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Bringing medicines to life
Scotland

Public Petitions Committee

ABPI Scotland thanks the Public Petitions Committee of the Scottish Parliament for the opportunity to address issues surrounding the petitions under consideration on access to medicines for orphan conditions and the Individual Patient Treatment Request (IPTR) option for clinicians to prescribe medicines that are not recommended for use in NHSScotland.

We will be glad to provide references for the statements in this submission.

1. Background

In 2000, EU regulations came into force aimed at providing a system to designate orphan medicinal products (OMPs) and to create incentives for pharmaceutical companies to develop treatments specifically for rare conditions. EC 141/2000 and 847/2000 include an extended period of exclusive marketing rights and assistance with clinical trial protocols

The legislation appears to be successfully fulfilling its purpose as the number of orphan medicines licensed per year has increased from 8 before 2000 to 74 by 2011. Despite the availability of these medicines on the UK market, SMC mechanisms and methodology are such that Scottish patients face significant challenge in gaining access to them, usually due to the data available from the inevitably small number of patients that can be recruited into any clinical trial.

A study conducted by the Office of Health Economic which shows that as of May 2011, the SMC has issued 55 decisions for the 74 orphan indications approved to date by EMEA. Because of resubmissions and reviews for some, a total of 69 decisions have been published, of which 43 were rejected. 29% of the rejections were due to no submission (which may be due to the manufacturer's perception that orphan medicines have a lower chance of achieving a successful outcome) and the remaining 71% were rejected due to the 'economic case not being demonstrated'. This means that either the cost per QALY estimate was too high, there was too much uncertainty associated with the model, or that no cost effectiveness model was provided. For 23 of the 31 rejected, the level of clinical evidence available at the time of the review was considered inadequate by SMC.

http://www.ohe.org/news/2011/08/23/recent-statistics-on-orphan-approvals-in-scotland-and-england/

The introduction of a policy statement on orphan drugs by SMC in 2007 allowed SMC to consider other factors in addition to the clinical and cost effectiveness in assessing OMPs. However, there is no significant difference in the distribution of decisions before and after (61% were 'not recommended' in the period 2003-2007 and 63% in the period 2008-2011).

In summary, cost utility, QALY based modelling as employed by the SMC, we believe, fails to recognise the value orphan medicines bring to patients suffering both life threatening or chronically debilitating conditions. The reasons for this are manifold but include the lack of any suitable comparator medicines and the relatively small number of patients enrolled in trials both of which can lead to high degrees of uncertainty resulting in unreliable QALY estimates.

2. What actions has the industry taken in order to make orphan medicines more economical?

The cost of OMP development is at least as high as for a non orphan medicine. Orphan medicines are expected to achieve similar standards of safety and efficacy, both in development and in manufacture. The recouping of the costs incurred in development must come through sales. As OMPs are specifically licensed to treat rare disease there is a dramatically lower level of usage compared to non-orphan medicines. This factor inevitably leads to high costs per patient, although the overall budget impact of OMP's is low. In 2007 orphan medicines accounted for 1.0% of the total drug spending in the UK.

In order to ensure patient access to their medicines in Scotland it is sometimes possible for some manufacturers of OMPs to offer a Patient Access Scheme whereby companies put forward proposals to reduce the budget impact of new medicines to allow them to reach patients. The medicines industry in Scotland has worked closely with the NHS to develop a robust system for assessing Patient Access Schemes through a central body, the Patient Access Scheme Assessment Group (PASAG). A report on the proposed PAS from PASAG is then considered alongside the company's submission to SMC. So far three medicines have been accepted for use in NHSScotland with a PAS, though several companies have had their PAS proposals accepted by PASAG but not subsequently by SMC.

It is important to note that Profits made by pharmaceutical companies within the UK including Scotland are regulated and capped through the Pharmaceutical Price Regulation Scheme. Medicine pricing is the key driver in profit generation for pharmaceutical companies and therefore, pricing is controlled to regulate profit.

3. What discussions has ABPI had with the Scottish and UK Governments concerning the availability of orphan medicines?

The issue of access to orphan medicines has been raised with the Scottish Government a number of times by patients, patient groups, industry and MSPs, while a briefing on medicines for very rare conditions was circulated earlier this year.

The response has been that such access decisions are a matter for the Scottish Medicines Consortium with Ministers unwilling to interfere in what they see as clinical decisions made by an independent NHS body.

On 11 November 2008, the European Commission adopted a Communication on Rare Diseases as well as a proposal for a European Council Recommendation. These two documents call for the establishment of an overall and comprehensive, EU wide and integrated strategy to support Member States on issues including diagnosis, treatment and care for rare disease patients throughout Europe. The Recommendation calls on member States to adopt plans or strategies for rare diseases by 2013.

The Orphan Medicines Industry Group (OMIG) of the ABPI, which brings together member companies of the ABPI to ensure patients with rare diseases can access the orphan medicines they require, sent a briefing note to MSPs in September.

The Health Secretary responded to OMIG that the Scottish Government is working alongside the other UK nations to explore areas of possible collaboration and progress in the provision of services for people with rare diseases and will report on the implementation of its actions by 2013. Ms Sturgeon added "The Scottish government is keen to ensure that people with rare diseases, and their families and carers, receive the care and support they need wherever in Scotland they live".

The medicines industry is keen to support the Scottish Government to meet the 2013 target. However, we believe that the petitioners are highlighting a problem that needs to be addressed far sooner, that of an apparent inability of clinicians to make successful IPTR requests for medicines to treat patients with rare conditions. ABPI Scotland has commissioned research on this and we will share this with the Committee as soon as it becomes available.

In England, industry was involved in consultation with Government leading to the establishment by the Department of Health of a specialist commissioning body the Advisory Group for National Specialised Services (AGNSS) to provide access to certain orphan

medicines on a national basis. Members of the AGNSS team have presented their process at a meeting organised by ABPI Scotland attended by representatives of the Scottish Government and SMC. AGNSS takes a broader approach to assessing clinical and cost effectiveness by also considering societal benefit parameters. The medicines industry, through the SMC User Group Forum, is in discussion with SMC on evaluating the outcomes of the AGNSS approach and examining the elements of the AGNSS process that differ from those used by SMC in the assessment of medicines for rare conditions.