

## **SPECIALISED HEALTHCARE ALLIANCE RESPONSE TO A NEW VALUE-BASED APPROACH TO THE PRICING OF BRANDED MEDICINES**

The Specialised Healthcare Alliance is a coalition of 61 patient-related organisations supported by nine corporate members, which campaigns on behalf of people with rare and complex conditions requiring specialised care. Examples are numerous but include certain cancers, cystic fibrosis, haemophilia, neurological conditions and a wide range of services for children. Accidents or complications of more common conditions can also trigger the need for specialised services such as burns, pain management and spinal injuries.

Over the last three years, the Alliance has taken a particularly keen interest in how the ethical dimension of assessing treatments should be incorporated in decision-making, bearing in mind that per capita costs for smaller patient populations may well be high. This response largely draws on that work.

**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?**

It is hard to quarrel with the overarching objectives of the new approach but delivering them in practice will be challenging and, in some cases, beyond the scope of pricing. Innovation is already stimulated in the field of severe conditions affecting fewer than five people in 10,000 through the EU Orphan Drugs Regulation. To be effective, such signals need to be delivered across larger markets than the UK alone. The problem is aligning the positive results of the EU regime with market access at national level.

**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?**

In theory, value-based pricing should be able to accommodate medicines for all patient populations. In practice, this may be difficult to achieve as costs for treating rare conditions tend to be higher and the evidence base thinner. It would therefore seem sensible to keep in place the arrangements developed for the Advisory Group on National Specialised Services (AGNSS) in the short to medium term ie for ultra-orphan patient populations of less than 500. There is a need for greater clarity about which evaluation pathway is appropriate for medicines taking patient numbers and other factors into account. The decision-making framework developed for AGNSS provides a useful starting point for value-based pricing, especially in the wider field of orphan medicines.

**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?**

**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?**

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

The Alliance attaches great importance to medicines and other forms of treatment for unmet need. Innovations which address severe unmet need would normally expect to command the highest value, influenced by the number needed to treat. A holistic approach is, however, required taking into account a wider range of considerations. For example, greater value could be attached to broadly the same product if it can be injected by the patient at home rather than infused in a hospital or injected less frequently and so on.

It is debatable whether price signals can be given to incentivise the development of innovative medicines but manufacturers should have confidence that innovative medicines and other products will be able to command a premium should they be successful in obtaining a licence.

**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?**

**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.**

The Alliance is concerned that a value-based pricing system should not be overly focused on misleading numerical exactitude. The QALY is a useful mechanism for assessing value but encompasses many debatable assumptions. The Alliance therefore strongly favours a multi-criteria decision assessment method of the kind developed for AGNSS, recognising that judgement will be required in weighing the different elements to determine the extent to which a company's list price represents fair value.

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

**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 AGNSS framework is broken down into four clusters covering health gain, societal value (including innovation), reasonable cost and best practice, with the patient's need at the heart of the process. This provides a good basis for exploring the burden of illness and therapeutic benefit in the NHS more

generally. The new system will only work if the evidence required can reasonably be expected at launch in most cases. There will, however, be medicines, especially for rare conditions, where evidence of cost effectiveness is more difficult to assimilate. A properly constituted assessment panel should be asked to exercise its judgement in such cases, applying a transparent process against accepted criteria.

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

**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?**

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

There is insufficient detail in the document to allow answers to these questions. The Department needs to be clear as to its objectives and to be honest about the tensions inherent in a system which seeks to obtain the best value for the taxpayer, the best outcomes for patients and the maintenance of a strong, research-led industry in the UK. There might be merit in running a value-based pricing pilot for orphan medicines drawing on the AGNSS framework.

**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?**

From a patient perspective, the greatest risk is that the way in which the scheme is calibrated between its competing priorities will lead to very slow decision-making and delayed access to new medicines or the rejection of medicines typically available in other comparable health systems. It will also be important that a value-based price, once agreed, is applicable across the NHS to avoid postcode problems arising from subsequent negotiations at local level.

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

The suitability of the scheme should be measured by its ability to reduce inequalities within the UK and between the UK and other comparator countries.

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

In considering the affordability of medicines, it is important to look at the nature of expenditure, past and prospective. This grew strongly in primary care fuelled by major blockbusters but is now tending to fall back as patents expire. Expenditure in secondary care is rising more strongly, though from a lower base. The net effect is perhaps less explosive growth than frequently reported, though the value of medicines relative to the majority of NHS expenditure still needs to be carefully weighed.