PE1399/M

The Association of Glycogen Storage Disorders (UK) welcomes the opportunity given to us to respond to points contained within The Scottish Government and the SMC responses to the Public Petitions Committee on PE1398, PE1399 and PE1401

To Review the SMC Mechanism and Methodology to Appraise the Value of Medicines to Treat Rare Diseases

- England, Wales and Northern Ireland have alternative arrangements for assessing conditions with a UK prevalence of less than 1 in 50,000; would the Scottish Government agree to put other bodies in place in Scotland for appraising medicines other than the SMC and HIS?
- The AGSD-UK accepts that the SMC operates independently from the Scottish Government when assessing medicines for use within NHS Scotland, but who decides upon the policy under which the SMC operate? Innovations in both medicines and technology development have been made in the last ten years since the inception of the SMC; what changes and updates have been made to the SMC to account for innovation?
- We commend the Scottish Government for monitoring the NHS Board progress in implementing the guidance issued to NHS Boards on the introduction and availability of newly licensed medicines in the NHS in Scotland and we are pleased to see that the 14 NHS Boards have arrangements in place. However, patients with rare diseases still have problems accessing medicines as the latest Chief Executive's letter (CEL) from the CMO states that:

"The responsibility for an application for an IPTR rests with the clinician who supports prescribing the requested medicine. It is the clinician who is expected to demonstrate the clinical case for the patient to be prescribed a medicine within its licensed indication(s) where the following criteria apply:

The patient's clinical circumstances (condition and characteristics) are significantly different from either:
(i) the general population of patients covered by the medicine's licence; or
(ii) the population of patients included in the clinical trials for the medicine's licensed indication as appraised.

These circumstances imply that the patient is likely to gain significantly more benefit from the medicine than would normally be expected. Such considerations should be taken on a "case by case" basis reflecting clinical opinion and, as such, should not be generalised."

Due to the fact that there are so few patients suffering from Pompe disease, these criteria will not be met. What does the Scottish Government intend to do about this?

Individual Patient Treatment Requests (IPTRs)

Applications have been made to access therapy via IPTRs as submitted by UK specialists and the applications have been rejected – not because clinical need has not been justified – but because the patient's clinical circumstances cannot be significantly different from the general or clinical trial population of Pompe patients. We believe that this makes it impossible for a patient suffering from Pompe disease to access therapy via an IPTR in Scotland. Again, what does the Scottish Government intend to do about this?

In an article published in The Herald on 19 April 2011, a spokeswoman for the Health Secretary said: "When a clinician decides that a patient requires access to specialised treatment for a rare condition we expect health boards to look favourably and flexibly at such cases and to take the clinician's recommendation seriously."

UK specialists have recommended that Myozyme should be prescribed for eligible patients in Scotland, but these patients have been turned down for funding when trying to access therapy via an IPTR. How does the Scottish Government explain this in the light of the Herald article above?

Will you undertake a review as requested by the petitioners?

We thank the Scottish Government for giving consideration to the extant arrangements for appraisal of medicines to treat rare diseases. When will the outcome be known?

The Committee was told that AGNSS has two observers from the Scottish Government's Health Department. What consideration has the Scottish Government given to adopting a similar approach for Scotland, please give reasons?

There is an Orphan Drugs Risk Share scheme in Scotland which is administered by the NSD and administers pooled NHS board funds for the provision of some specific high cost drugs required by very small numbers of people in Scotland for a range of conditions. We understand that Myozyme for the treatment of Pompe disease is not recommended by the SMC but is included in the risk share if there has been an IPTR whereby inclusion in the risk share is conditional on treatment being consistent with UK protocols, and being initiated, overseen and reviewed by the Enzyme Replacement Therapy specialists in one of the designated English centres; or by the metabolic specialist at the Royal Hospital for Sick Children in Glasgow.

Patients suffering from Pompe disease tend to require therapy for life. However we also understand that this risk share budget has remained flat for the past several years. Is this the reason why patients cannot access therapy for Pompe disease as monies have not been uplifted? Will the Scottish Government suggest that this budget is raised to allow patients suffering from Pompe disease to access therapy in Scotland?

Within the context of the PPRS and procurement legislation what opportunities are there for the NHS in Scotland to improve the procurement of orphan drugs in order to mitigate against the high cost of these medicines and improve availability?

- We would recommend that you monitor the procurement process currently being adopted in England.

- See also the response as sent out by National Procurement.

Additional points

The Mackie Report was published by the Cross Party Group on Muscular Dystrophy in the Scottish Parliament in September 2010. The CPG and the MDC which called upon "the Scottish Government to review the situation regarding the unequal treatment of the small number of patients with Pompe disease living in Scotland. While some patients are currently receiving enzyme replacement therapy, others are being refused this treatment. In England all patients are able to access this treatment."

Has the Scottish Government reviewed this situation, and what is their response?

Orphan Medicines

"Ultra-orphan" is indeed a term used by NICE, defined as conditions with a UK prevalence of less than 1 in 50,000. However, under the new arrangements a very limited number of products and technologies may be considered by the Advisory Group for National Specialised Services (AGNSS).

AGNSS will consider the suitability for national commissioning of services, products and technologies, which meet the entry criteria of their decision-making framework:
The product, service or technology will usually consist of no more than 500 patients (1 year period prevalence) and/or four centres in England. Lysosomal Storage Disorders, including Myozyme, come under the remit of AGNSS in England.

Northern Ireland has an agreement to follow AGNSS agreements for patients.

All Wales Medicines Strategy Group have defined Ultra-orphans on their website as of September 2011: Ultra-orphan medicines are orphan drugs that are licensed for the treatment of diseases with a prevalence of less than 1 in 50,000 persons in the European Union at the time of submission of the designation application to the European Medicines Agency.

Will the SMC consider using a separate process to assess ultra-orphans as in England, Wales and Northern Ireland?

SMC advice to date on orphan medicines

What recommendations have the SMC made for drugs that are licensed for the treatment of diseases with a prevalence of less than 1 in 50,000 persons in terms of accepted, accepted for restricted use and not recommended?

What is the highest cost per QALY accepted for use in NHS Scotland, and when?

Why is there a lower acceptance rate for orphan medicines submitted to the SMC than the acceptance rate for medicines without orphan status?

Societal considerations of valuing rarity

The AGSD are aware of the NICE's Citizens' Council report from November 2004 which also included the following that we would like to bring to the attention of the PPC:

The majority (20 out of 27) of Citizens' Council members came to a conclusion that it is sometimes, or always, justified for the NHS to pay premium prices for ultra-orphan drugs. For twenty of us, the NHS should vary its normal assessment of cost effectiveness to allow expenditure on ultra-orphan drugs where necessary.

Most of us felt strongly that everyone should have fair and equally high standards of care – and in order to achieve this, it may be necessary to spend more on some people than on others. We don't feel that the minority should be penalised for the sake of the majority, and we were concerned that once we start to discriminate against people with rare conditions, who knows which group we may decide that we can't afford next.

NICE have said **"**The conclusions of the Citizens Council, and the judgment of the board, suggests there is public support for the NHS to meet the reasonable treatment costs of expensive treatments for ultra-orphan conditions. This would accord with the NHS's egalitarian principles."

There is a more recent NICE's Citizens' Council report from November 2008 which came to the following conclusions when asked in what circumstances should NICE recommend interventions where the cost per QALY is above the threshold range of £20-30,000? 27 of 29 Council members favoured taking account of each of a list of various possible circumstances; Patients suffering with Pompe disease meet most of these criteria.

The survey of the Norwegian population, as mentioned by the SMC, had two final sentences:
However, the authors point out that there could be *"unexplored ethical reasons"* that would support a special funding status for orphan drugs. Furthermore, the authors concede that *"…majority opinion is not necessarily a good measure of what is ethical".* It is also a fact that Norway does fund Myozyme for patients with Pompe disease.

The best estimates arising from the Council of the European Union's Recommendation from June 2009 suggest that 1 in 17 people will be affected by a rare disease at some point in their lives if the rare disease affects less than 5 in 10,000 of the general population. However, the vast majority of patients affected by a rare disease will not have a therapeutic treatment available.

Pompe disease has a prevalence of far less than 1 in 50,000 of the general population. 4 patients have been identified in Scotland who would be eligible for therapy if residing in one of the other three administrations which make up the UK.

Is it not inequitable that these patients would receive therapy if residing elsewhere in the UK?

SMC views on the issues raised by Petitions PE1398, PE1399, PE1401

We would like to point out the Budget Impact of making therapeutic treatment available for the 4 eligible current patients suffering from Pompe disease in Scotland is very small compared to the spend on regular pharmaceuticals, so Budget Impact should be looked at rather than “opportunity cost”. Would the SMC agree to do this?

The SMC state that 13 medicines with a Patient Access Scheme (PAS) have been accepted for use or restricted use in NHS Scotland; how many have been for medicines which treat conditions with a prevalence of less than 1 in 50,000?