

Value Based Assessment of Drugs



In 2010, the Government proposed moving towards a broader value-based system for assessing and pricing branded drugs. Such an approach aims to ensure that the price the NHS pays for a medicine better reflects its benefits. This briefing outlines recent proposals to change the current assessment methods.

Drug Pricing and Value Assessment

The NHS drugs bill is considerably more than £10 billion a year. In theory, spending money on new treatments, including new medicines, diverts funds from elsewhere in the NHS. The interests of patients who stand to benefit from a treatment, and patients elsewhere in the NHS who will miss out on treatments that are not funded, must be balanced. Various mechanisms are in place to decide which drugs the NHS should provide. This note describes current drug pricing mechanisms for branded, in-patent, medicines. It considers attempts to incorporate any broader benefits of a drug into appraisals and how these might inform price negotiations. Current mechanisms include the:

- **Pharmaceutical Price Regulation Scheme (PPRS)**¹, a voluntary UK agreement between the pharmaceutical industry and the Department of Health (DH) to regulate the price of most branded drugs (Box 1). It caps the profits that companies can make from the NHS but leaves them free to set the price of individual drugs.
- **National Institute of Health and Care Excellence (NICE)** appraisals of the clinical and cost-effectiveness of most new drugs (Box 2). NHS commissioners in England are legally required to make funding available for drugs and treatments recommended by a NICE technology appraisal within three months. Wales (All Wales Medicines Strategy Group) generally follows recommendations made by NICE. Scotland (the Scottish Medicines Consortium) and Northern Ireland (Department

Overview

- Value based approaches aim to link more closely the price the NHS pays for a drug with the clinical and other benefits it delivers.
- After several rounds of consultation no agreement has been reached on how to widen current assessment methods to take fuller account of a drug's benefits.
- The Cancer Drugs Fund (CDF) was set up to allow patients to access cancer drugs otherwise unavailable on the NHS. It was intended as an interim measure until a wider value-based appraisal system was in place.
- The next Government will have to decide the future of the CDF beyond 2016. There may also be a need for wider reform to align the various drug appraisal and pricing systems.

of Health, Social Services and Public Safety) both have separate organisations to make decisions.

- **Cancer Drugs Fund (CDF)** which allows patients in England access to cancer drugs which are not routinely available in the NHS, because they have not yet been appraised by NICE or have not been recommended by NICE as being cost effective (see Box 3). It was set up in 2010 as an interim measure until the proposed value-based approach (see next section) was implemented. The CDF will cost £340 million between April 2015 and April 2016; its future thereafter is uncertain.

Evolution of Value Based Approaches

In May 2010, the Government stated its intention to introduce Value Based Pricing (VBP) with more detailed plans outlined in a July 2010 report.² It has been argued that

Box 1. The Pharmaceutical Price Regulatory Scheme (PPRS)

The PPRS is a voluntary agreement between the Government and the UK pharmaceutical industry which is renegotiated on a regular basis. The 2014 PPRS operates from January 2014 until December 2018. It:

- limits the price of branded medicines supplied to the health service, and the profits that companies can achieve through sales
- will keep the bill for the branded medicines covered by the PPRS flat for the first two years and then allow it to grow at set rates
- ensures that companies make payments to DH as a percentage (set nationally each year) of their net eligible sales, for any growth above the agreed limit
- apportions the payments between the four UK Health Departments.

Box 2. NICE appraisal of new drugs

NICE is an executive non-departmental public body that completes appraisals of medicines by comparing the clinical effectiveness (health effects) and cost effectiveness (value for money) of a new drug with established practice in the NHS in England.³ The process includes:

- evidence from clinical trials and peer reviewed research showing how well a medicine or treatment works, including its likely impact on mortality and quality of life (such as pain or disability)
- economic evidence on how much it costs the NHS
- the views of clinicians, patients and other stakeholders.

The basic NICE appraisal method calculates how much it costs on average for a drug to deliver an extra year of good quality life (a quality adjusted life year or QALY) when compared with established NHS practice. In the calculations the QALY difference for a new medicine compared to the established treatment is combined with the cost difference between the new medicine and the established treatment. A QALY is a uniform measure of the impact a treatment has on the patient; it allows comparisons to be made between diseases and conditions and takes into account both the quantity of extra life (years) and the quality of that extra life. To ensure effective use of NHS resources NICE operates on the basis that:

- Treatments with a cost per QALY gained of less than £20,000 are usually considered to be cost effective.
- As the cost per QALY gained for a medicine increases in the range of £20,000 to £30,000, the judgement about the acceptability of the medicine takes into account a number of factors. For example whether health-related quality of life benefits were adequately captured in the QALY calculation, the degree of certainty around the calculations and whether a treatment is innovative or meets other (non-health) NHS objectives.
- For treatments assessed as having a cost per QALY gained of higher than £30,000, NICE has to identify a stronger case for supporting the medicine. Consideration is given to whether the technology meets end of life (EoL) criteria that apply to patients with a short (two years) life expectancy *and* where there is good evidence that the treatment will extend life by at least three months *and* where the technology is licensed for small patient populations.

NICE appraisals may not capture the full value of a drug. For example, they may not sufficiently value drugs that treat very severe conditions or which have wider societal benefits (to patients, carers and other public service users). The proposed VBP approach aimed to broaden the scope of the NICE appraisal to incorporate these aspects and take them into account in determining how much the NHS would pay for new drugs. As an interim measure the Cancer Drugs Fund (Box 3) was announced in July 2010.

Government Consultation on Value Based Pricing

In December 2010, DH launched a consultation on VBP.⁴ It suggested VBP would result in better access for patients to innovative drugs and better value for the NHS. A higher cost for each QALY gained could apply for drugs that:

- tackle a disease for which there was high 'burden of illness' (a measure of the severity of a disease that has two components: quality of life and length of life)
- demonstrate wider societal benefits such as a patient's ability to return to work or contribute to society; such benefits vary, for example, with age, gender, disease and quality of life
- show greater therapeutic innovation or improvements compared with other products.

Box 3. The Cancer Drugs Fund (CDF)

The Cancer Drugs Fund was introduced in 2010 to enable patients to access drugs that may not be available on the NHS. This includes drugs NICE has not recommended as being cost effective and those it has not yet appraised. The idea was that, once in place, a VBP approach would be able to capture the wider benefits associated with end-of-life treatments and there would be no further need for the CDF. In addition, the UK had been highlighted as lagging behind some comparable countries in its uptake and use of innovative new cancer drugs.⁵

In its first year (2010/11), DH allocated £50 million to the CDF. This subsequently rose to £200 million a year in 2011/12, 2012/13 and 2013/14; and then to £280 million a year in 2014/15 and £340million a year in 2015/16. Access to the CDF is based on clinical recommendations and is limited to patients who have already considered all other treatments available for their type and stage of cancer. There is a national list of drugs available through the CDF (the National Cancer Drugs Fund list) and the Fund also considers applications on behalf of individuals for other drugs not on the list, usually to treat rare cancers or for unlicensed use.

More than 60,000 people have accessed drugs through the CDF and there are around 2,000 new patients accessing it every month. Nearly half of all patient notifications to the fund are for drugs supplied by three manufacturers: Roche, Janssen and Novartis. The fund covers the cost of a drug but the costs of delivering the treatment are met by NHS England. The Chemotherapy Intelligence Unit in Oxford was commissioned to collect data on the CDF. No figures have been reported to date but these will be published before the 2015 election.

The consultation proposed that if the price of a medicine was considered too high, the Government would ask for further evidence on its efficacy, or ask the company to lower its price. The assessment process also had the potential to increase prices if, for example, it found evidence of greater efficacy. The feedback to this consultation was largely positive with most responders supporting the move to VBP.⁶ However, there were concerns over how to determine price, including how to price a drug that can be used to treat different conditions or patient groups (for which its value could be varied). Other concerns included potential discriminatory effects and how unmet need (medical need that is not adequately met by an existing therapy), innovation and real world evidence could be taken into account. VBP was planned to apply to all new branded medicines introduced from 2014. Medicines already covered by the PPRS would be subject to a new scheme from 2014.

Role of NICE in Value Based Assessment

In March 2013, the Government announced that NICE would take a central role in assessing the value of new medicines.^{7,8} The assessment process would be based on an amended version of existing NICE methods. In June 2013 DH confirmed that VBP would be introduced in January 2014. A month later, DH provided NICE with terms of reference for the development of what was referred to as Value Based Assessment (VBA).⁹ The different terminology (VBA as opposed to VBP) reflected the fact that NICE would conduct the value based assessment of the drug and DH would use it as the basis of price negotiations with industry. The terms of reference stated that NICE should:

- adopt the same benefit perspective for all technologies within the scope of VBP, and for displaced treatments
- include a “simple system of weighting for Burden of Illness (BoI) that appropriately reflects the differential value of treatments for the most serious conditions”
- include a “proportionate system for taking account of Wider Societal Benefits” (WSB) by measuring the effects of a condition on productivity (such as ability to work) and consumption (for example of health or care services).

NICE Consultation on Value Based Assessment

In November 2013, the Government announced that a new PPRS had been negotiated (Box 1) but confirmed that it was still committed to introducing VBA for new branded medicines. In early 2014, NICE produced proposals for amending its appraisal methods to take into account BoI and WSB.¹⁰ Rather than trying to measure wider societal benefits as recommended by DH - by assessing effects on productivity and consumption - NICE proposed to focus on the loss of health caused by a condition which it termed Wider Societal Impacts (WSI). The consultation suggested using QALY shortfalls to measure WSI and BoI:

- Wider Societal Impacts would be assessed by calculating absolute shortfall. This is the total QALYs expected due to a condition subtracted from the total QALYs expected for people with the same age and gender without the condition. This varies with age; the younger the patient the greater the loss of QALYs.
- Burden of Illness would be assessed by calculating proportional shortfall. The absolute QALY shortfall calculated above is divided by the number of future QALYs expected by people the same age and gender without the condition at the time of treatment. This is less sensitive to age.

NICE proposed setting a maximum cumulative weight of 2.5 for WSI and BoI combined. This is the same as the current maximum weighting that can be currently applied by Appraisal Committees for end of life (EoL) treatments. This would effectively cap the maximum acceptable threshold at £50,000/QALY gained ($£20,000 \times 2.5 = £50,000$).

More than 900 consultation responses were published in September 2014. A meeting of the NICE Board in the same month concluded that it could find no agreement in the responses to the proposed changes. The NICE methods thus remain as they were prior to the consultation. The consultation itself raised wider issues (discussed in the following sections) including the possibility of discrimination in use of BoI/WSI and weighting and threshold concerns.

Discrimination

Any system of decision making which chooses between different possible uses of healthcare funds by definition discriminates between patients groups or conditions. Measures that attempt to capture WSI and BoI will discriminate in favour of those treatments that provide the highest value to society. For example, measures that count the cumulative BoI reflect the fact that younger people have a greater potential health loss (they experience a condition

for longer, or death is more premature). Conditions that predominantly effect older people have lower BoI, so measures involving BoI may discriminate against older people because they discount the duration of QALY loss. Evidence from surveys on what society values is limited and mixed. What little evidence there is suggests greater public support for placing more value on QALYs for those with a higher BoI, than for those needing EoL treatments.

Weighting and threshold

The rationale for the proposed 2.5 weighting for BoI and WSI was that they both overlap with the EoL criteria. This has led to concerns about the arbitrary nature of, and the lack of supporting evidence for, this proposed weighting.

There is also debate among health economists over whether the NICE £20,000 threshold is appropriate.^{11,12} Setting this too high (as some are concerned it currently is) could result in displacement, in which patients elsewhere in the system will be denied health benefits from other types of treatments.¹³ Too low could deny patients access to innovative new drugs. There is evidence that NICE currently operates nearer to its upper threshold of £30,000, raising concerns over an ‘acceptance creep’, in which higher drug prices are accepted for less clinical benefit. However, industry groups point to the fact that the threshold has not been formally reviewed since NICE was first established.

The Cancer Drugs Fund and Wider Reform

The attempts to introduce a value-based approach to drug-pricing raise issues concerning the future of the CDF and the need for wider reform, discussed in the sections below.

Future of the CDF

The CDF has been confirmed until 2016, but opinions over its future thereafter differ. For instance Breakthrough Breast Cancer is calling for the CDF to be extended across the UK, until 2020. Cancer Research UK’s (CRUK) campaign to ‘Cross Cancer Out’ calls for greater public awareness and equal access to innovative radiotherapy, surgery and effective cancer drugs, including drugs targeted to patients’ tumours. Some cancer specialists argue that money spent on the CDF displaces money for other treatments. They suggest better outcomes might be obtained by channelling funds into prevention, early diagnosis, radiotherapy and surgery rather than into expensive new drugs.¹⁴ In 2012, underspend on the CDF was invested into radiotherapy which CRUK suggest had huge benefits.¹⁵ The CDF could also potentially reduce the incentive for companies to gain a positive clinical and cost effectiveness recommendation from NICE. Finally, the lack of consistent data collection and the tendency for overspend is also a concern.¹⁶

Consultation on the CDF

In October 2014, the CDF consulted on proposed changes to the way in which it assesses cancer drugs.¹⁷ It proposed:

- using the median cost per patient of a drug treatment, in conjunction with clinical benefit, to evaluate treatments

- determining thresholds for removing (de-listing) drugs from the national list (Box 3) and including new drugs on it depending on the amount of money left in the fund
- that all patients will finish treatments they have started but de-listed drugs will not routinely be given to new patients (unless a clinician submits an individual application)
- that patient access schemes (PAS) be considered when assessing drugs (these are used in current NICE appraisals when proposed by a drugs company, to reduce the price the NHS pays for a drug where it is not going to be determined cost effective at its current price).

Responses to the CDF consultation

There was general support to remove those medicines that have not demonstrated clinical benefit to patients. Following the consultation the Standard Operating Procedures of the CDF have been modified to introduce the new proposals.¹⁸ An initial review of the CDF drugs has been completed and 25 treatments, involving 16 drugs, will be de-listed from March 2015. These include treatments for breast, pancreatic and bowel cancer. However, the consultation identified a range of other concerns. Chief among these were that reducing access to drugs might lead to patient suffering and that the changes will both reduce the transparency of decision making within the CDF and result in duplication of effort between the CDF and NICE. Other concerns focused on the different cost-effectiveness measures used by the CDF (primarily based on the cost of the drug) and NICE (cost for each QALY gained) and whether the CDF will de-list drugs “that are the only proven drug treatment therapy for a particular condition”. CDF has pledged not to do this but there is debate about which drugs fall into this category.

Wider Reform

There is widespread consensus about the need for a fair mechanism for pricing and appraisal of all drugs. The Association of the British Pharmaceutical Industry (ABPI), NICE and others suggest reforming the way that drugs for rare conditions (orphan drugs) are funded, as well as the funding of innovative new drugs and cancer drugs.^{19,20} ABPI further notes the trend towards developing ‘personalised’ drugs, which are only suitable for small groups of patients, and suggests there is a need to determine the NHS’s willingness to pay for such treatments. The ABPI is keen that the PPRS payments that cover overspend on medicines (see Box 1) should lead to further NHS spending on drugs (as in Scotland’s New Medicines Fund, see below). It further suggests that spending above and beyond the PPRS cap represents an opportunity for the Government to ensure access to innovative new drugs. However there are concerns that this strategy could fuel unsustainable growth in spending on drugs and make future drug price renegotiation difficult. Future changes to drug pricing and appraisal could include consideration of:

- real world data on quality of life and clinical benefit from sources such as those in Box 4
- NICE’s Highly Specialised Technology evaluations that appraise drugs for very rare conditions

Box 4. Data collection for drug appraisal

Data on quality of life and the effectiveness of drugs arising from routes other than clinical trials can inform decision making, particularly for rare diseases. More real world sources of data are becoming available and could be used to inform appraisals. These include:

- Commissioning Through Evaluation, a scheme that enables a small number of new treatments to be funded to allow evidence on clinical and cost effectiveness to be collected.
 - Outcome-based schemes that are used where limited or no data are available to assess a treatment’s cost effectiveness. The Multiple Sclerosis Risk-Sharing Scheme is one such example.
 - The Systemic Anti-Cancer Therapy Dataset which collates national data on the use of chemotherapy.
 - Early Access to Medicines, a scheme that aims to give patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorisation where there is a clear unmet medical need.
 - Adaptive Licensing, which allows the early authorisation of a medicine in a restricted patient population. Iterative phases of evidence-gathering may then allow adaptation of the authorisation to allow broader patient populations to access the medicine.
- Scotland’s New Medicines Fund, financed by the rebate made to the Scottish Government under the PPRS
 - Scottish Medicines Consortium’s Patient and Clinician Engagement group that gathers information about the impact of a drug on patients’ lives that may not always be fully captured by conventional assessments

In November 2014, the government announced the ‘Innovative Medicines and MedTech Review’. This will be a thorough review of discovery, development, evaluation and adoption of new treatments. One of things it will have to consider is whether the NHS should only be aiming to maximise health gain from its budget or whether it has a wider role to play, for example in stimulating UK medical innovation. The Review will report in summer 2015.²¹

Endnotes

- 1 *The Pharmaceutical Price Regulation Scheme 2014*, Finance and NHS/Medicines, Pharmacy and Industry Group/17080, Dec 2013
- 2 *Equity and excellence: Liberating the NHS*, DH, Jul 2010
- 3 *Guide to the methods of technology appraisal*, NICE, Apr 2013
- 4 *A new value based approach to the pricing of branded medicines*, DH, 2010
- 5 *Extent and causes of international variations in drug usage*, Sir Mike Richards Jul 2010
- 6 *Eighth Report, National Institute for Health and Clinical Excellence*, Health Select Committee, Jan 2013
- 7 *NICE will take responsibility for assessing the full value of medicines*, DH press release, Mar 2013
- 8 *Expert body given responsibility to look at the benefits medicines bring to wider society*, DH press release, Jun 2013
- 9 *VBA, The response to public consultation on changes to aspects of NICE’s methods for appraising new health technologies*, NICE, Sep 2014
- 10 *Value Based Assessment of Health Technologies*, NICE, Mar 2014
- 11 Dakin et al. *Health Economics*, 10.1002/hec.3086, 2014
- 12 McCabe C et al, *PharmacoEconomics*, 26,9, 733-744, 2008
- 13 Claxton K et al, *Health Econ*, 24, 1, 1–7, 2015
- 14 *Eight thousand patients to benefit from advanced cancer treatment*, DH, 2012
- 15 *The Radiotherapy Innovation Fund. An Evaluation*, CRUK, Jul 2013
- 16 The Kings Fund Blog, John Appleby, *The Cancer Drugs Fund: Inequitable and inefficient?*, Sep 2014
- 17 *Operation of the Cancer Drugs Fund 2014/15, Consultation Report.*, NHS England, 2014
- 18 *Standard Operating Procedures: The Cancer Drugs Fund (CDF) Guidance to support operation of the CDF in 2014-15* NHS England, 2014
- 19 *2015 Manifesto, Our agenda for change*, ABPI, 2014
- 20 *NICE calls for a new approach to managing the entry of drugs into the NHS*, NICE press release, Sep 2014
- 21 *Major investment in life sciences*, DH/BIS press release, Nov 2014