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

Reply Form

Closing date for responses: ***17 March 2011***

Please fill in and/or tick the appropriate response.

Response form

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Before submitting your response to the Department, please make sure that it has been saved in a name that will make it easier for us to track. Many thanks.

Freedom of Information

We manage the information you provide in response to this consultation in accordance with the Department of Health's **Information Charter.**

Information provided in response to this consultation, including personal information, may be published or disclosed in accordance with the access to information regimes. The relevant legislation in this context is the Freedom of Information Act 2000 (FOIA) and the Data Protection Act 1998 (DPA).

If you want the information that you provide to be treated as confidential, please be aware that, under the FOIA, there is a statutory Code of Practice with which public authorities must comply and which deals amongst other things, with obligations of confidence. In view of this, it would be helpful if you could explain to us why you regard the information you have provided as confidential. If we receive a request for disclosure of the information we will take full account of your explanation, but we cannot give an assurance that

confidentiality can be maintained in all circumstances. An automatic confidentiality disclaimer generated by your IT system will not, of itself, be regarded as binding on the Department.

The Department will process your personal data in accordance with the DPA and in most circumstances this will mean that your personal data will not be disclosed to third parties. However, the information you send us may need to be passed on to colleagues within the UK Health Departments and/or published in a summary of responses to this consultation.

I do not wish my response to be passed to other UK Health Departments ☐

I do not wish my response to be published in a summary of responses ☐

Please indicate all the countries to which your comments relate:

UK-wide	√	*and/or:*	
England	☐	*Northern Ireland*	☐
Scotland	☐	*Wales*	☐

Are you responding:

- *as a member of the public* ☐
- *as a health or social care professional* ☐
- *on behalf of an organisation* √

Country of qualification

Please indicate as appropriate:

UK ☐ Other EEA ☐ Rest of World ☐

Area of work:

NHS	
Social Care	
Private Health	
Third Sector	
Regulatory Body	
Professional Body	
Education	
Trade Union	
Local Authority	
Trade Body	
Other (Please give details)	
Independent Contractor to NHS	
Manufacturer	
Supplier	
Other (where relevant)	

If you are responding on behalf of an organisation, please indicate which type of organisation you represent:

NHS	
Social Care	
Private Health/Independent Secto	
Third Sector	
Regulatory Body	
Professional Body	
Education	
Trade Union	
Local Authority	
Trade Body	
Other (Please give details)	√

Other: Medical Royal College

In which of the following areas do yo live: (please tick one box only)	
North East	
North West	
West Midlands	
South East	
London	

Humberside/Yorkshire	
East Midlands	
East of England	
South West	
No answer	

1 What is your sex? *
Tick one box only.

Male	
Female	
Prefer not to say	

2 Date of Birth *

e.g.

03	06	1975

3 Are your day to day activities limited because of any health problem or disability which has lasted, or is expected to last at least 12 months?

The Disability Discrimination Act (DDA) defines a person with a disability as someone who has a physical or mental impairment that has a substantial and long-term adverse effect on his or her ability to carry out normal day to day activities.
Tick one box only.

I have a longstanding illness	
I have a disability	
Prefer not to say	

4 Do you look after, or give any help or support to family members, friends, neighbours or others because of either long term physical or mental ill-health/disability or problems related to old age?
Tick one box only.

Yes	
No	
Prefer not to say	

5 What is your ethnic group?
Tick one box only.

A White

British ☐

Irish ☐

Any other White background, write below

B Mixed

White and Black Caribbean ☐

White and Black African ☐

White and Asian ☐

Any other Mixed background, write below

C Asian, or Asian British

Indian ☐

Pakistani ☐

Bangladeshi ☐

Any other Asian background, write below

D Black, or Black British

Caribbean ☐

African ☐

Any other Black background, write below

E Chinese, or other ethnic group

Chinese ☐

Any other, write below

5 What is your religion or belief?
Tick one box only.

Christian includes Church of Wales, Catholic, Protestant and all other Christian denominations.

None	☐
Christian	☐
Buddhist	☐
Hindu	☐
Jewish	☐
Muslim	☐
Sikh	☐
Prefer not to say	☐

Other, write below

☐

6 Which of the following best describes your sexual orientation?
Tick one box only.

Only answer this question if you are aged **16** years or over.

Heterosexual / Straight	☐
Lesbian / Gay Woman	☐
Gay Man	☐
Bisexual	☐
Prefer not to say	☐

Other, write below

☐

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

Consultation Questions

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

Yes X No

Comments

- Achieving all aspirational objectives within a single new system will be challenging and some of the objectives are achieved within existing systems eg the breadth of NICE and SMC.
- We always want better outcomes for patients. The College is not convinced that medicines which offer great benefits but are presently denied to patients will necessarily be purchased and used appropriately in the new scheme.
- The number of new chemical entities achieving licence status has dropped sharply, so stimulation of innovation is a good thing.
- There has been, and will continue to be whatever system is used, uncertainty in the comparative aspects of clinical and cost-effectiveness evaluation of medicines.
- Good value for money for the NHS is clearly important.

- **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 X No

Comments

Given the rate of accrual of new medicines into practice, it will be necessary to apply some retrospective decisions if this scheme is to have any impact.

"Me too" drugs, product line extensions, medicines that replace those about to go

off-patent, licensed products that replace unlicensed simple chemicals, are some possibilities.

However, this will require a substantial additional body of work to undertake the extensive health economic analysis to justify any retrospective price change and, of course, some prices could increase.

- **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 **X** **No**

Comments

The entire orphan (more precisely, ultra-orphan) situation is unsatisfactory and goes to the heart of the dilemma facing doctors and politicians – the conflict between utility (efficiency) and equity (fairness). Ultra orphan conditions are by definition very rare, and conducting clinical trials to establish efficacy of medicines are difficult at best, impossible at worst, so licensing arrangements are already different. Prices seem to be set high because of low throughput, but these are not always medicines that have gone through extensive R&D. By definition these need to be treated differently, but NOT preferentially.

- **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 **X** **No**

Comments

The definition of unmet need is important, and difficult. For example, many cancers are not curable with current regimens, and that could be defined as unmet need. However, a new medicine approved on that basis might give only slight and marginal improvement over existing therapy. If such a medicine attracted a high price, the resulting CE ratio would be high, possibly higher than under the present scheme.

If, on the other hand unmet need is defined on a population basis, a new clinically effective treatment might also attract a high-ish premium and the budget impact could be enormous if there is high volume of use. However, it would be important

not to miss opportunities to support real innovation in common conditions where need is mostly already met.

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

Comments

This is difficult and is likely to impose a big administrative burden. One option would be to base pricing on the lower cost of the different indications or to license the drugs separately with different names and using different doses.

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

Comments

As above. It would be important to guard against the user switching to the cheapest preparation.

- **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 **X** **No**

Comments

Ideally we would like to see new medicines, possibly for diseases that have few effective treatments, that give great benefits. That seldom happens, but it does occur, although not all innovations will bring sustained benefit in the longer term.

The suggestion of higher reward for innovation is fine for first-in-class medicines. How should we then treat successors, some of which may confer modest benefits over the originator product, but huge benefits over the previous untreated situation?

Incremental change and modest gain is the usual picture. This is especially true in cancer where new combinations and mixed regimens may confer some small benefit, but these small gains may be cumulative over time. The proposal would go against that type of approach.

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

Comments

Innovations will arise in novel mechanisms of action (receptor, body system) or in novel means of administration (oral vs IV).

The QALY may not be the way to do this – a cheap drug that does little may have a good CE ratio.

4.23 - refers to innovation reflecting health gain that cannot be captured by usual pharmacoeconomic assessment. The College is unclear what is meant by this. Subjective aspects of health gain are captured by a range of QOL instruments. If they mean that societal aspects of pharmacoeconomic evaluation need to be considered, then that can be done, but it means drawing a much wider circle on the map of outcomes (or effectiveness).

4.24 - refers to the qualitative assessment of innovation reflecting, for example, a new mode of action. That would not be based on outcome, and as such, goes against the thrust of the paper.

4.26 - refers to calculating "patient health benefits" and is challenging in terms of finding a fair comparator and is often uncertain at product launch.

The College commends a recent BMJ article by Ferner Hughes and Aronson "NICE and new: appraising innovation" (BMJ. 2010; 340: b5493).

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

Comments

This must encompass quantity of life gained, quality of life gained, and age. If we are to cast the net wider then ability to return to work, consumption of resources eg carers, nurse time, other community resources, and a host of additional variables will need to be taken into account. Some of these are already quantified in standard economic assessment. Weights are currently assigned by a combination of economic evaluation, expert opinion and patient opinion. Reference to SMC methodology gives more information on this. It represents a pragmatic approach, part objective and part subjective, that already takes in some of the ideals that this new initiative seeks to render purely objective. Inevitably,

weights cannot be rigidly set or pre-specified, as a medicine-disease pair is often unique. Thus, it has never been appropriate to set a rigid CE ratio for the acceptance of medicines by HTA bodies such as NICE or SMC.

Pharmacoeconomics is inexact at best. Constructing a new more complex methodology will increase the uncertainty. Consequently, there will be great need for sensitivity analysis, and knowledge of the uncertainties thereof.

It may be necessary to construct a vast array of these weighting variables covering the entire universe of human illness if this tool is to be evenly applied. How do you compare cancer with acne, depression with heart attack, and how do you differentiate the impact on individuals versus society?

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

Comments

See above, in particular the comments about changing the NHS perspective to encompass a broader societal perspective. Although the QALY has some limitations, it still seems the best way forward.

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

Comments

This is difficult but criteria for "innovation" might include:

- Is this a new class of medicines with a new mode of action?
- Does it show clinical benefit over existing therapies?
- Does it show economic benefits (however defined) over existing therapies?
- What measures are in place to update benefit information as experience of use in clinical practice grows?
- What are the possible safety issues, and how are these being addressed as the medicine becomes more widely used?

Steps must be built in to review, possibly in a short time frame, the putative benefits. It is not unusual for early promise to be unfulfilled on grounds of efficacy or safety as experience accumulates – as stated previously, innovation may disappoint in the

longer term.

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

Comments

A decision is required as soon after licensing and launch as possible to address the problem of removal of drugs from the market post-licence. This allows access where appropriate, and gives clinical guidance to prescribers. It also prevents "decision blight" and provides the best chance of minimising regional variation in availability. If that rapid decision is to be made, inevitably there must be compromise in terms of comprehensiveness, as a number of clinical questions will be unanswered at that time and the value of the drug will not be fully understood. Thus, in certain cases, rapid guidance should be considered as an interim measure. Review and update at whatever time is appropriate in the given case then becomes an essential part of the process. Starting with high prices until sufficient evidence is accumulated to justify a reduction may help, with appropriate financial reimbursement for the manufacturer at that later stage if appropriate

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

Yes **No** **X**

Comments

The College presumes this should read "rapid access to medicines and affordability".

The manufacturers may price up to the £20-30k per QALY threshold and little may change. Affordability is somewhat arbitrary and is relative to other potential expenditure on health and, indeed, other public services. There is too much uncertainty in the proposals to predict with any certainty that this scheme offers rapid access and affordability.

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

Comments

This should be the default position where the evidence base has changed and this should also determine the frequency of review.

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

Comments

The priority given under the Cancer Drugs Fund is contentious. Drugs in other therapeutic categories are equally important, perhaps more so, as implied in the substance of this consultation. Cancer is given priority for emotional and politically expedient reasons, but if issues described here, such as clinical and cost-effectiveness, burden of illness, unmet need and societal opinions are taken into account then, for example, rheumatology, psychiatry and ophthalmology may turn out to have higher priority. In the meantime, cancer drugs should be treated like any others and subjected to the same scrutiny.

- **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?**

Answer: **In part.**

Comments

Clearly that is the hope. It seems likely that this approach will deliver improved patient outcomes and support innovation, but much less clear whether it will deliver better value for money and more transparent assessments.

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

Comments

How will the system avoid "price inflation" of new drugs to maximise returns to the manufacturer (eg threshold pricing)?

How will the system avoid postcode prescribing through autonomy of the new GP consortia?

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

Comments

There is a risk that the introduction of this new approach will discourage industry engagement, and that some manufacturers will move their R&D facilities from the UK. Rewards will go to those companies that create valuable treatments in areas of unmet need – it is unclear whether this will be reinvested in the UK for public benefit.

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

Comments

Nothing to add.

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

Nothing to add.

Before submitting your response to the Department, please make sure that it has been saved in a name that will make it easier for us to track. Many thanks.