Pricing pharmaceuticals: Value based pricing in what sense?

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Summary: Reimbursement of pharmaceuticals ought to provide both incentives for and reward innovation, as well as set a price which reflects the market value of the product. In other words reimbursement must reward both dynamic and static efficiency in the pharmaceutical sector. Within the UK a debate has emerged concerning whether the Pharmaceutical Pricing and Reimbursement Scheme, a profits based regulatory scheme, should be replaced with a value based pricing scheme. This article defines these competing reimbursement schemes and outlines their relative costs and benefits.

Keywords: Pharmaceutical Pricing, PPRS, Value Based Pricing, UK

The continuing debate over the pricing of pharmaceuticals has emphasised the relationship of pricing to value. The recent UK Office of Fair Trading report on pricing within the UK recommends the replacement of the Pharmaceutical Pricing Reimbursement Scheme (PPRS), essentially regulation through profit control supplemented with ad hoc intervention on prices through the imposition of price cuts if deemed necessary with a specific system of value-based pricing (VBP). It begins by defining the objectives of the regulatory environment and follows with a discussion of the PPRS and proposed VBP structure, with accompanying critical appraisal at both the conceptual and practical levels.

The aim here is neither to support, nor reject either system, but rather to provide a critical overview of both. In assessing the replacement of the PPRS with VBP a critical overview of both the conceptual and practical levels. The the fair market return achieved on sales. The value derived from regulated products and their reliance on high costs are normally presented as the aggregate of producer and consumer surplus, defined as the welfare gain from consumers purchasing products at the lowest possible market price and producer gain in terms of producer and consumer surplus, defined as the aggregate of producer and consumer surplus, defined as the welfare gain from consumers purchasing products at the lowest possible market price and producer gain in terms of market price and producer gain in terms of the fair market return achieved on sales. Given the nature of pharmaceutical products and their reliance on high costs concentrated in the research and development (R&D) process, there is an argued tendency towards monopoly production, as these costs tend to prohibit entry into the market.

Monopoly is associated with the exploitation of consumer surplus through control over prices, with a monopolist gaining a higher return (profit) through selling reduced quantity at higher price. Conversely, monopoly power could be associated with higher levels of innovation, given the potential use of profits to protect future market status. With an estimated ten thousand molecules to be screened for every product developed for the market, it takes on average around £350 million (€443 million) to produce a pharmaceutical product.

Indeed, patent protection is given as an incentive to invest in such high R&D costs, thus protecting producer profits. While such protection is offered against molecule structure rather than specific products, patent protection can co-exist with product competition. Moreover, it can be noted that pure monopoly is never really consistent with investment in R&D. If monopoly protection exists there is little incentive to invest; investment in R&D is only pursued if there is potential future competition in the market, which leads to current competitive strategies that manifest themselves in investments in R&D processes.

PPRS

Of course regulation can also reduce consumer surplus if it distorts the rate at which products bestowing health benefits to individuals flow onto the market. The existing form of regulation within the UK is based on the PPRS which couples rate of return control with price control. The scheme imposes profit controls, through setting maximum and minimum achievable profit levels for individual company sales to the National Health Service (NHS), coupled with price controls which allow initial price setting freedom on launch but then impose restrictions on subsequent price increases. Increasingly there have also been price cuts on existing products when the PPRS has been renegotiated every five years or so. Finally, price modulation is allowed through firms altering prices within their product portfolio as...
long as expected volume-weighted revenue remains constant. The price cutting aspects of the scheme are arbitrary and open to gaming; higher price setting in anticipation of cuts.

The PPRS is often portrayed as being dominated by the profit controlled element, although it has been argued this has increasingly become less and less true as the profit rebate associated with PPRS is implemented with less and less frequency.² It is well known that the rate of return regulation that characterises the PPRS tends to over-capitalisation. Interpretation of the PPRS in this form could lead to an argument that it supports possible subsidisation of R&D capital. However, this is unlikely within the specific form of the PPRS as individual companies are unlikely to over-estimate costs, thus protecting profits, as costs are subject to a form of benchmarking.

Of course, given the multinational dominance of the industry, internal cost shifting remains a possibility. Indeed the lack of payback within the PPRS over recent years is consistent, as the OFT pointed out, with transfer pricing across different geographical areas, leading to the PPRS being ineffective. Moreover, even if Towse³ is correct in suggesting that, as the PPRS is a profit regulation rather than a regulation of economic return, with R&D merely being an expenditure, it remains the case that the allowance of relatively high rates of return in the pharmaceutical industry may lead to an over-investment in R&D facilities coupled with high producer surplus.

The “may” is emphasised to highlight the uncertainty that surrounds this issue. The optimal degree of investment is of course determined by the return in terms of social value. This depends on the combined value of consumer and producer surplus. Producers may acquire consumer surplus through price differentiation with little impact on total surplus value. They may also achieve excessive profits through abuse of monopoly power and therefore reduce total surplus or may influence future producer and consumer surplus through investment decisions. The first two possibilities are associated with static considerations, the later with dynamic considerations. There may also be equity concerns over who should realise any surplus value; what share of the value inherent in the R&D process should be returned to consumers and producers? The motivation to move to a VBP regulation appears to be that the current reimbursement of pharmaceutical products in the UK has tended to support delivery of producer surplus rather than protection of consumer welfare. The balance between dynamic and static efficiency has tended towards a regulatory environment that has supported producers rather than consumers.

This conclusion is consistent with the view that the PPRS does appear to provide healthy incentives for R&D investment through a profits allowance accompanied with pricing freedom for individual products within the given profit level, albeit moderated by the possibility of imposed price cuts. These price cuts are somewhat inefficient as they are implemented across the board and possibly with some time lag, depending on when the product is launched and PPRS negotiations take place. A tentative conclusion is that the PPRS provides incentives aimed at securing dynamic efficiency, indeed some argue that such incentives are provided by a scheme that featherbeds individual firms’ dynamic efficiency, while at the same time allowing considerable latitude with respect to static efficiency.

The role of NICE

PPRS regulation of price is currently complemented, although by no means comprehensively, by the cost-effectiveness analysis of specific interventions undertaken by the National Institute for Health and Clinical Evidence (NICE) in England, Wales and Northern Ireland. While obviously this is not a regulatory authority dealing with pricing mechanisms per se, for those drugs which are assessed it does imply some additional pricing constraints if the product is going to be purchased by the NHS. In circumstances where a product is assessed by NICE, this weakens the ability of companies to modulate prices across products in their portfolios. The complementary role of other regulators on the operation of the PPRS therefore ought not to be overlooked.

The existing system, combining patent protection with the PPRS and occasional NICE cost-effectiveness evaluations, could operate to underpin static and dynamic efficiency within the UK with respect to pharmaceutical products. Patent protection relates to molecule structure and not product protection; for example, a number of statins remain under patent protection competing with each other for market share. The operation of the PPRS allows free pricing within a profits constraint across different products within a firm’s portfolio. The cost-effectiveness evaluation of some interventions then ensures an implicit control on price, if not across the board, then at least through threat. The role of NICE could, of course, be extended to further augment the PPRS without replacement.

Value Based Pricing

VBP regulation has been suggested as a replacement for PPRS in the OFT report.¹ This would establish a maximum price for a pharmaceutical based on an ex ante evaluation for new products and a rolling ex post evaluation of existing products. This might be supplemented by risk sharing contracts if there was insufficient evidence to allow a full ex ante appraisal, with the price being contingent on the realisation of treatment benefits. There would be non-linear pricing arrangements for different indications and sub-group applications, with generic pricing once off-patent if a generic was available. The evaluation would be based on the existing NICE type cost-effectiveness evaluations. In other words VBP would appear to be an extension of the type of evaluation already conducted as part of the valuation of a range of therapies that NICE deems to be cost-effective within the existing system. The proposed system would retain patent protection and combine this with a widened role for cost-effectiveness in pricing to pursue VBP. The emphasis therefore moves towards static efficiency, with the emphasis on value for money at launch, and away from dynamic efficiency.

A number of problems exist in using cost-effectiveness analysis, pertinent to both its existing use and future role in establishing VBP. One major issue relates to the use of clinical trial data to establish effectiveness. The objectives of such trials are normally to establish safety, tolerability and efficacy within a tightly controlled population. Such trials are normally short-term and therefore do not establish the long-term health effects required for a comprehensive cost-effectiveness analysis. The results from such trials are currently aimed at a different set of regulatory bodies than those concerned with pricing and reimbursement. Modelling, based on increasingly accepted methods, must therefore be undertaken not only for this reason but also as health economic data on endpoints and resource use are not routinely incorporated within clinical trial studies. For example, if Quality Adjusted Life Year (QALYs), the preferred outcome measure
for NICE, are to reflect outcomes over which the surplus is to be evaluated, then most products will have to transform clinical trial outcome measures into QALYs. Given that pricing and reimbursement is required on launch, the ex ante fast track appraisal method envisaged by the OFT will place heavy demands on evaluation data. This is not impossible to achieve, but it is open to uncertainty; hence the combination of ex ante and ex post evaluations.

Currently, NICE uses the Single Technology Appraisal Process (STAs) as a means of assessing comparator products within a limited time period. If used as the basis for VBP, as envisaged by the OFT, the data would have to be available quickly. This would, in principle, require head-to-head studies or indirect comparisons through some form of meta-analysis of a new product with existing comparator therapies. It is unlikely that this information would be readily available in all situations or clinical trials, increasingly designed with a global perspective, tapered to fulfil regulatory criteria in one market for pricing purposes. There may, in any case, be different standard comparator therapies in different geographical markets. Data limitations will therefore be inevitable, as within the current STA assessments, where there is already great pressure given the objective of realising a market price to ensure access to the product under evaluation. NICE however, currently lives within these data constraints, so it may not be impossible for VBP to tolerate such constraints.

NICE allows a considerable threshold of between £20,000 (£25,000) and £30,000 (£37,500) per QALY gained for acceptable treatment up-take. If this form of analysis is to be used for VBP a stricter threshold value, based on the changing opportunity cost of new treatments, will be required. This would only not be the case if sub-group analysis and non-linear pricing, as proposed in the OFT report, were permissible. While this could lead to a more flexible regulatory pricing mechanism, and in the extreme giving perfect pricing discrimination with all surplus being acquired by the company, in most circumstances this is very unlikely given the data required to substantiate such claims under the proposed VBP system. Even substantiating claims across a small number of subgroups would be highly data intensive. Moreover, if VBP is attached to a risk-sharing analysis, as allowed in the OFT report, given circumstances where there is a lack of data available to perform an ex ante analysis (for example, with chronic disease treatments), sub-group analysis will be even more unlikely as the risk transfer to companies increases with an increasing number of sub-groups. It can also be noted that such risk sharing schemes erode patent protection in any case as the length of time required to establish regulatory worth is increased.

Challenges

It is clear that both the PPRS and the envisaged VBP schemes have drawbacks. The efficiency of their implementation is largely concerned with the relative costs of implementation. VBP essentially drops the PPRS and considers an extended role for NICE type evaluations. This is supplemented with sub-group and risk-sharing analysis. Data availability is the major constraint. Ex post risk-sharing is only envisaged as a means of supporting situations where there is not enough available data for an ex ante consideration. The lag time for the implementation of ex post risk-sharing is of obvious interest. Too short a lag will not overcome data constraints and will not provide much incentive to participate; too long could lead to distortion of the perceived gains in static efficiency with firms gaining undue producer surplus.

Non-linear pricing within a VBP environment relies on the greater availability of data and a greater willingness of companies to accept risky pricing strategies. It is unlikely that non-linear pricing could result in perfect price discrimination, given the data requirements on sub-groups which would necessarily have to support pursuit of such a policy. Indeed, given that VBP is premised on an incentive with respect to dynamic efficiency that is meant to persuade firms to invest in those areas where health benefit is greatest, it is not clear that non-linear pricing will necessarily work towards this objective.

Long lead time mitigates against a firm ex ante considering non-linear pricing as a strategy, unless pursuing from the beginning of their investment a very sophisticated data collection and pricing strategy. As investment progresses the firm would have to pursue evidence on sub-groups and a range of indications, assuming that it had the foresight to see the aggregate rewards early in the investment cycle. Alternatively if a firm became aware of potential benefits of market segmentation, it would have to start collecting data at a late stage of development.

While such data constraints are not insurmountable, they are substantial and have to be faced as an additional investment to secure value for money pricing. It would seem of doubtful regulatory efficiency to allow firms to pursue extensive ex post evaluations or risk-sharing agreements on the basis of non-linear pricing proposals.

Investment in R&D

Most of the discussion above relates to issues of static efficiency. The impact of VBP has been less discussed with respect to dynamic efficiency. The envisaged regulatory environment is one where companies would pursue investment over a long time frame, given that there is a chance of reward based on a product price set in accordance with achieved health benefit. It is envisaged that firms will have an incentive to invest in areas where achievable health benefits are greatest. Areas of high disease prevalence where there is unmet need are obvious areas for high returns. However, investment in R&D may be mitigated if these are also areas characterised by a long lag between research and product development or by high risks to individual firms. Firms may place a lower value on R&D projects than society in some areas leading to general under-investment.

Even if VBP leads to a firm adopting a concentration of investment in those areas where there is perceived to be greatest health gain, this may result over time in a narrowing of the general R&D base, with subsequent loss in the external economies of scale which tend to characterise larger R&D establishments. A narrow based focus may tend to cause risk-avoidance within firms, without the broad base to spread risk, which may lead to a lower valuation of research projects, in the absence of external economies, than in society at large. Large R&D programmes in the pharmaceutical sector may have inherent advantages through economies of scale and scope that are difficult to identify but are nonetheless present.

The size of a firm may itself encourage innovation and a wide range of potential products. In this sense VBP seems consistent with a narrowing of firms’ technological capacities as they become more specialised in those areas with the greatest potential health gain for their investment portfolio. This seems to undermine one aspect of a productive R&D capability; the
Pharmaceutical pricing in France: a critique

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Summary: In France, pharmaceutical pricing relies on an ex ante evaluation of the medical value of drugs. Prices are negotiated on the basis of an industry-wide contract between drug manufacturers and the Health Products Pricing Committee (Comité Economique des Produits de Santé). Together the Committee and the drug companies sign a number of contractual agreements, which give the national health system a variety of flexible means to monitor prices and drug use, also ensuring that public resources are properly allocated. Some drugs have different levels of therapeutic value, depending on who takes them. These products in particular need close monitoring. Rebate policies are one of the tools available to control such spending. The economic efficiency of such rebates should not be overestimated; in practice they do not significantly decrease spending.

Keywords: Pharmaceutical Policy, Reimbursement, Rebates, Evaluation, Added Therapeutic Value

A few words on pricing and reimbursement

Pricing and reimbursement decisions in France are taken on a step by step basis. Firstly there is the market authorisation of a drug at a national or European level. The product is then evaluated by an independent scientific committee, prior to price negotiations. This Transparency Committee, named after the European Transparency Directive, assesses the therapeutic value, or clinical benefits of a drug, and proceeds to compare it with existing therapies. Drugs are evaluated against two sets of complex criteria: their therapeutic value (service médical rendu) and added therapeutic value (amélioration du service médical rendu).

Therapeutic value takes into account the severity of the illness and the efficacy of the drug. Although a drug’s therapeutic value does not impact on pricing, it helps determine its reimbursement rate. The added therapeutic value of a drug is a relative notion, as it is measured through a comparison with the clinical benefits of existing drugs or therapies. Thus, it represents the ‘added health gain’ or the ‘relative effectiveness’ of a drug, compared to its alternatives. The main problem in assessing this added therapeutic value is the time it takes, as well as the lack of proper clinical trials against alternative products on the market.

Nonetheless, such evaluations are necessary and need to take place prior to any decision on reimbursement. They should also fit within the timeframe (normally ninety days) to reach a decision on reimbursement set out in the 1989 Transparency Directive (Directive 89/105/EEC). The duration of such evaluations have in fact decreased in recent years and now meet this requirement, while fast track procedures were also introduced to help assess those drugs of great significance.

REFERENCES

ability to maintain a broad technological base which would otherwise provide a form of insurance against inevitable research dead-ends. Within the context of VBP this occurs not only within but also potentially across companies as they will have an incentive to compete for areas of highest health gain.

Conclusion
The design of optimal regulation is not straightforward. The current discussion of how to regulate pharmaceutical prices in the UK highlights this. The existing UK regulatory environment has been voluntary and has allowed high rates of return as an incentive to motivate R&D. The proposed system emphasises that the presumed high prices consistent with these returns have eroded consumer surplus. Such a debate brings a clear perspective to the tensions associated with the pursuit of static and dynamic efficiency concerns. With either regulation there is a trade-off; with the PPRS the trade-off is that static efficiency concerns are relaxed to allow the pursuit of dynamic efficiency through the provision of incentives for R&D investments and, hopefully, a quick rate of market launch of beneficial products. Under the proposed VBM the trade-off would be to tighten static efficiency concerns against the cost of potentially reduced incentives for R&D. The actual judgement regarding which is, of course, an empirical one.