Clinical Medicine 2011, Vol 11, No 3: 247–50

The NHS: assessing new technologies, NICE and value for money

A Stevens, K Chalkidou and P Littlejohns

ABSTRACT – The healthcare system in the UK, essentially the NHS, is an open economic system subject to the same pressures as any other economic system. The pressures concern limited resources coupled with powerful drivers for increasing spending: invention, demography and inflation. There have only ever been three types of economic system: steady state (everything, as in a feudal system, stays as it was the year before), market capitalism (supply and demand are allowed to find their own equilibrium) and some version of central planning.[1] In healthcare, most advanced countries favour the last of the three. This is for three reasons: distribution (not only are the poor less able to pay for sickness, but sickness exacerbates poverty), information (markets operate poorly when providers can easily outsmart customers) and externalities (it is in the interest of everyone that infectious diseases and the other knock-on consequences of ill health are ameliorated). So in the UK, the state, with a good deal of cross-party consensus, directs most of health service supply. This system has become more complex over the decades since the formation of the NHS in 1948. A notable element of the complexity is the regulation of the introduction of new technologies. A key element of the regulatory system has been the National Institute for Health and Clinical Excellence (NICE), and a key aspect of NICE's decisions has been not just value, but also value for money. This has not been without controversy.

KEY WORDS: health technology assessment, National Institute for Health and Clinical Excellence (NICE), NHS, regulation, value for money

The current regulatory framework is often described as comprising four hurdles for new medicines and other 'technologies' to jump: safety, quality, efficacy and cost effectiveness (value for money). The first three were established in the UK by the predecessors of the current Medicines and Healthcare products Regulatory Agency (MHRA) and European Medicines Agency (EMA), following the thalidomide catastrophe.[2] There was a 30-year time lag in between the establishment of the Medicines Act and the establishment of a national agency, the National Institute for Health and Clinical Excellence (NICE) to deal with the fourth hurdle (cost effectiveness). That period, however, saw the international emergence of health technology assessment (HTA) reflecting doubts about the presumed effectiveness of many licensed medicines, and concerns about their costs and opportunity costs (the benefits foregone when money has diverted). The publication of McKeown's *The role of medicine: the dream mirage or nemesis,* and Cochrane's *Effectiveness and efficiency: random reflections on health services* were landmarks in the stimulation of 'evidence-based medicine'.[3,4] The international collaboration to prepare systematic reviews of controlled trials in pregnancy and childbirth, the predecessor of the Cochrane Collaboration, was established in 1985.[5] The UK's HTA programme was established in 1993 with funding for both systematic reviews and new randomised controlled trials. The 1980s also saw a number of regional development and evaluation committees set up to act as a local fourth hurdle. NICE itself was established in 1999 and produced its first guidance in March 2000.

A Stevens, professor of public health, University of Birmingham; chair, Appraisals Committee, National Institute for Health and Clinical Excellence (NICE); **K Chalkidou,** director, NICE International; **P Littlejohns,** clinical and public health director, NICE

National Institute for Health and Clinical Excellence

NICE was created by the incoming Labour government. Frank Dobson, the former secretary of state for health, was keen to reassert the principles of the NHS at a time when there was increasing evidence of variability in the delivery of healthcare and in patients' access to expensive new drugs. Established as a special health authority, the aims of NICE were to:

- speed the uptake by the NHS of interventions that are both clinically and cost effective
- encourage more equitable access to healthcare (reduce the 'postcode lottery of care' – a term adopted by politicians when referring to different decisions made by local healthcare commissioners on whether to fund new drugs)
- encourage better and more rational use of available resources by focusing the provision of healthcare on the most cost-effective intervention
- encourage the development of new and innovative technologies.[7]

This was to be achieved by appraising the clinical and cost effectiveness of new and existing treatments and producing clinical guidelines. In 2005 its remit was expanded to include disease prevention and health promotion. Over the last 11 years its remit has expanded again to issue guidance on interventional procedures, diagnostics, devices and, more recently, the provisio of evidence as well as guidance though the web-based NHS Evidence (www.evidence.nhs.uk).

NICE guidance has always been considered as 'advisory' to clinicians, who are expected to fully take it into account when considering patient treatment options, while the final treatment decision remains their responsibility. However, in 2002 it was brought to the government's attention that while local health commissioners were complying fully with NICE guidance when its recommendations were not supportive of introducing new drugs, they were tardy in providing the funding for supportive guidance. The Department of Health (DH) issued a direction to the NHS that (unless directed not to in special circumstances) commissioners had to make the funding required to implement positive NICE appraisal decisions within three months of publication.[8]

This dramatically changed the nature of NICE appraisal guidance, for while it remained advisory to clinicians, it became national policy. While patients, doctors and the industry welcomed this development, local health commissioners were anxious that local health priorities would have to be forgone to fund new (mainly cancer) drugs of marginal cost effectiveness.

Over the last 11 years, while NICE's individual decisions were often considered controversial it gained an international reputation for its robust methods. These have combined scientific rigour (based on a close working relationship with the National Institute for Health Research, the Medical Research Council and the university sector) with careful attention to the process of decision making, which includes openness, transparency and inclusivity within a defined social and ethical framework.

Steering a path between its purpose and political/patient/pharmaceutical and media pressure

As a national policy organisation formally given the task of assessing the costs as well as the benefits of the whole spectrum of healthcare interventions, there were many claims that it meant explicit rationing in the NHS. NICE itself was eager to present the view that it was providing a new rational approach to prioritisation, the latter having always been necessary in a fixed budget NHS. Prioritisation of healthcare investment decisions is a political process. And institutions set up to carry out such functions are inherently political. When NICE was created, it was not only to 'give new coherence and prominence to information about clinical and cost effectiveness', but also do so with broad stakeholder engagement.[9]

In the 11 years since it was established, NICE has been striving to balance evidence, stakeholder views and changing government priorities in every aspect of its operation. Its first decision was against the use, in the NHS, of zanamavir (Relenza®), an anti-influenza drug manufactured by the British company, Glaxo Wellcome. Glaxo's chairman, Sir Richard Sykes, complained to Frank Dobson that '…NICE's handling of Relenza has confirmed the industry's worst fears about the institute – that it is an instrument for holding down the NHS budget and has nothing to do with treating patients'.[10] NICE (and government) held its ground.

This was just the beginning. In 2001–2, NICE said 'no' to drugs for multiple sclerosis (MS), a decision that was an anathema not just to pharma but also to an increasingly influential patient group. In response to NICE's suggestion that industry and government should work together to identify ways of improving the drug's cost effectiveness, the DH established (what has turned out to be a very controversial) 'risk sharing scheme'.[11] In 2005–6, NICE approved Herceptin® for early breast cancer amid a media and patient organisation campaign and a high-profile public comment by the then secretary of state, Patricia Hewitt.[12] A few months earlier, in 2005, NICE had started to rely more on industry evidence to improve the timeliness of its guidance through the single technology appraisal process.[13] Herceptin was the first technology to be considered under this new process. In 2006–7, NICE fought a lengthy legal battle on its guidance to restrict access to drugs for Alzheimer's disease, a decision eventually reversed by its committee in 2010.[14] In 2008–9, NICE asked its decision-making committees to apply less strict criteria (ostensibly giving greater value to the last months of life) when assessing the value of expensive cancer drugs prolonging survival at the end of life.[15] NICE's public health guidance, first launched in 2005, has also not been without controversy: NICE's calls for antenatal care at schools, stricter regulation of the food industry and increased taxation of alcohol have sparked a mixture of positive and negative reactions among the media and government.[16–18]

Over the years, the pharmaceutical industry, NICE's most suspicious stakeholder, and NICE, have learned to work together. In the beginning NICE was perceived to be a hindrance, unnecessary and even 'illogical':

> *It is quite wrong of NICE to delay further the introduction of medicines that have already proved their efficacy to the Government's own, well-respected regulatory authority. Medicines are one of the most cost-effective means at the disposal of doctors in fighting disease, and patients should not be denied their benefits. It is completely illogical to have a new medicine licensed as safe and effective by one Government body to then to be blocked for NHS use by another.*[19]

Ten years later, industry announced that it takes pride in working with NICE to improve access to medicines and listed the NHS Constitution guaranteeing access to NICE-approved drugs, among one of its major achievements.[20]

A reason for this is probably that the great majority of NICE's appraisal decisions have been 'yes', or at least partially so. It was hoped, perhaps forlornly, that NICE would, on the whole, be able to say yes to exciting new technologies, especially new cancer drugs, on the grounds that they would be good value to the NHS as well as being highly desirable for patients. In adopting a threshold for acceptance of approximately £20,000–30,000 per quality adjusted life year (QALY),[21] it seemed likely, given that even the dearest forms of care, such as neonatal intensive care and renal dialysis, were arguably below this threshold, this would also be the case for new developments. Unfortunately, the experience has not quite been that. Typically the new cancer drugs have afforded weeks or months of extra life rather than years, and typically their prices have escalated from the old-fashioned level of (eg 5FU) costs in the hundreds to

prices in the tens of thousands of pounds. So NICE's decisions concerned many new drugs right at the margin, or even above, what might be considered affordable. The 'no' decisions have therefore been few and well beyond the margin of what might be considered affordable, but many, including zanamavir, drugs for MS and drugs for Alzheimer's disease (above) have become causes célèbre.

The 'yes' decisions in marginal technologies have therefore been made by giving the technologies the benefit of the health economic doubt. In the case of the anti-tumour necrosis factor drugs for rheumatoid arthritis, the acceptable final cost per QALY (of at least £24,600) was achieved by assuming long-term benefits in the face of relatively early discontinuation of the drug.[22] In the case of paclitaxel for ovarian cancer, the relative benefits over platinum therapy were inferred from the assumption that the two trials which showed benefit (GOG111 and OV10), outweighed the trial (GOG132) which did not. This assumption was allowed to remain in the guidance even when the new, much larger and independent ICON 3 trial corroborated GOG132 rather than the other two (industry sponsored trials) demonstrating benefit.[23] Ranibizumab to prevent monocular blindness in age-related macular degeneration was accepted even though not cost effective, by assuming that the analysis for these patients should be merged with the patients at risk of total blindness for whom the treatment was cost effective.[24]

The problem for appraisal decisions has undoubtedly been the escalating prices of new pharmaceuticals. The appraisal committees have been bound to accept a set price from the manufacturers, and all the arguments that have taken place during an appraisal have concerned how to model effectiveness. In more straightened financial times for NHS, the need to achieve acceptable levels of cost effectiveness will be the greater.

The international scene

NICE's reach has been broader than the English and Welsh NHS. In the USA, President Barack Obama's efforts to reform the health system have been met with resistance from those fearing that a NICE-style organisation might be established to make decisions on priorities based on scientific evidence and values (to replace the current rationing-by-ability-to-pay system). The Manhattan Institute, the Heritage Foundation, the American Enterprise Institute and media channels such as Fox, Forbes, the *Wall Street Journal* and the *American Spectator* have been among NICE's fiercest critics, with the *New York Times* and channels such as NPR, adopting a more balanced view.[25–28]

What may be NICE's most lasting international legacy though is its influence over emerging market economies. Over the past three years, it has been working with ministries of health from countries such as Brazil, China, Colombia, Serbia, Thailand and Turkey to help inform the establishment of new, or strengthen existing, institutions making prioritisation decisions. With the support of global aid donors including the World Bank, the UK's Department for International Development, the Pan-American Health Organization and the Inter-American Development Bank, NICE has been helping with institution building, training and hands-on piloting of the development and implementation of value pathways in high priority (mostly chronic) disease areas.[29] A NICE in a country like China, effectively single-handedly driving growth in the global pharmaceutical sector, may have a much more lasting impact globally than the original, English version could ever have wished for.[30,31]

The future of NICE and the regulatory environment

Since 1999, NICE has been subject to two World Health Organizaton and four parliamentary enquiries, most of them supportive of its function. In 2008, the Health Select Committee stated:

> *We conclude that NICE does a vital job in difficult circumstances…Healthcare budgets in England, as in other countries, are limited. Patients cannot expect to receive every possible treatment. NICE requires the backing of the Government. NICE must not be left to fight a lone battle to support cost- and clinical effectiveness in the NHS.*[32]

Most recently, with a caveat, NICE survived the most radical reorganisation of the NHS since it was established in 1948. The incoming coalition government has given NICE a modified role to play in its new vision for the NHS. The series of consultation documents following the *Liberating the NHS* White Paper shifts the assessment of NHS performance away from indicators of process to outcomes which NICE will develop through expanding its clinical standards programme. Payment schemes for primary (Quality and Outcomes Framework) and secondary (CQUIN and Best Practice Tariff) care providers, commissioners' behaviour and performance measurement and patient entitlements in clinical and pubic health and in social care, will be driven by NICE standards. 'Quality standards, developed by NICE, will inform the commissioning of all NHS care and payment systems. Inspection will be against essential quality standards…'.[33] NICE is to be re-established under new legislation as a non-departmental government body, with an expanded remit to include social care and inform research priorities.

The caveat is that the coalition government is also radically revising the country's drugs pricing policies, and with it NICE's regulatory role. This started with the establishment of the Cancer Fund.[34] Further announcements on reforming the Pharmaceutical Price Regulation Scheme (PPRS) (and the implications for NICE's role) signalled that the government is putting the emphasis on the drug price being set in relationship to its value:

> *NICE will continue to appraise drugs until we implement our plans (as value-based pricing from 2014…. (Thereafter) there will continue to be a role for technology appraisals … NICE's role will increasingly focus on giving authoritative advice to clinicians…*[35]

The exact role that NICE and the equivalent organisations in Wales and Scotland will have in influencing drug pricing and

availability through determining cost effectiveness is still undecided (as the government proposals are out for consultation), but it seems probable that the final decision regarding drug availability in the NHS will be taken elsewhere. Clearly service prioritisation decisions will be required of the new general practice commissioners. General practitioners are already expressing concern over the impact of this new responsibility on the doctor–patient relationship. They would rather NICE decides.

References

1 Heilbroner RL. *The worldly philosophers: the lives, times, and ideas of the great economic thinkers,* 7th edn. New York: Touchstone, Simon & Schuster, 1999.
2 Penn RG. The state control of medicine: the first 3000 years. *Br J Clin Pharmacol* 1979;8:293–305.
3 McKeown T. *The role of medicine.* Oxford: Blackwell, 1979.
4 Cochrane A. *Effectiveness and efficiency: random reflections on the health service.* Leeds: Nuffield Provincial Hospital Trust, 1972.
5 Chalmers I. The Cochrane Collaboration: preparing, maintaining and disseminating systematic reviews of the effects of healthcare. *Ann N Y Acad Sci* 1993;703:156–65.
6 Stevens A, Milne R. A knowledge-based health service: How do the new initiatives work? *J R Soc Med* 1998;91(Suppl 35):26–31.
7 Statutory Instrument 2005 no. 497 (2005). The National Institute for Clinical Excellence (Establishment and Constitution) Amendment Order 2005. www.opsi.gov.uk/si/si2005/2005497.htm
8 Department of Health. *Directions to primary care trusts and NHS trusts in England concerning arrangements for the funding of technology appraisal guidance from the National Institute for Clinical Excellence (NICE).* London: DH, 2003. www.dh.gov.uk/en/Publicationsandstatistics/Publications/PublicationsLegislation/DH_4083088
9 Department of Health. *A first class service: quality in the new NHS.* London: DH, 1998.
10 Glaxo awaits Relenza verdict, *The Guardian*, October 1999.
11 McCabe C, Chilcott J, Claxton K *et al.* NICE multiple sclerosis technology appraisal, 2002. Continuing the multiple sclerosis risk sharing scheme is unjustified. *BMJ* 2010;340:c1786.
12 BBC. Hewitt has left the NHS toothless. 10 November 2005. http://news.bbc.co.uk/1/hi/health/4422662.stm
13 Wailoo A, Pickstone C. A review of the NICE Single Technology Appraisal Process. NICE Support Unit, Sheffield 2008. www.nicedsu.org.uk
14 BBC. Alzheimer's drugs court challenge, 25 June 2007. http://news.bbc.co.uk/i/hi/health;6250530.stm
15 National Institute for Health and Clinical Excellence. *Appraising life-extending, end of life treatments.* London: NICE, 2009.
16 Antenatal care in schools? Controversy hides the real problems, *The Daily Telegraph*, September 2010. www.telegraph.co.uk/health/healthadvice/maxpemberton/8023540/Antenatal-care-in-schools-Controversy-hides-the-real-problems.html
17 Free choice isn't healthy for the food industry's menu, *The Guardian,* June 2010. www.guardian.co.uk/commentisfree/2010/jun/23/trans-fat-food-industry-health-costs
18 The price of Britain's drinking problem, *The Independent,* June 2010. www.independent.co.uk/opinion/leading-articles/leading-article-the-price-of-britains-drinking-problem-1989756.html
19 ABPI statement in response to NICE's Relenza decision, 2000. www.abpi.org.uk/press/press_releases_00/001121.asp
20 ABPI 2008/09 Annual Report, 2009. www.pharmafield.co.uk/news
21 National Institute for Health and Clinical Excellence. *Guide to methods of technology appraisal.* London: NICE, 2008.
22 NICE Technology Appraisal Guidance No 130. Adalimumab, etanercept and infliximab for the treatment of rheumatoid arthritis.
23 NICE Technology Appraisal Guidance No 55. Guidance on the Use of Paxlitaxel in the treatment of ovarian cancer, 2003.
24 NICE Technology Appraisal Guidance No 155. Ranibizumab and pegaptanib for wet age-related macular degeneration, 2008.
25 Of NICE and men, *Wall Street Journal,* July 2009. http://online.wsj.com/article/SB124692973435303415.html#articleTabs%3Darticle
26 Live or let die, *American Spectator,* August 2009. http://spectator.org/archives/2009/08/14/live-or-let-die
27 FoxNews. Another sneaky Obama move, July 2010. www.foxnews.com/opinion/2010/07/09/phil-kerpen-obama-recess-appointment-dr-donald-berwick-senate-baucus-medicare/
28 British balance benefit vs cost of latest drugs, *New York Times,* December, 2008. www.nytimes.com/2008/12/03/health/03nice.html?_r=4&pagewanted=all
29 Chalkidou K, Levine R, Dillon A. Helping poorer countries make locally informed health decisions. *BMJ* 2010;341:c3651.
30 Bloomberg, China demand to help push drug sales to $880 billion, IMS says, October 2010. www.bloomberg.com/news/2010-10-06/china-demand-to-help-push-drug-sales-to-880-billion-ims-says.html
31 China puts healthcare under scrutiny, *Financial Times*, September 2010.
32 Parliamentary Select Committee on Health. National Institute for Health and Clinical Excellence. First Report of the Service 2007–8. London: HMSO, 2008.
33 Department of Health. *Equity and excellence: liberating the NHS.* London: DH, 2010.
34 Department of Health. *The Cancer Drugs Fund. A consultation.* London: DH, 2010.
35 Department of Health. *A new approach to the pricing of branded medicines.* London: DH, 2010.

Address for correspondence: Dr A Stevens.
Email: stevenaj@adf.bham.ac.uk