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Value-based pricing for pharmaceuticals: Its role, specification and prospects in a newly devolved NHS

CHE Research Paper 60

Value-based pricing for pharmaceuticals: Its role, specification and prospects in a newly devolved NHS

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February 2011

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Summary

In December 2010, the Government launched its consultation on its plans to change the way medicines are priced in the UK. By 2014, a system of 'value-based pricing' (VBP) will replace the current Pharmaceutical Price Regulation Scheme (PPRS), which is a voluntary agreement between the Department of Health (DH) and the pharmaceutical industry whereby companies negotiate profit rates from drug sales to the National Health Service (NHS) every 5 years utilising price and profit controls. The concept of VBP emerged from the Office of Fair Trading (OFT) report in 2007, which recommended that prices of individual pharmaceutical products should reflect their 'clinical and therapeutic value to patients and the broader NHS'. The introduction of VBP provides an opportunity to found pharmaceutical pricing and access to new health technologies on sound principles; reflective of social values and the reality of a budget constrained NHS. This requires: clarity on key issues of principle and social value; as well as critical details of how it will be implemented and operate. This Research Report describes a potential framework for VBP in the type of devolved NHS envisioned in recent government reforms. It also considers some of the critical features of VBP arrangements which need to be appropriately specified if the potential benefits are to be realised.

Any assessment of value must be founded on the type of transparent scientific assessment of the costs and benefits offered by a technology which is already embodied in the National Institute for Health and Clinical Excellence (NICE) appraisal process. It is also important that the assessment of the health expected to be forgone elsewhere in the NHS due to additional costs displacing other NHS activities, i.e., the cost-effectiveness threshold, is evidence based. In addition, transparent rules of how prices might be negotiated based on such assessments are required. Rules, which are reflective of markets in other sectors where investment is also protected by patent, provide a suitable model. The triggers for when VBPs, once set, would be reconsidered must include patent expiry and the entry of cheaper generics and significant new evidence becoming available. Without the former, the NHS may never benefit from innovation even in the longer run and, without the latter, incentives for manufacturers conducting evaluative research will be undermined and/or the NHS will not realise the benefits of publically funded research.

The details of how the VBP scheme will be implemented and operate are also important. For example, an effective vehicle for price negotiation might be value-based rebates through a PPRS-type arrangement rather than reductions in UK list prices. Since the UK represents only 3% of the world market the influence of VBP in the UK on global investment appears limited. However, there is an opportunity to use the scheme to align incentives for local prescribing, which, in the absence of mandatory NICE guidance on the use of health technologies after 2014, is especially important. Since VBP is necessarily linked to particular indications and subgroups, there is an inevitable link between price and volume. Therefore, any agreed rebate needs to be combined with volume agreements which might be specified at a national or local level.

The introduction of VBP also poses the question of whether there are other aspects of social value which ought to be included. Although supporting innovation is important, the notion that paying 'innovation premiums' for particular products has little to commend it. There are also other aspects of value, however, which might be included, such as effects outside the NHS and/or different weights that might be attached to health improvement in different circumstances. Critically, extending the scope and/or weights applied to the benefits of a drug must be matched by accounting for those same aspects of benefit that are likely to be given up to release resources for new technologies. Finally, uncertainty in the assessment of costs and health benefits, the need for further evidence and the irrecoverable costs committed when the NHS purchases a technology also needs to be reflected in initial VBP at launch and in subsequent reappraisal and renegotiation.

1. Introduction

Since the advent of the National Institute for Health and Clinical Excellence (NICE) in 1999, the NHS has used formal methods of cost-effectiveness analysis as a major input into its decisions about the use of new medical technologies, principally pharmaceuticals. NICE's rationale, processes and preferred methods are grounded in the reality of a fixed NHS budget constraint such that, if a new and more costly medication is to be funded, other services will need to be curtailed or eliminated to release the necessary resources which will have a negative impact on the health of other types of patients.[1] Throughout this period it has been clear that the value for money of new pharmaceuticals is strongly influenced by the prices set by manufacturers which are typically determined with a view to the international market rather than the specific requirements of the NHS. Unlike some other countries like France and Italy, the NHS does not engage in a process of determining the price at which it is willing to adopt a specific new product. In contrast, the UK government's approach to the financial regulation of the pharmaceutical industry has been defined over the last 50 years or so through the Pharmaceutical Price Regulation Scheme (PPRS).[2] This voluntary agreement with the pharmaceutical industry has sought to strike a balance between creating the conditions conducive to a thriving pharmaceutical sector and value for money in the medicines purchased by the NHS. However, the potential has been identified for improving upon the current system of regulating the overall rate of return a company achieves on capital invested by assessing the price at which a given product would represent good value to the NHS.[3] In 2007 the Office of Fair Trading (OFT) recommended an alternative approach for the PPRS by which the prices of individual pharmaceutical products reflect their 'clinical and therapeutic value to patients and the broader NHS'.[4] This concept of 'value-based pricing' (VBP) was broadly welcomed by the previous administration[5] and is now a key feature of Coalition Government's policy towards the PPRS which is due to be renegotiated in 2014.[6]

This fundamental change in pharmaceutical regulation offers an opportunity to align the incentives of manufacturers, the NHS and individual prescribers. To realise these gains, however, there needs to be careful specification of the scheme, with emphasis on a clear definition of value. The principles of value-based pricing (VBP) might be better described as 'benefit based pricing': the price at which the health benefits of a new product are no less than the health benefits forgone as a result of services displaced to fund it. The debate following publication of the OFT report[4] focused primarily on the role VBP would have in aligning the incentives for investment in research and development with the needs of the NHS. However, the type of devolved NHS envisaged in the recent white paper[6] suggests that VBP has the potential also to have a much more significant role in aligning incentives for prescribers. In other words, VBP has a role in aligning incentives 'up and out' of the NHS but also ‘down and in’. This paper examines the principles of value-based pricing in a collectively-funded health care system like the UK NHS (Section 2). It also develops a series of proposals for how these principles might be best implemented, highlighting particularly critical issues (Section 3). Finally it examines whether and how other aspects of value might be taken into account (Section 4).

2. Principles of value-based pricing

The value of a technology to the NHS requires a clear definition of the social objectives of collectively funded health care and a means of measuring whether the technology, at the price offered to the NHS, meets these social objectives. If the primary purpose is to improve health across the NHS then a technology can be said to be of value if the health expected to be gained from its use exceeds the health forgone as other NHS activities are displaced by its additional cost, i.e., if it is expected to improve overall health outcomes across the NHS. These assessments of health gained and forgone are already central to the NICE Technology appraisal process.[1]

2.1 Value price and cost-effectiveness

The concept of value and its relationship to price and cost-effectiveness is illustrated in Figure 1.[7] The new technology offers a gain of two units of health outcome, here expressed as quality adjusted life years (QALYs), at an additional cost to the NHS of £20,000 (an incremental cost-effectiveness ratio of £10,000 per QALY gained). The key question is whether the expected gain of 2 QALYs from this innovation is greater than the health outcomes (in QALYs) forgone elsewhere due to other NHS treatments being displaced by the additional cost.

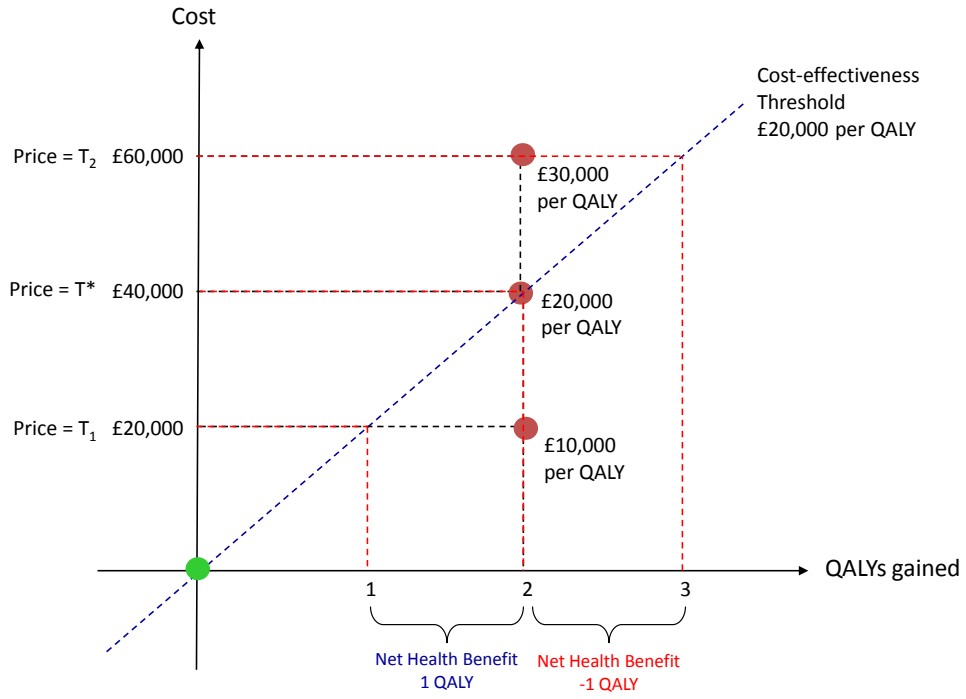
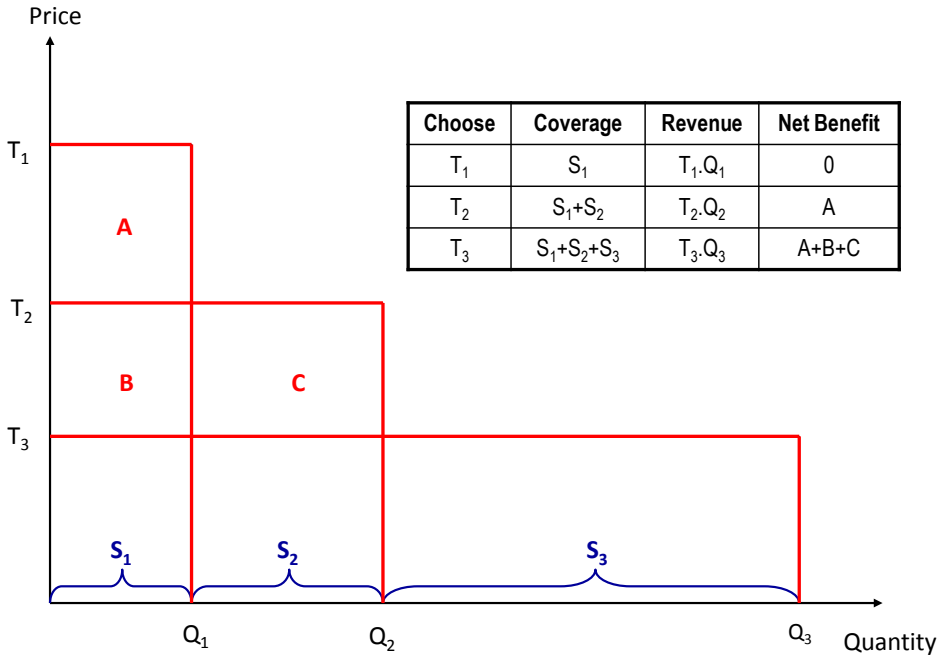


Figure 1 Value, price and cost-effectiveness (adapted from Claxton et al.[7])

If the transaction price is set at T_1 then the additional NHS costs of £20,000 will be expected to displace 1 QALY elsewhere, based on the lower range for cost-effectiveness threshold adopted by NICE (see Section 3.6.3). Therefore, at a price of T_1, the technology offers net health benefits to the NHS (it is expected to be cost-effective). At higher prices more health will be displaced elsewhere, and when price is equal to T* the expected gains in health outcome are just offset by the health expected to be forgone by the higher NHS costs (£40,000). At this point, there are no net health benefits to the NHS, so T* represents the maximum transaction price the NHS can afford to pay for the benefits offered by this technology. T* represents the value (benefit) based price so, if the NHS was to pay more than T*, e.g., T_2, the technology would damage overall health outcomes because the health expected to be forgone as a consequence of the higher costs (£60,000) would exceed the health expected to be gained.

Since manufacturers already have an incentive to price their products to the point where net benefits are zero (at T*), with a single indication and no identified subgroups all the value of the innovation will be appropriated by the manufacturer through the sales revenue they receive (the total value of the innovation to the NHS for the volumes associated with the indication (Q*) is T*.Q* and all of this value is appropriated by the manufacturer in the form of revenue). The NHS has no incentive for early uptake of the technology and, in the short run at least, ought to be indifferent between early access and delaying until patent expires and cheaper generics enter the market (see Figure 3)[a].

If there are multiple indications and/or subgroups within a single indication an 'average' value-based price, which would make the technology just cost-effective 'on average' over the relevant indications and subgroups, would allow all value to be appropriated by the manufacturer (it would be equivalent to price discrimination). Some share of value will accrue to the NHS (providing an incentive for early uptake) if the rules of VBP are reflective of other markets in other sectors which are also protected by patent. Elsewhere temporary monopolies (created by patent protection) can freely select their price but have to be mindful of the implications that a higher price will have on sales. Applied to pharmaceuticals, manufacturers may choose a high price but they must then accept lower sales volumes. Alternatively, if they choose a lower price which provides unrestricted coverage across all indications and subgroups (where there are zero net benefits for the marginal indication or subgroup), there ought to be some positive net benefit for some patient groups. In other words, the value of the innovation is shared to some extent during patient protection just like in other markets.[7-8] This principle of 'marginal' value-based pricing is illustrated in Figure 2 where the technology is most cost-effective for subgroup S_1 and least cost-effective for subgroup S_3.



Choose	Coverage	Revenue	Net Benefit
T_1	S_1	$T_1.Q_1$	0
T_2	S_1+S_2	$T_2.Q_2$	A
T_3	$S_1+S_2+S_3$	$T_3.Q_3$	A+B+C

Figure 2 A menu of price and volume (adapted from Claxton et al[8])

The assessment of expected costs and effects across indications and/or subgroups essentially presents the collective NHS demand curve to the manufacturer. Just as a temporary monopolist in other markets may chose where to locate, pharmaceutical manufacturers may choose from the menu of price (T_i) and associated coverage (Q_i). For example, if they were to choose T_1 their sales volume would be restricted to Q_1 (revenue of $T_1.Q_1$). Alternatively choosing a price of T_3 would be associated with Q_3 of sales across all the subgroups of indications (S_1 to S_3 with, in this case, higher revenue of

[a] Once a drug has been developed, the longer term benefits which follow generic entry (see Figure 3) will be realised whether or not the NHS purchases the more expensive brand. However, in the longer run such a policy would undermine incentives for development of future drugs which will also offer net benefits on patent expiry, although the adverse impact on dynamic efficiency for the NHS depends in part on the UKs share of the world market.

$T_3.Q_3$). The NHS would receive some share of the total value with net benefits of A+B in S_1 and C in S_2 (T_3 is the VBP for S_3, the marginal subgroup). Setting aside the marginal production costs, manufacturers have an incentive to maximise revenue (located at unit price elasticity) which in Figure 2 is at T_3,Q_3. However, this need not be the case depending on how subgroups are defined and how cost-effectiveness and coverage changes across subgroups. It should be noted that a choice of T_1,Q_1 is no worse, for the NHS as a whole, than an 'average' value-based price which would make the technology just cost-effective 'on average' over Q_3 (also equivalent to price discrimination, i.e., T_1 for S_1, T_2 for S_2 and T_3 for S_3). In both cases the net health benefits to the NHS will be zero. The only difference between these outcomes is the way health gains and health losses are distributed. Clearly manufacturers will have a strong incentive not to identify any subgroups since the 'average' VBP will allow them to appropriate all the value.

Sometimes other mutually beneficial 'deals' could in principle be done, e.g., a manufacturer who would otherwise choose T_1, Q_1 may be willing to agree to price greater than T_3 for Q_3, which would still offer net health benefits to the NHS while increasing their revenue. Such 'deals' are not available to temporary monopolists in other types of market as they depend on negotiation with a monopsony purchaser. Although such beneficial deals are possible, they must be balanced against the potential adverse outcomes once the principle of transparent rules mirroring other markets is conceded. Firstly, there will be a danger that the monopsonist may start to exploit its power, forcing down prices below T_3 by offering all or nothing deals to manufacturers and undermining patent protection. Even the possibility of this type of outcome now or in the future will undermine confidence that investment returns can be realised, making research and development more costly. Secondly, there may be a danger that those negotiating on behalf of the NHS could be 'captured' by the manufacturer especially when 'deals' are possible rather than following simple and transparent rules for which accountability and redress is possible (see Section 3.6.4). Thirdly, it is not at all clear that the monopsonist will have the upper hand in such negotiation if the level of transparency and accountability expected of public bodies is greater than that expected of manufacturers. This seems especially problematic when there is inevitable political pressure to provide NHS access to a technology, i.e., negotiators may be susceptible to all or nothing deals from manufacturers which damage the NHS (e.g., T_1, Q_3). Finally, if there is a proper analysis of subgroups within the relevant indications,[9] it is very unlikely that high price and low coverage options will be revenue maximising (as long as list prices are preserved – see Section 3.1) . However, if 'deals' are possible, T_1,Q_1 is very likely to be taken as a negotiating starting point by manufacturers even if it does not represent their revenue maximising position. For all these reasons simple and transparent rules that mirror other markets (a choice of 'marginal' value-based prices and associated volumes) should be regarded as the most appropriate basis for VBP. This balances the interests of all sides (including other sectors which compete for government support, public money and private capital) and offers clear, accountable and predictable signals with the possibility of redress.

2.2 The future value of a product

A key issue with VBP is whether the concept of value fully reflects the benefits to health systems in the longer term.[7-8 10-11] Even where there are no net benefits during patent protection, in the longer run, if cheaper generics become available, following patent expiry then the health care system may ultimately benefit. This requires certain conditions to be satisfied – most importantly, that the generic market remains competitive and that the prices of future newly patented drugs reflect their value when compared to the cheaper generic versions of the old brand and, if they do not, then all prescribing switches to the generic version of the old brand. If any of these conditions are not met, the heath care system, as a whole, may never benefit from innovation and the low net benefits observed in the short term will be realised in the longer run as well. However, even when these conditions are met the private sector may still retain a significant proportion of value in the long run.[11]

An example of this is illustrated in Figure 3 which shows the cumulative value of an innovation over time (discounted at a rate of 3.5%) and how this total value is shared between the manufacturer and the health care system. Initially, during patent protection at the VBP (for a product with a single indication and no identifiable subgroups), all value is appropriated by the manufacturer. At 15 years from launch the patent on the product expires and, assuming that competitive generics enter, the market price of that product will then fall. Assuming that: i) competitive generic entry occurs reducing prices to 25% of the brand; and either ii) all prescribing is switched to generics (and/or the price of the brand is reduced to generic levels); and iii) any new patented drugs are priced relative to the generic

version of the old, then the health care system will begin to benefit from the innovation and start to share some of the value of the innovation with the manufacturer. However, the manufacturer will still retain most of the value even in the long run. In this example, at 30 years the manufacturer appropriates 72% of the value and even at an unbounded time horizon (i.e. assuming that the innovation is forever relevant) it still retains 57%. Of course, the manufacturer's share will be higher (and the NHS share lower) if any of the three assumptions above do not hold and none can be taken for granted.

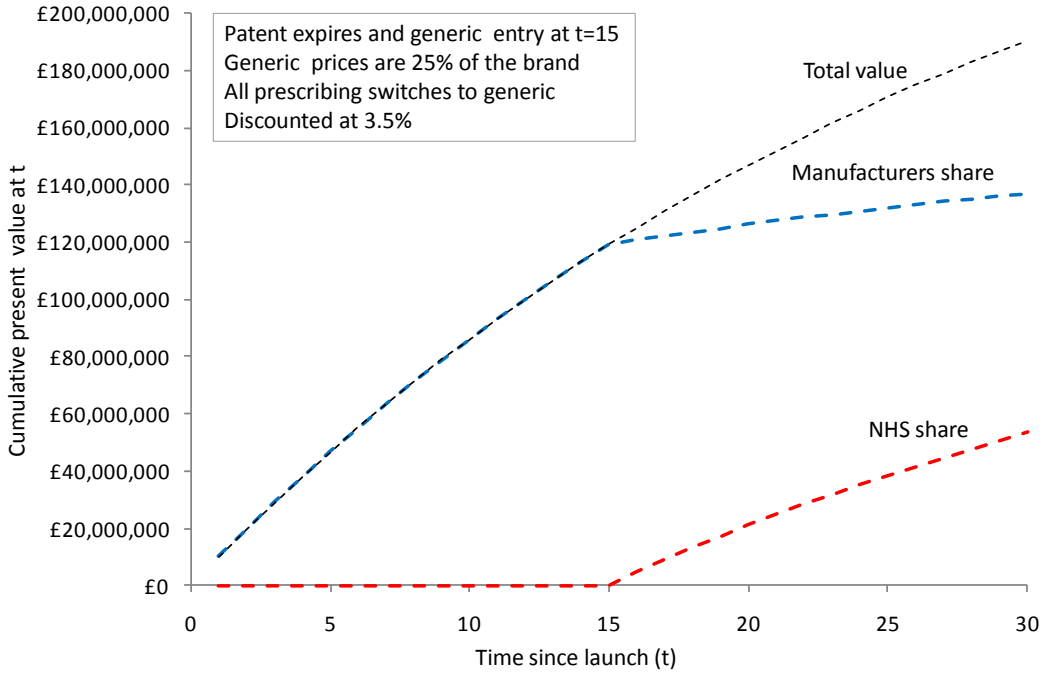


Figure 3 Sharing value over time

Any system of pricing and reimbursement should not undermine the protection offered by patents by exploiting the monopsony power of collective purchasing. The current system does not do so nor do the proposals for VBP: both provide manufacturers with an incentive, and the freedom, to price their products up to the point where net benefits are zero. The NHS will start to share the value of the innovation with the manufacturer following patent expiry, but should this potential future value also be transferred to the manufacturer? This would mean incurring negative net benefits during patent protection which may only be recovered later if the three assumptions described above hold. For the NHS this would be worse than indefinitely extending patents (that would assure zero rather than negative net benefits). It is also unlikely to be efficient since the health care system and other parts of the public sector contribute directly and indirectly to innovation through, for example, the infrastructure of basic science and clinical research, so should share the rewards to align investment incentives appropriately. Finally there are legitimate questions of social value: how the value of innovation should be shared across society (NHS, the pharmaceutical and other sectors for the economy) and between NHS patients, UK citizens and manufacturers and their shareholders often located outside the UK.

Value-based pricing aligns returns in the UK with NHS needs (with some, albeit limited influence on global investment decisions) as long as the measure of benefit captures those aspects of outcome that are socially valuable (see Sections 4.2, 4.3 and 4.4) and an assessment of what is likely to be displaced is empirically based (see Section 3.6.3). Nevertheless, it is beyond dispute that some innovations today will provide the basis for subsequent innovations which may be even more valuable in the future. The question is who should anticipate the benefits of the possibilities of further innovation and when should the NHS pay for them (see Section 4.1).

3. Value-based pricing in a devolved NHS

Although currently there is little detail about how VBP will work in the NHS, more details have emerged about the future role of NICE. Importantly, from 2014, not only will NICE guidance no longer be mandatory, it will no longer issue guidance about whether or not a technology is or is not cost-effective or whether it is recommended for NHS use.[12] Taken in isolation this announcement appears, at first sight, to be a significant step back from evidence based policy in the NHS. However, the Government has also made clear that VBP, based on NICE appraisal, will be introduced at the same time.[12] If successful, VBP could well mean that the type of mandatory guidance issued by NICE based on prices offered by manufacturers will become unnecessary. However, the success of a VBP scheme for a newly devolved NHS depends critically on details of the type of scheme envisaged and of how it might be implemented.

3.1 An effective vehicle for price rebates?

A substantial segment of the world market references UK drug prices (estimated at 25% by OFT[12]). Therefore, manufacturers will typically be unwilling to lower their list price in the UK to match a VBP relevant to the NHS. Over recent years there has been an increase in patient access schemes offered by manufacturers during the NICE appraisal process.[13] These schemes essentially offer a discount to the NHS whilst maintaining a product's list price. This is achieved by defining a marker of clinical success which has to be achieved in each patient in order for the full list price to be paid. However, such schemes are already costly to administer, and there is evidence that the discounts offered are not always recovered by the NHS.[14] Such schemes are unlikely to provide an effective vehicle for administering VBPs after 2014 when the scheme will apply to all new drugs.

A more effective and less costly mechanism would be to use the current PPRS mechanism to recoup any discrepancy between the list and the VBP. For example, where the value-based or transaction price (T*) was less than the list price (L), manufacturers would be asked to agree to pay the rebate (L-T*) through the PPRS. The rebate might be paid directly or recovered through price cuts (with 'modulation' where companies have flexibility in deciding which products have prices reduced) for those drugs not subject to VBP. This mechanism has a number of advantages. Firstly, it removes the administrative burden of patient access schemes from the local NHS. Secondly, it allows manufacturers to maintain their list price which means they can isolate the effect of VBP from international referencing and be more likely to agree to such rebates. Finally, manufacturers retain freedom to set list prices and might also retain freedom to modulate the price of other products not covered by VBP.

3.2 Incentives for NHS prescribers

It is important that any VBP scheme aligns incentives for local prescribing, especially in the absence of mandatory NICE guidance after 2014. This will require the DH to hold back that part of drug expenditure which will be covered by value-based pricing and allocate L or T* to local prescribers based on use and whether or not a rebate agreement with a manufacturer is in place. This should not be regarded as an administrative budget for pharmaceuticals but rather an allocation mechanism from DH to local prescribers based on value. Some assessment will be required of likely expenditure to ensure that DH retains sufficient resources to reimburse local prescribers.

For example, those drugs included in the VBP scheme and for which there is an agreed value-based rebate, the DH could reimburse NHS prescribers at the list price in the knowledge that they will recover the difference from the manufacturer through the agreed rebate. Importantly, NHS prescribers would retain an incentive to negotiate local discounts (d) on the list price. Hence incentives are aligned: NHS prescribers will pay L-d, but receive L from the DH; DH will pay L to prescribers but receive a rebate of L-T* from the manufacturers; and manufacturers will receive L-d but pay a rebate of L-T* to the DH. Alternatively, DH could only reimburse local prescribers the difference between list price and value (L-T*)[b]. The difficulty is that this type of reimbursement would

[b] Local prescribers would face the value-based cost of the drug when making prescribing choices between drugs within the VBP scheme and those not covered by VBP. However, if list price is reimbursed by DH then the local cost of drugs with a VBP rebate in place will be much lower than those outside the scheme, providing a strong incentive to prescribe the VBP drug. As long as the VBP are set appropriately and volume agreements are in place (see Section 4.4) the accelerated uptake of VBP drugs may well be appropriate.

reveal T*, which, given the implications of international reference pricing, would make manufacturers reluctant to agree rebates.

Where the list price is already below the VBP no rebate is necessary so manufacturers will receive L-d from prescribers and DH will pay L to prescribers. Any local discounts achieved (L-d) can be used locally to provide other NHS care.

It is possible that, for particular products, manufacturers will not be willing to agree to a value-based rebate of (L-T*). This is much more likely if they believe that local prescribers will be willing to pay L anyway. Therefore, it is important that VBP is used to align incentives for prescribers which, in turn, will provide an incentive for manufacturers to agree to a rebate. This might be achieved by the DH not reimbursing prescribers when an agreement is not in place. In these circumstances incentives are also aligned: NHS prescribers will remain free to choose to purchase the drug. If the appraisal is reflective of the costs and benefits they face locally, they ought to be willing to pay up to T*. Therefore, they may nevertheless choose to prescribe if they can negotiate local discounts so that L-d=T*, or pay more than T* if they believe that there are greater than expected benefits for particular patients.[c]

This has a number of advantages. Firstly, it leaves prescribers free to negotiate local discounts and/or chose to prescribe because the value to a particular patient is believed to be higher than T*. Secondly, manufacturers have an incentive to agree a rebate since prescribing costs (L-d) will fall on local prescriber's budgets. Local prescriber's decisions may , however, be informed by the VBP that would have been acceptable to DH. This may provide an additional incentive for the manufacturer to agree rebates since failure to do so will reveal T*, with potential consequences in other markets through reference pricing. If an agreement is in place, local prescribers do not need to know T* and what rebate was agreed between manufacturers and the DH. Whether a scheme which retains transparency and accountability can also effectively withhold the information which would allow T* to be derived when an agreement is made is discussed in Section 3.6.4.

Rebate agreements alone, however, would incentivise over prescribing of those products where an agreement is in place, putting the NHS at risk of unjustified growth in expenditure on branded pharmaceuticals. Since any VBP relates to particular indications and or subgroups (see Figure 2), any rebate also needs to be linked to volumes associated with the agreed T*.

3.3 Value-based rebate with volume agreements

To establish accountability and to give clear and predictable signals to manufacturers, it is important that price 'negotiation' avoids undermining incentives. On the one hand, the process needs to ensure that incentives for innovation on the part of manufacturers are preserved by avoiding the NHS exploiting or appearing to exploit its monopsony power. On the other hand, manufacturers should not be permitted to appropriate all the value of new products through either price discrimination (that is, agreeing a different T for each indication and sub-group - see Figure 2) or all or nothing deals (see Section 2.1). To achieve this balance, it is important to have transparent rules which mirror markets in other sectors where innovation is also protected by patent. In other markets the temporary monopoly created by patent protection may choose where to locate on the market demand curve.

Any VBP will relate to a particular indication, groups of indications or particular patient subgroups within an indication. Where there are a number of subgroups and/or indications, the NHS demand curve is represented by the combinations of evidence based T_i and associated Q_i (see Figure 2). The manufacturer should be free to choose from this menu, i.e., they may choose a higher T_i but associated with a narrower indication/subgroup and, therefore, a lower volume, Q_i, associated with it. Therefore, for each drug once a value-based rebate is agreed there would be a single T^*, Q^* chosen from the available T_i,Q_i combinations available.

Mandating that prescribing be restricted to the indication(s)/subgroup(s) associated with the particular T* included in the agreed rebate would seem to undermine the principles of a more devolved NHS, where NICE guidance informs rather than determines which drugs should be used and for which

[c] This would avoid DH effectively reducing the local price of these drugs compared to those not included in the VBP scheme. That part of drug expenditure retained by DH to reimburse the drug if an agreement had been reached would need to be allocated back to the local NHS in the following period to avoid an effective cut in local resources.

particular types of patients. However, since prescribers will be reimbursed at list price, there is a danger of over prescribing as there would be no incentive to restrict their prescribing to the indications and subgroups (Q*) associated with the agreed T*. This would place the NHS and DH at considerable risk of unjustified growth in prescribing expenditure for indications not covered by the rebate agreement where T* exceeds the value in these groups. There are broadly two responses to this problem.

The first is that reimbursement could be made conditional on the prescriber demonstrating that it was for the indication(s) and subgroup(s) covered by the agreement. The costs of prescribing beyond the coverage of the rebate would not be reimbursed and fall on prescribers' budgets. There are number of ways in which the administrative burden of such conditions might be reduced. For example, reimbursement from DH could be paid periodically based on routinely collected information about the number of patients treated which fall under the rebate agreement, i.e., the prescriber will be reimbursed at L up to the Q* relevant to their local patient population (if the subtleties of distinctions between subgroups are not routinely recorded, the Q* for the local prescriber might be based on national estimates of the proportion of the indication accounted for by relevant sub group). However, there are a number of alternative ways in which the rebate paid can be restricted to Q* without recourse to imposing mandatory guidance on local prescribers - a new role for the Healthcare Commission is one of many possibilities.

Alternatively (or in addition), a value-based rebate agreement could also include a volume agreement at a national level, where, for volumes up to Q*, a rebate of L-T* is paid. However, for volumes greater than Q* a greater rebate will be required because T* (what the DH ultimately pays) will exceed the value to the NHS. Any volume agreement should not place the manufacture at risk of incurring costs when they cannot directly control the volume of NHS prescribing. This could be achieved by setting the rebate for volumes greater than Q* as the difference between list price and the marginal production costs (C). Prescribing beyond Q* will not impose costs on the manufacturer since sales revenue will cover their costs. It may impose costs on the NHS if the value of the drug beyond Q* is less than C but then it is appropriately the responsibility of the NHS to ensure that prescribing is focused on the most valuable indications and subgroups (informed by NICE guidance and/or making reimbursement by DH conditional as discussed above). Of course, the marginal production costs of a new product are unlikely to be revealed by manufacturers, even if they have that information available. However, such costs are revealed by a competitive generics market. Therefore C and the greater rebate beyond Q* can be based on generic prices for comparable types of drugs. This arrangement has a number of advantages:

- The DH is not put at risk of significant increases in expenditure due to over prescribing where a value-based rebate is in place.
- The small risk pool of some local prescribers will not place them at financial risk and deter uptake when an agreement is in place for drugs with high list prices (the DH essentially pools this risk centrally).
- The speed of uptake of new drugs with high acquisition costs but where a value-based rebate is in place would be expected to increase with claimed positive spin offs, e.g. incentives for manufacturers to launch in the UK and an improved research environment within the NHS.
- Manufacturers are not put at risk of incurring net costs as a consequence of NHS prescribing above Q*.
- The costs to the DH and the revenue to manufacturers becomes more predictable than is currently the case where there is no VBP or Q* agreed.
- There is little incentive for manufacturers to devote resources to marketing beyond the indication(s) and/or subgroup(s) associated with the agreed T*.
- Manufacturers when offered the choice between higher T_i but with a lower Q_i, or lower T_i but with wider coverage of the agreement (a higher Q_i) are more likely to choose the latter if there is no strategic or financial advantage of agreeing a higher T_i but then hoping to market beyond the associated Q_i.
- Only volume would need to be monitored (possibly with an assessment of a local Q*) rather than monitoring the reasons for prescribing and then putting effort into restricting prescribing to indications and subgroups associated with T*, i.e., the incentives for prescribers are aligned with evidence of value embodied in NICE guidance while leaving them free to exercise clinical judgement.

- The type of appraisal conducted by NICE, which would inform value-based prices, also informs the assessment of Q_i for each T_i that might be agreed, based on evidence relevant to the NHS. This would provide some transparency and accountability in the assessment of both T_i and Q_i which, with current consultation rights in the NICE process as well as redress through appeals and judicial review, would provide a reassurance to manufacturers that both the T_is and Q_is on offer are an evidence based expression of the NHS demand curve for their products. Importantly, they would also be to a large extent predictable, providing a clear signal of likely returns for those making investment decisions in drug development.

Whether a national rebate-volume agreement would itself be sufficient, or whether incurring the additional costs of monitoring and/or establishing local volumes for conditional reimbursement would be appropriate might be established case by case. Clearly, where there is a large discrepancy between L and T* and where the agreed Q* is associated with only a small subset of all the patients who may benefit from the drug, then the latter is more likely to be worthwhile.

3.4 Reducing postcode prescribing

Postcode prescribing can be defined as geographical variation in access to NHS treatments for patients who are similar in terms of the characteristics which are relevant to the expected costs and benefits of those treatments. The existence of such variation may be regarded as unjust in a collectively funded *national* health care system. One fundamental problem has been that the way resources are allocated to localities has not necessarily reflected the local resource implications of national guidance issued by organisations such as NICE. NICE guidance issued through the Technology Appraisal (TA) Programme has been mandatory since 2001. The mandatory nature of this guidance should, in principle at least, have reduced variation in access to those drugs subject to TA guidance. The problem is that access to other health care, including other drugs, must be restricted to accommodate the additional NHS costs of such guidance. Therefore, reducing postcode prescribing for part of NHS prescribing can only increase geographic variations in access to other care not covered by such guidance. This unintended consequence of mandatory guidance is inevitable if resource allocation does not precisely match the costs of such guidance for local patient populations.

The type of value-based rebate scheme described above would to, some extent, overcome this problem since resources would flow from DH to localities based on the value of using particular drugs for agreed indication(s) and/or subgroups. Where a value-based rebate agreement is in place there seems little reason why geographic variation in access should persist even without mandatory national guidance. But more importantly, and unlike mandatory guidance, this will not lead to geographic variations in displaced health care as long as local prescribers are reimbursed by DH at the list price. However, when a value-based rebate is not agreed some variation in use would be expected, partly reflecting different assessments of value for particular patients, but also variations in local efficiency as well as the possibility that the allocation of the greater part of NHS resources may not fully reflect the local cost of providing access to the same care given the mix of the local patient population.

3.5 Implications for NHS costs

The DH could accept initially higher NHS costs as the coverage of VBP expands from 2014 due to the lag before agreed rebates are received, i.e., NHS initially pays L but receives L-T* in the following period. Alternatively the agreement could include provisions for ‘payment on account’ in the first year based on L-T* and an estimate of the likely volume (Q*) associated with the indication for which T* was estimated. In the following year a ‘balancing payment’ based on actual volumes could be made.

It should be noted that applying VBP only to new drugs will not offer any saving to the NHS or any net health benefits in the short term. In the longer run, as generics enter, the NHS will start to accrue benefits so long as VBP for future branded drugs are relative to generic versions of the old brand available at that time (see Section 2.2). However, applying VBP to existing technologies where the current price paid by the NHS is thought to be greater than its value (e.g., where branded drugs at premium prices continue to be prescribed despite equivalent generics being available) would offer an opportunity to reduce costs and generate benefit. Therefore, it would seem sensible to include into the VBP scheme at the outset some existing technologies (those where L>T* and which generate

large NHS expenditures) as well as new technologies. This could be used to ensure the scheme is cost neutral after the first year.

There may be circumstances when L<T*, in which case no value-based rebate agreement would be needed. However, in these circumstances, manufacturers will have a strong incentive to increase list prices so that L=T, increasing NHS costs. Therefore, including some existing drugs in the VBP scheme could also be used to offset the cost implications of these incentives.

3.6 Role of NICE

Existing methods[1] and processes[15-16] can already establish value-based prices of technologies. However, there are some critical issues which, if not properly addressed, could mean that VBP does more harm than good: increasing NHS costs and reducing overall health outcomes as expensive new technologies displace more valuable NHS activities. Maintaining the independence and robustness of evidence based methods of appraisal developed by NICE is fundamental. However, four other considerations are particularly critical for the success of VBP.

3.6.1 NICE appraisal process

Through its methods of appraisal and the process of deliberation by its advisory committees, NICE issues guidance on whether technologies represent a cost-effective use of NHS resources based on the list prices offered by manufacturers at launch. Therefore, NICE appraisal can already establish the price at which a technology could be regarded as cost-effective and of value to the NHS. In other words, NICE has been undertaking the fundamental aspects of appraisal required to establish evidence based assessment of value since its inception. The restriction has been that NICE is unable to negotiate prices with manufacturers. Nevertheless, over recent years it has become increasingly common for manufacturers to offer patient access schemes (PAS) either before or, more commonly, during the NICE appraisal process when it is apparent that the technology is unlikely to be regarded as cost-effective at the list price. In some ways the increased use of patient access schemes, which effectively offer different forms of discount to the NHS but without formally changing the list price,[13] has been a form of *de facto* value-based pricing[d].

The appraisal process also commonly considers the cost-effectiveness of the technology, not just for the whole indication under consideration, but also for subgroups within the indication. Therefore, the current appraisal process is already capable of establishing the combinations of values (T_is) and coverage levels (Q_is) from which the manufacturer can choose T*,Q*. However, in the past, NICE has tended to explore subgroups only when the technology would not be considered cost-effective for the whole indication at the list price. What should be apparent is that, once value-based rebates are possible, NICE should always explore subgroups so that manufacturers can always be offered the choice of value-based prices with the associated coverage levels.

This is important because it mirrors other markets similarly protected by patents where the temporary monopolist selects where to locate on the market demand curve for their product. It is also important for the NHS because, just like other markets, it means that value is more appropriately shared between the NHS and manufacturers during patent protection. A failure to explore subgroups and to offer a menu of prices and coverage levels to manufacturers will mean that, at a VBP for the whole indication, the additional health benefits offered by the technology will just be offset by the health expected to be forgone elsewhere in the NHS due to the additional costs. All value will be appropriated by manufacturers and new technologies will not improve health outcomes overall. Indeed, it would be equivalent to allowing perfect price discrimination (see Section 2.1). In other markets, with the exception of natural monopolies, the temporary monopoly offered by patent is regarded as sufficient.

The exact role NICE will undertake remains to be determined. It may supply an assessment of expected health benefit and expected costs to a pricing authority which will translate this into a menu

[d] In principle PAS could continue to be considered as part of a VBP scheme (Ti,Qi based on costs and effects with the PAS in place). This would offer manufacturers an opportunity to price discriminate, i.e., offering PAS for lower value subgroups. Therefore, if permitted, a greater use of PAS might be expected with attendant monitoring and transactions costs.

of VBPs and coverage levels (T_i,Q_i) using an empirically based assessment of the cost-effectiveness threshold for the NHS (see Section 3.6.3). Alternatively, NICE may apply the cost-effectiveness threshold to the results of its appraisal and then supply the combinations of Ti,Qi to the body negotiating value-based rebate agreements. What is critical is that, whichever organisation uses it, the cost-effectiveness threshold is empirically based and estimated and applied in a transparent and, therefore, accountable way (see Section 3.6.3). Without this, the clarity and predictability of the signal provided by VBP will be undermined, thus negating the dynamic benefits. Failure to use an appropriate and empirically based threshold would, on one hand, introduce additional uncertainty into investment decisions for manufacturers and a fear that monopsony power will be exploited now or in the future. On the other hand, the NHS and wider public might fear that limited NHS resources, expected to be employed to provide health care, are in fact being used to provide a hidden and unwarranted subsidy to a particular sector of the economy. This is a particular danger (in reality or appearance) and especially in hard pressed times when the spending department responsible for representing the NHS as a consumer of drugs (the DH in negotiating the value-based rebates) still has the necessarily conflicting responsibility of representing the longer term interest of the pharmaceutical sector in the UK. The latter responsibility would surely be more appropriately allocated to the Department of Business, Innovation and Skills as is the case in most other sectors which are also legitimately competing for the support of government and public expenditure to ensure their longer term interests.

3.6.2 Scope and reappraisal

NICE considers new technologies at launch under the Single Technology Appraisal (STA) process, where the appraisal is primarily based on a submission from the manufacturer which is reviewed by an independent Evidence Review Group (ERG). All NICE guidance also includes a date at which the guidance will be reviewed and if, at that time, there are sufficient changes to the evidence base or other relevant technologies then, commonly, all the technologies available for the indication are reappraised under the Multiple Technology Appraisal (MTA) process, where an independent Technology Appraisal Review (TAR) group develops an analysis of the whole area taking account of submissions from manufacturers and other stakeholders. This process of an initial STA followed by later MTA matches the proposals made by OFT of what they termed an ex-ante assessment at launch followed by ex-post assessment some time later (5 years suggested by OFT).[4]

The initial assessment of Ti, Qi could be undertaken as part of the existing STA process if methods and process were strengthened in some respects. For example, it would require either a greater role for the ERG in revising manufacturers' submissions to provide acceptable estimates of Ti,Qi, or more iterations with manufacturers so that their analysis reflects the social and scientific judgments of the appraisal committees, and routinely exploring subgroups (see Section 3.6.4).

However, any initial assessment based on a STA will need to be revised as: more comparator technologies enter through STA; evidence accumulates; and, importantly, generic versions of technologies with VBPs enter as patents expire. A review of a whole indication requires a comparison of very many technologies utilising evidence generated by different manufacturers. Therefore, the MTA process seems most appropriate rather than asking manufacturers to make submissions about their competitors products when they may not have access to the complete evidence base. There also needs to be a balance between stability of VBPs in the short term, the costs of multiple appraisals in the same area (for NICE and manufacturers) and making sure that changing conditions are reflected in VBPs. Explicit criteria, including requests from manufacturers and other stakeholders, could be used to initiate reappraisal and to renegotiate the VBP. These criteria ought to include the entry of a generic version of the VBP drug or additional significant evidence, e.g., a large randomised trial reporting - whether publically funded or sponsored by a manufacturer. It should be noted that reappraisal triggered by new evidence may increase VBP if the evidence suggests that the benefits of the technology were originally underestimated, providing incentives for evaluative research where manufacturers believe there are additional benefits not yet evidenced (see Section 4.4).

3.6.3 Evidence based cost-effectiveness threshold

An assessment of whether the health expected to be gained from the use of a new technology exceeds the health likely to be forgone elsewhere is critical for decisions currently made by NICE and is fundamental to establishing value-based prices/rebates. This requires an estimate of the health that is expected to be forgone, on average across the NHS, as other activities are displaced to accommodate the additional costs of new technologies. The cost-effectiveness threshold range currently used by NICE (£20,000 to £30,000 per QALY gained) is intended to represent precisely that. However, it is widely recognised that this informal assessment has limited empirical basis and that more robust estimation of the NHS threshold is urgently required. The Methodology Research Programme funded by the National Institute for Health Research and Medical Research Council has funded research to establish appropriate methods to estimate the NHS threshold based on routinely available sources of data. This research extends earlier econometric analysis[17-18] which utilised NHS programme budget data and mortality data for different programmes of care to estimate the relationships between programme expenditures and health outcomes. The results of this initial work are summarised in Table 1.

This analysis is being extended in a number of ways by the current research. Firstly, it will estimate these relationships for more programmes of care to cover a greater proportion of overall NHS expenditure. Secondly, the relationship between changes in the overall NHS budget and expenditure by individual programmes will be estimated which will provide a key link to the estimate of the NHS threshold. Thirdly, routinely available mortality data will be more robustly translated into estimates of life years and QALYs gained for each programme to reflect better the impact of expenditure on morbidity as well as mortality. In addition, this work will characterise the uncertainty in any estimation. It will also exploit recently available panel data to explore how the threshold is expected to change over time and examine how the threshold changes with the sign and scale of changes in expenditure. The structure of this research and how each element combines to provide an estimate of the NHS threshold (k) is illustrated in Figure 4. Initial estimates are due to be presented in Spring 2011 and the final report is due in summer 2012, in time to inform VBP in 2014.

Table 1 The relationship between programme expenditure and life-years gained[18]

	Cancer	Circulation	Respiratory	Gastro-int	Diabetes
04/05 Cost per LY	£13,137	£7,979			
05/06 Cost per LY	£13,931	£8,426	£7,397	£18,999	£26,453

The empirical basis of the cost-effectiveness threshold will also need to be re-assessed over time, but with whom should this responsibility rest? It should be apparent that the type of threshold required is not a social value judgement about the value of health, but a scientific question about the current productivity of the NHS, i.e., the relationship between changes in expenditure and outcome. Therefore, the threshold is not something that NICE can choose or that can be negotiated between Ministers, DH and manufacturers. Nor can the value of an estimated threshold be raised (or lowered) to reflect the social value of different types of health improvement. If there are other values or weights to be applied to health benefits then they must also be applied to the type of health likely to be forgone when estimating the threshold, i.e., each alternative set of value implies a different but single threshold for VBP (see Section 4.2). It is important that the threshold used to generate the menu of T_i, Q_i is known and that the methods and data for its estimation are open to scrutiny. Indeed, the threshold ought to be based on the best available evidence and most appropriate methods. This, however, will require scientific value judgements which might best be made independently of DH, NICE or manufacturers. Periodic reassessment of the threshold, based on agreed methods and data, will provide some predictability for manufacturers making longer term investment whilst reflecting changes in overall NHS expenditure and productivity.

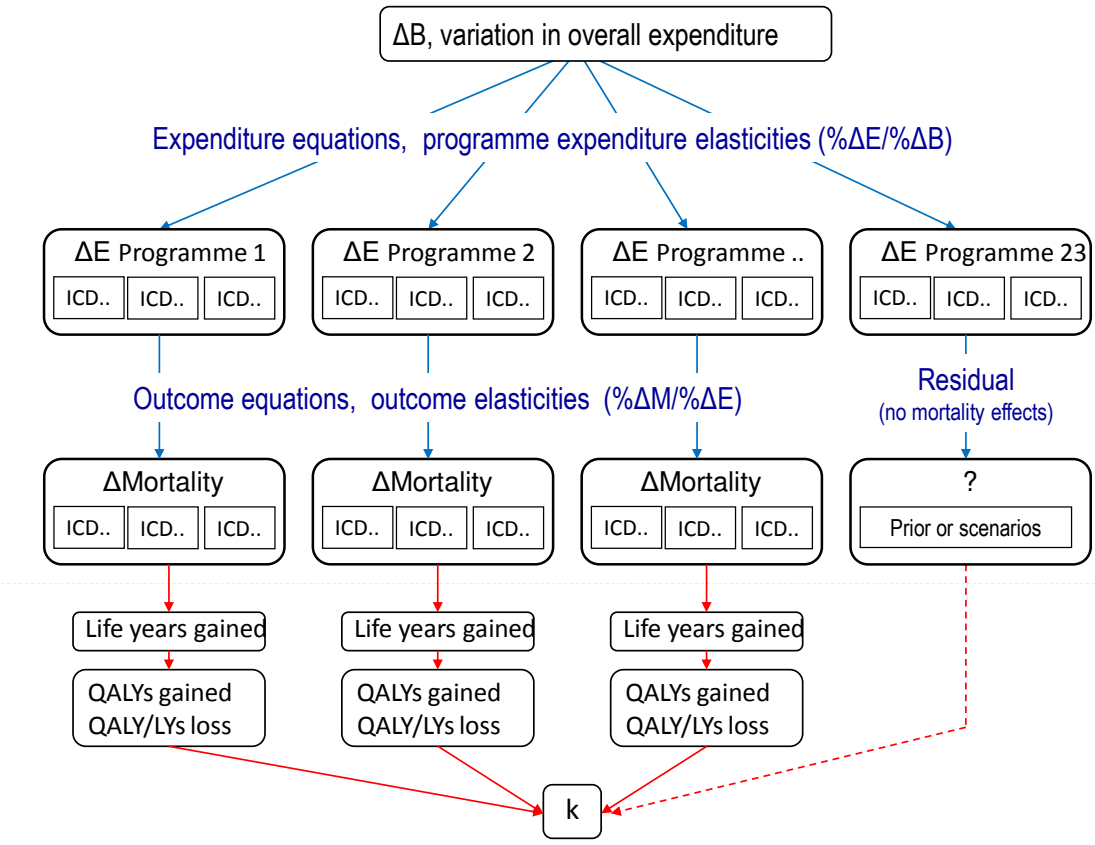


Figure 4 Estimating the threshold for the UK NHS

3.6.4 Transparency, accountability and redress

It is critical that a VBP scheme offers transparency and the opportunity for accountability and ultimately redress. However, for value-based rebates to be agreed it is also important that the value-based transaction price T* is not revealed in a way which would impact on reference pricing in other countries. If it is, then manufacturers will be unlikely to agree to value-based rebates[e]. There are three discrete tasks which must be undertaken and each may require different levels of transparency and routes of accountability and redress. These tasks include: i) an assessment of the costs and benefits of a new technology for each indication and identifiable subgroup within an indication; ii) applying an evidence based threshold to turn these estimates of costs and benefits into a menu of TiQi combinations; and iii) negotiating the agreed T*, Q* from this menu ensuring that the rebate (L-T* up to Q* and L-C for volumes greater than Q*) are recovered.

The first task is already undertaken by NICE with unprecedented levels of transparency, consultation, rights of appeal and ultimately redress through judicial review. Maintaining the transparency and accountability of this task seems crucial for all sides and there seems little to be gained by making it less so (it does not reveal the particular T* for reference pricing). The third task cannot be undertaken by NICE (without primary legislation) but by DH. This separation of duties might also be appropriate as it clearly distinguishes these tasks and reduces the potential conflict between making a balanced assessment of what current evidence suggests might be the costs and benefits of a product, from what price the NHS will actually pay. It seems that this final task need not have the same level of public transparency as the first as long as the process (a set of rules which mirror other markets) is well specified. If they are, then accountability and redress can be achieved with a suitable appeal

[e] If the VBPs for the NHS are expected to be lower than transaction prices likely to be agreed in other jurisdictions which reference UK prices, then the UK NHS will benefit from helping to facilitate such international price discrimination by making any agreed T* more difficult to reference.

process which might use similar methods to handle commercial in confidence (CIC) information as existing NICE appraisal. Ultimately access to judicial review can establish whether the rules have been followed without making CIC information public.

The second task requires the application of an empirically based threshold to provide the menu of T_i, Q_i. It is critical that the threshold used is known and that the methods and data for its estimation are open to scrutiny. Indeed, the threshold ought to be based on the best available evidence and most appropriate methods. This, however, will require scientific value judgements which might best be made independently of DH, NICE or manufacturers, i.e., an independent scientific threshold committee whose evidence, processes and deliberation should be transparent, accountable with routes for redress if its decisions appear perverse or its processes are not followed. Once estimates of costs and effects are established for each indication and subgroup, it is trivial to produce the menu of T_is based on an agreed threshold. However, additional evidence will be required to estimate the Q_i associated with each T_i. NICE appears best placed to undertake this second step which would be conducted only after appraisal has been completed, i.e., Final Appraisal Document (FAD) issued and any appeal resolved. This second step may not require the same level of public transparency and consultation as the first so long as there is sufficient detail provided to make scrutiny of whether it has been done correctly possible and an assessment of whether the Q_is are reasonable estimates. The different ways in which each of these three tasks might be undertaken can be judged on the basis of which allow the greatest transparency and accountability while withholding the agreed T* on which prices might be referenced in other countries (this will depend on whether national or local volume agreements are included – see Section 3.4).

4. Other aspects of value

The principles of VBP outlined above can be implemented based on existing methods and process of appraisal adopted by NICE. Such a scheme would be wholly appropriate if the measure of benefit captures all important aspects of social value. However, there are four aspects of value discussed below which require further consideration.

4.1 A premium for innovation?

Although competing definitions of innovation are not particularly insightful, all make clear that it refers to new methods, ideas or products which are claimed to offer benefits over existing ones.[19] In recent debates it seems more commonly invoked to support particular views of what ought to be given additional value or consideration when considering new patented products (pharmaceuticals and devices).[20] It should be noted however, that there are other sources of innovation relevant to the NHS including new ways to deliver services as well as new surgical, diagnostic and other procedures.

The question of how to value innovation and how to ensure that there are sufficient incentives for private investment in the development of socially desirable innovations requires a clear view of the social value of a health technology, its relationship to price, and the incentives this provides for private sector investment decisions. These important issues are central to the principles of VBP (see Sections 2, 4.2 and 4.3) founded on the type of appraisal conducted by NICE in providing clear and predictable signals to the private sector of what is of value to the NHS.

The policy question is not whether innovation should be supported. Self evidently it should be and it is through patent protection, public investment in infrastructure and the research and development environment in the UK (including tax and other incentives). Any VBP scheme should not undermine the protection offered by patents which is why rules are required which mirror markets in other sectors also protected by patent i.e., a free choice of the T_i, Q_i combinations so that the NHS cannot exploit its monopsony power and manufacturers cannot price discriminate or claim more than the current value of the product. In these circumstances are there any reasons why the NHS ought to pay more that the current value of a product to encourage innovation?

4.1.1 Anticipating future benefits of innovation

It is beyond dispute that that some innovations today will provide the basis for subsequent innovations which may be even more valuable in the future. The question is who should anticipate the benefits (and associated returns) of the possibilities of further innovation and when should the customer (the NHS) pay for them? In other markets in other sectors, also protected by patent, it is the role of the private sector, supported by the Government's industrial policy, to anticipate the benefits of further innovation and the future market returns which flow from better products when making investment decisions. No one suggests that consumers should be compelled to buy products (compulsion would indeed be required) merely to provide an additional incentive for future innovation!

In any case, the NHS is not best placed to anticipate future benefits: some products may and some may not offer future value, and even those that do, the investment required may not be the best use of private capital. Such assessment is appropriately the responsibility of the private sector because: i) it is best placed to anticipate the potential benefits and returns across global markets; ii) such returns must be compared to the cost they will commit during the development process; and iii) it is their decision (not the NHS') to risk private capital in such an endeavour. Those manufacturers that are better at making investment decisions based on anticipating and then delivering future benefits at lower development costs will succeed and those that are not will rightly fail.

If the NHS was to start to pay premiums for current technologies based on anticipated benefits, the incentives for efficient development would be perverted, damaging not enhancing future innovation. Even if the NHS was able to anticipate future benefits and pay an associated premium on current products, all value would have been given to the incumbent (manufacturers with the current patent). There would be no value left to offer future innovators who might be best placed to deliver the anticipated future benefits more quickly and at lower cost. Furthermore, of course there would also be little incentive for the incumbent to put effort into making sure the anticipated benefits are actually realised, unless they are to be paid twice. Since there is little evidence that these perverse

incentives have been properly considered in the recent policy debate about premiums for innovation, it is reasonable to suspect that the NHS is actually being asked to pay twice for the same benefits, i.e., payment for current and future value now and then payment again for the same value if and when it is realised. If the NHS is not to pay twice, an innovation premium for particular products would be self defeating (dynamically inefficient): disincentivising the unknown innovators of the future. If the NHS is to pay twice for innovation then it would be both statically and dynamically inefficient: reducing health outcomes across the NHS as well as blunting incentives within the sector and encouraging overinvestment and higher costs compared to other sectors of the economy which do not receive such excessive rewards.

4.1.2 Incentives for innovation and location

It is, however, quite proper to be concerned whether the protection offered by patents is sufficient and whether the UK offers an environment in which research and development activities might be located. Indeed, a case could quite reasonably be made that some share of NHS resources as well as other public money might be devoted to this if the returns from such public investment are greater in this sector compared to others. In fact government already does this in a number of ways including using resources which could have been devoted to provision of health care. For example the investment in translation research following the Cooksey Report,[21] as well as more fundamental science and biomedical research, can be viewed as an attempt to subsidise research and development and make the UK a more attractive location for such activities. However, the case for an innovations premium within a VBP scheme to achieve the same policy goals cannot logically be made.

It is not possible under EU law only to offer innovation premiums for products associated with manufacturers who locate in the UK. Therefore, if a premium is to be offered it must be offered irrespective of location. Although manufacturers would no doubt be grateful for this subsidy to their global research and development efforts, one presumes they will chose to invest it wherever offers the best environment. Since other countries compete for inward investment, those that devote similar resources to improving their environment, rather than offering a per product premium, will have the advantage. Therefore, a policy of innovation premiums will result in a perverse situation in which the NHS pays premiums for particular products only to find that the subsidy is invested in other countries which, quite sensibly, have devoted similar resources to improving the research and business environment instead. This is not to suggest that the pharmaceutical sector suffers from a lack of gratitude, or that innovation should not be supported through other more effective means, but simply an observation that the discipline of international capital markets means that incentives which are not excludable by location will not provide any incentive for inward investment. Innovation premiums are not excludable so cannot achieve the intended policy goals. In fact they make things worse as there will be less resource available to improve the research and business environment if they are paid; a policy imperative better aligned with the responsibilities of the Department of Business, Innovation and Skills.

4.1.3 Innovation and unmet need

The principle of VBP aligns the incentives for investment in research and development with NHS needs. Those manufacturers which develop products and present evidence that indicates they represent a ‘step change’ in treatment will achieve higher prices and greater returns since VBPs are based on an assessment of the expected benefits of the technology. Therefore, insofar as areas of unmet need also offer opportunities to develop technologies which offer significant improvements in treatment, VBP will provide a clear signal and incentive for such investment. Equally, insofar as innovation is incremental, each smaller step will be rewarded, although at lower premium prices reflecting smaller realised benefits; preserving rewards for future innovators (see Section 4.1.1).

As currently, competitors independently developing similar products but who launch after the originator (early followers) or those who have developed similar products based on the originators patent (me toos), will take some of the market share and reduce the total revenue for the originator. Although VBP will mean that similar products will achieve similar prices, during the originators patent, it will also mean that early followers or ‘me toos’ will have less time to build market share and generate revenue before the VBPs of their and the originator's product are reduced by generic entry. Compared to the current situation this will tend to disincentivise development focused on existing patents and encourage research in less well worked areas.

If the incentives offered by VBP are not regarded as sufficient then the fundamental problem is on the supply side, i.e., patent protection is potentially too narrow and too low (which may be reasonable if the opportunities for step changes are now more limited, e.g., the limitations of cell receptor biology). However, there are trade-offs here. Insofar as 'me toos' are really early followers (independent investment in similar areas) then any shift of reward to the product which happened to be first to market will also make future investment in step change innovations more risky. Therefore, policy considerations ought to be based on evidence that there is a problem to solve; especially once VBP sharpens the incentives. At present there is little evidence that returns in the sector are lower than others or that the there are insufficient returns for those first to market.[22-24] Even if compelling evidence existed, it is far from evident that manipulating the demand side (paying more for the originators product than other similar products) would be an effective or practical policy tool. There are a number of supply side policy options which may be superior in these regards. For example, as well as addressing the width of patent protection (short but wide patents are more appropriate if step change innovation is more limited), prizes could be offered for improvements in treatment in particular areas as well as continued public investment in the development of basic science which will provide the foundation of future innovation.

However, the policy concern for areas of unmet need might instead reflect a demand rather than supply side problem: that some aspects of social value are not fully reflected in the VBP described in Section 2. For example, significant health benefits may be regarded as worth proportionately more than a relatively minor one (for patients and society). Such social values might also be related to concepts of need (other than capacity to benefit) such as 'severity' or burden (defined in terms of current health, or past health experience, or the length and quality of life expected to be lost as a consequence of the condition) and/or particular characteristics of patients.

The two alternative reasons for a concern for unmet need discussed above are very different and need to be clearly distinguished as they require very different policy responses and will have different consequences. The first may mean static losses for the NHS (if the greater value appropriated by the originator is at the expense of the NHS's share of value) but the potential dynamic gains may be very dilute as UK revenue is unlikely to have measureable influence of investment decisions (the UK only represents 3% of the world market). The second reason, if supported by evidence of social value and implemented appropriately (see Section 4.2), may offer static gains to the NHS and dynamic gains although equally dilute.

4.2 Severity, burden and need?

NICE's formal methods of technology appraisal have consistently viewed a QALY unit of health improvement to be equally weighted no matter what the characteristics of the recipient - sometimes known as 'a QALY is a QALY is a QALY'.[1] When the appraisal committee considers the available evidence, the characteristics of patients may play a role in its decisions, but this is currently achieved through a deliberative process rather than formally weighting the QALYs gained. NICE's end of life considerations are perhaps an exception to this process whereby the reasonableness of the QALY weighting necessary to make a technology cost-effective is explicitly considered by the appraisal committee. However, this happens late in the appraisal process, and the cost-effectiveness analyses in manufacturers' submissions are required to include non-weighted QALYs.[25]

It may be judged that other aspects of social value are not fully reflected in the measure of health gain used in NICE appraisal and that some additional weighting of different types of QALYs gained might be deemed appropriate. For example, a larger per patient QALY gain might be regarded proportionately more socially valuable and carry proportionally more weight than a more modest QALY gain. Alternatively, or in addition, QALY gains in areas where the burden of the disease is regarded as more severe (which might be defined in terms of current health, or past health experience, or the length and quality of life expected to be lost as a consequence of the condition) might be regarded as more socially valuable and carry greater weight. We don't comment here on whether we believe any particular set of weights might be appropriate or how a particular set of weights might be arrived at. We only note that there is an extensive literature on the estimation of such weights, including studies commissioned for NICE[26-27] but no consensus on what weights ought to be used or how they should be arrived at. The use of any set of weights will inevitably disadvantage some patient groups. Therefore, without some consensus such a policy may become

socially divisive and unsustainable in the longer run; undermining the predictability of the signals offered by VBP.

Setting aside the question of what weights might be appropriate, consistency demands that any weights that might be attached to the health likely to be gained by a technology must also be used to weight the health likely to be forgone elsewhere as a consequence of additional NHS costs. This is crucial because if the social value of the health that is likely to be forgone is not properly accounted for then the VBPs will not reflect the social values embedded in any set of weights that might be chosen. If there are weights that are applied to the type QALYs gained then the same weights (which might be expressed in monetary terms) must also be applied to the QALYs likely to be forgone, i.e., there will be a single and common estimate of the threshold which will differ depending on which set of weights are adopted. The availability of programme budget data and estimation of expenditure and outcome equations described in Section 3.6.3, in principle at least, allows any set of weights to be applied to the QALYs forgone within programmes (by ICD chapter). For example, if QALYs gained in cancer are to have a greater weight than QALYs gained in respiratory health then that weight must be reflected in the programme specific QALY effects when estimating the threshold of the NHS (see Figure 4).

It would be wholly inappropriate to apply additional weights to the benefits (QALY gained) but fail to use the same weights in calculating the opportunity costs (QALYs forgone). The danger would be that the QALYs forgone might have just as much or even more social value than the QALYs gained. Similarly, trying to reflect social value by applying different threshold to different types of QALY gains in calculating VBPs would also be inappropriate as it conflates two very different questions: i) what social value ought to be placed on different types of QALY gained and forgone and ii) what is the social value of the type of QALYs are likely to be forgone as a consequence of additional costs falling on the NHS. It would be impossible to find the appropriate threshold to apply to unweighted QALYs to reflect a consistent set of social values.

4.3 Wider economic benefits?

Since 2008, NICE methods of appraisal have generally restricted attention to health benefits and costs which fall on the NHS and Personal Social Services budget. This NHS perspective may well be reasonable if the objective of collectively funded health care is to improve overall health; that the measure of health gained and forgone captures enough aspects of social value to be useful; and that the budget for health care ought to be regarded as fixed by the decision making body. It also requires that either the effects outside the health care sector to be of limited socially value, compared to the effects within the health sector, or that wider economic benefits are closely related to improvements in health. If they are then those technologies which are regarded as cost-effective from an NHS perspective (they improve overall health) will also offer net benefits to the wider economy, but those that are not will reduce overall health and impose wider net costs. Therefore, on average, the NHS perspective is perhaps appropriate even if wider effects are important and deemed socially valuable. However, there will be exceptions, where the wider benefits associated with the health gained from a particular technology for a specific patient group is expected to be substantially greater or less than other displaced NHS activities. The questions of fact and value on which the question of appropriate perspective turns and alternative policy responses have been evaluated in recent DH funded report.[28]

Wider effects fall into two broad types: i) direct costs of care that do not fall on the health care budget; and ii) the indirect external effects on the rest of the economy. Some of the direct costs of care are borne by patients (e.g., out of pocket costs, time in accessing care) and may include the direct financial consequences of ill health (and financial benefits of earlier recovery) for patients and families if these are not fully captured in measures of health related quality of life. It will also include the time and resources devoted to caring for patients outside the health care system. Such direct costs may fall on marketed and non marketed activities (e.g., time and informal care). Importantly, an effective health technology may reduce these costs (e.g., a quicker recovery) or increase them (e.g., prolong survival in a chronic state). There are also indirect effects external to the patients, their family or informal carers but which are valued by the rest of society. For example, returning a patient to active participation in the labour market may add to production in the economy. This will be a net benefit to the rest of society if the value of the additional production exceeds the individual's additional consumption over their remaining life expectancy. Therefore, an effective health technology may provide external benefits by reducing mortality in economically active groups whose production is

likely to exceed their consumption. However, it may also impose external costs on the economy if it reduces mortality in populations where remaining life cycle consumption exceeds the value of production.

Although the implications of taking account of some external effects might be regarded as undesirable, once wider consumption effects are taken into account it becomes difficult to justify why only some effects should be included and others not. If conflicts arise this is because there are other aspects of social value which are difficult to specify and codify. It should also be clear that properly taking account of external effects must work both ways: that although some technologies might achieve higher VBPs as a consequence, the VBPs of others will be lower than they would have been with a narrower NHS perspective.

4.3.1 Appropriately valuing opportunity costs

All these direct and indirect external effects fall on consumption (gained or lost) outside the health sector rather than on health (gained or forgone) within it, i.e., the type of opportunity costs differ. This ought to be reflected in the weight attached to the different types of effects. For example, external benefits (e.g., a reduction in patent and career costs) cannot be used by the NHS to provide additional health care and generate health. Equally additional costs which do not fall on the NHS budget will displace consumption rather than health. Therefore, external costs and benefits cannot be treated in the same way as NHS costs, which displace health at a rate given by the cost-effectiveness threshold (see Section 3.6.3). A means of valuing health generated by the NHS relative to wider consumption effects is required, i.e., an agreed consumption value of health. Since those who bear the external costs or enjoy the benefits are generally free to chose how to consume, an appropriate value might be the amount of consumption individuals are willing to give up to improve their own health. There are a number of reasons why such a consumption value of health is likely to be higher than the cost-effectiveness threshold for the NHS.[28] Therefore, when taking proper account of external costs or benefits only some weight, between zero and one (based on the ratio of the cost-effectiveness threshold to the consumption value of health), ought to be applied to them. This effectively places policy between the two extremes of either disregarding external effects or ignoring the implications of a budget constrained NHS.

4.3.2 Wider economic benefits forgone

The additional health care costs of new technologies will displace other health care activities, not only resulting in forgone health elsewhere, but also forgone benefits to patients' carers and the wider economy. Therefore, it is not sufficient to observe wider economic benefits associated with a new technology, but there is a need also to establish that these exceed the benefits which may be forgone elsewhere as other NHS activities are displaced. A failure to take proper account of the wider economic benefits likely to be forgone would result in doubly false positive decisions, i.e., technologies priced such that the health benefits offered are less than the health forgone and, in addition, the wider economic benefits are also exceeded by those that are forgone elsewhere. Including as assessment of external effects in a VBP scheme provides a strong incentive for manufacturers to search assiduously for evidence of wider benefits associated with new technologies, but offers little incentive to identify those external benefits which may be forgone. Therefore a proper and rigorous assessment of external benefits forgone is essential.

Identifying precisely which activities are displaced at a local level in the NHS and estimating their associated cost-effectiveness from an NHS perspective is notoriously difficult.[29] It would be an even greater challenge to also try to estimate the associated wider costs and benefits. However, at a more aggregate level, evidence suggests that improvements in health generally offer net consumption benefits to the wider economy.[30-32] Therefore, on average across the NHS, both health will be forgone due to additional NHS costs and this forgone health will also be associated with displaced consumption benefits. The amount of health likely to be forgone is provided through the estimate of the costs-effectiveness threshold (see Section 3.6.3) but further work would be required to estimate the wider benefits associated with it. It might also be possible to reflect the impact of the type of health likely to be forgone since estimates of the threshold can now be based on evidence of where displacement is likely to occur (which programme budget category and associated ICD chapter – see Figure 4). The programmes and ICD chapters are also associated with different types of patient

characteristics (e.g., age at diagnosis) which could, in principle, also be used to improve the estimates of the wider economic benefits that are likely to be forgone.

Nevertheless, it should be recognised that extending the perspective for VBP will impose additional costs on the appraisal process and introduce the possibility of a biased assessment if the economic benefits forgone elsewhere are more difficult to identify. The problem may be more manageable if the consideration was restricted to those exceptional cases where an NHS perspective is more likely to be inadequate, i.e., where the external economic benefits are likely to be substantially greater or less than current NHS activities which may be displaced.

Even if properly conducted, VBP which includes an assessment of wider economic benefits will have static and dynamic effects that may or may not be socially desirable. Technologies will be VB priced to the point at which the overall benefits, to the NHS and the wider economy, will be zero. Insofar as new technologies tend to offer net benefits to the wider economy these will be appropriated by manufacturers through higher prices, at least during the period of patent protection, turning what were originally external benefits into higher internal NHS costs, so overall health will be reduced. Therefore, there is a danger that immediate static losses for the NHS might never be compensated for by later dynamic gains if the limited incentives by the UK market do not sufficiently influence future global investment decisions about the type of technologies that are developed.

4.3.3 Questions of fact and value

Extending the perspective for appraisal beyond the NHS poses a series of empirical questions which would need to be resolved. As well as an empirically based estimate of the cost-effectiveness threshold (which should also reflect non-marginal impacts on the NHS budget), an agreed estimate of the consumption value of health is also needed. Critically, an estimate of the wider benefits likely to be forgone is required. Finally, robust estimates of the cost of care not borne by the NHS, and the external effects on the wider economy would be needed. There are a number of questions of social value posed in resolving how these elements should be measured and valued. Taking account of effects outside the health care sector requires a social value judgement about the rate at which society is willing to trade social arguments including health and consumption. A key question is whether it is possible or desirable to specify such a description of all possible social states which will have implications for decision across all sectors, not just health. If a complete specification of all social arguments is not possible or if any particular welfare function does not carry a broad consensus or obvious legitimacy, then attempts to formalise and codify these trade-offs might be undesirable because the prescriptions may well conflict with other objectives of social policy and lead to undesirable and socially divisive changes to the health care system.

4.4 Uncertainty, evidence and investment

An assessment of expected cost-effectiveness, or net health benefits, relies on evidence about effectiveness, impact on long-term overall health and potential harms, as well as the costs which fall on the NHS budget with some assessment of what health is likely to be forgone as a consequence. Such assessments are inevitably uncertain, especially at launch when the evidence base is least mature.[33] Without sufficient and good quality evidence, subsequent decisions about the use of technologies and the appropriate VBP will also be uncertain, i.e., there will be a chance that the resources committed by agreeing a VBP for a new technology may be wasted if the expected net health benefits are not realised. Equally, VBPs which restrict the use of a new technology only to the most cost-effective subgroups, will risk failing to provide access to a valuable intervention if the net health benefits prove to be greater than expected. Therefore, if the social objective is to improve overall health for both current and future patients, then the need for and value of additional evidence is an important consideration when making decisions about the use of technologies and the prices that ought to be paid.

4.4.1 Value of access and the value of evidence

The value of access to a new technology with uncertain benefits and the value of additional evidence about the technology is illustrated in Table 2.[34]

Table 2 The value of early access and the value of evidence. Two treatments are compared: A (current treatment) and B (new treatment).

	NHB (A)	NHB (B)	Max NHB
1	4	1	*4*
2	10	10	*10*
3	16	22	*22*
Average	**10**	**11**	***12***

Value of access

Value of evidence

In this example, on average, the net health benefit of the new technology (NHB(B)=11) is expected to be greater than current treatment (NHB(A)=10), so at current prices early access to the new technology is expected to offer 1 additional QALY per patient. However, this assessment is uncertain (represented by the 3 possible resolutions of NHB) and there is a chance that the new treatment may not be cost-effective, i.e., a probability of 1/3. If more evidence could be acquired to resolve this uncertainty then better decisions could be made, improving net health benefit (Max NHB=12, as shown in the final column). The upper bound on the value of additional evidence is the difference between the best that can be done if the uncertainty could be resolved and the best that can be done if decisions are based on existing information, i.e., also 1 QALY per patient. Therefore, evidence about the performance of a new and effective technology is valuable for the same reasons as access to it, both improve health outcomes, and the value of evidence may be as great, or greater, than the value of the technology itself.

If the type of research which is required can be conducted once the technology is available for use in the NHS, the VBP at launch can be based on estimates of expected cost and effects (as described in earlier sections). However, even in these favourable circumstances, there is an important link between VBP, uncertainty and the need for evidence. As discussed in Section 3.6.2, reappraisal of VBP, among other things, ought to be triggered by significant new evidence becoming available; increasing the VBP if the evidence suggests that benefits were originally underestimated, or reducing it if they where overestimated. This means that manufacturers retain an incentive to conduct further evaluative research if they believe that there are additional benefits which could not be evidenced at launch. Publically funded evaluative research, however, will still be required where these incentives are insufficient and especially in those cases where the original evidence is likely to have overestimated the benefits. However, it should be noted that linking VBPs to the results of publically funded research means that the NHS will only benefit if the results lead to lower VBPs (thus avoiding the losses of net health benefit at the original VBP). Manufacturers will, however, be able to appropriate the value of evidence when it suggests that net health benefits were originally underestimated and this leads to higher prices. This might inform whether manufacturers should be expected to generate evidence through approval with research (AWR) agreements or make some contribution to the costs of publically funded research. Some of these considerations are reflected in the flexible pricing arrangements of the current PPRS.[13]

4.4.2 Price and the value of evidence

These considerations are even more critical once it is recognised that the approval of a technology for widespread use might reduce the prospects of conducting the type of research that would provide the evidence needed. For example, the early diffusion of a technology means that future clinical trials are less likely to be supported or regarded as ethical by the clinical community, even when public funds are made available for such research; and patients are unlikely to enrol in clinical trials once they have unrestricted access to the new technologies. In these circumstances there will be a trade-off between the expected net benefits for current patients from early access to a cost-effective technology and the health benefits for future patients from withholding approval until valuable research has been conducted, i.e., an only in research (OIR) recommendation.[35-36]

For a VBP which fails to account for the value of evidence, the expected benefits of the technology just offset the health likely to be forgone, so the expected net benefits of early access across current NHS patients will be zero. Therefore, insofar as there is value associated with the type of evidence that cannot be acquired once the technology is in widespread use, the NHS would be better off (improve net benefits across current and future patients) by restricting access to the technology until the research can be conducted. It is important to retain this option of restricting access because it provides an incentive for manufacturers to provide the type of evidence needed to support NHS use at launch or to negotiate lower prices which reflect the value of evidence forgone by early access. For example, if there is uncertainty about the scale of the benefits offered by a technology then, an appropriate VBP would provide sufficient positive net benefits of early access for current NHS patients to just offset the value of evidence (net benefits) for future patients which would be forgone, i.e., the VBP will be lower than the price which would make the technology just expected to be cost-effective (with zero net benefits). This lower VBP, which accounts for the need for evidence, provides an important incentive for manufacturers: higher prices can be achieved by developing effective technologies and by generating the type of evidence needed to support clinical practice. In this way a VBP scheme that incorporates a consideration of uncertainty can provide signals and incentives for the type of evidence needed by the NHS.

A failure to take account of the need for further evidence at launch will tend to undermine the evidence base for future clinical practice. If an assessment of uncertainty and the need for evidence is incorporated into a VBP scheme then, in some circumstances, forgoing the evidence may be appropriate but only at prices lower than the VBPs described in Section 2. However, there will be circumstances in which any reduction in VBPs will not eliminate the need for further research (e.g., when uncertainty primarily surrounds whether the technology is effective rather than its magnitude), so the possibility of withholding approval or restricting access until research is conducted, i.e., an OIR recommendation, must be retained.

4.4.3 Investment, uncertainty and price

Investment costs are those which, once committed, cannot be recovered if a decision is revised at a later date. These are generally considered to be costs with a long life expectancy such as those incurred in providing new facilities or equipment necessary to use the new technology.[37-38] These are commonly annuitised in appraisal and included in the expected costs of the technology, but the impact of their irrecoverable nature is often not explored. Irrecoverable costs also include situations where initially negative net heath benefits of a new technology are offset by later positive net benefits. This is illustrated in Figure 5 where the initial losses (area A) are more than offset by the later gains (area B) - the technology is expected to be cost-effective overall. However, if approval is revised (e.g., due to research revealing that the technology is not as effective as expected) then initial losses will have been incurred which will not be compensated by later gains. Overall losses will tend to be greater if decisions are more likely to change and in the more immediate future.

There are many common circumstances where initial losses are only offset by later gains. For example, when practitioners must learn how to best use a new technology by experience, then the initial learning costs imposed on patients (who will not get the maximum benefit from the new technology) are compensated by later gains once the technology can be used to its full potential. Similarly, when it is not possible for practitioners to identify those patients likely to respond to treatment without first treating them, the initial population net health benefit will be lower as non-responders impose a cost without any additional benefit. As non-responders are identified and treatment is withdrawn, overall treatment costs fall and net benefit increases. In many circumstances the initial per patient costs of a technology can be very high and far in excess of the immediate health benefits in the initial period of treatment. However, these losses tend to be offset by future health benefits and sometimes reductions in future NHS costs. Therefore, Figure 5 represents a common pattern for technologies with mortality affects.

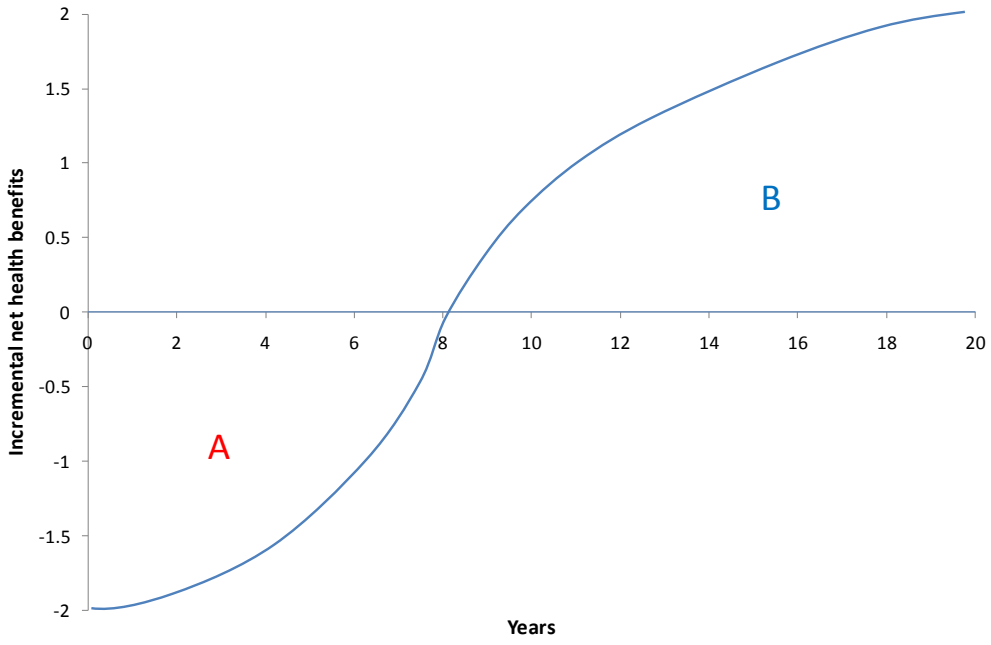


Figure 5 Uncertainty, investment and price

The irrecoverable nature of these costs can have particular influence on whether or not purchasing a technology represents a worthwhile investment.[35 37] Even if further research is not required, as long as there are other sources of uncertainty which may resolve over time, an assessment of the benefits and costs of early approval is needed. This should take account of investment costs and the risk that the expected longer term benefits will not be realised and more immediate losses incurred. Therefore, even though a technology might be expected to be cost-effective, it may not represent a valuable investment because the decision to purchase the technology will commit immediate costs that the NHS cannot recover once the other sources of uncertainty have resolved. A VBP which reflects the irrecoverable costs and the risks of incurring losses would make the investment just worthwhile for the NHS. This will be lower than the price at which the technology is expected to be cost-effective (the price at which area A=B in Figure 5).

The impact of irrecoverable costs is compounded when further research is also required because it adds to the risk that a decision to purchase the technology will be revised. In these circumstances, even when research could be conducted while the technology is in widespread use, restricting access through an OIR recommendation may be better for the NHS if the expected benefits of early access do not offset the expected losses.[39-40] Again, a lower VBP would be required before the NHS should take the risk of committing irrecoverable costs in purchasing the technology while also awaiting the results of research. Where the research needed cannot be conducted, once a technology is approved for NHS use, the opportunity costs of early approval now include the impact of investment and reversal costs when decisions might be revised as well as the value of evidence that will be forgone by early access. A VBP which would allow immediate access ought to reflect both these aspects of opportunity costs and will be lower than the VBPs discussed in Sections 2 and 4.4.2.

Although aspects of investment or reversal cost are almost always present, their potential significance depends on their scale relative to expected population net benefits offered by the technology. Since the latter may be zero under VBP, the proper assessment of investment costs becomes especially important. Critically, their impact depends on the risk that decisions (approval and pricing) will be revised in the near or distant future due to new evidence becoming available, the launch of new technologies or changes in the prices of existing technologies. Irrecoverable costs are a common feature of very many pharmaceuticals which are likely to be included in the VBP scheme post 2014. Failure to take account of the impact the irrecoverable nature of the costs in establishing VBPs would place the NHS at risk of purchasing technologies where the expected long term benefits which are expected to offset the immediate losses are not realised.

5. Conclusions

The introduction of VBP after 2014 provides an opportunity to found pharmaceutical pricing and access to new health technologies on sound principles; reflective of social values and the reality of a budget constrained NHS. This requires: clarity on key issues of principle and social value; as well as critical details of how it will be implemented and operate.

The type of robust, scientific and accountable assessment of the costs and benefits offered by a technology already embodied in the NICE appraisal process is, of course, essential. However, it is equally important that the assessment of the health expected to be forgone elsewhere in the NHS due to additional costs displacing other NHS activities, i.e., the cost-effectiveness threshold, is equally robust, scientific and accountable. In addition transparent rules of how prices might be negotiated based on such assessment are required. Rules, which are reflective of markets in other sectors where investment is also protected by patent, provide a model which would give confidence that the NHS would not undermine patent protection by exploiting its monopsony power. It would also protect the NHS from manufacturers offering all or nothing deals or engaging in price discrimination. The triggers for when VBPs, once set, would be reconsidered also need to be clearly established. These triggers must include patent expiry and the entry of cheaper generics and significant new evidence becoming available. Without the former, the NHS may never benefit from innovation even in the longer run and, without the latter, incentives for manufacturers conducting evaluative research will be undermined and/or the NHS will not realise the benefits of publically funded research.

The details of how the VBP scheme will be implemented and operate are also important. For example, an effective vehicle for price negotiation might be value-based rebates through a PPRS-type arrangement rather than reductions in UK list prices. Since the UK represents only 3% of the world market the influence of VBP in the UK on global investment appears limited. However, there is an opportunity to use the scheme to align incentives for local prescribing, which, in the absence of mandatory NICE guidance on the use of health technologies after 2014, is especially important. Since VBP is necessarily linked to particular indications and subgroups, there is an inevitable link between price and volume. Therefore, any agreed rebate needs to be combined with volume agreements which might be specified at a national or local level.

The introduction of VBP also poses the question of whether there are other aspects of social value which ought to be included. The notion that premiums ought to be paid for innovation itself has little to commend it as there are other much more effective ways to provide incentives for future innovations of value to the NHS and for inward investment in research and development in the UK. It is no doubt possible that different types of health benefits might be valued differently by society. This is already recognised in the NICE appraisal process where the reference case of equal QALY weighting is taken as a useful starting point for deliberation. VBP would require any values or weighting to be made much more explicit. The difficulty is that the social values or weights that ought to apply and how they should be established is far from self evident. What is critical, however, is that any values or weights which are assigned to the health benefits associated with new technologies must also be applied equally to the health likely to be forgone as services are displaced to fund the new ones. In other words a clear distinction is required between the social values that might be applied and any empirical estimate of the social value of the health likely to be forgone, i.e., a single common threshold is required which is reflective of any weights applied to the type of health benefits offered.

Other effects outside the NHS, such as reduction in the cost of care born by patients and their carers or external benefits to the wider economy, might also be included in assessment of value. All these direct and indirect external effects fall on consumption (gained or lost) outside the health sector rather than on health (gained or forgone) within it, i.e., the type of opportunity costs differ. This ought to be reflected in the weight attached to the different types of effects. For example, external benefits (e.g., a reduction in patent and career costs) cannot be used by the NHS to provide additional health care and generate health. As well as requiring a number of social and scientific value judgements, it will also require some assessment of the external benefits which are likely to be forgone as other NHS care is displaced. In other words, and consistent with the position for social values or weights discussed above, it is not enough to establish that a new technology offers benefits to patients, carers and the wider economy, but that these benefits exceed those likely to be forgone as a consequence of the additional costs of the technology.

Finally, there needs to be a recognition that appropriate VBPs depend in part on uncertainty, the value of additional evidence, whether it can be acquired once the technology is in widespread NHS use and the scale of irrecoverable costs committed by the NHS when purchasing the technology. The link between VBP and evidence provides an incentive for manufacturers to conduct evaluative research relevant to the NHS prior to or after launch. There will be circumstances, however, when the type of research required cannot be conducted once VBP is agreed. Since manufacturers may be unable or unwilling to reduce the price sufficiently to compensate the NHS for the value of evidence that might be forgone, the NHS must retain the right to withhold access until further research is conducted by the manufacturers or through publically funded research.

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