



A new value-based approach to the pricing of branded medicines: A response from Rare Disease UK

About Rare Disease UK

It is estimated that 1 in 17 people will be affected by a rare disease¹ at some stage in their lives. This amounts to 3.5 million people across the UK. Collectively, rare diseases are not rare.

Rare Disease UK (RDUK) is the national alliance of people with rare diseases and all who support them. We have over 800 members including over 140 patient organisations, clinicians, healthcare professionals, professional bodies, researchers, academics, industry and individuals with an interest in rare diseases.

RDUK is an initiative of Genetic Alliance UK, the national charity of over 150 patient organisations supporting all those affected by genetic conditions, in conjunction with other key stakeholders.

RDUK is supported by an unrestricted educational grant from the Association of the British Pharmaceutical Industry's (ABPI) Orphan Diseases Industry Group and the Orphan Diseases Industry Group Partnership representing companies outside of the ABPI.

RDUK aims to work with policy makers and the NHS to inform and aid the development and implementation of an effective strategy for rare diseases in the UK in accordance with the Council of the European Union's Recommendation on an action in the field of rare diseases. The Recommendation which calls for the development of plans or strategies for rare diseases by 2013 was adopted unanimously by each of the EU's Member States in June 2009.

A strategy for rare diseases should coordinate:

- Research
- Prevention, diagnosis and screening
- Commissioning and planning
- Access to treatment
- Multidisciplinary Care
- Access to information and support

As well as securing better outcomes for patients, a strategy for rare diseases would enable the most effective use of NHS resources.

¹ A rare disease is defined as any disease affecting fewer than 5 in 10,000 of the general population

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An initiative of



Genetic Alliance UK
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A charity registered in England and Wales (no. 1114195)
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RDUK published a report outlining comprehensive recommendations for a strategy for rare diseases in February 2011. The report, *Improving Lives, Optimising Resources: A Vision for the UK Rare Disease Strategy* is available here: www.raredisease.org.uk/documents/RD-UK-Strategy-Report.pdf

RDUK welcomes the opportunity to respond to this consultation.

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Question 1: Are the objectives for the pricing mechanisms 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?

RDUK welcomes the objectives behind the value-based pricing approach. Rare diseases represent an area of significant unmet need; for the majority of the over 6000 known rare diseases there is no effective treatment or therapy. As a result RDUK supports initiatives to stimulate innovation and the development of high value treatments for rare diseases.

RDUK's report *Improving Lives, Optimising Resources* found that patients with rare diseases are being denied access to orphan medicines that have been granted European marketing authorisation. Many orphan medicines are not evaluated through the NICE health technology assessment (HTA) process and the few that are appraised are often rejected on the basis of their high estimated cost per quality-adjusted life year (QALY). In the absence of NICE guidance, decisions on whether or not to fund treatments are often made by PCT individual funding request (IFR) panels, groups with little experience of the specific issues surrounding the appraisal of orphan medicines. Not only does this lead to duplication of effort, with over 150 bodies making funding decisions on the same medicines, it also inevitably leads to inconsistent outcomes. This in turn leads to inequity of access to the medicines being considered. Some medicines for very rare diseases are available as part of a nationally commissioned service and the Advisory Group for National Specialised Services (AGNSS) now has a remit to consider a limited number of medicines. This only applies to diseases affecting fewer than 500 patients in England. RDUK supports efforts to facilitate better, more equitable access to effective medicines which improve patient outcomes and to improve the process for assessing new medicines for rare diseases. This includes consideration of a wider range of benefits to the patient alongside clinical effectiveness.

RDUK strongly supports the efficient use of NHS resources. A strategy for rare diseases would ensure the optimal use of NHS resources whilst ensuring better outcomes for patients. Patients with rare diseases already make heavy demands on NHS resources, however, services are too frequently inefficient or poorly coordinated, and patients struggle to access the care, support and treatments they need. Not only does this lead to a reduction in patients' quality of life and potentially their life expectancy, it also creates waste through delays in diagnosis, duplication of services, unnecessary interventions, repeat visits to hospital, increased emergency admissions due to poor management of the condition, etc.

Question 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?

In developing the model for value-based pricing, issues specific to orphan medicines should be taken into account during the design process. These include the difficulties in

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gathering data from small population groups, the absence of an appropriate approved comparator treatment, the recognition of the unmet need or significant benefit that orphan medicines bring and the small patient population for industry to recover investment in the development of a drug. The current HTA process has proven to be ineffective in capturing these unique characteristics; only three of the over 60 approved orphan medicines granted European marketing authorisation have been appraised and approved by NICE. As a result, consideration must be given to how particular considerations such as these can be built into mechanisms for assessing value.

A single model of assessing value should be the aim in order to minimise the need for parallel pricing mechanisms. If analysis shows that one pricing model would have an unjustifiably restrictive impact on the accessibility of orphan medicines, then separate arrangements should be considered so as not to replicate the current situation whereby appraisal processes cannot effectively consider orphan medicines. This is especially so if QALYs continue to be used as suggested in the consultation document.

AGNSS provides a positive example of an initiative to capture value in the context of medicines for rare diseases. AGNSS's Decision-Making Framework considers four domains: health gain, societal value, reasonable costs and good practice. This framework applies to services, products and technologies. This framework is currently undergoing a pilot study and the outcomes should be used to inform the development of the value-based pricing model. A caveat to this is that this framework is only used only for medicines to treat very rare diseases affecting 500 or fewer patients. A single rare disease could affect up to approximately 26,000 people in England (most rare diseases will affect far less than this amount). As a result there is currently a significant gulf currently between those medicines for less than 500 patients which could be eligible for AGNSS assessment and those medicines for between 500 and 26,000 patients which as described in question 1, frequently fall to IFR panels. A considered approach to value-based pricing has the opportunity to rectify this situation.

Question 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?

Little detail is provided in the consultation paper on what will be taken into account in measuring value. RDUK believes that unmet need and burden of illness are some of the important criteria for the measurement of a medicine's value. In principle it follows that the greater the value the medicine provides, the higher the price the healthcare system should pay for that medicine.

The value of medicine should not only be measured in relation to the disease itself, but also the value it can bring to healthcare system as a whole and to wider society. In this respect

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it is important to consider the cost of not being able to access a medicine when considering value.

Question 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?

RDUK supports the principle that a higher price should be paid for medicines that deliver a step-change in benefits to patients in comparison to those which do not deliver a step change. This is important to encourage the development of drugs that deliver added benefits to patients.

The concept of innovation is closely related to other aspects of value. Innovation *per se* is not necessarily a good thing; we do not believe it should be valued for its own sake. Innovation should be valued in these cases:

- where it addresses an outcome valued by society;
- where there is potential to build on the innovation to bring benefits in broader indications than its initial target;
- where a technology can bring benefits across a wider range of disease areas.

The need to offer incentives to develop medicines for rare diseases was recognised by the adoption of the EU's orphan medicinal product regulation which offers incentives to develop medicines for small patient populations. Without incentives, the pharmaceutical industry would be unlikely to invest in developing medicines due to the small patient population to recoup costs. Criteria to be designated orphan status include aspects of unmet need, disease severity and innovation.

Question 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.

As discussed previously, QALYs are unable to capture the full impact of rare diseases. We believe other options should be explored to determine value. The experience of the Scottish Medicines Consortium (SMC) demonstrates that even when applying “modifiers” such as whether the drug: treats a life threatening disease; substantially increases life expectancy and/or quality of life; can reverse, rather than stabilise, the condition; or bridges a gap to a “definitive” therapy, traditional HTA processes cannot respond adequately to rare diseases. As of May 2010 the SMC had appraised 46 orphan medicines – 18 had been recommended, 17 rejected and 11 had been recommended for limited use only.

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RDUK strongly supports involving patients to capture the value to patients and experts to assess value to the health service.

Question 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 issue described above is likely to be a particular issue for medicines for patients with rare diseases due to the problems of generating evidence from small patient populations. Patient access should not be unduly constrained whilst data is being generated. An interim pricing arrangement should be developed, coupled with an agreed development programme, in order to allow for the generation of data from clinical use.

Question 17 and Question 18: Are there other factors not mentioned in this document which the new system should take into account? 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.

RDUK is concerned that in the absence of a positive binding opinion from NICE there may be a reluctance at GP consortia level to purchase new medicines. This is an especially important issue for orphan medicines which by their very nature are likely to be high-cost, not only due to the small patient population, but also, because an orphan medicine is likely to be valued highly under a value-based pricing system as they are likely to satisfy many areas of value such as unmet need, disease severity and innovation. This could perpetuate the current “postcode lottery” of access to medicines and lead to a situation where only those patients who shout the loudest are able to access the most “valuable” treatments.

In our *Improving Lives, Optimising Resources* report, RDUK recommends that funding for medicines for rare diseases should be organised nationally from a central source in order for decisions to be made at the level where the best expertise is available. This would alleviate the potential problem of reluctance by GP consortia to fund these medicines.

There is little mention of patient input in determining value in the consultation. RDUK believes that this is a significant oversight. Patient testimonies need to be properly taken into account in the determination of the potential value of a medicine. Most patient organisations for rare diseases are small or entirely volunteer-led; this should not preclude their involvement and measures should be put into place to facilitate patient input.

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

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In order to ensure that value-based pricing has a positive impact on equalities, the issue of access needs to be examined alongside consideration of determining value. As described previously, patients with rare diseases face unequal access to medicines across England.

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