The Scottish Parliament
Pàrlamaid na h-Alba

| PUBLIC PETITION NO. | PE01399 |
|---|---|

## Name of petitioner

Allan Muir on behalf of Association for Glycogen Storage Disease (UK) Ltd

## Petition title

Equitable access to therapy for Pompe disease

## Petition summary

Calling on the Scottish Parliament to urge the Scottish Government to instruct the Chief Medical Officer (CMO) to revise the criteria to access IPTRs for Orphan diseases as these criteria are detrimental to patients suffering from Pompe disease.

## Action taken to resolve issues of concern before submitting the petition

Ayrshire & Arran NHS Board and Greater Glasgow & Clyde NHS Board have reviewed Individual Patient Treatment Requests since the publication of CEL 17 and have rejected them, although treatment was recommended by a UK specialist. The UK Guidelines have been written by specialists and the patients rejected by their NHS Boards in Scotland do fit within these Guidelines.

www.specialisedservices.nhs.uk/library/23/Guidelines_for_Late_Onset_Pompe_Disease.pdf

An appeal has been made to the Medicines Appeal Panel of Ayrshire & Arran NHS Board which has also been rejected on the grounds of cost efficacy, despite representation by a UK specialist.

A patient has written two letters to John Scott, her MSP, at the suggestion of Jackie Baillie, MSP.

This Parliamentary Question has been asked -
S3W-40287 - Jackie Baillie:
To ask the Scottish Executive what review it has undertaken on Recommendation 11 of the Mackie Report: Access to specialist neuromuscular care and social care in Scotland by the Cross Party Group on Muscular Dystrophy.
Nicola Sturgeon (Wednesday, March 16, 2011):
“The enzyme replacement therapy for Pompe disease, Myozyme, has been appraised by the Scottish Medicines Consortium, but was not recommended for routine use within NHS Scotland. In such circumstances, NHS boards have arrangements in place to consider treatment requests from individual patients. Such decisions are made on a case by case basis, exercising professional judgement on clinical need.”

The Cross Party Group on Muscular Dystrophy in the last Scottish Parliament recommended that “The Scottish Government reviews 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." This means that approximately 90 adult patients residing in England receive this treatment.

www.muscular-dystrophy.org/assets/0001/9335/Mackie_Report.pdf

Robbie Warner, Chair of the Muscular Dystrophy Campaign's Scottish Council is quoted in the Mackie report hoping for service improvements:

"I would want advice available for people living with muscular dystrophy, and I would want the planning to be done and for people to acknowledge it. I would want the whole system streamlined in such a way that you don't have to fight to get everything, because if you get it at the end of the fight, you wonder whether all that energy was wasted in terms of them resisting it. I would like to see a change of attitude that would say that you need it and you plan for it, and get into a working relationship."

First Minister Alex Salmond has previously intervened to support a case for one of his constituents to receive treatment for Pompe disease.

http://www.heraldscotland.com/news/health/families-launch-an-appeal-for-rare-disease-treatment-on-nhs-1.1092516?57380

From this same article, published in The Herald on 25 March 2011:

• Allan Muir, development director of the Association for Glycogen Storage Diseases – which supports families affected, said: "Access to therapy for Pompe disease patients in Scotland currently depends on which health board a patient belongs to, and the vast majority of patients are being denied treatment. This is hugely frustrating for the families affected, especially given that patients with other related rare diseases are being treated with similar medications."
• The Muscular Dystrophy Campaign is supporting the drive by Scotland's sufferers to obtain access to the drug. Director of care for the charity Nic Bungay said: "We know of only 11 Scottish Pompe disease patients, yet the vast majority of them are being denied access to a ground-breaking treatment automatically available to people living in England, on the grounds that the therapy is not cost-effective."
• A spokesman for the Scottish Medicines Consortium, which approves new drugs for use by the NHS, said: "We evaluated Myozyme in 2007 and were disappointed to find that it wasn't a value for money medicine. This decision was based on analysis of the manufacturer's own information. All NHS boards have mechanisms in place in order to consider drugs that are not deemed to be cost-effective."

The Daily Record have also publicised the plight of a patient:

http://www.dailyrecord.co.uk/news/real-life/2011/03/21/facing-life-in-a-wheelchair-and-on-oxygen-because-nhs-will-not-provide-vital-drug-86908-23005660/

An article was published in the Scotsman on 1 May 2011:

"Scottish Labour's health spokeswoman Jackie Baillie said: "It is vital that those suffering from rare conditions get access to the medicines they require and we must ensure that patients in one part of Scotland are not disadvantaged compared with patients living in England and Wales." An SNP spokesperson said: "Everyone recognises the importance of decisions on medication and treatment being made by health professionals and not politicians. When consultants recommend access to medicines we expect health boards to respond flexibly and favourably to requests."

http://news.Scotsman.com/health/Family-forced-to-fund-life-saving.6760470.jp

In addition: "First Minister Alex Salmond has met the anti-cuts protesters who ambushed Labour leader Iain Gray and Scottish Conservatives leader Annabel Goldie…. issues include the reversal of a decision to move a Glasgow day-care service and the potential funding of treatment for people with Pompe disease, a genetic muscle disorder." Following the meeting, Mr Salmond said: "Pompe disease has to be dealt with sensitively but there might be a way forward there as well."

http://news.stv.tv/election-2011/243383-alex-salmond-to-meet-protestors/

Finally, “Scottish patients have demanded Health Secretary Nicola Sturgeon end the “postcode lottery” in drug provision… However a Scottish Government spokeswoman said: “Health boards have arrangements in place to provide this drug for individual patients in certain circumstances. This is a clinical decision based on a robust national framework – not a postcode lottery.”

http://www.heraldscotland.com/news/health/sturgeon-urged-to-end-scotlands-drug-lottery-1.1111791

But 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.”

http://www.sehd.scot.nhs.uk/cmo/CMO(2011)03.pdf

Due to the fact that there are so few patients suffering from Pompe disease, these criteria will not be met.

Therefore, although applications have been made to access therapy via IPTRs as submitted by UK specialists, 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 Pompe patients.

**Petition background information**

The AGSD 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 refusals are being given when IPTRs are done for patients with Pompe disease as they cannot meet the referral criteria for IPTRs within the latest CMO letter (see page 7 of attachment below).

http://www.sehd.scot.nhs.uk/cmo/CMO(2011)03.pdf

There are currently just 11 patients diagnosed with Pompe disease living in Scotland. Three of these patients are receiving enzyme replacement therapy, Myozyme. A further two of these patients have suffered rejections for funding for Myozyme from their NHS boards, even when recommended by a specialist who has exercised professional judgement based on clinical need. There is no other therapy available, just palliative care.

The best therapeutic results are achieved when enzyme replacement therapy is started early in the course of symptom development and before irreversible muscular damage has occurred.

Without therapy, progression will occur with the disease spreading to the respiratory muscles, which may precede limb muscular weakness. Over a 2 year period the need for respiratory support increases as functional activity decreases. It does not matter how

old or young a patient is; the longer the time since diagnosis, the higher the probability of wheelchair or ventilator dependency.

Late-onset Pompe disease should be viewed as a progressive disorder for which timely intervention is required to prevent further loss of function.

One patient with late onset Pompe disease who resides within NHS Tayside is receiving Myozyme, whereas another patient with late onset Pompe disease who resides within NHS Ayrshire & Arran cannot receive Myozyme – even when recommended by a specialist who exercised professional judgement based on clinical need – as the recommendation “provided no further important information to uphold the appeal, or impact on the QALY cost, and subsequently on the opportunity cost implications for NHS Ayrshire & Arran.” Both the NHS Tayside & NHS Ayrshire & Arran patient were assessed and recommended for therapy by the same specialist.

Since September 2001, all new medicines have been assessed for use in NHS Scotland by the Scottish Medicines Consortium through Health Technology Appraisals. The very small population associated with an Orphan Medicine, along with high development costs, make it extremely difficult to attain a QALY anywhere near the accepted QALY by an HTA group. This means that the SMC tends not to approve products to treat an Orphan Disease as these products will never be cost effective.

It appears that the Deputy First Minister and Cabinet Secretary for Health, Wellbeing & Cities Strategy, Nicola Sturgeon, is under the impression the decision on whether an orphan medicine can be given or not is based upon clinical need as evidenced by a specialist whereas in reality the decision is based upon cost efficacy.

This means that NHS Boards are not responding flexibly and favourably to IPTR requests, even when the access to a medicine is recommended by a consultant.

Therefore the Association for Glycogen Storage Disease (UK) calls upon the Committee to ask the Scottish Government to ask the CMO to rectify this iniquitous situation immediately by revising the referral criteria for IPTRs for patients with Pompe disease. This will allow the few patients residing in Scotland to access therapy, when recommended by a specialist exercising professional judgement based on clinical need, and not for NHS Boards to rely upon cost implications for decisions to impact upon access to therapy for this orphan disease.

Resources:

AGSD-UK Pompe disease website: www.pompe.org.uk

Pompe Bulletin: www.pompe.org.uk/images/stories/pompebulletin%2017.pdf

AGSD-UK website: www.agsd.org.uk

**Unique web address**

http://www.scottish.parliament.uk/GettingInvolved/Petitions/PE01399

**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