



PUBLIC PETITION NO.

PE01401

Name of petitioner

Lesley Loeliger and Professor Peter Hillmen on behalf of PNH Scotland and the PNH Alliance

Petition title

Access to therapy for paroxysmal nocturnal haemoglobinuria

Petition summary

Calling on the Scottish Parliament to urge the Scottish Government to review the mechanism and methodology used by the Scottish Medicines Consortium to appraise medicines for rare diseases and to instruct the Chief Medical Officer to revise the criteria by which health boards assess Individual Patient Treatment Requests in order to improve access to therapy for patients with paroxysmal nocturnal haemoglobinuria.

Action taken to resolve issues of concern before submitting the petition

- The PNH Alliance wrote to the Cabinet Secretary for Health and Wellbeing on 28 February 2011 requesting a meeting to discuss inequality in access to therapy for paroxysmal nocturnal haemoglobinuria (PNH) between health boards. The meeting was declined on the basis that the Cabinet Secretary's diary was "very pressured".
- The PNH Alliance wrote to the Scottish Medicines Consortium (SMC) on 15 March 2011 requesting a meeting to discuss the appraisal of Eculizumab for the treatment of PNH. The meeting was declined but the SMC has since published updated guidance on the therapy. However, it is still not recommended for use in Scotland.
- The PNH Alliance wrote to the Chief Executive of NHS Greater Glasgow and Clyde, where a patient was being denied access to therapy for PNH, on 11 February 2011 and 11 April 2011 requesting that the health board reassess its policy on the use of Eculizumab. The Chief Executive declined to reassess its policy.
- The PNH Alliance and PNH Scotland have advised MSPs on barriers to access to therapy for PNH which has led to a number of written questions being tabled and correspondence with the Cabinet Secretary (February 2011 onwards).
- The PNH Alliance and PNH Scotland have secured significant media coverage on access to therapy PNH, including in the Herald, the Sunday Post, and on the BBC and STV.
- The PNH Alliance wrote to the Cabinet Secretary for Health and Wellbeing again on 1 July 2011 requesting a meeting following the death of a patient who had been declined access to therapy in NHS Greater Glasgow and Clyde. The meeting was declined on the basis that "decisions relating to IPTRs are a matter for each NHS Board". This misses the point as we believe that political intervention is required to improve the way local boards assess Individual Patient Treatment Requests.

Petition background information

Paroxysmal nocturnal haemoglobinuria (PNH) is a very rare, chronic, life threatening disease which causes red blood cells to lyse (explode). This can lead to a range of debilitating symptoms including severe fatigue, anaemia, abdominal pain, difficulty with swallowing, erectile dysfunction, kidney dysfunction and thrombosis. Patients are most commonly diagnosed in their 30s and median survival from diagnosis is in the range of 10-22 years.

The only licensed and effective treatment for PNH is Eculizumab, which is nationally commissioned for patients in England and Wales and fully funded in Northern Ireland.

Eculizumab is a highly effective treatment for PNH with successive studies demonstrating excellent outcomes for patients and improved quality of life. Recent data shows that patients being treated with Eculizumab have a normal life expectancy.

Eculizumab was not recommended for the treatment of PNH by the Scottish Medicines Consortium (SMC) in 2010 on the basis that "cost-effectiveness was not demonstrated in an independent economic analysis".

However, the SMC's appraisal process is not appropriate for the appraisal of medicines for very rare diseases and it regularly rejects these medicines. For example, as of May 2010, the SMC had reviewed 46 orphan medicines and of these 37 % (17) had been rejected and a further 24% (11) granted only highly restricted use.

We are therefore calling for the Scottish Government to review the mechanism and methodology used by the Scottish Medicines Consortium to appraise medicines for rare diseases to ensure that more are recommended for use in Scotland.

As Eculizumab is not recommended by the SMC, patients access the medicine via Individual Patient Treatment Requests (IPTRs). This has resulted in a very serious inequality of access to the medicine for patients with PNH across Scotland.

The refusal of NHS Greater Glasgow & Clyde to fund Eculizumab for one patient led to his death in late April 2011. Another patient has recently been declined access to the medicine in the same health board. The board has stated that it acts in accordance with the guidance that is provided.

We are therefore also calling for the Scottish Government to instruct the Chief Medical Officer to revise the criteria by which health boards assess Individual Patient Treatment Requests.

Unique web address

<http://www.scottish.parliament.uk/GettingInvolved/Petitions/PE01401>

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1

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