Value Based Approach to the Pricing of Branded Medicines: Consultation Response from the Association of the British Pharmaceutical Industry

Submission to the Department of Health

14th March 2011
About the ABPI

The Association of the British Pharmaceutical Industry (ABPI) has 150 members including the large majority of the research-based pharmaceutical companies operating in the UK, both large and small. Our member companies research, develop, manufacture and supply more than 80 per cent of the medicines prescribed through the National Health Service (NHS). In addition, there is affiliated membership for other organisations with an interest in the pharmaceutical industry in the United Kingdom.

The pharmaceutical industry is immensely valuable for the UK and its medicines contribute greatly to improving both the health of the population and the economy as a whole. It is committed to working together with Government and the NHS to deliver value for money from medicines, better patient access to medicines and to ensuring that innovation and research are appropriately and fairly rewarded. Medicines have contributed significantly to increasing overall UK life expectancy and quality of life. The pharmaceutical industry invests more in R&D than any other industry within the UK and one fifth of the most prescribed medicines globally were developed by UK companies. It directly employs around 72,000 people and generated a greater contribution to the UK economy than any other industry sector in 2009 with a trade surplus of more than £7bn.

However, the global competitiveness of the pharmaceutical industry in the UK needs to be better supported through a more pro-innovation adoption culture within the NHS which seeks to redress the balance between embracing the benefits which are obtainable from the appropriate use of new medicines versus simply viewing them as an additional cost pressure to be minimised.

About this Response

The ABPI welcomes the opportunity to respond to the DH consultation on a new value-based approach to the pricing of branded medicines. This response has been developed in discussion with our member companies and we have also consulted with a wide range of external stakeholders.

The response has been approved by the ABPI Board of Management.

Context for Value Based Pricing

The Pharmaceutical Price Regulation Scheme (PPRS) has provided over many years a stable and predictable environment for the regulation of branded medicines and improvements have been made to it at each successive renegotiation.
The development of value based pricing (VBP) for new medicines from 2014 provides significant opportunities to both improve patient outcomes through the more appropriate use of medicines and to stimulate development of the new innovative medicines of the future, in line with the potential of the current Healthcare and Life Sciences Growth Review. If we get the reforms of the current system right, UK patients may be able to benefit from earlier and more consistent access to medicines, but if we get these wrong, patients may face even longer delays before they are able to benefit from innovative medicines. The UK has been an attractive market supporting the early launch of new medicines for many years. The changes which will be introduced must not jeopardise this position.

Maintaining stability in the existing market segment (i.e. for medicines not reviewed under VBP) will be critical for both Government and industry; and in the early years, the size of this existing segment will remain close to 100% of total medicines expenditure. For medicines which do go through VBP, key to success will be ensuring that value medicines with a value-based price are used optimally within the NHS without the need for any further local re-evaluation or appraisal.

ABPI agrees with the objectives stated in the VBP consultation of improving access to medicines and therefore patient outcomes, stimulating innovation, improving the comprehensiveness of value assessments and processes and ensuring value for money for the NHS. However, a new UK pricing and reimbursement environment must deliver against a number of additional objectives for it to be acceptable to the pharmaceutical industry covering both industrial and health policy. Stability and predictability will be needed for companies with existing medicines and with those that undergo value-based pricing. Changes in the pricing and reimbursement environment will need to be fully aligned with those taking place within the NHS as part of the broader reform programme.

At the completion of this consultation exercise, ABPI on behalf of the pharmaceutical industry, stands ready to work with Government on the co-creation of a new environment for the pricing, reimbursement and use of new medicines which will be fit for the future and which will deliver against jointly agreed objectives.

We have nearly three years to co-create the optimum environment which will meet the needs of patients, the NHS and the pharmaceutical industry and must use the time we have to best effect to achieve this. We must complete the required research needed to answer key questions, and test out the proposals thoroughly to
ensure that unintended consequences for patients, the NHS, Government and the pharmaceutical industry are minimised. Extensive modelling, analyses and piloting between now and 2013 will help deliver a new pricing and reimbursement environment which meets the required objectives.

**Key Strategic Issues**

There are a number of key strategic issues on which early dialogue will be essential between Government and industry as part of the co-creation process prior to entering into more detailed discussions about value based pricing.

These issues are set out below:

**Linking value to access and uptake**

The introduction of value based pricing alone will not be enough to ensure improved access to and uptake of medicines and thus deliver on what Government has stated as a central objective for a new pricing and reimbursement environment. Patient outcomes will only improve if uptake of medicines follows the granting of access. The structural and commissioning reforms of the NHS alongside the challenging fiscal climate over the next three years can be expected to have an impact on the uptake of medicines. ABPI believes that additional measures must be built into value based pricing to ensure improved access and uptake and further measures outside of value based pricing will also be needed.

There are multiple mechanisms available within the ‘new NHS’ which could be harnessed to ensure better access to medicines. These include but are not limited to: the Commissioning Outcomes Framework; the Quality and Outcomes Framework; NICE Quality Standards; NICE Technology Appraisal Guidance; Payment by Results Tariffs; CQUIN; the NHS Constitution; and Best Practice Commissioning Guides. However, these mechanisms are largely available in England only.

Decisions on access to medicines are a devolved responsibility and so the devolved administrations will need to be involved early on in order to ensure that the new environment provides equitable access to medicines for patients in all four nations of the United Kingdom and avoids driving any inequities.

It is in the mutual interest of both Government and the pharmaceutical industry to ensure that there are no further barriers to access and uptake of medicines once a value based price has been agreed. In other words, the new system will need to ensure that there are no secondary regional / local blocks once a medicine has gained an agreed value based price and so has theoretical national access.
There are a number of additional approaches which could be explored to ensure appropriate uptake of medicines within the new environment such as providing an equivalent mechanism to the current PCT mandatory funding direction for NICE guidance or top-slicing local NHS budgets, each with their own pros and cons. However, if we get the design of the new system right through the process of co-creation such that it does effectively drive patient outcomes through the appropriate use of medicines, then these additional measures may not need to be negotiated.

ABPI recommends that the NHS Commissioning Board is involved in the further development of value based pricing proposals which relate to access to medicines, with relevant input from the devolved nations.

**A single coherent UK pricing and reimbursement environment**

A new UK pricing and reimbursement environment will need to consist of two components: value based pricing for medicines launched after 1st January 2014 and a PPRS-like scheme to cover medicines already on the market. The two components need to be fully integrated as part of a Voluntary Agreement in order to create a single workable system which provides stability and predictability. This environment will also need to be aligned with the broader NHS reforms.

It should be noted that in the early years, a maximum of around 20 – 30 new medicines per annum are likely to be assessed under value based pricing, whilst the thousands of existing branded medicines already on the market will need to remain as part of a PPRS-like scheme. Over time, the proportion of medicines introduced under value based pricing will increase and the medicines covered by a PPRS-like scheme will decrease as these medicines lose their exclusivity. In theory the transition from a PPRS-like scheme to value based pricing might therefore take around 20 years to complete.

There may be synergies between the components which can be utilised to help achieve the agreed overall objectives for the new environment. As an example only, whilst the product-level value based system might generate value based prices, the portfolio-level PPRS-like scheme for existing medicines might be utilised to reconcile rebates for differences between list prices and value based prices where this was required. How the PPRS-like scheme and value based pricing components of the new environment inter-relate as part of an overall voluntary agreement will need to be determined as a next step.

ABPI has assumed that the new environment will retain freedom of list price setting by companies for all new medicines at launch, as with the current arrangements, and that value based pricing will therefore be used to determine a ‘value based price’ (which is the term used throughout the remainder our response) which in many cases may of course be the same as the list price.
ABPI recommends that a process of co-creation between Government and industry is used to develop value based pricing and a PPRS-like scheme as part of a voluntary agreement for existing medicines at the same time.

**Providing a fair reward for R & D investment which delivers value**

The pharmaceutical industry has produced waves of innovation that have eradicated infections, cured previously incurable diseases, reduced catastrophic events from chronic diseases and extended productive life for millions of people.

It is a laudable goal of value based pricing to further incentivise such innovation. However before determining how exactly this is to be done, it is vitally important to understand how the innovation cycle works. Firstly, it unfolds over decades: research programmes typically take 10-12 years from idea to medicine. Secondly, medicines often end up treating conditions for which they were not originally conceived. Thirdly, new mechanisms of action may initially appear to offer only small benefits but ultimately go on to influence the creation of new classes of therapeutics that replace all previous treatments.

'First of kind' molecules are rarely those that turn out to be the treatment of choice: earlier medicines may carry side effects or have lower efficacy. In many cases the most valuable treatments bring important benefits for patients, such as more convenient administration, that may not seem to be major advantages but are hugely valued by patients.

The medicines in the pipeline today (including around 600 for cancer, 300 for cardiovascular disease and 100 for Alzheimer's Disease) are by definition already in development: the choice is therefore whether these medicines should receive fair reward in the UK, not whether they are worthy of development.

If too narrow a definition of value-adding innovation (such as clear major clinical [breakthrough] advances demonstrable at the time of launch) had been applied historically, many of our most important medicines would not exist today. Broader definitions of innovation have been developed, including via the EU High Level Pharmaceutical Forum.

Overall, ABPI is concerned that the industrial policy implications of the value based pricing proposals have not been adequately investigated. The UK market for medicines must be included alongside other factors being considered in the Healthcare and Life Sciences Growth Review. It is unclear whether the new system will provide a fair reward for R & D investment or not, particularly that which delivers incremental innovation, which is the most common, as opposed to breakthrough innovation which is relatively rare and often generated as a result of incremental innovation. Both types of innovation are important, and both must be fairly rewarded. The current DH impact assessment is incomplete and appears inconsistent with the consultation document in this regard. ABPI considers that
significantly more research is required to inform a more robust impact assessment to inform the design of VBP.

The current measure of therapeutic improvement and innovation is too narrow in its definition, neglects important aspects of value and needs to be redefined. ABPI recommends that the definition is broadened to encapsulate aspects of innovation not presently captured during the co-creation process. This needs to be done with particular care since UK thinking on this is certain to be carefully scrutinised and perhaps may be adopted internationally. Our responses to the consultation questions explore the issues further.

The need for a pricing framework

ABPI notes that the consultation focuses largely on the assessment of the value elements of ‘value based pricing’ and does not give consideration as to how a value based price negotiation process would work once an assessment of value has been completed. Substantial work will be required on a framework for value based price determination. There are further questions which need to be answered and ABPI has set these out in our response to consultation Question 13.

It will be important that the development of this mechanism considers the potential for value based prices or value based commercial arrangements to be non-disclosed and remain “commercial-in-confidence”.

Importantly, once a value-based price has been agreed for a medicine it should not be subject to further local NHS commercial negotiation or agreements.

Cost-effectiveness thresholds and weightings

The mechanism of how the cost effectiveness threshold (willingness to pay) will be set and used as part of value based pricing and how it will be managed over time needs to be agreed. The value of the threshold will be one of the key metrics in VBP as indeed will be the values assigned for the various weightings. ABPI recommends that the process of co-creation between the pharmaceutical industry and the Government is used to discuss and agree the mechanisms to be used. The Office of Fair Trading originally recommended that threshold considerations should be negotiated as part of the PPRS.
Dealing with uncertainty in evidence and absence of evidence

Evaluations of value undertaken via health technology assessments generate plausible ranges of cost effectiveness rather than point estimates. Whilst the pharmaceutical industry recognises the need to generate appropriate evidence at launch, under value based pricing, there will be no less uncertainty than at present and this needs to be reflected in the value based assessment process.

In exceptional cases where there is considerable uncertainty in the evidence base or when there is inadequate or immature evidence upon which to base a firm value based pricing decision, it will be necessary to utilise additional approaches to granting reimbursement in order that NHS patients are not denied access to important medical innovations. Coverage with evidence development, patient access schemes, risk share schemes or flexible pricing arrangements may need to be utilised. These will need to be explored further as part of next steps.

ABPI believes that the consultation is premature in suggesting there should be no role for patient access schemes within a new UK pricing and reimbursement environment at this early stage of development. Indeed it would appear to pre-empt the PPRS Review of Flexible Pricing Arrangements (patient access schemes and flexible pricing) presently underway.

Creating an environment which includes the devolved administrations

Medicines pricing is presently a reserved power. However, decisions on assessing value and on access to medicines are devolved. NICE, SMC and AWMSG all undertake assessments of clinical and cost effectiveness for new medicines. A new pricing and reimbursement environment should be applicable across the UK and avoid duplication in assessment processes. The devolved administrations should be involved in a single process that avoids duplication. Any new process should not delay access for patients in the devolved administrations compared to current timelines. There should not be an increase in decision-making layers that may create barriers to access. Once a value based price has been agreed for a medicine, all eligible patients should have the right to access that medicine in a timely manner.

The creation of a new UK pricing and reimbursement environment provides opportunities to streamline current medicines evaluation processes and eliminate unnecessary duplication that add to regulatory burden. Implementing a new system will have resource implications and there exists the opportunity to utilise current existing agencies to share or undertake specific non-duplicative roles within a new UK pricing and reimbursement environment. This may mean considering a change to the roles of some agencies to focus more on improving access to medicines rather than on assessing value.
ABPI recommends that early dialogue is established with the devolved nations of Wales, Scotland and Northern Ireland in order to agree the optimum way forward which is in the best interests of NHS patients, the devolved administrations and the pharmaceutical industry.

**Key Points**

The ABPI on behalf of the pharmaceutical industry in the UK seeks an agreement with Government and the NHS which - for the first time – both secures unhindered access for NHS patients to new medicines and ensures innovative companies are fully and fairly rewarded for the patient outcomes their medicines deliver.

ABPI recommends that:

- a well-planned process of co-creation between Government and the pharmaceutical industry is used to create a Voluntary Agreement embracing value based pricing and a scheme for existing medicines, at the same time

- the NHS Commissioning Board is involved in the development of objectives and value based pricing proposals which relate to medicines access and uptake, with relevant input and agreement from the devolved nations. The Board should play a key role in securing access and uptake for value-priced medicines

- the mechanism for setting the cost effectiveness threshold and weightings and the process for managing these over time is agreed between the pharmaceutical industry and Government

- the current measure of “therapeutic improvement and innovation” is significantly broadened. In its present form it will have a negative impact on valuable forms of innovation to the long term detriment of patients. The whole approach to measuring and quantifying innovation needs to be further explored

- early dialogue is established with the devolved nations of Wales, Scotland and Northern Ireland to agree the optimum way forward in the best interests of NHS patients and to avoid duplicative decision making or delays in access to medicines

- further extensive work is undertaken on a possible value based pricing negotiation framework during which the further questions we have identified are addressed (and are set out in our response to Question 13)
• it will be important that where the value based price differs from the list price, the value based pricing framework potentially permits value based prices or arrangements to be subject to non-disclosure and remain “commercial-in-confidence”

• value based pricing should, in general, be for new medicines and indications launched on or after 1st January 2014. However, there may be circumstances where it might be appropriate to review medicines launched before this time. Mutually agreed criteria need to be developed to identify these medicines

• the processes for undertaking value based pricing should be as simple and efficient as possible and low in bureaucracy

• all further barriers to access and uptake of medicines are identified and removed within the NHS once a value based price has been agreed. This is in the mutual interests of patients, Government and the pharmaceutical industry, in order to meet the objectives set out in the consultation

• re-assessments of value should only be undertaken when new evidence on the effectiveness of a medicine becomes available. Mutually agreed criteria need to be developed

• the immediate next steps set out in our response to Question 20 are planned out between Government and the pharmaceutical industry as the next stage in the development process following the completion of the consultation process.
Responses to Consultation Questions

ABPI has responded as fully as is currently possible to the questions about value based pricing which have been raised in the consultation document. However, many of the questions are prematurely detailed and should be addressed once broad agreement is in place on the strategic issues which have been raised in the earlier part of our response. To answer many of the questions properly, research will need to be conducted along with further analyses and modelling.

The strategic issues and broad framework requirement for value based pricing will need to be addressed during the co-creation process between Government and the pharmaceutical industry.

Government requirements which limit increasing regulatory burden will also need to be considered in redesigning the new pricing and reimbursement environment in the context of one-in-one-out regulation.

ABPI has summarised the immediate next steps which need to be carried out following the completion of the DH consultation process in conjunction with the ABPI and the pharmaceutical industry. These steps are set out in response to Question 20.

1 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 WITH ADDITIONS

These are all laudable objectives but to them needs to be added the long term health of the pharmaceutical industry that brings new medicines to patients. The objectives need to address both industrial and health policy requirements. In August 2010, ABPI and DH reached an agreement about the positive features of the current PPRS 2009 Agreement.

These features are set out below:
## Key positive features of PPRS - agreed with DH in August 2010

### Access
- Freedom of pricing at launch for new medicines enables early introduction which supports normal commercial decision making and quicker access for patients
- New flexible pricing provisions enable supply of medicines at lower initial prices to promote uptake, with the option of higher prices linked to new indications or new evidence at a later date
- Provides for more systematic use of patient access schemes which reduce the effective cost of a medicines to the NHS

### Promotion of Innovation
- R&D allowances designed to reward innovation: the highest allowances are achieved by those companies that supply the NHS with innovative new medicines
- Includes a specific number of measures aimed at promoting both uptake of medicines and innovation

### Low bureaucracy
- "Light touch" regulation
- Low staffing and costs for DH
- Low staffing for industry with exemptions for SMEs
- Minimises disruption in commercial operations and supports “business as usual”

### Conceptual Appeal
- Designed to deliver a balance between investment in R&D and innovation and secure reasonable prices for the NHS
- A voluntary scheme, negotiated by both parties promotes government – industry relationship and mutual understanding

### Stability
- 5 year term provides a stable regulatory environment for the industry
- Certainty for business planning and supports industry investment decisions

### Flexibility
- Clear framework, but with flexibility to negotiate precise terms between DH and individual companies
- Modulation allows market responsiveness
- Adapted over the years to take account of changes in the NHS and elsewhere
Whilst the objectives for VBP proposed so far are to be welcomed they are incomplete and ABPI recommends that a next step between Government and industry would be to gain joint agreement on the health and industrial policy objectives for the new system. The agreed key features of the PPRS set out above provide a good starting point for this dialogue.

More than ever before in the context of the NHS reform programme, some of these objectives will need to be owned explicitly by the NHS. For example, a key objective of the new system is to improve access to and uptake of medicines for NHS patients. Once the value of a medicine has been established and a value based price agreed, there must be strong mechanisms to ensure that value is realised by enabling access and uptake for NHS patients. Specific mechanisms will be needed both within and out with VBP to ensure that this is the case. ABPI therefore recommends that some of the VBP objectives need to be worked up in close collaboration with the NHS Commissioning Board and with relevant input from the devolved administrations.

Improved patient outcomes will only be achieved through the timelier uptake of innovative medicines and the ABPI would like to agree specific objectives relating to both access and uptake of innovative medicines.

ABPI recommends that once the objectives for the new system are agreed they will need to be supported by a series of metrics and indicators (both lead and lag) which will permit regular reporting of progress against the objectives. These should be jointly agreed between Government and industry as part of next steps.

ABPI recommends that there are regular planned joint reviews of the new arrangements between Government and industry. Five year reviews with possible mid-term reviews (as available for the PPRS) provide the opportunity to assess how well arrangements are working based on monitoring and evaluation and to make any necessary changes.

2. 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 an individual basis, or are there particular groups of drugs which might be considered?

YES ON A CASE-BY-CASE BASIS

ABPI recommends that the value based pricing component of the new UK pricing and reimbursement environment should, in general, be for new medicines and indications launched only on or after 1st January 2014. However, there may be specific circumstances when it may be appropriate for individual medicines and indications already on the market before this time to be assessed under value based pricing.
The circumstances when such a situation might apply include cases where medicines launched before 1st January 2014 obtain a marketing authorisation for a new indication on or after 1st January 2014 or potentially for some medicines which were included in the Cancer Drugs Fund (see response to question 15) or for some medicines which have previously been turned down by NICE.

Further work will be required on this issue giving consideration to the fact that the PPRS-like component of the new UK pricing and reimbursement environment will likely remain as a portfolio-wide scheme whilst the value based pricing component is a product-based scheme. The implications of products moving or being split between the two components will need to be investigated. ABPI does not generally support the inclusion of entire groups or categories of medicines which are already on the market before 1st January 2014 being considered under value based pricing.

The volume of medicines which can be considered under value based pricing at any one time will also need to be evaluated in order to create a workable system which avoids creating bottlenecks that might result in delays to patients being able to access new medicines in a timely manner. NICE will need to be appropriately resourced to be able to carry out the estimated number of planned assessments.

It will be necessary to draw up and mutually agree a set of criteria to govern the selection of medicines which might be subject to value based pricing (both for those launched before 1st January 2014 and for those launched on or after 1st January 2014). ABPI recommends that a next step will be to jointly agree the appropriate criteria to inform case-by-case decision making and dialogue with companies.

3 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

Whilst a single holistic pricing and reimbursement environment for all branded medicines could have advantages, this would only be viable if sufficient flexibility could be demonstrably guaranteed within the new system to accommodate the particular circumstances of specific categories of medicines. In practice, this would mean that measures of severity, level of unmet need and therapeutic improvement and innovation utilised within value based pricing would have to be able to influence value based price discussions in such a way that an adequate return on R & D was achievable for these categories of medicine. Additionally, effective approaches for dealing with limitations in the clinical evidence base and for handling uncertainty would be required. In particular, smaller numbers of patients
are involved in clinical trials for medicines for rare diseases and there is often an absence of suitable comparators. If these issues are not adequately taken account of then the UK may become a less attractive market to launch medicines for rare diseases and UK R & D investment in these areas may be disincentivised.

The co-creation of a system which is as inclusive as possible is desirable, subject to it being able to demonstrably accommodate the unique requirements of particular categories of medicines.

Also, there may be further circumstances where a medicine should not be subject to a full value based pricing assessment. For example:

- **Proposed value based price \( \leq \) relevant comparator medicine value based price(s) then a value based assessment only might be required to demonstrate equivalent clinical effectiveness between the medicine and its comparator(s)

- **Low budget impact:** in the case of a medicine or an indication for a medicine with a low budget impact (for example, \(< £5m\) per annum) or where it would be disproportionate to undertake a full value based pricing assessment on the part of DH, the NHS or the company (in this latter case, this might be where the cost of making a value based assessment submission might outweigh any subsequent sales of the medicine).

In these cases, an abridged or shortened value based pricing process may be desirable.

ABPI recommends that the criteria for identifying medicines which are to be included in value based pricing are agreed upon during the co-creation process along with any requirement for an abridged value based pricing process.

**4 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?**

**YES**

This is one of the most fundamental areas of value based pricing and a thorough review of the evidence base in this area will need to be conducted. The research is fairly sparse in relation to understanding society’s preferences for being willing to pay more for treating diseases which are severe or pay more in areas where there is a high unmet medical need. It will be for the detailed design stage of VBP to identify exactly how unmet need and severity are to be measured and to what levels of granularity. There are industrial policy issues here and these will need to
be considered along with a full understanding of how the R & D lifecycle works in the pharmaceutical industry.

Catalá-López, et al. (2010) provides a helpful analysis of medicines licencing over time categorised by type of intervention, disease seriousness and other factors and concludes that only 17% of medicines launched in the period 2005-2009 were for non-serious diseases.

The lifecycle of a branded medicine usually spans more than 20 years with the first 5-10 years generally being in research and development prior to commercialisation. The short-term pipeline of companies is generally fixed in nature (“work-in-progress”) and largely not subject to change. In order to stimulate future R&D investment in the mid-term an adequate return on the investment made in the short-term will still be needed. The impact of rewarding R & D investment through a value based price alone for new medicines launched after 1st January 2014 as opposed to through a portfolio based approach will need to be carefully evaluated.

A new value based pricing process will need to take account of product life cycles and associated pricing, and the timescales associated with bringing new medicines to market. For example, a new innovative medicine may provide significant benefits, but because of the length of time taken for research and development or because of regulatory delays may enter the market at a point in time after the comparator medicine has lost patent or its period of exclusivity. The reduced generic price of the comparator at that point in time may significantly affect the pricing of the new medicine. The new system should look to minimise risks and uncertainties in this regard. For example, the price of a comparator medicine could be taken over a period of time (say the last 5 years) rather than a point in time at launch of the new medicine.

An impact assessment of introducing a value based pricing component into a new UK pricing and reimbursement system will need to be completed from an industrial policy perspective. ABPI notes that this has not yet been undertaken and that the impact assessment accompanying the consultation has been largely limited to a health department perspective. In any case this impact assessment is somewhat inconclusive regarding the desirability of introducing value based pricing.

ABPI suggests prior to any policy decisions being taken on value based pricing that a more complete impact assessment is undertaken from the industrial policy and the health perspective and considered further as part of next steps.

ABPI supports the proposals to use unmet need and severity to compute burden of illness as part of the assessment of value but believes the precise definitions / scope of each of these criteria need to be agreed as an early part of next steps.
5 How should we approach the issue of a single drug which delivers significantly different benefits in different indications?

ABPI notes that it has always been the case that value is assessed at the level of the individual indication of a medicine. An increasing number of medicines are now being licensed for multiple indications (for example, those in cancer and rheumatoid arthritis). This presents significant challenges for companies in agreeing a single value based price which can reflect the value being delivered by each individual indication. This is made more complex because it is not only the value to an individual patient which must be considered but also the number of patients being prescribed a medicine in each indication (this latter component not being easy to estimate accurately at any one particular point in time). For this reason, NICE has so far found it difficult to deal with proposals from companies which utilise ‘blending’ approaches to combine different levels of value utilising averages or weights. This has also proved problematic for designing and evaluating patient access schemes for the same medicine but across different indications.

Linking value most closely with a value based price would require a solution which permitted multiple value based prices for multiple indications. The largest practical hurdle to achieving this lies within the NHS where existing clinical, pharmacy, finance and management information systems (such as PPA) would need to be enhanced to accommodate such requirements. It would be necessary to link at the patient level, clinical diagnosis to prescribed medicine to indication. There would be many additional benefits if this were possible within existing IT systems but it is likely that there would be a significant cost to achieve this. The use of patient access schemes in the NHS has highlighted the challenges and difficulties in this area. Early dialogue and involvement of the NHS would be required in order to investigate possibilities in this area. It may be the case that solutions could be found which are not onerous, for example, by exploring the possibility of whether a medicine/indication combination could be registered within existing pharmacy systems by using different naming conventions.

In the absence of being able to link a value based price to a specific indication for a medicine then some form of blended or averaging approach might be required to combine the value delivered by different indications into a single value based price. Alternatively, we need to retain the potential for a value based price to be agreed for a specific indication or sub-group of patients and then the use of the medicine to be restricted to that specific cohort of patients (as is presently the case where NICE guidance is ‘optimised’ to a specific sub-group of patients).

Further dialogue is needed on this aspect of value-based pricing between Government and industry.
6. 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?

As stated in question 5, whilst indication-specific pricing might be closest to the intended spirit of value-based pricing, the absence of appropriate NHS information systems to manage this is a key limiting factor. A full review of the evidence base should be undertaken in order to answer this question more fully.

Wherever possible, companies will want to agree a value based price that is reasonable for as many indications as possible. It is important to recognise that within this, it is inevitable that some indications will provide better value than others.

Where there is a clearly differentiated value proposition (e.g. for different indications of the same medicine) then the potential for different naming conventions on NHS pharmacy systems could be explored. Companies should retain the option to focus on different indications to allow for example for the continuation of ‘optimised’ guidance where the value based price only refers to a sub-group or limited indication whilst other sub-groups are not recommended for use on the NHS.

A dialogue between Government, the NHS, the pharmaceutical industry and representatives of the main pharmacy-related information systems suppliers might be helpful to explore what might be feasible within this area.

7. 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?

YES

In the current health technology appraisal system, NICE assumes the goal of the NHS generally is to maximise QALYs, whilst society appears to value additional goals such as reducing inequalities and supporting the least advantaged. NICE adopt a narrower definition of value – largely considering increases in quality of life and improvements in life expectancy, and can appear to pay less attention to other factors that are valued by patients e.g. convenience and dignity. Generic quality of life instruments do not always measure health gain as well as they should in domains such as cognition, vision and hearing. NICE generally considers costs borne by the NHS and Personal Social Services (PSS) and excludes costs borne by patients and their families, assuming that all health gain is equal, irrespective of who gets it and when, whilst society might argue that is not the case. A new system of value based pricing might seek to explore and resolve these issues.
ABPI agrees with the proposition that more innovative medicines should be rewarded more highly than less innovative medicines subject to further discussion about the definition of innovation and to clarification of the issues raised below.

The current PPRS agreement states that one of its objectives is 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 new system will need to embrace this objective also. Pharmaceutical research is an extremely uncertain and long-term investment. Rewards for appropriate degrees of risk taking and commitment are essential to enable a dynamically efficient industry: researching and developing the right mix and quantity of new medicines without wasting resources.

It is vital to understand that innovation in medicines is not binary, on or off, but is a matter of degree. New medicines are innovative to different extents. There are also as many dimensions to innovation as there are potential benefits of medicines, i.e. innovation is not limited to health gain but can include, for example, cost savings in other parts of the health budget, cost savings in the wider public sector and economy, greater convenience and tolerability and other process benefits for patients. Public health and environmental aspects of innovation may also need to be considered, including for example, innovation which reduces the carbon footprint of the NHS. The OHE Consulting report “The Many Faces of Innovation” [OHE Consulting, 2005] provides a concise discussion and summary of these issues, plus key references to the relevant economic literature: http://www.ohe.org/lib/ldownload/48/Many%20faces.pdf?CFID=4451270&CFTOKEN=29136583).

Paragraph 4.21 of the consultation indicates a misunderstanding of the innovation process. Companies do not aim for incremental innovations at the expense of larger “breakthroughs”. Incremental innovations are the result of dynamic competition and are in themselves valuable. A succession of incremental innovations can produce a cumulative breakthrough improvement in treatment that if achieved in a single step would be judged a major breakthrough. It would be damaging to overlook this. Rewarding incremental innovation paves the way to making advancements in science which can lay the foundations for new therapeutic advances to be made.

For example, since 2000 there has been a considerable increase in the number of available treatments for colorectal cancer. In 2000, 5-FU/FA was the standard treatment and if this treatment failed there was no other option. However, since then the standard first line treatment has progressed and now includes other medicines in combination with 5-FU/FA (such as FOLFOX or FOLFIRI) or other agents alone. Moreover, the introduction of biotechnology products (monoclonal
antibodies) has also improved overall survival – and these drugs can either be taken in combination with existing chemotherapy or as monotherapy. The cumulative gain to patients with colorectal cancer has been substantial despite the absence of a single major “breakthrough”.

Any pricing approach which leads to increased uncertainty for companies about their future earnings if they fail to launch a first-in-class breakthrough medicine could deter much worthwhile R&D, rather than encouraging it. This point is demonstrated below using the example of colorectal cancer treatments.

Innovative developments in therapy for colorectal cancer can be clearly linked to years of extended survival

It is crucial that competition is encouraged between companies as it is desirable to have a number of medicines in the same class. A fast follower in the same class needs to be able to achieve any innovation premium that the first in class medicine achieved although companies may of course choose to lower value based prices for competitive reasons.

The definition of innovation proposed in paragraphs 4.23-4.25 of the consultation is too narrow. It focuses solely on “clinical performance” and “QALY benefit”. The definition of innovation needs to take into account improvements on any or all of the dimensions of potential benefits of medicines, for example: patient benefits (route and frequency of administration, mode of action, drug delivery mechanism, dignity, improved experience of the healthcare process) and cost savings within or beyond health care and personal social services.

Paragraph 4.23 states that the “therapeutic innovation and improvement” measure would “reflect any additional health gain not captured by the normal pharmacoeconomic assessment of health gain because of measurement difficulties.” This is unusual as part of a definition of innovation, but it is nevertheless an important point. As discussed earlier, it is well documented that the generic instruments of health gain typically used to generate QALY estimates – principally the EQ-5D in the current UK context – are insensitive to some types of health gains that are highly valued by patients, e.g. improvements to sight and hearing. Value based pricing must recognise and incentivise / reward all relevant gains, and not merely those picked up by standard instruments.

8 In what ways can we distinguish between levels of innovation?

ABPI noted above that innovation is a matter of degree and is multi-dimensional. Consequently, defining “innovation” as being either present or not would be a mistake in any system of value based pricing. Similarly, socially worthwhile R&D might also be discouraged if a crude three-level definition were to be used, of the kind: ‘breakthrough’ vs ‘incremental’ vs ‘no’ innovation. An appropriate level of granularity will be required in any sliding measurement scale which will need to be applied to the dimensions of innovation, including those which cannot be valued as part of the QALY.

From an industrial policy perspective, clarity is needed about how within the new system investment risk will be rewarded (static versus dynamic efficiency). It may be the case that risk taking may need to be subsidised directly rather than indirectly through a premium on the value based price of a medicine, e.g via mechanisms such as extended patent life or other such reward mechanisms.

ABPI considers that the present definition being proposed for therapeutic improvement and innovation is too narrow and that this should be broadened to encapsulate aspects of innovation not recognised in the current proposals.
ABPI recommends that the approach to measuring and quantifying innovation is explored further. The solution is likely to include the creation of a framework which can be implemented by a panel of experts, including those who understand pharmaceutical research and development. It may be useful to jointly review the approaches taken to measuring innovation in other pricing and reimbursement systems (EU and beyond) and understand the pros and cons of the various approaches (e.g. in France and Italy) as part of this process.

Sir Ian Kennedy’s independent study on how NICE establishes the value of innovation and the ABPI submission to inform that study is a useful further reference.

9 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?

ABPI recommends first, that the elements potentially included in VBP need to reflect a wider set of concerns, including industrial policy perspectives that may not be adequately reflected in stated preferences regarding health care. Second, relying on stated preference 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 infer a spurious degree of precision. While it is important to be broadly consistent in the approach to pricing across technologies, a practical approach is more likely to involve establishing a plausible range of weights, informed by a range of methods and viewpoints and the available literature, including the perspectives of NHS commissioners, clinicians, Government and the general public.

ABPI considers that further research is required in order to establish the appropriate weights for the different elements in value-based pricing.

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 extensions of life. However, there are some limitations as to how this evidence might inform the development of VBP. Studies so far have often focused on a single issue (such as severity, or end of life, or age) and explore trade-offs between that issue and QALY gains.

An important consideration in deriving the weights is whose opinions should count. Much of the research to date has used stated preference methods with samples of the general public. While the use of general public preferences is in keeping with
other methods that are a standard part of health technology assessment (HTA) – for example, the quality of life weights used in QALYs – the argument for relying exclusively on these approaches to determine VBP weights needs careful thought.

Regardless of how the weights are derived, and whose preferences they are based upon, it will be crucial that modelling is undertaken to provide a level of validation and to ‘sense check’ how they will operate in practice in order to manage unexpected consequences and to mitigate against risks. All weightings will need to be fully tested to ensure that they deliver in practice what was expected in theory. Different weightings may be applicable for different disease states. As stated earlier, whilst sophisticated mechanisms may deliver some benefits, the use of more pragmatic approaches, or a combination of approaches might be more beneficial.

10 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.

Value based pricing will require joint agreement between Government and industry on the elements of value to be considered; a means of measuring the magnitude of these elements for each medicine; a plausible range of weights to be applied to each; and a means of aggregating these into an overall assessment of value.

A first step will be to reach agreement on the elements of value which need to be considered as part of a comprehensive value assessment, and to reach agreements on the definition of the terms: unmet need, severity, societal benefit, therapeutic improvement and innovation. This detail will aid understanding of the fundamental principles being supported by VBP any will help to highlight related operational issues of how the various factors are defined, measured, weighted and combined for decision-making purposes.

The consultation document focuses on 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 a medicine (or by flexing the cost/QALY threshold, which amounts to the same thing).

The ABPI has a number of concerns with this approach. First, it relies on the QALY being an adequate measure of health gain in all cases. In disease areas where quality of life (QoL) is difficult to adequately 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 (vision and hearing) are known to pose challenges. Garau et al (2010) notes the potential limitations of QALYs with respect to cancer. NICE notes the challenges involved in measuring and valuing QoL in children, and indicates that
this as a reason why children are given special consideration in its HTA process (Rawlins et al 2010).

Moreover, some types of healthcare intervention are simply not appropriate to value using QALYs (for example, infertility treatments). In other cases where patients’ problems prohibit self-completion of standard QoL instruments, measurement relies on proxy completion. 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 these shortcomings.

Second, weighting QALYs is an intuitively plausible approach for some but not all of the additional types of ‘value’ being considered. Some criteria may be thought of as broadly proportional to the number of incremental QALYs gained by a medicine. 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. However, other criteria are not self-evidently proportional to the QALYs produced – for example, degree of innovation or process-of-care benefits to patients. Incorporating the value of these benefits by weighting QALYs is less defensible, since the size and value of these will vary by medicine in a way that is not necessarily linked to the size or value of the QALYs gained. Combining these other sources of value requires a means of describing and measuring them (e.g. on a scale of innovativeness) and some common currency for reflecting the relative value of them alongside QALYs.

A 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); and productivity effects on the wider economy. These are best combined with the costs of treatment to provide a net cost measure (which can of course be negative).

ABPI recommends that a range of approaches to valuing medicines are explored, including MCDA type approaches for combining the different elements of value during assessments prior to any decisions being made.
ABPI recommends the inclusion of societal costs and benefits as part of the assessment of value for a medicine, where these are relevant for the particular medicine being reviewed. We propose that further work is required on how to achieve this, including making an assessment of the evidence requirements in this area along with any impact on medicines assessment processes and timelines in order to ensure that these are proportionate.

Given the range of means of measuring, valuing and aggregating disparate sources of ‘value’, the implications for pricing are complex and, at present, there is a lack of evidence about what might be the preferred approach. ABPI recommends that a range of approaches to valuing medicines be explored fully, to help better understand the advantages and disadvantages of each approach.

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

The means by which technologies are assessed on these criteria (burden of illness, therapeutic innovation and improvement) might entail the use of specific ‘categories’ or ‘continuous measures’. Options include:

- A series of ordered levels/categories, e.g. very severe, severe, moderately severe, not severe, with greater levels of severity accorded higher scores/weights

- A continuous measure, e.g. severity might be measured as the pre-treatment quality of life score, and a sliding scale of weights be applied to that using a weighting function.

Using discrete categories might have an advantage in terms of simplicity, and in facilitating the use of stated preference methods to derive appropriate weights. However, categories have the disadvantage of requiring somewhat arbitrary judgments about what constitutes an appropriate number of levels, and what are the relevant cut-off points that define those levels.

As noted in our response to Question 10, given the non-trivial implications of these judgments, careful testing of a range of alternative approaches and the sensitivity of VBP to them should be undertaken prior to agreeing policy.

Whichever approach is taken, VBP should avoid a simplistic approach of pre-assigning diseases to a small number of categories defined in terms of, for example, level of unmet need and disease severity. Within any given disease area, the relevant weights will depend also on the stage of the disease, and any other factors regarding existing treatments or patient characteristics that determine the burden of the disease or the ability of patients to benefit from new
technologies. The criteria and weights should be developed in generic terms, and medicines assessed in terms of those on a case by case basis.

A further key question which will need to be addressed relates to the patient population against which the assessments of value are undertaken. Whilst the licensed indication may be used to undertake the initial assessment, it may be the case that during the value assessment process, particular sub-groups are identified for further scrutiny which might require the revisiting of the scoring of each of the individual value elements (e.g. for NICE 'optimised' recommendations)

ABPI recommends that the issues addressed by Questions 9, 10 and 11 will all need to be underpinned by further research work which will need to be planned, specified, commissioned and reported during the period of the VBP creation process through 2011 and 2012.

12 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?

The pharmaceutical industry recognises that as part of the process of developing new medicines it has a role to ensure that evidence is developed that supports not only the licensing process but also assessments of value to inform value based pricing decisions. However, as recognised in the current NICE Methods Guide, “there are always likely to be deficiencies in the evidence base available for health technology assessment”. Current methodologies within health technology assessment allow for the exploration of the impact of such deficiencies or uncertainty in the form of probabilistic sensitivity analyses and other analytical methodologies. These allow decision making committees to explore the ranges of potential estimates as well as the most likely value (e.g. the best estimate). It is critical in such exploration that the potential that a medicine will be at the higher end of value estimates is fully explored as well as more conservative assumptions. It is likely that the assessment will result in a range of plausible estimates and in this context it is unlikely that a maximum value based price based on a point estimate can be identified without an upper and lower range also being included. Indeed, as uncertainty can never be wholly eliminated, it is the case that the maximum, value based price of any given medicine will at best be identifiable within a range.

However, as outlined in the consultation there are some medicines where the data that will be required to estimate value fully are unlikely to be available at launch – for example, where longer term data are required or where for ethical or logistical reasons such evidence cannot be produced (such as for orphan medicines for rare diseases) or is difficult to produce in parallel with the licensing process. The current PPRS agreement permits a number of approaches such as flexible pricing and outcomes-based patient access schemes that potentially allow for an initial
value based price to be agreed (based either on current or future value) and for a subsequent value based price adjustment and/or rebates when the evidence becomes available. Whilst there have been some concerns about the impact of such schemes and the burden they place on the NHS, the current PPRS Review of these schemes should identify improved approaches to permit the more systematic use of schemes.

The UK is well placed to be able to deliver real world evidence that can be utilised as part of a value-based pricing system. For this to be realised, it will require greater health records linkage and the ability to link medicines utilisation to indication to health outcomes. Delivery on this may also help to provide the UK with competitive advantage in attracting future clinical research and development.

ABPI recommends that the proposals for the ‘Innovation Pass’ are revisited during the dialogue about value based pricing. The Innovation Pass allowed for initial central funding for drugs for small patient populations where data to demonstrate value were limited mainly because of small numbers of patients. The specific criteria for medicines to be included within the Innovation Pass could also be usefully reviewed in the broader context of how to deal with uncertainty.

In the circumstances where it is unlikely that further evidence will be developed, then decisions need to be made on the basis of appropriate modelling or reasonable assumptions rather than insisting on an unreasonably high burden of proof. ABPI has already undertaken work in the area relating to coverage with evidence development and this is a well established approach in other countries which enables patients to access medicines whilst the evidence base is extended.

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

NO

On medicines pricing......

ABPI is concerned that the consultation is unclear about how value based prices will be agreed. The consultation focuses primarily and in detail on how a new system to assess value might work. It does not address or provide any detail on the proposed processes for the agreement of a value based price based on the utilisation of the outputs from a value assessment process, i.e. the process for linkage of value to price.

It is important to understand that medicines prices are set in a global context and by the global headquarters of pharmaceutical companies.
Further questions which will need to be answered include the following:

- What should the process be for linking assessed value to price?
- How should the process of value based price negotiation and agreement work?
- Which organisation(s) should be involved in the process, alongside the company?
- How should agreement on value based price be reached when company estimates of cost effectiveness or overall value differ from those of NICE?
- How can the system ensure that value based prices or agreements remain non-disclosed and commercial-in-confidence when necessary?
- What alternative courses of action could be open, in those cases where initial negotiations fail to reach agreement on a value based price?
- What arbitration or appeal processes will be required to support value based pricing?
- How long should the value assessment and pricing discussions take respectively? For example, SMC currently aims to provide guidance within three months of launch – whereas NICE presently takes longer
- How much organisational infrastructure, staffing and resourcing levels will be needed to operate the value assessment and pricing process respectively?
- At what stage should discussions on value assessment and on pricing begin – part way through the regulatory process, at EMEA / MHRA positive opinion or at EU licensing?
- What would be the indicative transaction costs for a full value assessment and pricing process based on the criteria for the selection of medicines to be subject to value based pricing (for Government, NICE and the pharmaceutical industry)?
- How will the processes accommodate consideration of commercial proposals from manufacturers including: risk share agreements; coverage with evidence development; commercial propositions utilising approaches other than discounts; patient access schemes or flexible pricing arrangements?
In relation to the devolved administrations:

- What role should the devolved administrations play in a new value assessment and pricing process?

- What role should the SMC and AWMSG play in a new value assessment and pricing process and in enabling medicines access and uptake?

- How would the sequencing and scheduling of value assessment and pricing processes be undertaken across the four nations if they undertook these processes separately?

In relation to regionally based medicines evaluation agencies (such as LNDG and MTRAC):

- Is there a continuing need for these organisations within the NHS since they may become duplicative after value based pricing is introduced? Does their role need to change?

In relation to impact on NHS commissioners, GPs and clinicians:

- What information about value based pricing agreements will be communicated to NHS stakeholders?

- How will guidance be provided to NHS stakeholders about the place of a particular medicine in the treatment pathway?

- How will NHS stakeholders ensure that a particular medicine is made available for all eligible patients in a particular locality?

Only after these questions have been fully answered and positions have been agreed between Government and industry is it possible to fully answer Question 13.

The processes for undertaking value based pricing should be as simple and efficient as possible and low in bureaucracy, which is consistent with Government commitments made in this area. EU legislation (EU Transparency Directive 89/105/EEC) recommends decision making within 90 days.
On affordability……

ABPI is concerned that the question of affordability has not been considered adequately in the consultation. Affordability relates to the budget impact of medicines and the availability of appropriate and timely levels of funding to meet this budget impact. Affordability, amongst other key factors, is a key driver of medicines access in the NHS (for example, see ABPI Response to CDF Consultation, question 10). ABPI recommends that improving the use of medicines must be a primary objective for the new system since the UK lags behind many other comparable countries in relation to usage of new medicines, as reported in: “Extent and Causes of International Variations in Drug Usage Report” prepared for the Secretary of State for Health by Professor Sir Mike Richards and published in July 2010.

The consultation document in section 5 does not explain fully how the optimum uptake of medicines post value based pricing will be ensured, although there is reference in the consultation to GP Consortia being ‘…held to account for outcomes…’. Physicians will indeed be motivated to improve outcomes but without further direction on the optimal use of medicines in the form of national guidance they may lack the necessary information to support local decision making about medicines. Moreover, the optimum uptake of medicines will not be achieved without adequate local funding being set aside to meet the budget impact to treat all eligible patients using those medicines which have a value-based price. This is further compounded by the fiscal situation within the NHS in the context of the requirement to deliver £20bn of efficiency savings over the next three years.

It should also be remembered that decisions on affordability are devolved and the consultation does not address the different health systems within the four nations of the UK.

To strengthen the affordability perspective, ABPI recommends that the outputs of value based pricing need to extend beyond just value based price agreement and be accompanied by key levers which will ensure appropriate uptake of medicines for all eligible patients. During the co-creation process, consideration might need to be given as to whether the PCT funding direction to implement NICE guidance (Clause 221, Health and Social Care Bill) needs to be replicated with an equivalent mechanism for the ‘new’ NHS.

In summary, compared to many EU countries the UK has relatively low uptake of new medicines, lower medicines prices and poorer outcomes in a number of diseases. Spending more on improving access to and uptake of medicines may be part of the solution to these issues. VBP therefore has the potential to improve upon the current position.
14 In what circumstances should a value-based pricing assessment be subject to review

In the spirit of value based pricing, ABPI recommends that it would be appropriate to review an assessment in cases when new evidence on the effectiveness of a medicine becomes available. New evidence might be generated from both clinical trials or as a result of real-world use of the medicine in clinical practice.

Additionally, it may be necessary to undertake a review in circumstances when a medicine initially used to treat one condition gains licences in additional indications. This position would be consistent with the existing provisions on flexible pricing in the 2009 PPRS Agreement Chapter 6.

(Note that when reviews are undertaken, the burden of illness and therapeutic improvement/innovation measures and the weightings applied to these may also change).

Reviews should not be triggered by changes in the market such as patent expiry of comparator medicines. Companies should be able to obtain a reasonable return during patent life and for periods of exclusivity. To trigger pricing reviews due to market changes such as this would be unacceptable.

Reviews of medicines should in general be scheduled in advance to align with published regulatory timelines. ABPI recommends that clear criteria for triggering reviews are mutually agreed upon as part of next steps.

15 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?

ABPI notes that the Cancer Drugs Fund (CDF) was set up to address issues concerned with access to cancer medicines. It is primarily about funding and affordability not about the assessment of medicines’ value or pricing. It is also important to recognise that there are other medicines that are presently restricted by NICE (and further medicines will no doubt also be restricted over the period to the end of 2013) and similar consideration should be given to their pricing post 2014. ABPI has gathered data which demonstrates that there remain regional variations in access to some cancer medicines approved for use via the Interim CDF. This situation would present challenges were it to occur for medicines which have undergone assessment via value based pricing post 2014. As noted earlier, consideration might need to be given if necessary during the co-creation process to NICE guidance issued for medicines after 2014 carrying with it a mandatory implementation requirement, as facilitated by Clause 221 of the Health and Social Care Bill.
ABPI suggests that there should no assumption that medicines funded by the CDF up until the end of 2013 should automatically be subject to a value based pricing assessment.

However, it may be reasonable to assume that access arrangements that have been put in place for medicines in use prior to 2014 (including for example, those with patient access schemes or flexible pricing arrangements) should continue beyond 2014 under the original agreements.

ABPI recommends that this question needs to be revisited between Government and industry once the lessons from the Interim Cancer Drugs Fund and the full Cancer Drugs Fund are distilled and analysed.

16 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?

PARTIALLY

If agreement between Government and industry can be reached on the key strategic issues and on the areas set out below, we hope that it would be possible to deliver upon the agreed objectives.

On patient outcomes……

Patient outcomes will only be improved if all eligible patients are able to access the medicines which will improve those outcomes and in a timely manner. The linkage of value assessment to medicines access and uptake is critical in this regard. A cost effective medicine has no value if patients are unable to access it or only limited value if its use in practice is not at the point in the treatment pathway at which its cost effectiveness was evaluated. Taking a broader definition of the value of medicines through value based pricing should enable additional measures to be defined which can be used to monitor progress towards improving patient outcomes. ABPI recommends that NICE guidance about medicines post 2014 includes a clear statement about where in the treatment pathway a medicine is to be utilised and that equivalent guidance is provided in the devolved nations.
On greater innovation……

As discussed earlier, innovation occurs generally on an incremental basis. Any mechanism of measuring innovation needs to be able to distinguish – and reward – each type of innovation and not simply a new first in class medicine. Innovation often occurs for medicines within their lifecycle – for example, controlled release medicines or the redevelopment of medicines that are generic but add significant patient benefits – for example, tolerability and adherence – which may not be measureable by direct health utility. Unless innovation – both incremental and breakthrough - is appropriately rewarded then the lifecycle of investment in R & D may break down.

On a broader and more transparent assessment……

The proposals in the consultation support a broader assessment of the value of new medicines. The transparency levels of the assessment processes have yet to be defined but ABPI would be supportive of greater transparency of decision making processes but within a framework which, where necessary, permits non-disclosure of value based prices or commercial-in-confidence information.

On better value for money for the NHS…..

Medicines prices in the UK are already amongst the lowest in Europe. It is too early to say whether the proposed value based pricing system will deliver better value for money for the NHS. Extensive modelling and analyses will be required to prospectively understand the impact value based pricing will have on both medicines pricing and value for money for the NHS, where prices are agreed based on a fuller assessment of the value provided by a medicine.

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

ABPI has set out the broader strategic issues which relate to the creation of a new UK pricing and reimbursement environment at the beginning of our response. These issues should be resolved early on prior to the commencement of more detailed work on value based pricing and are not repeated again here for the sake of brevity.
18 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?

ABPI supports the principles of value based pricing. Medicines already deliver significant value to patients and the NHS and efforts to ensure that their full value is more broadly recognised and appropriately rewarded are to be welcomed.

However, there are substantial risks associated with the introduction of value based pricing. The pharmaceutical companies in the UK operate in a global commercial market and it is from this standpoint that the following points are made:

**Limiting access to medicines for UK patients**

**Risk**

Companies may delay or not launch some medicines in the UK because reimbursement is only available at an inappropriate commercial level which will undermine global revenues in other key markets and provide an insufficient return. This may have a subsequent negative impact on R & D and on the attractiveness of the UK as a location for clinical trials because if medicines are launched late, or not at all, these may not become established as a standard of care in the UK in time to be used as comparators in subsequent clinical studies.

NHS patients may face protracted delays in getting access to new medicines. This may result in a ‘two-tier’ health service where some patients are only able to receive basic medicines, but others who can afford higher prices or have private healthcare insurance are able to access more innovative medicines which are not reimbursed, undermining the fundamental principles of the NHS.

**Mitigation**

The cost effectiveness threshold and the weightings applied in value based pricing should be set in a way which allows companies to charge prices that reflect the full value of the medicine and allow a reasonable return on investment whilst ensuring the NHS achieves value for money.

Ensure that where required value based prices or arrangements are not disclosed in the public domain and are shared only on a commercial in confidence basis.
Distorting the market

Risk

Companies might be disincentivised from competitive pricing if they bring to market a second or third in class medicine where the expected value based price is anticipated to be at or just below the first in class medicine and may not be as low as would have occurred in the absence of value based pricing.

Mitigation

Ensure that the approaches introduced to assess levels of innovation are sensitive and granular enough to identify and appropriately recognise differences between medicines in the same class and in such a way that can be reflected in medicines pricing.

Jeopardising the UK life sciences sector as a key growth area

Risk

The attractiveness of the UK for R&D investment will be eroded if UK patients are unable to gain access to new medicines. Issues which affect life sciences investment are explored by Gambardella et al (2000) and in an EU Commission Report (2009).

Mitigation

Undertake full impact assessment on value based pricing from an industrial policy perspective to fully understand impact on life sciences sector and revisit impact assessment undertaken from health department perspective when further agreement has been reached about the overall design of the new system.

Patient outcomes do not improve

Risk

The UK already lags behind developed countries in many disease areas such as cancer, where outcomes lag behind that of many other comparable countries [Coleman et al (2010). If access to medicines reduces under the new system of value based pricing or the broader NHS reform programme then this might be expected to have a detrimental impact on patient outcomes.
Mitigation

Ensure mechanisms to deliver improved access to and uptake of medicines for NHS patients are built into value based pricing and into NHS commissioning processes and that metrics are identified to measure progress.

Increasing the costs of managing the NHS medicines bill

Risk

Running a pricing and reimbursement environment with two components, i.e. a value based pricing system and a successor to the current PPRS agreement for existing medicines available before 2014 (and any other mechanisms which might need to cover certain categories of medicines such as rare disease medicines) may involve an increase in transactions costs. This is recognised by the Impact Assessment accompanying the value based pricing consultation [DH (2010)].

It is not clear whether these higher transaction costs will necessarily be offset by the gains anticipated from value based pricing and at a time when the NHS needs to make unprecedented efficiencies and is facing the lowest growth rates for many years, as identified for example by the Nuffield Trust (2010).

Mitigation

Undertake further impact assessment on value based pricing proposals when the framework for the new system has been agreed between Government and industry.

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

Once the value based price has been agreed for a medicine then it should be made available for all eligible patients who will benefit from it. Equality of access to medicines across the NHS is an important goal for a new UK pricing and reimbursement environment. Where appropriate, it will be important to specifically consider approaches to ensuring access to medicines for patients in the following categories:

- different socio-economic groups where it is reasonable to assume uptake in medicines will vary
• patients with a lower overall life expectancy and health state in areas of deprivation may benefit more from new medicines. These may presumably also be the areas with highest budget pressure due to demand and mechanisms should be developed to ensure uptake in lower socio-economic areas is equitable with more affluent locations.

The new system should avoid introducing cross border inequalities between the four nations of the UK.

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

ABPI stands ready on behalf of the pharmaceutical industry to start work on the co-creation of a new UK pricing and reimbursement environment.

The detailed areas which will need to be worked on together as part of the next steps in the process are set out below:

a) Initiate early dialogue with the ABPI and industry on the broader strategic issues which have been set out in our response and agree joint positions on each strategic issue

b) Develop work programme and prepare high level project plans (2011 to 2013) and detailed project plans for remainder of 2011

c) Obtain joint agreement as to the objectives for the new system. The agreed key features of the PPRS can provide a good starting point for this dialogue

d) Develop a series of metrics and indicators (both lead and lag) which will permit regular reporting of progress against the objectives

e) Identify how the PPRS-like component and value based pricing component of the new environment either stand-alone or are integrated together in practice as part of a new voluntary agreement

f) Initiate early dialogue between DH, the devolved administrations and ABPI to ensure the development of a new pricing and reimbursement environment that is applicable to all four nations of the UK

g) Draw up and agree a specific set of criteria to govern the selection of medicines which might be subject to value based pricing (both for those launched before 1st January 2014 and for those launched on or after 1st January 2014)
h) Explore further for use in exceptional circumstances mechanisms for coverage with evidence development; patient access schemes; risk share schemes or flexible pricing arrangements

i) Explore further the categories of medicines with unique pricing and reimbursement requirements which will need to be accommodated within VBP or otherwise may require separate consideration outside of VBP

j) Define how unmet need and severity are to be measured and to what levels of granularity. This will need to consider aspects of industrial policy

k) Develop revised impact assessment from the industrial policy and the health perspective

l) Resolve how to deal with the linkage of indication specific value assessment to price

m) Establish a dialogue between Government, the NHS, the pharmaceutical industry and representatives of the main pharmacy-related information systems suppliers

n) Revisit the approach to measuring innovation including considering how to deal with incremental innovation alongside breakthrough improvements in treatment

o) Review the approaches taken to measuring innovation in other pricing and reimbursement systems (EU and beyond) and understand the pros and cons of the various approaches

p) Explore a range of approaches to valuing medicines, including MCDA type approaches for combining the different elements of value during assessments

q) Undertake further work on the inclusion of societal costs and benefits within the value assessment framework, assess the evidence requirements and administrative burden to do this and conduct review of the evidence base

r) Consider how to manage the setting and updating of the cost-effectiveness threshold and weightings and how these should be managed over time

s) Identify how to deal with sub-groups which might be identified on an iterative basis during the value assessment process
t) Plan and commission further research to underpin the development of the detailed approaches to identifying weights, measurements and derivation of categories (Questions 9, 10 and 11)

u) Revisit the proposals for the 'Innovation Pass' to understand any learnings, including the selection criteria utilised

v) Undertake further work to answer the questions which have been set out in response to Question13 regarding the requirements of a pricing framework

w) Agree the specific circumstances / criteria for undertaking reviews of value assessments which have been completed.

A number of the above key work programme areas will require the commissioning of specific research, analyses and modelling projects. It may be appropriate to jointly collaborate on some of these projects across Government and industry. ABPI stands ready to support the joint coordination, commissioning and funding for a number of the key projects with the Department of Health and / or Department for Business, Innovation and Skills.
References


