Access to new licensed medicines

Rare Disease UK (RDUK)

Rare Disease UK (RDUK) and The PNH Alliance are grateful to the Health and Sport Committee for taking forward Public Petitions PE1398, PE1399 and PE1401. For the benefit of Committee members, the Petitioners have prepared a short briefing document detailing our key messages for consideration at the evidence session on access to newly licensed medicines on the 18th September.

The Scottish Medicines Consortium’s appraisal process for orphan medicines

The petitioners recognise that there must be a robust system in place for assessing new medicines in Scotland and whilst there are many positive elements of the way the SMC conducts its appraisals, we remain fundamentally concerned that the process currently used cannot adequately appraise orphan drugs for rare disease patients equitably.

We believe that an equitable evaluation of an orphan medicine should be based on an appraisal against multiple criteria, and not primarily on a cost utility analysis. In Scotland, the current appraisal processes, including the modifiers employed by the SMC, do not enable an equitable judgement to be made. It remains unclear when and how modifiers are used, which criteria must be met in order for the SMC to take into account other factors and the extent of the impact the modifiers have on whether a medicine is recommended or not. In addition, statistics provided by the ABPI show that the introduction of the SMC policy statement on orphan drugs (regarding modifiers) has resulted in no significant difference in the distribution of decisions (61% were 'not recommended' in the period 2003-2007 and 63% in the period 2008-2011).

Rather than an appraisal process primarily based on an inappropriate cost-per QALY analysis, the petitioners suggest that the Health and Sport Committee recommend that the SMC appraisal process for orphan medicines be subject to an open and transparent public review, with input from all stakeholder groups, and that the expertise of bodies such as the Commissie Farmaceutische Hulp (Netherlands) and the AGNSS decision making framework (England) be acknowledged when considering appropriate appraisal processes for orphan medicines.

In addition, the petitioners suggest that the Health and Sport Committee request clarification from the SMC regarding the use of modifiers, specifically,

- Will all orphan medicines be assessed against SMC modifiers?
- What criteria must be met before modifiers are applied?
- What weight do the modifiers have on the final SMC decision?
Individual Patient Treatment Requests (IPTRs)

When an orphan medicine does not receive a ‘recommended’ appraisal from SMC, the only way it can be accessed by a patient is through the IPTR process. The IPTR referral criteria are extremely difficult to satisfy for orphan medicines and are more likely to result in rare disease patients with the greatest clinical need being refused access to potentially life changing/saving treatments compared to patients affected by more common conditions. Moreover, in the absence of a recommendation from the SMC, the reliance on the IPTR process leads to a postcode lottery in access in Scotland, as demonstrated by PE1398 and PE1401.

The petitioners are concerned that the appropriate clinical experts are not consulted when making IPTR decisions on orphan medicines. Health Boards must acknowledge that for many rare diseases, clinical expertise exists out with Scotland and in some cases, out with the UK. It is the Petitioner’s opinion that the term expert needs to be properly defined in relation to IPTRs to ensure the appropriate clinical experts are approached for their opinion in relation to an IPTR request for a rare disease. This will ensure members of the IPTR panel have a better understanding of the rare disease in question and the treatment options being recommended.

The petitioners suggest that the Health and Sport Committee recommend that the Scottish Government undertake a thorough review of the current IPTR criteria to assess whether or not this criteria is appropriate for orphan medicines. A review should involve all relevant stakeholders including patient organisations, health boards, clinicians and industry.

In addition, the petitioners suggest that the Health and Sport Committee request clarification from the various Health Boards regarding how they choose clinical experts when considering an IPTR for a patient with a rare disease.

**Rare Disease UK (RDUK)**
10 September 2012