THE OFFICE OF HEALTH ECONOMICS RESPONSE

to the Department of Health's consultation:

A new value-based approach to the pricing of branded medicines: a consultation

17 March 2011

The response was drafted by the staff of the Office of Health Economics. It should not be taken to represent the views of any individual.

Consultation Questions

- **Are the objectives for the pricing of medicines set out in Section 3 of this document – better patient outcomes, greater innovation, a broader and more transparent assessment and better value for money for the NHS – the right ones?**

  Yes ✓ **No**

Comments

The objectives are admirable as far as they go but in our view omit three important aspects:

1. The role of pricing in promoting a strong and productive pharmaceutical industry
2. The UK's responsibility as one of the richest economies in the world to share a reasonable part of the burden of developing new medicines that will benefit people globally
3. The wider context of other factors impinging on medicines' prices

1. The current PPRS includes an objective to: "Promote a strong and profitable pharmaceutical industry that is both capable and willing to invest in sustained research and development to encourage the future availability of new and improved medicines for the benefit of patients and industry in this and other countries." The 'greater innovation' objective of value-based pricing (VBP) of branded medicines should explicitly state that.

2. As discussed in Danzon (1997), R&D is a “joint cost” which does not vary with the number of users reached worldwide. That is, companies do not have to incur much if any extra R&D investment to launch a product in an additional market (variable costs such as marketing and distribution usually arise). In addition, because R&D costs are incurred largely in advance of product sales, they represent a large proportion of the total pharmaceutical costs (around 31% according to Danzon, 2007). As it is not possible to attribute R&D costs to a specific national market or user, individual country payers may seek to free-ride by driving prices down, leaving others to pick up the R&D bill. From an individual country perspective, this can lead to better outcomes in the short term, as it allows them to obtain more affordable prices, but in the long term it can have negative impact on R&D investment and therefore lead to less innovation.

Economic literature applying Ramsey pricing principles suggests that differential pricing across countries, with prices inversely related to demand elasticity is the (second-best) optimal way to pay for the substantial joint cost of R&D (Danzon, 1997; Danzon and Towse, 2003; Jack and Lanjou, 2005). Under plausible assumptions, countries’ demand variation in response to price changes is mainly determined by GDP per capita. Thus higher income countries, such as the UK, should accept paying higher prices than lower income countries. VBP does not conflict with a system of differential pricing across national markets based on GDP per capita. As shown by Danzon et al. (forthcoming) , as long as each government/payer sets an incremental cost effectiveness threshold based on its citizens’ willingness to pay for health care, optimal prices (achieving second best static and dynamic efficiency) will vary according with national incomes.

We propose that one objective of VBP should be to ensure that when pricing medicines for use in the NHS, the UK does not free ride on lower income countries.

3. Alongside the objectives it needs to be recognised explicitly that any scheme of “value based pricing (VBP)” of branded medicines in the UK does not exist in a vacuum and that the form and impact of that scheme are affected by other factors which act on medicines prices, particularly:

- Policies and initiatives which increase the price and quality sensitivities of prescribers of medicines, e.g. budgeting arrangements for GPs, activity-based funding arrangements in the hospital sector, pay for performance schemes, and many more;
- The existence of competition for some, but not all, newly launched medicines from other medicines (on- and/or off-patent) and non-medicine treatments. Where this competition is effective it renders VBP redundant;

The Competition Act (1998), which deals with abuse of monopoly power and competition-restricting agreements. Mestre-Ferrandiz (2006) concisely summarises how, and its impact on the NHS medicines market**.**

References:

Danzon P. (1997) Trade and price differentials for pharmaceuticals; policy options. OHE, London.

Danzon P. and Towse A. (2003) Differential pricing for pharmaceuticals: reconciling access, R&D and patents. *Int J Health Care Finance and Econ* 3: 183-205.

Danzon P., Towse, A. and Mestre-Ferrandiz J. (2011) Value Based Differential Pricing. NBER working paper (forthcoming). Available from atowse@ohe.org

EFPIA (2010) Overview of International Reference Pricing in Europe.

Jack W. and Lanjou J. O. (2005) Financing pharmaceutical innovation: how much should poor countries contribute? The World Bank Economic Review, 19(1), 45-67

Mestre-Ferrandiz J. (2006) The faces of regulation – Profit and price regulation of the UK pharmaceutical industry after the 1998 Competition Act. OHE: London. Available at: http://www.ohe.org/page/publications/publication.cfm?catid=28&itemid=77&archive=0

- **Should value-based pricing apply to any medicines that are already on the UK market before 1 January 2014? If yes, should this be determined on a individual basis, or are there particular groups of drugs which might be considered?**

Yes ☐ **No** ☑

Comments

Retrospective pricing does not give helpful price signals. Worse it undermines the credibility in the eyes of manufacturers and investors of the stability/durability of future prices, thereby undermining the intended incentivisation of R&D.

- **Are there types or groups of medicines, for example, those that treat very rare conditions, which would be better dealt with through separate arrangements outside value-based pricing?**

Yes ☑ **No** ☐

Comments

As noted in our comments on the first question above, there is competition for some, but not all, newly launched medicines from other medicines (on- and/or

off-patent) and non-medicine treatments. Where this competition is effective it renders VBP redundant – as a value-based price would then not be the binding constraint on price level.

VBP processes and corresponding information submission requirements should be limited to where either there is not alternative treatment or, if there is, to where the price sought by the manufacturer implies that treatment costs would be expected to rise. Where the medicine would not be cost increasing for equivalent or better treatment benefits, VBP processes should be waived.

For medicines likely only ever to have small sales in the UK it would be inefficient to require an elaborate 'value' determination process with corresponding information and administrative burdens and costs. Thus we suggest a *de minimis* rule be applied, similar to the £5million annual sales 'small company' definition used in the PPRS. Thus a new medicine whose UK annual sales are not expected by the Department of Health to exceed £5 million.

- **Do you agree that we should be willing to pay more for medicines in therapeutic areas with the highest unmet needs, and so pay less for medicines which treat diseases that are less severe and / or where other treatments are already available?**

Comments

We agree that the value of a medicine depends on social value judgements including an apparent willingness to value more highly, other things being equal, treatments for patients who are more severely ill.

We agree that it is not always clear how society's preferences are taken into account in the current system (paragraph 4.15). We therefore support efforts to develop a consistent method for incorporating societal concerns into the health care decision making process.

We are surprised by the lack of reference in the consultation document to NICE's existing position on social value judgements, which has been informed by the published literature on popular preferences, workshops and roundtable discussions involving members of the Institute's staff and experts in the field, and the deliberations of the NICE Citizens' Council (NICE, 2008a). Three members of NICE's senior leadership team have also described the 'special circumstances' to which special weighting has been given in the appraisal process (Rawlins et al., 2010). One of these special circumstances is described as 'severity of the underlying illness' – NICE's advisory bodies have in the past given relatively more generous consideration to the cost-effectiveness estimates of treatments for the severely ill. This is consistent with a growing body of evidence which suggests that society favours giving priority to the treatment of the severely ill (Shah, 2009). The consultation document proposes an adjustment of the basic price threshold to reflect severity of illness, but omits to mention many of the other special circumstances and criteria that are supposed currently to be taken into

consideration by NICE's advisory bodies.

References:

NICE (2008) Citizens Council Report: Quality Adjusted Life Years (QALYs) and the severity of illness; 2008, http://www.nice.org.uk/getinvolved/patientandpublicinvolvement/opportunitiestogetinvolved/citizenscouncil/reports/reports.jsp

Rawlins M. et al. (2010) "Pharmacoeconomics: NICE's approach to decision making." *British Journal of Clinical Pharmacology* 70: 346–349

Shah KK . (2009) Severity of illness and priority setting in healthcare: a review of the literature. *Health Policy* 93: 77-84.

- **How should we approach the issue of a single drug which delivers significantly different benefits in different indications?**

Comments

Patient Access Schemes (PAS) offer a route by which, *de facto*, non-linear pricing is possible, permitting higher prices to be paid for the first tranche of use of a medicine per year than for a second or subsequent tranche. The width of each tranche can equate to expected use for indications and patient subgroups with different abilities to benefit from the medicine. VBP would not obviate this. Hence we disagree with your statement in paragraph 2.15 that "[PAS] are not long-term solutions": they might be. The administrative burden of PAS needs to be considered, and may argue against particular PAS proposals, but it is not a reason to abandon all PAS, even after the introduction of VBP.

PAS provide a mechanism for handling uncertainty through the use of outcome based schemes, in effect a form of coverage with evidence development. This is in principle an efficient way to handle uncertainty for patients, the NHS, and companies subject to transaction costs and reversal costs (Towse and Garrison, 2010). PAS also provide a means for companies to offer confidential discounts to the NHS in an international reference pricing environment. Such reference pricing can lead to delays to market where countries are seeking lower prices. Although locally efficient (i.e. reflecting payer willingness to pay) such prices may not be offered (Danzon and Epstein, 2008; Garau, Towse and Danzon, 2011). Confidential discounts are an efficient way to price differentiate (Danzon and Towse, 2003). Such arrangements are beneficial to the NHS and also increase overall returns to R&D.

References:

Danzon P. and Towse A. (2003) Differential pricing for pharmaceuticals: reconciling access, R&D and patents. *Int J Health Care Finance and Econ* 3: 183-205.

Danzon P. and Epstein A. (2008) Effects of regulation on drug launch and pricing in interdependent markets, NBER Working Paper 14041

Garau M., Towse A. and Danzon P. (2011) Pharmaceutical pricing in Europe: is differential pricing a win-win solution? Office of Health Economics. Occasional Paper 11/01.
http://www.ohe.org/object/download.cfm?lib=liDownload&id=715

Towse A. (2010) "Value Based Pricing, Research and Development, and Patient Access Schemes. Will the United Kingdom get it right or wrong?" *British Journal of Clinical Pharmacology* Vol.70; Issue 3 pp360-366

Towse A. and Garrison L. (2010) Can't Get No Satisfaction? Will Pay for Performance Help? Toward an Economic Framework for Understanding Performance-Based Risk-Sharing Agreements for Innovative Medical Products. PharmacoEconomics.28(2): 93-102.

- **What steps could be taken to address the practical issues associated with operating more than one price for a drug, if we took such an approach?**

Comments

See our comments in response to the previous question.

- **Do you agree that – compared to the current situation – we should be willing to pay an extra premium to incentivise the development of innovative medicines that deliver step changes in benefits to patients but pay less for less innovative drugs?**

Comments

If value were to be fully recognised in all its aspects, and small improvements recognised and rewarded (proportionately) as well as large ones, there would be no need for separate incentivisation of innovation. There may also be other policy options for incentivising innovation (Sussex, 2010). We are concerned, however, that the drafting of the consultation document implies an incomplete understanding of key features of innovation and of the economics of the pharmaceutical innovation process. See our comments in answer to the next question.

Reference:

Sussex J. (2010) Innovation in Medicines: Can We value progress? Office of Health Economics.

- **In what ways can we distinguish between levels of innovation?**

Comments

Your paragraph 4.25 refers to "an appropriately increased incentive to companies to focus their resources on achieving ***genuine*** step changes in clinical performance, rather than seeking just to make incremental changes" (emphasis added). This comment implies a misunderstanding of the pharmaceutical R&D process. "Incremental" changes are genuine and worth having. As outlined in greater detail in OHE's "Many Faces of Innovation" report (which references the innovation literature in general), new products may be more or less innovative: innovation is a matter of degree, not a quality that is simply present or absent. Innovation is also multi-dimensional. The greater the improvement on the more dimensions, the greater is the degree of innovation.

Three important aspects of innovation need to be highlighted. First, the innovative process entails major uncertainty, so that its outcome is hard to anticipate. Thus, we believe that companies do not usually set out to achieve only small incremental changes.

Second, innovation is a cumulative activity. This means that small steps are important too.

Third, there is competition in pharmaceutical R&D. Different companies might be investing resources in R&D for the same therapeutic area without knowing whether or nor not they will be the first one to the market (which could be deemed as a "genuine step" as, by definition, no other therapeutic alternatives are available at that point). Development of follow-on drugs often occurs almost contemporaneously with that of the first-in-class. Thus much R&D in the pharmaceutical industry is simultaneous across competing companies, so it is hard to meaningfully distinguish between R&D that is directed to the first available treatment for any particular indication and to follow-on products. For example, Di Masi and Paquette (2004) show that in a substantial number of cases in recent periods, the first drug in a class to reach the US marketplace was not the first to enter clinical testing either in the US or anywhere in the world.

Moreover, the innovation race brings competition - periods of marketing exclusivity have been shrinking since the 1960s for first-in-class medicines as a result of therapeutic competition from follow-on medicines (Di Masi and Paquette, 2004). These authors also show that, by examining the therapeutic

ratings that the US FDA has assigned to follow-on drugs, approximately one-third of all follow-on drugs have received a priority rating from the US FDA. In addition, 57% of all therapeutic sub-classes have at least one follow-on drug that received FDA priority rating. This highlights that the first in class medicine is not necessarily the best in class.

One example of the importance of using a broad(er) definition of innovation for pharmaceuticals can be found in Berndt et al. (2006), where they investigate the appropriateness of traditional measures of innovation. In particular, they try to answer the question of whether there has been a reduction in important innovations that occur after a drug receives initial market approval. By looking at ACE-inhibitors, H2 antagonist/PPIs and SSRI/SSNI, they show that in these areas significant incremental innovation has been occurring in the form of supplementary approvals for new dosages, formulations and indications. These supplementary approvals account for a substantial share of drug utilisation in these areas.

Thus there are damaging consequences for not rewarding incremental innovations.

References:

Berndt E., Cockburn I. and Grepin K. (2006), The impact of incremental innovation in biopharmaceuticals, Pharmacoeconomics, 24, Suppl 2

DiMasi J. and Paquette C. (2004), The economics of follow-on drug R&D: trends in entry rates and the timing of development, Pharmacoeconomics, 22, Suppl. 2: 1-14

OHE Consulting (2005), The Many Faces of Innovation. Report for EFPIA. Available at: http://www.efpia.org/Objects/2/Files/Manyfacesofinnovation.pdf

- **How can we best derive the weights that will be attached to each element of the assessment? Are there particular elements we should put greater weight on?**

Comments

Some evidence already exists on social preferences regarding some of the decision making criteria mentioned in the consultation – for example, Dolan et al (2005) examine a range of factors; Shah (2009) reviews the evidence relating to the importance of severity; and Baker et al (2010) report evidence on the willingness to pay for improvements in quality of life as opposed to life saving or extension of life. However, there are limitations to the ability of that evidence to inform VBP. Studies often focus on a single issue (such as severity, or end of life, or age) and explore trade-offs between that and QALY gains. VBP requires weights for the combination of elements identified as relevant to price regulation. Further, existing evidence uses a wide variety of

methods, affecting the comparability of results. VBP will probably require new research to establish defensible weights for the particular combination of elements/criteria to be considered.

An important consideration in establishing the weights is whose opinions should count. Much of the research to date has used stated preference methods with samples of the general public**.** The use of general public preferences is in keeping with other methods that are a standard part of HTA – for example, the quality of life weights used in QALYs. Logically the weights should have at least some basis in general public preferences. However, the elements potentially included in VBP reflect a wider set of concerns, including government and industrial policy perspectives, that may not be adequately reflected in stated preferences regarding health and health care. Second, relying on stated preferences surveys to generate a specific set of weights to 'pre-populate' a VBP process is likely to be too restrictive and, given the wide range of methods that exist to generate those weights, to suggest a spurious degree of precision. While it is important to be broadly consistent in the approach to pricing across technologies, a practical approach is likely to involve establishing a plausible *range* of weights, informed by a range of methods and a range of viewpoints, including those of NHS commissioners, NICE technical appraisal committees and government in addition to those of the general public. Decision makers will need to retain some degree of flexibility in their initial consideration of the relevant weights, and in subsequent price negotiations.

Regardless of how the weights are derived, and whose preferences they are based on, it will be crucial that preliminary modelling and piloting work be undertaken to provide a 'sense check' on how these will operate to guide VBP, prior to implementation.

References:

Baker R. et al. (2010) Weighting and valuing quality adjusted life years using stated preference methods: preliminary results from the social value of a QALY project. *Health Technol Assess.* 2010 May;14(27):1-162.

Dolan P., Shaw R., Tsuchiya A. and Williams A. (2005), QALY maximisation and people's preferences: a methodological review of the literature. *Health Economics,* 14: 197–208. doi: 10.1002/hec.924

Shah KK . (2009) Severity of illness and priority setting in healthcare: a review of the literature. *Health Policy* 93: 77-84.

- **What measure should we use to define the weightings? Options might include using the existing Quality Adjusted Life Years (QALY) measure, patient experience and expert opinions or some combination of these.**

Comments

VBP requires agreement on the elements of value to be included; measurement of the magnitude of those for each medicine; a range of weights to be applied to each element; and a way of aggregating the different elements into an overall assessment of value.

Paragraph 4.27 of the Consultation Document proposes a shift from the use of a narrow perspective in which the benefits of a treatment refer only to the health gains accruing to the patient, to a wider perspective that includes consideration of all benefits that are important to society. We support this proposal as we believe that it is right that a tax-funded health system should consider all benefits to society. The true value to society of a given medicine may be over- or underestimated if its impact on elements such as patients' experience of care, their and their carers' convenience, time and travel costs, are ignored.

Similarly, we agree that the price threshold should in principle reflect all types of health gain, including those that are not captured by standard pharmaco-economic assessment tools due to measurement difficulties (paragraph 4.23).

The consultation describes a QALY-centric approach: the QALY is taken as the main measure of value, and other elements of value are handled by multiplying the incremental QALYs by weights intended to reflect the other benefits generated by that technology, or by flexing the £/QALY threshold, which amounts to the same thing. We consider this weighted-QALY approach inappropriate for the following reasons:

First, it relies on the incremental QALY being an adequate measure of health gain in all cases. In disease areas where quality of life (QoL) is difficult to measure and value, standard instruments and methods may fail to capture the effects of disease or be insufficiently sensitive to detect improvements in health. For example, sensory impacts (such as vision, hearing) are known to pose challenges. Garau et al. (2010) sets out some potential limitations of QALYs with respect to cancer. NICE notes the challenges involved in measuring and valuing QoL in children, and indicates this as a reason why children are given special consideration in its HTA process (Rawlins et al (2010). Some types of health care are simply not appropriate to value using QALYs (for example, infertility treatments). In all such instances, relying on QALYs gained as the baseline measure against which other sources of value are reflected as some multiple of QALYs will act to embed and amplify any shortcomings.

Second, weighting QALYs is an intuitively plausible approach for some but not all of the elements of value. Some criteria may be thought of as broadly proportional to the number of incremental QALYs gained by a technology. For example, severity of illness reflects the relative value society places on QALYs gained by those with relatively poor health. For this criterion it might be acceptable to weight the QALYs produced, i.e. to multiply the crude QALYs gained by a 'severity' adjustment factor, as seems to be proposed by the

consultation document. But other criteria are not proportional to the QALYs produced, e.g. process-of-care benefits to patients or their carers, savings of patient or carer time or expense. Combining these other sources of value requires a means of describing and measuring them and some common currency for reflecting the relative value of them alongside QALYs and (where appropriate) other measures of health gain.

One pragmatic way of achieving this is to assign scores/points to each type of 'value' and assess medicines using multiple criteria decision analysis (MCDA) approaches. Some types of benefits are best expressed financially: time and cost savings to patients and carers; cost savings to other parts of public spending (e.g. social care, education, the criminal justice system). These are best combined with the costs of treatment to provide a net cost measure (which can of course be negative). This kind of MCDA approach is already used at a local level in the NHS and in health care systems internationally. See: Devlin N and Sussex J (2011).

References:

Rawlins M. et al. (2010) "Pharmacoeconomics: NICE's approach to decision making." *British Journal of Clinical Pharmacology* 70: 346–349

Garau M. et al. (2010) *Using QALYs in cancer: a review of the methodological limitations.* OHE Research Paper 2010/01,

Devlin N. and Sussex J. (2011) "Incorporating multiple criteria in HTA: methods and processes" Office of Health Economics; March 2011 – in press but available from the authors: ndevlin@ohe.org and jsussex@ohe.org

- **How can we best derive the different categories for burden of illness and therapeutic innovation and improvement?**

Comments

We are not convinced that a categoric approach is the best. As indicated in our previous comments, continuous measures of all aspects of value are desirable, or at least a highly granular approach such as is common in multi-criteria decision analysis scoring systems.

- **What approach should be taken under value-based pricing where insufficient evidence is available to allow a full assessment of the value of a new medicine?**

Comments

Uncertainty surrounding the estimates of the value of a new medicine is an essential aspect to consider in any reimbursement decision. However,

producing further research to reduce this uncertainty is costly and will require an assessment of whether the value of this additional information, in terms of higher benefits in future, offsets forgoing the benefits of immediate access to new medicines. In a context where prospects of obtaining further evidence are undermined when a new drug is reimbursed, a key issue is whether, under the VBP, incentives will be in place for manufacturers to provide this information when needed.

A recent study by Griffin et al. (2011) proposes a framework where reimbursement decisions not only depend on the expected cost-effectiveness but also on the opportunity loss of immediate adoption of a new medicine (measured as the health benefits of future patients forgone if further research is not conducted). The opportunity loss has to be assessed in a formal, explicit and transparent analysis on which decision rules can be based on. This approach provides incentives to manufacturers to reduce the price or provide additional evidence; in fact they face a trade-off between these two options. Forms of coverage with evidence development may be possible as set out in Towse and Garrison (2010) involving the collection of observational data or the use of clinical trials either in the NHS or elsewhere. An MRC/NICE commissioned study on “Only in Research” options, exploring these and other issues, is currently underway led by the University of York.

We recommend that the design of VBP should include an evaluation of this and other frameworks that can help to incentivise companies to provide additional information when required.

Whenever there is insufficient evidence about the value of a new drug, the Consultation Document proposes an approach where initial price at launch would depend on the available evidence but this would be reviewed when new evidence becomes available (paragraph 5.8). As discussed above, proper incentives will need to operate in order to generate this additional information. Furthermore, the value assessing body should publish standards of the quality of the evidence required for any post-launch studies, to be endorsed by the public organisations or manufacturers commissioning the research.

As alternatives to randomised controlled trials (RCTs), NICE also recommends other studies such as indirect comparisons, mixed treatment comparison and meta-analysis. Observational studies might also be considered a fruitful source of evidence under VBP. Under VBP it would be desirable to establish a transparent process setting standards of further research and its operability.

In addition, there should be set criteria indicating how the new evidence gathered would lead to variations in price.

References:

Griffin S.C. et al. (2011) Dangerous omissions: The consequences of ignoring

decision uncertainty. *Health Econ*. 20: 212-224.

Towse A. and Garrison L. (2010) Can't Get No Satisfaction? Will Pay for Performance Help? Toward an Economic Framework for Understanding Performance-Based Risk-Sharing Agreements for Innovative Medical Products. PharmacoEconomics.28(2): 93-102.

- **Does the system set out above describe the best combination of rapid access to prices and affordability?**

Comments

That depends on the details, as indicated in our preceding comments.

- **In what circumstances should a value-based pricing assessment be subject to review?**

Comments

- **What arrangements could be put in place within the new medicines pricing system to facilitate access for patients who may benefit from drugs previously funded through the Cancer Drugs Fund, at a cost that represents value to the NHS?**

Comments

- **Will the approach outlined in this document achieve the proposed objectives of better patient outcomes, greater innovation, a broader and more transparent assessment and better value for money for the NHS?**

Comments

That depends on the details, as indicated in our preceding comments.

- **Are there other factors not mentioned in this document which the new system should take into account?**

Comments

Yes, two:

1. The impossibility of precision implies there will be negotiation

The drafting of the Consultation Document gives the impression that if appropriately designed the VBP process will yield a single, precise, maximum price the NHS would be willing to pay for a new branded medicine. We consider this an unrealistic expectation. There is uncertainty – some of it quantifiable but much of it not – at every stage: whether all appropriate aspects of value are being taken into account; are they being accurately measured (if they can be); are they being aggregated with other elements of value using the right weights; and is the measure of value being converted into a money value at the right rate? That is quite apart from the uncertainty that surrounds the capabilities of the medicine, i.e. the inevitably incomplete information about effectiveness etc., at time of launch.

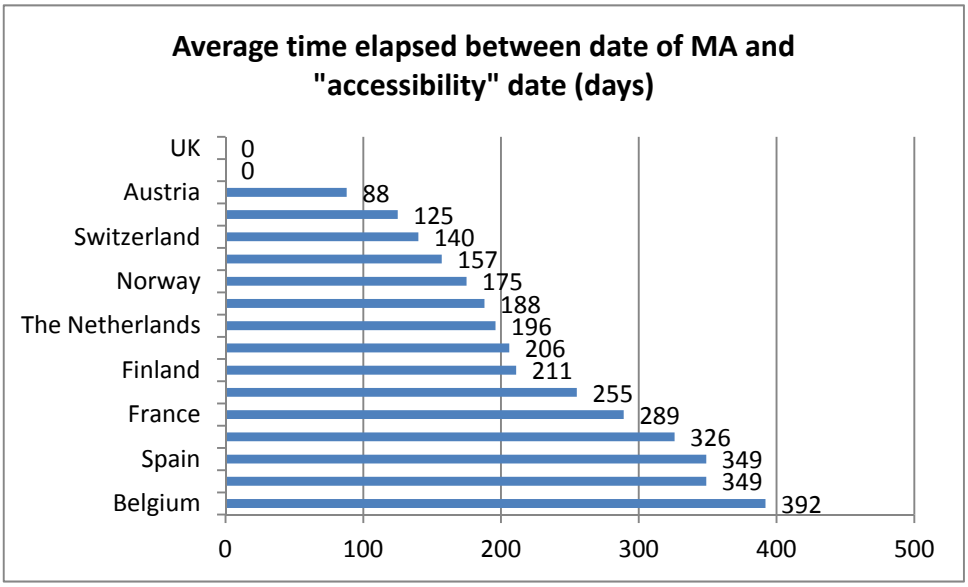
For all these reasons, even a well-defined VBP process can only hope to identify a range of reasonable monetary values, not a precise single value, for a medicine. Determining ***the*** maximum price the NHS is willing pay will, we believe, inevitably include some element of negotiation with the manufacturer. Experience from pricing and reimbursement systems around the world backs this up. We are not aware of any system internationally that attempts to determine value which identifies a precise price without an element of negotiation being involved.

2. Speed is of the essence

The Consultation Document does not consider in detail the impact VBP could have on the current position of the UK as a relatively early launch country for new medicines. For this purpose, we provide evidence on two related aspects. First, on how delays between marketing authorisation (MA) and patient access compare between the UK and other European countries. Second, on how regulatory (i.e. pricing and reimbursement) mechanisms affect launch delays and the possibility of non-launches. In summary, the evidence provided below suggests that (i) pricing and reimbursement delays are currently shorter in the UK than in most other European countries; and (ii) medicines are launched in the UK relatively early compared to other European countries. These results are important for UK patients, as they give them early access to new medicines.

(i) Figure 1 shows the most recent 'Patients WAIT Indicator' (EFPIA, 2010). It shows, for new medicines with first EU marketing authorisation in the period 2007-2009, the average time between EU market authorisation and patient access, measured by the number of days elapsing from the date of EU market authorisation to the day of completion of post-authorisation administrative processes, including pricing and reimbursement processes.

Figure 1 Patients WAIT Indicator – 2010 Report



Source: EFPIA's WAIT Indicator (2009 Report). For more information on the methodology used, please refer to: http://www.efpia.org/Content/Default.asp?PageID=517 (accessed 22nd February 2011)

The UK (and Germany) has a reported delay of 0 days, because in the pre-VBP UK there is no pricing and reimbursement process to be completed before new medicines can be prescribed to patients, unlike in most other European countries. The implementation of a VBP system might add delays that are currently not happening in the UK, and this should be considered when exploring how the VBP system is designed and implemented.

(ii) A number of studies confirm the relationship between pricing and reimbursement regulation and launch delays and non-launches.
Danzon et al. (2005) analyse the effect of price regulation on delays in launch of new drugs. They focus on 85 new chemical entities (NCEs) launched between 1994 and 1998 in 25 major countries, including the UK and 13 other EU countries. Several results are worth mentioning. First, the US (73 launches), Germany (66) and the UK (64) had the most launches. The authors point out that these countries did not require price approval before launch. Second, the US had the highest launch probability (80% launch probability in 14 months and 86% in 30 months). The launch probability in the UK was among the highest (around 63% in 14 months and 80% in 30 months). Third, average launch delay ranged from 4.2 months (US) to 23.5 months (Japan). UK's launch delay was second lowest, at 7.2 months. Figure 2 shows the relationship between number of launches and average launch delay. Five countries are highlighted, including the UK.

Figure 2 Number of launches and average launch delay

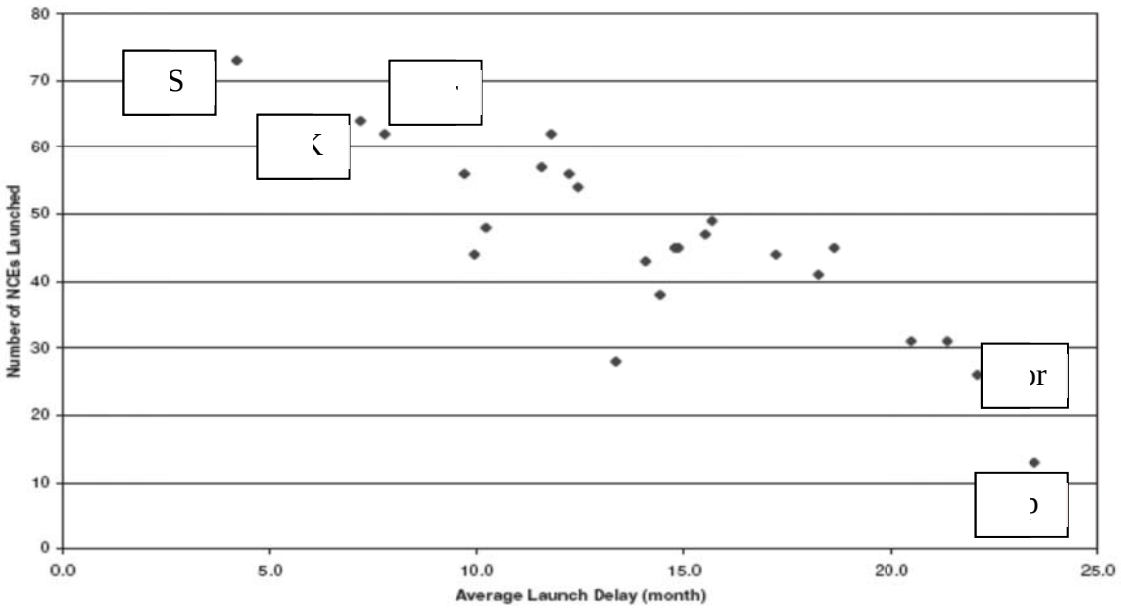


Figure 2. Number of NCEs launched and average launch delay, by country

Source: Danzon et al. (2005)

Danzon and Epstein (2008) supplement the previous work by looking at launch experience in 15 countries over the decade 1992-2003. They use a similar methodology to the Danzon et al. (2005) paper described above and again find that the UK has shorter launch delays than most EU countries, with the exception of Germany, the Netherlands and Sweden.

References:

Danzon P. and Epstein A. (2008) Effects of regulation on drug launch and pricing in interdependent markets, NBER Working Paper 14041

Danzon P. and Furukawa M. (2003) Prices and availability of pharmaceuticals: Evidence from nine countries, *Health Affairs*

Danzon P., Wang Y. and Wang L. (2005) The impact of price regulation on the launch delay of new drugs - evidence from twenty-five major markets in the1990s, *Health Economics*, 14: 269–292

- **Are there any risks which might arise as a result of adopting the value-based pricing model as outlined above? If so, how might we try to reduce them?**

Comments

- **What steps could be taken to ensure that value-based pricing has a positive impact in terms of promoting equalities?**

Comments

- **Are there any other comments or information you wish to share?**

Comments

The proposed VBP approach set out in the Consultation Document applies only to branded medicines. However, in order to achieve an efficient allocation of resources across the NHS and provide efficient price signals to developers of all types of health care technology, the scope must widen to all forms of health care, not just pharmaceuticals. Since its inception, NICE's remit has broadened to include specific focus on public health, devices and diagnostics. The same level of rigour is expected in the assessment and appraisal of all types of health technology, so we would expect that the methods used to assess the value of medicines should be applied to other technologies, which can be just as costly to the NHS.

The NHS is also expected to implement NICE Technology Appraisals, i.e. to make funding available to ensure patients have access to technologies deemed good value for money for the NHS. It is unclear how this will happen under VBP if the "NICE Mandate" disappears. Retention of the NICE Mandate needs to be considered at least until the efficiency of new local Commissioning arrangements is proven, or alternatives such as budget "topslicing" as proposed by Claxton et al. (2010) considered.

Reference:

Claxton K., Schulpher M. and Carroll S. (2010) Value-based pricing for pharmaceuticals: Its role, specification and prospects in a newly devolved NHS. Centre for Health Economics, University of York. CHE Research Paper 60.