the NHS pays for the first 14 injections of the drug. If the patient needs any further injections, Novartis picks up the bill.