



RARE DISEASE | UK

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

Good practice in prescribing and managing medicines and devices: 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 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 and the Orphan Medicines 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.

RDUK welcomes the opportunity to respond to this consultation.

Q. 21 – Do you agree with the draft guidance at paragraph 58 that doctors can prescribe off-label or unlicensed medicines if satisfied, on the basis of authoritative clinical guidance, that it is as safe and effective as an appropriately licensed alternative?

Rare Disease UK supports the importance of licensing of medicines and as a result we are concerned that the wording of the proposed guidance places other considerations above patient safety. In practice many people affected by a rare disease will use an off-label or unlicensed medicine as, for example:

- A large proportion of rare diseases affect children and as the consultation document recognises, many medicines are not licensed for use in the treatment of children.
- Most rare diseases do not have a medicine licensed for that particular condition.
- There are difficulties in obtaining the necessary level of evidence to secure a license for treatments for diseases with small patient populations.

Rare Disease UK

Unit 4D, Leroy House, 436 Essex Road,
London, N1 3QP

www.raredisease.org.uk

T: +44 (0)20 7704 3141 | F: +44 (0)20 7359 1447

E: info@raredisease.org.uk

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in Scotland (no. SC039299).

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Nevertheless, licensing is an important assurance that a medicine meets rigorous safety, quality and efficacy standards. We are concerned that by implying that it is acceptable for doctors to prescribe off-label or unlicensed medicines above a licensed alternative on the grounds of cost, that patient interest and safety is not being prioritised and there is a risk of undermining the purpose of the regulatory process. At a time where the NHS faces considerable financial challenges and with doctors set to take greater control over commissioning decisions, this sends an inappropriate message regarding the prioritisation of patients' interests against cost saving.

RDUK supports the retention of the 2008 guidance which states that 'when prescribing a medicine for use outside the terms of its license, you must... be satisfied that it would better serve the patient's needs than an appropriate licensed alternative'. This version of the guidance rightly prioritises patient's best interests over other considerations.

We are aware that in the field of rare diseases, there have been some high-profile examples whereby there has been a considerable increase in price in a drug when a company has licensed a slightly modified version of a previously unlicensed drug. Whilst we are concerned at these situations, we do not feel that prescribing guidance is the right forum to address these issues.

Q.22 Do you agree with the guidance at paragraph 60 that it may not be necessary to draw patients' attention to the licensing status of medicines routinely used off-label and for which there is authoritative clinical guidance?

Rare Disease UK does not agree with this section of the guidance. Patients should be given all the information relevant to their treatment in order to be able to make informed decisions. Whilst the receipt of this information may not change a patient's decision on whether to consent to treatment or not, this doesn't remove the importance of providing that information in the first place.

The stance taken here seems inconsistent with the guidance on 'Consent to prescribe' as well as with current NHS policy of "no decision about me, without me".

Q.23 Do you have any other comments on the *Prescribing off-label and unlicensed medicines* section?

A framework is necessary for the evaluation of off label use of medicines. Some patients affected by rare diseases do benefit from off label medicines, however, access is inconsistent as some doctors are more willing than others to prescribe these medicines. At present, there is little or no incentive for doctors to take an informed risk as evidence about the effectiveness of off label use is not captured in a systematic way.

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