

PNH Alliance and PNH Scotland response to the Public Petitions Committee (PE1401)

1. Introduction

The PNH Alliance and PNH Scotland welcome the opportunity to respond to the Scottish Government response to recent questions from the Committee in relation to PE1401. Our comments are set out below, referenced to the associated response from the Scottish Government.

As a general comment, it is our assertion that it is not simply individual elements of policy that are failing PNH patients, rather that the overarching policy for appraising medicines for PNH is not working effectively which has led to the death of one patient and seriously endangers the lives of others. Of particular concern is the failure of Greater Glasgow & Clyde NHS Board not to take into account accepted clinical expertise for the treatment of PNH and associated failure of the Scottish Government to ensure that relevant clinical expertise is considered.

2. Commentary on Scottish Government response

3. The Scottish Government states that the good practice guidance on IPTRs should have time to 'bed in' before any further changes are considered. When asked by the Public Petitions Committee (PPC), however, every NHS Board indicated that they were already in compliance with the guidance. As the guidance is already being implemented by NHS Boards, and IPTRs for patients with rare diseases are still being rejected, there is no need to allow the guidance to 'bed in' and the Scottish Government should consider amendments to the guidance immediately.
5. The IPTR Good Practice guidance makes no reference to ensuring that recognised clinical specialists input into the decision making process. The Outreach Clinic for PNH was established to ensure that specialist clinicians are available to treat Scottish patients despite there being no recognised specialist clinician in PNH within any Scottish NHS Board. The lack of specialist clinical input into the IPTRs significantly compromises the ability of NHS Boards to make clinically correct decisions.

Note that the recently updated guidance from the Scottish Government (*Guidance to further strengthen the safe and effective use of new medicines across the NHS in Scotland*), that was published after the Scottish Government response to the PPC questions, does state that: "IPTR panels are expected to include a practicing medical consultant with (or access to) specialist knowledge of the relevant clinical area."

Whilst this is a welcome development, Boards are continuing to fail PNH patients by not accessing specialist knowledge but claiming they are doing so. This is supported by the widely accepted recognition in the clinical community that there are only a small number of consultants with significant experience of managing patients with PNH, particularly with expertise in the use of Eculizumab, with only one of which living in Scotland. The PNH Outreach

Clinic was therefore established in Scotland to allow access to Scottish patients the required clinical expertise to treat the disease.

37. The phrase “not been considered clinically and cost effective” is misleading as Eculizumab has demonstrated exceptional clinical efficacy. Furthermore, Eculizumab is only recommended for treatment from specialist clinicians where the patient is expected to gain significantly from the medicine.
57. In response to the petition’s statement that “it is the responsibility of the Scottish Government to set the criteria and framework within which the SMC operates in order to improve access”, the Scottish Government states that the SMC “operates independently from the Scottish Government”. This response is not satisfactory. The SMC was established by, and is funded by, the Scottish Government, which grants it “statutory authority for Health Technology Appraisal”. The Scottish Government is therefore responsible for ensuring that the SMC’s health technology appraisal processes are fit for purpose and for amending its statutory authority as necessary.
61. There is significant inequality of access to Eculizumab in Scotland due to the inconsistencies between Greater Glasgow & Clyde NHS Boards and all other NHS Boards to IPTRs. This needs to be addressed as a priority.
62. The Scottish Government states that the SMC’s appraisal arrangements are “robust”. In paragraph 32, however, it notes that acceptance rates for orphan medicines are lower than acceptance rates for medicines without orphan status, but describes this difference as “justifiable”. On what basis does the Scottish Government consider this difference to be justifiable and does it consider this difference to constitute discrimination against people with rare diseases?
- 63-64. See points 1, 3, and 4 above.
65. One PNH patient, Joyce Juszcak, had an IPTR declined by NHS Greater Glasgow and Clyde and was unsuccessful in her appeal. It is our understanding that it was only when she suffered a potentially fatal blood clot that her IPTR was accepted. The PPC should ask the Scottish Government how it justifies allowing patients to risk their lives and become more unwell before allowing them access to treatment.
66. The Scottish Government makes reference to IPTRs being decided on a case-by-case basis reflecting clinical opinion. The PNH Alliance is not aware of any clinician working within NHS Greater Glasgow and Clyde with expertise on PNH or who has published on the treatment of PNH. In fact, the PNH outreach clinic at Monklands Hospital was commissioned specifically to overcome this lack of clinical expertise. Nevertheless, NHS Greater Glasgow and Clyde has rejected IPTRs on the basis of clinical opinion even when treatment had been recommended by specialist clinicians at the outreach clinic.