



National Voices

People shaping health
and social care

National Voices

NATIONAL VOICES' RESPONSE TO THE CONSULTATION:

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

March 2011

About National Voices

National Voices is the coalition of national health and social care organisations that ensures a strong voice for all those who come into contact with the NHS and care services, and for the voluntary organisations that work for them. Our broad membership, rooted in people's experience, represents millions of people, and covers a diverse range of conditions and communities.

Summary

Some National Voices members can see potential advantages from a future system of value based pricing for medicines. However, they also express the following concerns about the proposals:

1. There is no detail on how the new system of weightings would work, which makes it impossible at this stage to judge the likely impacts, or to form conclusions about the worth of the government's proposals.
2. There is no reference anywhere in the consultation to the involvement of patients, patient organisations and the public, either in designing the new system or in implementing it.
3. While it is possible that access to effective therapies may be increased by the new system, there are also risks to equity. National Voices members do not want a new form of postcode lottery for access to new medicines.
4. The future role of NICE is not clear.

National Voices notes that, while the high level objectives contained in the consultation document are admirable, it is inherently difficult to serve multiple policy objectives through a single set of measures (for example, incentivising the pharmaceutical industry and increasing the benefits to patients). This is particularly the case with price sensitivities.

With regard to the proposed new weightings to be used in the evaluation of new drug treatments, National Voices believes some of these may, in due course, prove to have merit. However, we question the assumption that it is possible to make *ex ante* calculations of the future benefits of a specific medicine in relation to, for example, the 'burden of illness'. It is not clear that such calculations could take account of the differential effectiveness of a medicine for different individual cases; nor predict patients' preferences as they consider the risks of all available treatments and make decisions in partnership with their clinicians.

We note the absence of any consultation question on the issue of ‘wider societal benefits’, which is the least developed and the most concerning of the proposals for new value weightings.

Given the lack of detail on the new weightings, the impossibility of assessing their impact, and the lack of recognition for the role of patients and the public in technology appraisal, National Voices suggests that the government should not bind itself to a target date for the implementation of these proposals.

We suggest that the next step forward should be for the Department of Health to map out a process of further policy development which includes commissioning robust, independent, exploratory research into how value based pricing might be further developed, how the measures might work in practice, and what the likely effects would be – on pharmaceutical company behaviour, on outcomes for patients, and on NHS commissioning and prescribing.

Integral to this policy process and the research programme should be the involvement of people and organisations who can represent the views and experiences of relevant patients.

CONSULTATION QUESTIONS

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

The high level objectives contained in the consultation document are admirable. However, National Voices notes the inherent difficulty in trying to serve multiple policy objectives through a single set of measures (for example, incentivising the pharmaceutical industry and increasing the benefits to patients). This is particularly the case with price sensitivities.

The consultation document, in our view, contains greater clarity on how the industry incentives would be altered, than it does on how the outcomes for patients would be improved.

Q: 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?

Q: 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?

It is not possible, based on the proposals contained in this consultation, to assess the impact of value-based pricing on the valuation of, and access to, drug therapies – whether these are existing or new medicines.

National Voices therefore suggests that the government does not set an implementation date for these proposals, but rather maps out a policy development process to test the ground further.

Q: 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?

The first new weighting which the government proposes could lead to a higher cost threshold for a new treatment is ‘burden of illness’.

The consultation document states that: “The most important factors contributing to the measurement of “Burden of Illness” would be the severity of the condition and the level of unmet need.”

Some of National Voices’ comments on this question will also be common to the other new weightings that are proposed.

First, throughout this consultation document there is a very mechanistic approach to evaluating treatments. There is an assumption that:

- a. a new treatment is proven to have potential benefits ‘x’ for patients ‘y’
- b. the treatment is therefore relevant and appropriate for all patients ‘y’
- c. clinicians will commission and prescribe the treatment to these patients ‘y’
- d. on the basis of a headcount of patients ‘y’ it is therefore feasible and appropriate to make value assessments of the treatment (based on multiple courses of treatment ‘z’)
- e. the figures derived can then be ‘weighted’ for the potential quantity of illness dealt with in the population as a whole.

This is not how clinical applications work, and nor should it be how patient decision-making works.

Especially in cases of severe or life-threatening illness, which is the stated focus for this proposal, the appropriateness of a treatment for an individual patient is dependent on many factors (not only the clinical indications).

Some patients may wish to reduce the severity of certain symptoms, or to attempt to prolong life, leading to a decision to prescribe a new treatment. Others may not wish to manage their illness that way. Patients will wish to balance the risks of the new treatment – its potential benefits and harms – against their circumstances, values and preferences. For example, a particular side effect may be an acceptable risk to one patient but not to another.

The very welcome emphasis in the government’s health reforms on prioritising shared decisions about treatments – and the relevant rights in the NHS Constitution – should help to ensure that many more patients are enabled to participate in making these choices.

This means it is now, and will increasingly be, dangerous to try to estimate beforehand the ‘burden of illness’ that a particular new treatment might alleviate.

Second, as with the other areas of new weighting, there is no explanation here of:

- who will set the criteria or benchmarks by which the ‘burden of illness’ will be assessed
- what these are likely to be
- how or by whom the appraisal of a treatment for the purpose of this weighting will be carried out
- whether or not there will be any involvement of patients, the public, or patient groups.

Third, we the proposal to calculate impact on ‘unmet need’ raises more questions than it answers.

Who will identify where there is ‘no effective treatment’? Will this include illnesses for which there are some treatments, but their effectiveness could be improved upon? If so, to what degree must new treatments offer improvement, if they are to be weighted towards a higher cost threshold? Will these calculations actually work against ‘incremental improvements’ for these illnesses – which may be strongly welcomed by patients, but which, in general, this consultation seeks to disincentivise?

These unanswered questions imply that calculations in this area would need to be very sensitively nuanced. However, the consultation document implies that the government does not want to make calculations complex. In the same section, it notes that “Unmet need could be calculated for different indications for the same medicine... but it would be a far more complex system and there could be significant practical issues in implementing such an approach”.

Q: 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?

As a high-level principle, it is possible to support this proposal. Again, however, many questions arise when considering any implementation.

This type of value weighting, like the ‘burden of illness’ proposal above, is subject to our concerns about calculating beforehand the value to patients of a treatment which, in the context of shared decision-making, they may in fact choose not to access.

It is also subject to our set of questions about the lack of explanation of the ‘who, how and what’ in relation to how the criteria for these judgements are set, the appraisals carried out, and the weightings applied.

A third issue relates to the point made above about illnesses for which there is currently ‘no effective treatment’. Would patients want to see the NHS pay less for new treatments that may offer incremental improvements for these illnesses?

A fourth question that arises is: what is the risk of policy failure in this area? To what extent would an NHS valuation, and an increased cost threshold, be influential on the long term research, development and marketing agendas of multinational pharmaceutical industries? In order to justify the policy, answers to that question would need to be properly researched.

Q: 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?

There are fundamental issues in relation to the evidence available for value based pricing.

First, under these proposals, the source of the evidence for assessment would be the pharmaceutical industry itself:

“If, however, the manufacturer considered that a higher price was warranted, they would need to provide robust evidence demonstrating that the new medicine merited a higher

weighting in terms of burden of illness, therapeutic innovation and improvement, or clinical and wider societal benefits.”

Industry will have a natural incentive to overclaim the benefits of its new products. Also, it is not obvious that the industry is the best placed actor to provide evidence of, for example, ‘wider societal benefits’.

It is therefore worrying that there is no mention of mechanisms to ensure there is rigorous external/independent appraisal of the evidence for these higher weightings.

The body best positioned to develop a system for providing external/independent appraisals would be NICE. However, the consultation is highly equivocal about whether NICE would be involved in this part of the appraisal:

“NICE will be the key source of advice on the relative cost-effectiveness of new medicines, although in future this will be combined with other aspects of value before a reimbursement price is determined. Given the expertise of NICE, they will play an important role in any new system, but the details of their role will depend on, amongst other things, the responses to this consultation.”

Second, there are fundamental problems with the concept of making *ex ante* evaluations of the additional benefits new treatments will offer, in relation to the burden of illness and to wider societal benefits. As we have noted in previous answers, the extent to which treatments are actually commissioned, and are chosen by patients in partnership with their clinicians, is by no means easily foreseeable. Also, the effects and benefits of treatments may appear somewhat different once they begin to be used by numbers of patients in real life situations -- outside the context of clinical trials.

Therefore, there must be a very large extent to which any system based on *ex ante* assessment of potential future benefit would be making only provisional judgements. This creates a risk that, for many medicines, there will need to be periodic reviews, creating a further burden of cost and process.

Q: 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?

It is currently impossible to answer this question.

The Department of Health itself cannot answer the question. The Impact Assessment issued alongside the consultation states:

“The effects of the policy depend entirely on the system design, and the mechanism used to calculate value-based prices – neither of which are yet known.”

It further states:

“The magnitudes of costs and benefits depend entirely on the design of the system, and the weightings applied to different aspects of new treatments. As these details are not yet determined, the impacts of the policy have not been quantified.”

National Voices accepts that this is an early sketch of the policy, and welcomes the opportunity to be consulted at such an early stage.

However, given the sketchiness of the proposals, the impossibility of assessing their impacts, and the absence of any detail on how they would work in practice, it does not seem advisable to work to a definite implementation timetable as the government has proposed.

We would suggest that the government should not set a target date for implementation until much more work has been done, to develop the policy detail, to research the means by which it might be given effect, and to assess the likely consequent impacts on patients and the NHS.

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

1. 'Wider societal benefits'

National Voices notes that, while there are consultation questions on the 'burden of illness' and 'innovation' proposals for new value weightings, there is no equivalent question for the 'wider societal benefits' proposal.

Let us imagine that there had been such a question. We would respond that this is the least clear of the proposals, and has triggered a number of concerns among National Voices members. For example, there is a strong concern that if the additional 'benefit' of enabling sick people to become economically active again was used as an incentive, this might create disinvestment from treatments targeted more at children and young people or at older, non-working adults.

This proposed 'weighting' would particularly be subject to our previously expressed concerns about the 'who, how and what' of determining the additional value.

2. Role of patients, patient organisations and the public

We ask the government to note the absence from the consultation of any reference to the future involvement of patients, the public and patient organisations.

This has been a source of concern to all of the member organisations who have shared their views with National Voices.

Currently, NICE has a well developed system for patient and public involvement, both through the involvement of lay people in its Citizens' Council, and through the involvement of patients and patient groups in its technology appraisals.

We would wish to see an equivalent, sophisticated system of patient and public involvement in any new arrangements for implementing value based pricing. Patients, the public and patient organisations should be involved in contributing to:

- the development of approaches to value based pricing
- the development of criteria and weightings that might lead to higher cost thresholds
- future appraisals of specific new medicines for the purposes of assessing their 'additional benefits'.

3. Role of NICE

As noted above, the consultation is equivocal about the future role of NICE in determining the value to the NHS of new medicines. National Voices believes that NICE is in fact the best-placed organisation to help drive the development of new pricing policy. In part this is because of its existing expertise, and in part because of its well developed approach to the involvement of patients, patient organisations and the public in determining its ethical policies and in contributing to technology appraisals.

As a related issue, elsewhere in the government's reforms to the NHS it appears that NICE guidance is no longer to be mandatory for the NHS as a whole. Even where National Voices members can see the potential benefit to patients of, for example, the incentivisation of innovation in medicines, they would not want a new postcode lottery to develop, in which patients in some areas experience greater access to new treatments, but those in other areas do not.

4. Next steps

National Voices proposes that the government does not bind itself to a definite target date for the implementation of this new policy. Rather, it should map out a policy development process to which stakeholders may contribute. This should include commissioning a programme of robust, independent, exploratory research into the means by which value based pricing could be developed; and its likely consequences in relation to pharmaceutical company behaviour, benefits to patients, and impact on NHS commissioning and prescribing.

Integral to this policy process and to the research programme should be the involvement of people and organisations who can represent the views and experiences of relevant patients.

Our preference would be for NICE to play a significant role in driving this research.