Submission from the Association for Glycogen Storage Disease (UK)

The Association for Glycogen Storage Disease (UK) welcomes the decision by the Health and Sport Committee in Holyrood to continue their work in the area of Access to New Medicines and are pleased to learn that the Committee are sitting again on 6 November 2012, albeit in private.

However we would be grateful if you could consider the following points:

1. The title of the original Petition is ‘Equitable access to therapy for Pompe disease’. At the last meeting of the Committee on 18 September, Pompe disease and the enzyme replacement therapy available elsewhere in the UK for patients suffering from Pompe disease was not mentioned. Whilst it is understandable that many pharmaceutical companies wish to have their therapies, mainly for cancer, discussed by the Committee, the AGSD-UK feels disappointed by this variation from the original Petition which was: ‘Calling on the Scottish Parliament to urge the Scottish Government to allow all Scottish patients suffering from Pompe disease access to enzyme replacement therapy, when included in the risk share and consistent with UK protocols and initiated, overseen and reviewed by a suitable specialist.’

**Will the Health and Sport Committee consider enzyme replacement therapy for Pompe disease as in the spirit of the original Petition?**

2. There is currently only one patient in Scotland, who resides within Greater Glasgow and Clyde NHS Board, with a confirmed diagnosis of Pompe disease who fits within the UK Guidelines to receive enzyme replacement therapy. Other patients suffering from Pompe disease have relocated to England where they are now successfully receiving enzyme replacement therapy. The cost of providing treatment for this patient would amount to about £250,000 in any financial year.

**Will the Health and Sport Committee look at ‘budget impact’ when considering enzyme replacement therapy for Pompe disease?**

3. The NHS National Institute for Health Research has recently published *The effectiveness and cost-effectiveness of enzyme and substrate replacement therapies: a longitudinal cohort study of people with lysosomal storage disorders*¹.

This concludes that for Pompe disease:
“These data provide further evidence on the effectiveness of ERT in people with LSDs. The confidence with which conclusions can be drawn inevitably hinges primarily on the numbers of patients with a particular condition.”

¹ [www.hta.ac.uk/execsumm/summ1639.shtml](http://www.hta.ac.uk/execsumm/summ1639.shtml)
“In patients with Pompe disease these data provide some evidence of a beneficial effect on muscle strength and on mobility as measured by the 6-minute walk test.”

Will the Health and Sport Committee consider these conclusions when discussing enzyme replacement therapy for Pompe disease?

4. Enzyme replacement therapy for Pompe disease is included in the risk share scheme as ‘the effect is so financially significant that individual NHS Boards could be at financial risk’.

Will the Health and Sport Committee look at the cost and budget impact for NHS Boards when deciding whether to recommend enzyme replacement therapy for this one patient in Scotland?

5. Enzyme replacement therapy for Pompe disease is included in the risk share scheme as ‘clinical practice across Scotland is based on appropriate clinical evidence and/or national protocols (where available), such that services can demonstrate equity of access to treatments across Scotland (accepting there may be local and individual patient needs)’.

Will the Health and Sport Committee look at which NHS Boards are funding enzyme replacement therapy for clinically-similar patients with Pompe disease given that Greater Glasgow and Clyde have refused a funding application and subsequent appeal?

6. Whilst the Health and Sport Committee chose to take evidence on 18 September from the industry body the ABPI, which is the member body for the pharmaceutical industry, the ABPI do not typically represent the views of the companies developing treatments for the rarer diseases – which tend to be smaller companies who cannot afford or choose not to join the ABPI. So whilst the ABPI were able to communicate the views of their members at the last Committee meeting, they were not in the best position to give the view required to address the issues raised in the Petitions (PE1398/PE1399/PE1401).

Will the Health and Sport Committee make contact with EMIG (Ethical Medicines Industry Group) to obtain a representative view from the industry members’ body which represents companies largely developing medicines for rare diseases and understands the issues raised in the Petitions?

Allan Muir

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2 www.emig.org.uk