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PE1399/S

The Association of Glycogen Storage Disorders (UK) welcomes the further opportunity given to us to respond to points contained within The Scottish Government response to the Public Petitions Committee on PE1398, PE1399 and PE1401, as sent 1 February, 2012

The Scottish Government

- **In the response of 8 November 2011, the Scottish Government states that it will give consideration to the extant arrangements for appraisal of medicines to treat rare diseases.**

(1; 2): We are aware of the Guidance to Further Strengthen the Safe and Effective Use of New Medicines across the NHS in Scotland published on 13 February 2012.

However this problem still exists: "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, and the disease is so heterogeneous, these criteria will never be met. No patient will be distinctly different from the population studied as there are so few patients and therefore no subgroups.

Therefore, although applications have been made to access therapy via IPTRs as submitted by UK clinical experts, 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 trial population of patients with Pompe disease. In this regard the written procedures could be challenged as unfair or irrational. How does the Scottish Government intend to reconcile this anomaly?

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?

- **The new medicines approval process employed by Scotland does not adequately capture the unique nature of rare diseases and the inherent problems in developing medicines for rare diseases.**

(18): We are glad to read that Scottish Government policy regarding arrangements for the appraisal of new medicines to treat rare diseases in under consideration; by whom and what are the timelines for this policy review?

(20): Who are the „clinical experts"? How do you choose these and define clinical experts? The AGSD-UK considers that the clinical experts in the treatment of Pompe disease include Professor Ed Wraith, Dr Robin Lachmann, Dr Patrick Deegan and Dr Mark Roberts; will you make contact with these clinicians regarding the treatment of Pompe disease?

(28): The Healthcare Quality Strategy gives three Quality Ambitions which are not currently being met for patients with Pompe disease as their access to therapy is not Person-centred, Safe, Effective, Efficient, Equitable and Timely as stated "There will be no avoidable injury or harm to people from healthcare they receive…" and also "The most appropriate treatments, interventions, support and services will be provided at the right time to everyone who will benefit…."

The AGSD-UK still suggests that due consideration is given to the approaches taken by AGNSS in England and the AWMSG policy.

(29): What are the limitations found in the OHE analysis?

(31): Will the SMC list the orphan medicines which have been assessed and also list the date of decision, QALY and Budget Impact of each of these medicines?

(32): Why is the difference in the lower acceptance rate for orphan medicines justifiable?

(35): If the SMC"s modifiers truly capture those medicines deemed by NICE to come under the description of "ultra-orphan", can the SMC explain why the acceptance rate for these medicines, such as Myozyme, is not the same in Scotland as it is in England?

Due to the small numbers of patients suffering from Pompe disease, the extant IPTR criteria will not be met.

The AGSD-UK considers that this will still be the case. See answer to (1; 2).

(37; 38; 49): What does „significantly more benefit" look like? There are just four adult patients in Scotland who fall within the English Guidelines to receive Myozyme therapy (one of whom is receiving Myozyme) and they are all very different clinically as Pompe disease is so heterogeneous. Postcode prescribing has come into play here as one NHS Board is funding therapy whilst two NHS Boards have refused to fund, despite having

clinical experts in the treatment of Pompe submit the IPTRs. The efficacy of Myozyme has never been brought into question, only the cost. Would the Scottish Government contact a metabolic specialist from the MCN for IMDs, or AGNSS metabolic specialists, who have been involved in the care of Scottish patients and who have already contributed to their IPTRs? Would the Scottish Government and/or the Public Petitions committee be willing to hear evidence from a patient suffering from Pompe? Would the Scottish Government and/or the Public Petitions Committee be willing to call genuine clinical experts to present to them on Pompe disease?

QALY based modelling:

(40): As there is a high degree of uncertainty in the QALY estimates, would the Scottish Government look for a separate process for these orphan medicines to remove this degree of uncertainty?

(41): Which clinical experts in Pompe have influenced the SMC? Which orphan medicines has the SMC accepted?

(43): For orphan medicines, NICE does not use QALYs but patients go via AGNSS where Budget Impact is looked at, rather than QALYs. The Budget Impact for Scotland to allow all diagnosed patients who fall within the „Guidelines for the Investigation and Management of Late Onset Pompe Disease" to access Myozyme is currently a maximum of £1M.

What consideration has the Scottish Government given to adopting a similar approach to that of AGNSS for Scotland?

(45; 50): The NSD holds top-sliced funds to allow patients with Pompe to be referred to England. 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?

The Mackie report:

(54): What progress has been made with services and therapy for patients with Pompe disease? What is the date for the meeting taking place between the SMN and the IMD Network? Where is it taking place? Who is to attend? Which Healthcare professionals from England are attending? Is the Scottish Government willing to ask clinical experts in Pompe to attend? May the AGSD-UK attend as an observer? If this meeting is not to address issues around access to specific medicines for Pompe disease, what is the purpose of it? While some patients in Scotland are currently receiving Myozyme, 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?

(56): When is the draft UK Plan for Rare Diseases likely to be published for consultation?