



RARE DISEASE | UK

The National Alliance for people with rare diseases & all who support them

## All Wales Policy: Making Decisions on Individual Patient Funding Requests (IPFR) - A response from Rare Disease UK

### About us:

It is estimated that 1 in 17 people will be affected by a rare disease<sup>1</sup> at some stage in their lives. This amounts to 175,000 people in Wales. Collectively, rare diseases are not rare.

Rare Disease UK (RDUK) is the UK alliance of people with rare diseases and all who support them. We have over 900 members including over 170 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 140 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 Medicines Industry Group (OMIG) and the Orphan Medicines Industry Group Partnership representing companies outside of the ABPI.

RDUK aims to work with policy makers and NHS Wales to inform and aid the development and implementation of an effective strategy for rare diseases 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; planning of services; 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 Wales resources.

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](http://www.raredisease.org.uk/documents/RD-UK-Strategy-Report.pdf) The recommendations in this report will be referred to in this response.

### Rare Disease UK – August 2011

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<sup>1</sup> A rare disease is defined as any disease affecting less than 5 in 10,000 of the general population

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## Overview:

Rare Disease UK welcomes the aims of the policy to introduce a robust, transparent and fair decision-making process to consider requests for individual patient funding. We believe that it is an important step in ensuring equitable access to services, treatment, medicines, devices and equipment in Wales.

However, we remain concerned about how the policy will ensure equitable access to beneficial interventions for all patients affected by rare diseases in Wales. Some of our main concerns, or areas we would like clarification on, are discussed below.

## Key points:

### How the guidance is applied universally

Although an all-Wales policy on IPFR will go some way to improving consistency and transparency around decision-making, we are concerned that there remains scope for health boards to interpret the guidance in different ways leading to inconsistent and inequitable decision-making. To ensure that the guidelines are being applied consistently and robustly we propose the annual review of the policy, as stated in s.8.0, is extended to include a review of practice and outcomes. The outcomes of this review should be made publically available.

### Exceptionality

We are concerned that the guidance is framed in a way which places the onus on a patient with a rare disease to prove that they are exceptional. The guidance states that it will be determined that a patient receives funding through an IPFR "either because they meet agreed policy criteria (where it is available) or on the grounds of exceptionality". We are concerned that it may be difficult to prove that a patient affected by a rare disease is exceptional in comparison to other patients affected by that condition. Exceptionality may be clearer when dealing with a patient with particular characteristics among a wide group of patients where the condition is well-understood, but when dealing with a rare disease where there may be limited knowledge of that condition, trying to prove exceptionality can become an insurmountable obstacle. The exceptionality criteria should be modified for applications relating to rare conditions to take this into account.

### Evidence-based considerations

We welcome the acknowledgement that in certain circumstances decisions must be made in areas where the level of evidence is limited and that "there is not always an automatic "right" answer that can be scientifically reached" meaning consideration must be given to different value judgements as well as current scientific knowledge. Decisions will be made under these circumstances for most rare diseases due to the difficulty of collecting sufficient data on the effectiveness of interventions in small patient populations.

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Whilst guidance issued by NICE and the AWMSG is an important body of evidence to take into consideration, current health technology assessment (HTA) processes and the cost effectiveness thresholds that are applied as part of them may not always be appropriate for evaluating orphan medicines. Moreover, many orphan medicines are not evaluated by NICE through the 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). Whilst we recognise the AWMSG has a policy taking into account a wider range of considerations for ultra-orphan drugs (those designed to treat diseases with a prevalence of less than 1 in 50,000), this issue remains when making decisions on funding requests for orphan medicines to treat diseases with a prevalence of less than 5 in 10,000.

Due to the inappropriateness of relying on standard HTA evidence it is essential that a broader range of evidence is taken into account when considering IPFR in relation to rare diseases. This should include evidence from specialists in that particular rare disease or group of rare diseases (who may be located outside of Wales), patient testimonies and information from patient support groups (where they exist).

### Economic considerations

In s.5.2 it states that the “opportunity cost” will be considered under “ethical considerations”. “Opportunity cost” is an ambiguous term and care must be taken to avoid oversimplified calculations which do not take into account factors such as health gain, burden of disease and the impact to the NHS. Crude opportunity cost calculations inevitably work against expensive interventions (whether for rare diseases or not) which may nevertheless be highly beneficial to the patient and health service as a whole. In considering the opportunity cost, it is imperative that the cost of not treating a patient to health and social services is taken into account.

Further clarification is needed around whether a health board can “afford a request” as it is unclear whether this is in relation to a specific budget, or the health service as a whole. If a health board decides it cannot afford a request it should be explained fully why this is the case.

### Ethical considerations

We fully support the need to ensure a balance between the needs of an individual and the needs of the wider community. However, it is also important to ensure that patients with rare diseases are not prejudiced against on the account of the rarity of their condition. Patients affected by rare diseases already face many additional problems including a lack of information and awareness about their condition, fewer sources of support, less research being conducted into the condition to offer hope for future breakthroughs, and fewer treatment options and guidelines on how best to manage the condition. When potentially effective treatments become available it is unethical to unduly restrict access to these treatments.

The values and principles set out under s.5.3 are important considerations and we again welcome the recognition of the need to balance scientific and value-based judgement. However, RDUK would like to see a stronger emphasis on the effects of a positive or negative funding opinion on an individual as this is a vital ethical consideration. These should include (but not be limited to) the impact on an individual's

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health, quality of life, effect on family life and the psychological and social consequences for the patient and their family.

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