

Implementing Value Based Pricing for Medicines

An introduction

David Taylor

Professor of Pharmaceutical and Public Health Policy

The School of Pharmacy, University of London

There is a view amongst some commentators that the case for ‘value based’ pharmaceutical pricing has been proved, and that it will in future provide a means of calculating fair NHS prices for new medicines that in the UK will obviate the need for the current Pharmaceutical Price Regulation Scheme (PPRS). Some also argue that the latter has already become redundant because of the establishment of NICE, and the criticisms of it contained in the Office of Fair Trading’s 2007 report on pharmaceutical pricing (OFT 2007).

It is certainly the case that the PPRS no longer covers off-patent pharmaceuticals, which in volume terms already represent more than 80 per cent of all the drugs prescribed by GPs. Further, in the context of products such as, for example, high cost new cancer medicines the *de facto* situation is that there is already a VBP approach in place, which defines their maximum acceptable NHS prices. Even if the PPRS would allow higher prices (or, where market conditions permit, allow earnings from other products’ sales to carry a proportion of the costs attributed to such medicines) NICE interventions in the main restrict or block the use of innovations that incremental cost effectiveness analysis indicates cost in excess of around £25/30 thousand per quality adjusted life year yielded.

However, the situation relating to the appropriate pricing of new medicinal goods is more complex than is on occasions assumed. For example, although even within many pharmaceutical companies the PPRS is now seen as unsatisfactory, its provisions may in some respects be socially beneficial. This introductory analysis seeks to explain why pharmaceutical pricing remains a controversial area and to highlight questions that need to be further addressed if value based pricing approaches are to be used by the NHS (as a monopsony medicines purchaser in Britain) in ways consistent with protecting overall public interests in the pharmaceutical sector. Seen in the round, these include ensuring not only the affordability of new medicines, but also maintaining desirable levels of investment into pharmaceutical and allied forms of bio-medical/life science innovation.

The role of prices

In commodity markets like those for generic medicines prices act to balance supply and demand. Their role is to facilitate the efficient allocation of resources, which involves minimising good quality production costs and normalising the profitability associated with the supply of any given product or service. However, in more complex markets like those for health care innovations the role of price mechanisms is more plural. In addition to taking into account the immediate value attached to a good or service by consumers, they may also reflect factors such as the premiums needed to encourage highly risked investment that should – either directly or indirectly – in the long term contribute to very

significant societal gains, yet which in the short term (that is, within the likely effective patent life of an average medicine) may fail financially and/or produce only modest health benefits.

The pricing of new pharmaceuticals can be especially difficult for a variety of additional reasons. They range from the fact that in some instances drug costs are seen – rightly or wrongly – to be a key barrier to patient access to life saving treatment, through to the existence of the medical agency problem and that of moral hazard. For the purposes of this brief outline these last can be said to centre on the fact that typically doctors prescribe medicines, patients consume them and the state (or the insurer) funds them. This means that normal market mechanisms for preventing over-payment may be impaired. In the UK debate about pharmaceutical outlays has also sometimes been an apparent proxy for questioning the affordability of the NHS as a whole, about 90 per cent of the costs of which remain non-pharmaceutical.

The origins of the PPRS

The original policy debate around the post-war establishment of the NHS involved a vision of a post imperial Britain with an increasingly educated and productive domestic population, supported by welfare systems paid for via the international earnings of ‘sunrise’ industries like pharmaceuticals. The antibiotics of the 1940s were seen, not least by some socialist observers, as the first of a long wave of new products that would revolutionise health care and that could be exported to the rest of the world.

In the 1950s the original establishment of the then VPRS (the Voluntary Price Regulation Scheme) can in retrospect be regarded, at least in part, as an attempt to deflect what some civil servants and industry representatives regarded as unhelpful criticisms of health service pharmaceutical spending. In linking UK domestic prices to export prices (which in reality often tended to be high compared to those offered on the home market) the VPRS in its initial form did little or nothing to reduce NHS drug outlays. But it was nevertheless for a time a politically successful solution to the perceived problem of excessive NHS spending on medicines.

But after the 1960 Thalidomide tragedy attitudes began to change, and scrutiny of the pharmaceutical industry intensified. In time the VPRS evolved into the PPRS. The mechanisms adopted within the latter put a ceiling on the returns to capital employed that companies could enjoy from NHS sales, with allowable research, marketing and other expenses being calculated in relation to the core ROCE (return on – historic – capital employed) figures.

Critics argue that the PPRS has in the past encouraged excessive capital investment in this country, and led to the UK paying unduly high prices for individual products. But against this, aggregate NHS spending on medicines has been and remains relatively low, as compared to the figures recorded elsewhere in the OECD.

This country has in addition had an unusually robust record of pharmaceutical research and export from the 1950s through to the early twentieth century, coupled with what is in international terms low spending on advertising and other forms of promotion. PPRS advocates point out that it does not guarantee ‘as of right’ target returns, because of price competition between alternative medicines. The validity of this view depends in part on the extent of the medicines price sensitivity that can be found in the GP and, today more importantly, the hospital pharmaceutical markets. The proportion of NHS resources spent on drugs has fallen since the end of the 1990s, although within the total ‘drugs bill’ the proportion due to hospital costs is rising. (The current figure is in the order of 25 per cent, or <5 per cent of gross hospital costs.)

It is of additional note that low drug prices are not synonymous with low drug spending. Many countries with limited unit prices for medicines have relatively high total pharmaceutical costs, because of factors such as high volume prescribing (as in the case of France) and/or rapid take up of new medicines (as in Spain). Further, the OFT’s 2007 analysis indicated that while the UK remains a successful pharmaceutical research and exporting nation relatively high domestic prices should, at least in instances where other States use the NHS price as a reference, advantage the country. It may also be suggested that the free new product pricing environment in the UK permitted by the PPRS has fostered early medicine launches, so potentially enhancing public access to therapies.

However, against this suggestion there is substantive evidence that the NHS is typically slow to adopt innovative products and practices of any sort (Richards 2010), presumably because of the extent of institutional level financial and other bureaucratic controls on professional freedom of action. NHS pharmaceutical purchasers drive down the actual prices paid for medicines to below ‘list’ levels. But even so, this does not appear to have been effective in encouraging rapid innovation uptake rates. In the case of oncology, for instance, gross UK per capita spending on cancer medicines appears to be only about two thirds of the western European average. This contrasts with the fact that total per capita cancer research spending (public plus private plus charitable) in the UK is when expressed as a proportion of GNP probably the highest in the world (Kanavos et al 2009).

The VBP offer

Value based pricing techniques grew out of the – originally in large part pharmaceutical industry funded – health economics and health technology assessment research pioneered in centres such as the University of York from the 1960s onwards and subsequently institutionalised within the NHS via the creation of NICE in 1999. They rest on the calculation of the incremental cost per QALY generated by a given therapy (which is often referred to as the ICER, or incremental cost effectiveness ratio) set against an assumed NHS affordability threshold. This provides what VBP proponents may take to be an ‘objective’ indicator of whether or not a medicine at a given price offers acceptable value for money.

In this context the full meaning of the word incremental is frequently misunderstood in public debate. Put simply, it relates to the difference in outcome achieved between a new

medicine/treatment package (ie the extra gain derived from a new treatment, as compared to that offered by current therapy) relative to the difference in cost between the former and standard care. To illustrate the significance of this, an at one time ‘affordable’ drug, that could for a proportion of its users perform better than any other available, may deliver QALYs at an *average* price of £5,000. Yet its incremental cost effectiveness ratio may become in excess of £30,000 per QALY if, for instance, a similar medicine were to lose patent protection, and subsequently be supplied as a generic at a much reduced price.

Hence in 2007 the OFT argued that in the case statins the PPRS was causing the NHS to pay in excess of some £500 million per annum. This claim was based on the fact that the medicine simvastatin had recently lost patent protection and become available as a low cost generic, while other statins still under patent maintained price levels set at around the original simvastatin level (Taylor and Craig 2009). However, this last example does not necessarily suggest a pricing problem. It may be rather be indicative of a failure of the NHS to achieve cost effective purchasing and prescribing at a particular point in time. One possible downside of the VBP model employed by the OFT is in effect designed to curtail patent holders’ rights to charge a premium for their products during the full life of their patent.

As previous 2020Health publications have argued (2020Health 2009), medicine prices should fairly represent their value. But defining the latter is not straightforward, especially when long term gains and societal level costs and benefits are considered. One obvious problem is that it is normally impossible to assess the full clinical value of any medicine at the time of its initial launch. The types of difficulty this leads to with regard to implementing value based pricing are discussed below, following a brief discussion relating to the intended and/or possible unintended impacts of VBP based approaches on not only overall health service pharmaceutical spending but also the behaviour of companies and global industrial structures.

Would VBP have saved the NHS money if it rather than the PPRS had been in place between the 1950s and 2000?

Given that the actual working of any pricing and purchasing system in any economic sector may be subject to political and allied interventions (for example, with regard to the essentially arbitrary affordability thresholds set in the case of medicines VBP) questions like that posed above cannot be answered categorically. However, the last half of the twentieth century was one of very considerable success in terms of producing clinically effective new medicines. This implies that had value based pricing been in place instead of the PPRS during that period, then the NHS could well have spent as much or more on pharmaceuticals than was actually the case. However, investment levels in UK based industrial facilities and research might have been lower, while spending on drug promotion would (without the introduction of a new regulatory system) almost certainly have been higher.

Future pharmaceutical research and development productivity may for a variety of reasons be lower than it was in the last 50-60 years¹. In these circumstances a product by product VBP pricing approach may in theory at least be likely to reduce NHS medicines spending as compared to one based on the ‘traditional’ (ie pre NICE) PPRS approach. Yet a counterbalancing factor is that this would be at a probable cost in terms of lost UK investment and research activity (see below). The likely impacts of any such declines on the economy as a whole and its capacity to support employment and welfare systems cannot be reliably quantified, but they are likely to be significant. There is a case for believing that at the domestic economy level they could greatly outweigh any savings made.

As already indicated, the essence of the academic case against the PPRS is that, as with profit regulation based systems in other sectors, it may have allowed companies with new products of marginal incremental value to ‘price up’ in order to reach the highest permissible target returns. A product by product ICER based pricing approach ought to prevent this. Another possible benefit from an international perspective could be that the removal of other control elements embodied in the PPRS would allow companies more scope to focus production and research activities in the most cost effective settings. That is, they would have enhanced freedom to cut investment and activity in relatively high cost areas such as the UK and Western Europe more broadly whenever concentrating it elsewhere would enhance profitability.

Abandoning the PPRS and introducing VBP in the UK might therefore be expected to, over time, contribute to increased global economic efficiency. But factors such as the movement of pharmaceutical goods within Europe, the high level of generic prescribing in Britain, the current work and impact of NICE and the pressures on, for example, NHS hospital pharmacies to buy as cost effectively as possible complicate the local picture. Given the real-world situation of the UK market today, removing the PPRS would in itself probably have little effect either way on the true level of NHS pharmaceutical outlays, albeit that it might nevertheless accelerate possible reductions in domestic research activity and/or other industrial expenditure levels.

The limitations of VBP for medicines

Value based pricing clearly has a potential to help inform the fairness or otherwise of the amounts NHS bodies pay for patented medicines. Its introduction could at a political level, not unlike that of the original VPRS in the 1950s, for a time at least allay concerns

¹ Pharmaceutical research outlays have increased absolutely over recent decades, but have stayed relatively constant as a proportion of total health outlays in OECD countries. Similarly, research productivity has apparently fallen, but this is part a function of increased regulatory intervention (including more measures which stop drugs being marketed on safety and/or relative cost grounds) coupled with the changing nature of the underlying bio-medical problems being addressed. Successfully treating later life problems like cancers and neurological diseases may be more challenging than finding effective anti-infectious agents, albeit that in the long term its relative rewards may well be as great or greater. The view taken here is that there remains good reason to believe that investment in continuing pharmaceutical research will in time generate human/social benefits likely to make the initial costs of discovery and development appear relatively insignificant, but that the time scale over which such rewards will become manifest is (at least presently) extending.

about whether or not the use of tax payers' money to pay for NHS supplied 'free' medicines is legitimate. Yet at a fundamental level the techniques embodied in VBP do not address the issue of how much money it is right for the health service to channel into the private (or public) sectors for pharmaceutical research aimed at generating inherently uncertain future welfare or other benefits. Even ignoring such concerns, there also remain a range of lower level methodological and allied limitations relating to the practical application of VBP for medicines. They include:

1. The essentially arbitrary nature of the incremental gain affordability thresholds set. Associated with this there is the fact that in other areas (like maintaining public order and providing care for people considered a danger to the public) such thresholds may be very higher or lower than those used by NICE in relation to the QALY, or not be taken into account of at all in public policy making and service and product purchasing processes.
2. As recently highlighted by Professor Sir Ian Kennedy in a report commissioned by NICE (Kennedy 2009), there may be a lack of evaluation of the additional health related benefits of items such as new medicines for conditions like, say, dementias. In this last instance, for example, the model employed by the National Institute for Health and Clinical Excellence appears not to take into account the gains that may be enjoyed by informal carers and family members as a result of better symptom or disease management.
3. Beyond this, NICE is also not presently charged with evaluating the long term external benefits that will in time be generating as a result of, for instance, an improved understanding of cancer genomics and/or the provision of high quality employment in the pharmaceutical sector. Although it may in this context be argued that other agencies, such as the newly established Office for Life Sciences, may be better placed to take on an industry 'sponsorship' role, the arguable reality is that if the NHS is as the monopsony purchaser of prescription medicines only willing to pay medicine prices based on immediate individual patient level gains, that will be the *de facto* driver of the overall national system.
4. Problems associated with the use of aggregated data in circumstances where there is substantial variance within populations, and a lack of appropriate provision for identifying and meeting humanely the needs of people who can reasonably be regarded as 'exceptional cases'. The potentially severe harm to public confidence associated with NHS patients having to, or seeming to have to, 'plead for their lives' with the support of doctors who wish them to have access to non-approved treatments is one cost linked to this area.
5. Lags between best practice developments and the publication of supportive evidence. In the oncology context such problems may become apparent because although the effectiveness of anti-cancer drugs is normally first demonstrable in late stage disease treatment, their optimally effective use may be at an earlier stage. If because of an unduly crude application of VBP based pricing principles

clinically informed logical extrapolations cannot be used in a timely manner to justify new treatment applications, health outcomes will on occasions be impaired.

6. The inherent challenges of measuring and comparing utilities of different types, both within the health sphere and between that and other areas, including industrial development. The possibly special nature of end of life care illustrates this area of concern.

It would be beyond the scope of this brief introduction to attempt to analyse in depth all the questions that such factors raise for the further implementation of value based medicines pricing. But examples of possible solutions include:

- ***Employing variable affordability threshold levels?***

It may be that some areas of health related innovation are likely to be more valuable in the long term than others because, for instance, of their potential for application outside the health sphere. Alternatively, as suggested above, achieving some health related social goals may be widely regarded as inherently more desirable than attaining others, contrary to the assumptions currently underpinning incremental cost effectiveness analysis. Thus being able to cure life threatening or disabling diseases in young people may be regarded fundamentally more important than reducing mortality and morbidity amongst those who have enjoyed a 'fair innings'. Similarly, democracies may wish to pay special attention to the needs of people affected by rare disease on social inclusion grounds.

Were this so, it means there is a case for applying in a transparent manner varying affordability thresholds in differing health care fields, albeit that a more viable way forward in practice might simply be to raise the average incremental affordability threshold used. If as can reasonably be expected the genericisation of the high volume medicine market continues, one check to be employed in this context is whether or not overall medicines spending is remaining constant as a proportion of all health spending.

- ***Varying medicine prices – should they be increased post-launch, as and when a medicine's efficacy becomes better demonstrated?***

In response to the lagged evidence problem identified in point 5 above, some authorities have suggested that low initial reimbursable medicine prices could be raised if and when new evidence of treatment effectiveness comes available during the patent life. This is arguably especially relevant to areas like cancer care and/or long term neurological disease treatment. From a VPB perspective such a strategy would be entirely justified, and is theoretically attractive.

But in practice raising significantly the price of an established product is much more difficult than lowering it, especially in today's pan-European and wider global markets. The danger for innovators is that if they were to introduce an expensive to develop new medicine at a relatively low initial price, this would become its international reference

cost. From the perspective of assuring public interests in the ongoing financing of high risk research a more attractive option might be to permit a relatively high ‘free market’ reimbursable launch price, and to have a PPRS-like provision for repaying excess profitability as and when the full potential of the product is revealed.

- *Varying patent terms in relation to VB prices and returns on permitted investments?*

The duration of a product’s exclusivity of supply period is a critical determinant of the price needed to allow financial viability in relation to the marketing of any particular pharmaceutical innovation. In his recent evaluation of the work of NICE and its implications for the future, Professor Sir Ian Kennedy excluded from his analysis a consideration of issues like patent terms relative to changing research costs and productivity on the grounds that any change in current IPP provision would require pan-national agreement. However, this is although understandable unhelpful regarding the wider policy debate on pharmaceutical pricing.

If the time taken to develop new medicines is increasing and the number of successful new medicines produced per quantum of resource invested is for whatever reason falling, then one potentially sustainable way of keeping product prices down to VBP defined levels would be to allow variable patent life extensions, in part determined (regionally or ultimately globally) via principles like those embodied in the PPRS. Such an approach might also provide a way of addressing ‘evidence lag’ related concerns to be resolved more elegantly than may be possible via post marketing price increases.

- *Enhancing local clinical choice?*

As already noted, there is good evidence that the hospital sector is price sensitive in relations to medicines use, and that the combination of NICE judgments, PCT interventions and the reality of generic prescribing mean that past criticisms of GP medicine price awareness are of only limited relevance in today’s context. It may also be worth emphasising that in primary care the available evidence is that when it was in place GP fund holding succeeded in promoting increased price sensitivity and economically informed clinical decision making across a range of areas.

Further evidence of increased ‘demand side’ medicines use abilities might ultimately lead to a situation where the perceived need for formal NHS pharmaceutical price controls of any sort is reduced, although unregulated free pricing is not advocated here. Rather, what is important to stress is that greater reliance on cost and patient outcome informed clinician choice within the NHS could promote innovative practices and prevent unduly rigid interpretations of NICE or related judgments (or the lack of them) regarding what the NHS can or cannot afford to offer individual patients.

This could help promote more effective case by case local decision making and further prevent the sorts of unsatisfactory situation referred to in point 4 above. New or modified existing approaches could still be used to identify affordable NHS medicine price levels

in normal/average circumstances. But local clinician led care quality management processes could be used to make exceptional care decisions in progressively more humane and patient acceptable ways.

Conclusions: towards value based NHS medicines pricing within a modified PPRS structure

Rather than relying on private companies being able to fund pharmaceutical research and development through charging premiums for innovative (patented) medicines, some critics of current arrangements believe that it would be preferable to develop alternative mechanisms. They may point to initiatives such as the Gates Foundation, and the fact that there has already been some shift in the UK towards providing enhanced fiscal incentives for research investment. In future there could also be additional movements in the direction of offering ‘prizes’ for successful therapeutic ‘inventions’, coupled with more reliance on directly taxpayer funded research markets.

Taken to the extreme, developments in this direction could ultimately mean that the funding of all private sector medicines research will be dis-linked from selling medicines *per se*, and that all drugs will be supplied at a generic (ie commodity competitive) price from the time of their initial launch. Models of this sort may seem to be an attractive proposition to people or institutions wishing to cut the cost new medicines. Yet there is little or no evidence that they would in reality benefit the public, if members of the latter wish to combine continuing innovation with ensuring affordable prices for licensed drugs.

The use of ‘value based’ techniques for the pricing of patented pharmaceuticals may therefore appear to offer a more secure way of balancing these competing goals in a sustainable manner, depending where the affordability threshold is set. The higher this last the greater the likelihood of investors being prepared to accept the risks and uncertainties inherent in funding pharmaceutical research and development. The lower it is the lower the amount of money the NHS will have to find for delivering treatments.

However, people in countries such as the UK also have interests in issues like the indirect benefits that conducting pharmaceutical research and producing high quality medicines can bring to their communities. They may also be seeking assurances in contexts such as balancing NHS contributions to promotional as opposed to medicines research costs. The view suggested in this introduction is therefore that the linked health and wealth creating objectives of the ‘traditional’ PPRS should not be abandoned, but rather that future UK pharmaceutical pricing approaches should seek to combine the appropriate use of VBP methodologies with desirable elements of the former. Care should be taken to ensure that the overall approach to medicines pricing in the UK does not become unduly dominated by the in some respects limited calculus that VBP alone embodies.

The main body of this publication explores in technical detail issues relating to the further implementation of value based pricing for medicines within the NHS, via its existing or new institutions. But a key point to finish on here is that it is in common sense terms

unlikely that a more coherent set of twenty first century national health, industrial development and medicines related policies and controls will be achieved if the process of deciding what the NHS is prepared to spend on individual medicines becomes functionally split from that of protecting the community's longer term interests in pharmaceutical sector as a whole

Steps towards the end of establishing a better integrated system than that currently in place could include redefining and broadening the health related benefits included in cost effectiveness calculations, and recalibrating the 'cost per incremental QALY' affordability thresholds applied within the overall pharmaceutical price regulation framework employed in this country. It might also be worthwhile considering whether or not a secondary 'average cost per QALY' measure should be introduced as a check against unwisely judging beneficial treatments unaffordable for NHS service users.

But in conclusion an even more fundamental stepping stone towards the end point of creating a stable and appropriate twenty first century financial environment for medicines research and successful marketing could be provided by a rigorous re-evaluation of the appropriate duration of intellectual property protection for new medicines. Without provisions in place to assure appropriate levels of intellectual property protection in changing scientific and commercial environments, the implementation of 'value based pricing' can alone have only limited utility.

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