The Scottish Parliament
Pàrlamaid na h-Alba

PUBLIC PETITION NO.	PE01398

Name of petitioner

Alastair Kent on behalf of Rare Disease UK

Petition title

Access to therapy for orphan diseases

Petition summary

Calling on the Scottish Parliament to urge the Scottish Government to review the mechanism and methodology used by the Scottish Medicines Consortium to appraise the value of medicines for orphan diseases and to instruct the Chief Medical Officer to revise the criteria for accessing Individual Patient Treatment Requests by removing the term ‘exceptional’ from all health boards IPTR requests in relation to orphan diseases.

Action taken to resolve issues of concern before submitting the petition

Rare Disease UK (www.raredisease.org.uk/) is the national alliance for people with rare diseases and all who support them. RDUK was established by Genetic Alliance UK, the national charity of over 140 patient organisations supporting all those affected by genetic conditions, in conjunction with other key stakeholders in November 2008 following the European Commission’s Communication on Rare Diseases: Europe’s Challenges.

Subsequently RDUK successfully campaigned for the adoption of the Council of the European Union’s Recommendation on an action in the field of rare diseases. The Recommendation was adopted unanimously by each Member State of the EU (including the UK) in June 2009. The Recommendation calls on Member States to adopt plans or strategies for rare disease by 2013.

A report was developed by Rare Disease UK (RDUK) called ‘Improving Lives, Optimising Resources: A Vision for the UK Rare Disease Strategy’. The report contains a section on ‘Access to orphan medicines’ in which it states ‘UK patients with rare diseases are being denied access to orphan medicines that have been granted European marketing authorisation.’ The Recommendation is that ‘There is an urgent need to reassess the mechanism and methodology by which the value of medicines for rare conditions is appraised for reimbursement on the NHS, to ensure improved and equitable access to licensed medicines from which patients will benefit.’

www.raredisease.org.uk/documents/RD-UK-Strategy-Report.pdf

RDUK organised a parliamentary reception hosted by Jackie Baillie MSP on 22 February 2011 at which Mr Will Scott – as Head of the Long Term Conditions Unit in the Scottish Government Health Directorates - accepted the Report ‘Improving Lives, Optimising Resources: A Vision for the UK Rare Disease Strategy’ on behalf of the Scottish Government.

RDUK has been made aware of a number of recent IPTR’s for treatment of patients with

rare diseases, who have been refused funding despite the treatments being available as part of nationally commissioned services in England, or have been accepted by different health boards in Scotland.

www.specialisedservices.nhs.uk/info/agnss

RDUK hosted an event in conjunction with the Scottish Medicines Consortium on 23 June 2011. The aims of the event were:

• To build a dialogue between different stakeholders from the rare disease community and the Scottish Medicines Consortium.
• To gain clarity about the assessment process for access to medicines for rare diseases.
• To explore how we can proceed in a constructive partnership with the Scottish Medicines Consortium.

Speakers from the Scottish Medicines Consortium Executive Board were invited to present at the meeting to explain the Scottish Medicines Consortium's structure and decision making process. Following the presentations, attendees were asked to join in discussion in relation to the aims listed above. Representatives from different stakeholder groups, with an interest in rare diseases and the Scottish Medicines Consortium attended the event and were informed of processes used to appraise the value of medicines for rare diseases. Rare Disease UK remains concerned that the appraisal process does not adequately capture the unique nature of rare diseases and the problems developing medicines for rare diseases.

In addition, the following Parliamentary Questions have been asked and answered in relation to rare diseases and access to orphan medicines:

- S3W-40250 - Richard Simpson:
- S3W-39573 - Nanette Milne:
- S3W-40248 - Richard Simpson:
- S3W-40247 - Richard Simpson:
- S3W-38010 - Richard Simpson:

Petition background information

There are four distinct areas which are brought to attention of the Committee:

1. A National Plan for Rare Diseases

The Council of the European Union's Recommendation on an action in the field of rare diseases was adopted unanimously by each Member State of the EU in June 2009. The Recommendation calls on Member States to adopt plans or strategies for rare disease by 2013.

• RDUK calls upon the Committee to ask the Scottish Government what plans it has in place for the implementation of a National Plan for Rare Diseases in Scotland.
• RDUK calls upon the Committee to ask the Scottish Government what plans it has to allow a budget so that all patients can access therapy, if available, for rare diseases.

2. The current IPTR process is too onerous for orphan medicines

RDUK would like to commend the extensive work undertaken by the previous Public Petitions Committee in consideration of petition PE1108 which led, directly, to revised guidelines being issued by the Scottish Government on the 'end to end' process from licensing of medicines through to individual patient treatment requests (what was known as 'exceptional prescribing'). However, this has not improved access to orphan medicines for patients with a rare disease.
www.scottishmedicines.org.uk/files/CEL2010_17.pdf

Although the Scottish Government may no longer be referring to the terminology of 'exceptionality' within the IPTR process, the clinical case made by the requesting physician still relies on the principle that the patient is in some way 'exceptional' from the general population where the drug is used. The criteria that must be met as part of

this process are proving to be a particular challenge for patients with rare diseases. The CMO letter to the NHS Boards on 18 March 2011 states:

“The patient’s clinical circumstances (condition and characteristics) are significantly different from either:

• the general population of patients covered by the medicine’s license; or
the population of patients included in the clinical trials for the medicine’s licensed indication as appraised.

There is also evidence that the terminology “Exceptionality” is still applied within the IPTR process at a Health Board level. The Non-Formulary process from GG&C HB is very clear that the requesting clinician is responsible for “demonstrating exceptionality” for their patient. http://www.ggcformulary.scot.nhs.uk/Medicines%20Policies/Medicines%20Policies%20Home.htm

In rare diseases it is extremely difficult to demonstrate the above criteria. The small patient numbers who make up the clinical trial populations are those patients with the greatest clinical need for the drug and therefore the license will be based on this group of patients. It is therefore extremely difficult to show that a patient with genuine clinical need will be “More likely to benefit from the medicine than might be expected for other patients with the condition”. The patients who are likely to have the greatest need for the treatment will be the same as those patients within the clinical trials upon whom the license is based. Unlike in some of the more common conditions and even certain cancers where there is often more than one licensed treatment available, in the majority of rare diseases there is likely to be only one licensed treatment available, apart from just supportive care. In orphan diseases the above criteria are therefore more likely to lead to those patients with the greatest clinical need being refused access to therapies, which may be life changing and / or life saving.

This has been observed in several recent cases since the publication of CEL 17, whereby GG&C NHS Board and Ayrshire & Arran NHS Board have refused to fund therapy in patients with rare diseases. RDUK are aware of five cases in Scotland where patients have failed to meet the clinical circumstances highlighted in the CMO letter, despite very strong endorsements for treatment by UK opinion leaders.

• RDUK calls upon the Committee to ask the Scottish Government to review the CMO letter to the 14 NHS Boards dated 18 March 2011 which aims to provide guidance to the NHS Boards in relation to the implementation of their Individual Patient Treatment Request (IPTR) process regarding orphan medicines.

3. Assessment of Orphan Medicines

A recommendation from the report; ‘Improving Lives, Optimising Resources: A Vision for the UK Rare Disease Strategy’ is that ‘Evaluation [for orphan medicines] should be based on an appraisal of the technology against multiple criteria and not simply a cost utility analysis. A recent positive example of this approach is the decision making framework developed by the AGNSS in England. This framework involves a consideration of ‘value’ in four domains: health gain, societal value, reasonable costs and good practice. This mechanism will be reserved for medicines that are used to treat 500 or fewer patients in England. By contrast, the SMC appraises all new medicines coming to market in Scotland. As of May 2010 the SMC had appraised 46 orphan medicines, recommending 18, rejecting 17 and recommending the restricted use only of a further 11. This situation has come about despite the addition of modifiers to the SMC process designed to give special consideration to treatments for rare disease and terminal illness.’

The SMC has stated that a medicine has to be very special to be recommended if the cost per QALY > £30,000, which is the case with the majority of innovative products treating very few patients.

• RDUK calls upon the Committee to ask the Scottish Government to use expertise developed by AGNSS to explore how drugs for very rare diseases can be more appropriately assessed.

4. Orphan Drugs Risk Share Scheme

The ODRSA in Scotland is operated by the NSD. From their 2009 /10 Annual report (www.nssannualreport.scot.nhs.uk/nsd/orphandrugs10.html) it shows that the spend for the financial year for the risk share was £5.21 million against a budget of £5.2M. The risk share budget for the last three financial years has remained static with no increase on £5.2M despite having been slightly over spent in 2009/10. As this pays for therapy for rare disorders, and patients who have chronic long term conditions which require therapy for the duration of their life, this budget requires to be increased to allow new patients to access the therapies in the risk share. NHS Scotland announced that NHS bodies collectively underspent by £37 million on a revenue budget of £9.5 billion in 2009/10. Also Ms Sturgeon has said; “When we add this saving to the other non-clinical efficiency savings that health boards will make, it will release more than £100 million a year to be spent on vital frontline services.” (www.scotland.gov.uk/News/Releases/2010/12/15135337)

• RDUK calls upon the Committee to ask the Scottish Government to review whether the budget for the Orphan Drugs Risk Share Scheme is sufficient to meet the needs of patients in Scotland who would benefit from these treatments.

Unique web address

http://www.scottish.parliament.uk/GettingInvolved/Petitions/PE01398

Related information for petition

Do you wish your petition to be hosted on the Parliament's website to collect signatures online?

NO

How many signatures have you collected so far?

1

Closing date for collecting signatures online

N/A

Comments to stimulate online discussion