

## PE1401/N

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

#### 1. Introduction

- The PNH Alliance and PNH Scotland are grateful for the work of the Public Petitions Committee in taking further inquiries to ascertain the reason for the lack of access for PNH patients to Eculizumab.
- There presently exists a very serious situation in Scotland where a designated outreach clinic for all Scottish PNH patients is held in Monklands Hospital in Lanarkshire but the individual health boards of respective patients determine funding (or not as the case may be) for those that require Eculizumab. Globally recognised expert PNH physicians working at the Monklands Hospital can therefore only recommend Eculizumab for treatment with patient not guaranteed to receive it despite clinical opinion to the contrary. The situation is particularly acute for patients within the NHS Greater Glasgow & Clyde Board where several patients have been denied treatment. This has led to the death of one patient in May 2011 and is putting further lives at risk (see 2.i).
- It is noted from the responses that all of the organisations that the Committee contacted claimed to be correctly following agreed Scottish Government policy. Whilst this may be correct, PNH patients are still being denied access to life saving therapy, which suggests that there is a systematic failure between NHS Boards, the SMC and the Scottish Government.
- The PNH Alliance and PNH Scotland therefore propose that the Scottish Government establishes and implements a separate body or alternative pathway within the SMC for the appraisal of ultra-orphan medicines. Such a body or process would be in line with arrangements in England and ambitions set out by the Scottish Government for NHS Scotland in their Quality Strategy which states that: “The most appropriate treatments, interventions, support and services will be provided at the right time to everyone who will benefit.”<sup>i</sup>
- The proposed body should undertake a holistic evaluation of the value of an ultra-orphan medicine to patients taking into consideration the following criteria:
  - Data on clinical effectiveness showing improvements in quality and/or quantity of life and an assessment of patient outcomes on therapy
  - The number of patients whose condition improves as a result of the treatment compared to the total number of patients treated (NNT)
  - Burden of illness and severity of condition

- Availability of treatment alternatives
- Where possible, comparison with existing treatments and to what extent the medicines meet unmet needs
- Safety and risk profile of the medicine
- Societal value of the medicine including impact on carers or families, needs and expectations of patients (including productivity quality of life), patient voice and NHS Scotland priorities
- Benefit to society from research and innovation in the relevant area where conventional rules of investment may not apply

## **2. Comments on responses to the Public Petitions Committee**

### **(i) IPTRs / NHS Boards**

- The PNH Alliance and PNH Scotland welcome the input from all NHS Health Boards.
- There is however a gross inequity for PNH patients, affecting those living within the NHS Greater Glasgow and Clyde (NHSGGC) area. In relation to a recent case of a PNH patient highlighted extensively in the media and Parliament, the NHSGGC released the following statement: “The patient’s new consultant has completed a detailed clinical review and has presented the case to a multi-disciplinary team of 16 haematologists from across the west of Scotland for their expert opinion as to whether her condition supported the prescription of eculizumab. After assessment of her case the multi-disciplinary team did not support the view that her condition had changed significantly for the prescribing of eculizumab.”
- There are however no haematologists who are considered experts in PNH or who have published clinical papers on treatment of PNH in NHSGGC. Prof Peter Hillmen, Consultant Haematologist, St James University Hospital, Leeds and renowned expert in PNH has confirmed that another patient, whose appeal for funding of Eculizumab was rejected by NHSGGC earlier this year, died shortly after with severe anaemia, which was caused by his PNH. The PNH Alliance and PNH Scotland are therefore of the firm opinion that there is overwhelming clinical evidence for PNH patients to be treated with Eculizumab and considers the lack of treatment to be a financial decision as opposed to a clinical one.
- The situation for PNH patients living within the NHSGGC catchment area is further exacerbated by some patients receiving Eculizumab and some not despite living in close proximity. The NHSGGC bases decisions on whether to

fund therapy on exceptional criteria. However, given the very small amount of patients in NHSGGC the catchment area (estimated to be 4) there is a strong argument to suggest that all PNH patients are exceptional and exceptionality cannot be defined for such low patient numbers. Furthermore, PNH is a life threatening disease and therefore exceptionality is based on the patient becoming gravely ill. The patient, who passed away earlier this year, was not considered an exceptional case but nevertheless died from PNH, largely as a result of failure to fund treatment by NHSGGC.

## **(ii) Scottish Government**

- The Scottish Government states that the “SMC operates independently from the Scottish Government”. Whilst the PNH Alliance and PNH Scotland understand that there has to be some degree of independence the fact remains that the SMC is responsible for recommendations on large amounts of government spending for which elected politicians have to take responsibility. The PNH Alliance and PNH Scotland believe 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 PNH Alliance and PNH Scotland welcomes the work of the Committee associated with PE 1108 in producing guidance for access to newly licensed medicines. However, the guidance does not address the issue for PNH patients demonstrated by the continuing lack of access to Eculizumab for some patients.
- The associated Scottish Government guidance framework for NHS Boards published in March 2011 is not being implemented consistently as demonstrated by the inequity of access to Eculizumab.
- The Scottish Medicines Consortium has comprehensively failed the PNH community for failing to recommend Eculizumab despite the use of modifiers. Eculizumab has been demonstrated to be extremely effective in the treatment of PNH which would suggest that their modifiers do not work in that they fail to take into account the “evidence of substantial improvement in life expectancy and quality of life.”
- The PNH Alliance and PNH Scotland welcome the consideration of the Scottish Government to examine existing arrangements for the appraisal of medicines for rare diseases and the CMO to review criteria for individual patient treatment requests. We would however urge the Government to ensure that both of these

processes are undertaken in a publically transparent nature, potentially via a formal public consultation.

### **(iii) Scottish Medicines Consortium**

- PNH is an ultra-orphan condition and we recommend that the SMC formally recognises such conditions in line with definitions stipulated in England by NICE and in Wales by the All Wales Medicines Strategy Group. England and Wales have further recognised the need for separate policy mechanisms to review ultra-orphan therapies.
- The SMC does not provide data on recommendations on ultra-orphan medicines. However, analysis<sup>11</sup> by the PNH Alliance and PNH Scotland shows that of the 12 medicines licensed by the European Medicines Agency for ultra-orphan conditions, the SMC only recommends one for restricted use only.
- As stipulated in our response to the Scottish Government (2.iii), the PNH Alliance and PNH Scotland believe that the SMC system of modifiers does not work because successive studies of Eculizumab demonstrate “evidence of substantial improvement in life expectancy and quality of life”. However, the SMC still declined to recommend it for reimbursement by NHS Boards.
- The very small patient populations suffering from ultra-orphan diseases make it extremely difficult for manufacturers and clinicians to generate robust clinical cost effectiveness data required by the SMC HTA process. The very concept of health economic analysis was originally developed to facilitate comparison of products for large patient populations and for which there are alternative treatment options. Conversely, given the small patient populations and general lack of treatment alternatives, it is inappropriate to apply conventional cost effectiveness analyses and thresholds to ultra-orphan therapies. Doing so serves only to entrench the systemic denial of patient access to ultra-orphan medicines as it is extremely unlikely that the HTA Quality Adjusted Life Years (QALY) mechanism (central consideration of the SMC) will ever come close to traditionally accepted levels when assessing ultra-orphan medicines.
- The PNH Alliance and PNH Scotland would support work by the SMC to understand more regarding public views on health spending associated with rare diseases. Such discussions should be held with a high degree of public transparency.

#### **(iv) ABPI / National Procurement**

- The PNH Alliance and PNH Scotland note the comments from ABPI and NHS Scotland, and understand that medicines are broadly priced globally which provides for a lack of bargaining power by the Scottish Government due to the very small patient numbers involved.

### **3. Suggested considerations for the Public Petitions Committee**

- **Request that the Scottish Government undertake a public consultation on new means for appraising ultra-orphan medicines in line with arrangements in England and Wales.**
- **Request that in the interim the Scottish Government designates funding for PNH patients to prevent putting further lives at risk.**
- **That NHS Greater Glasgow and Clyde make publicly available their “expert clinical opinion” on the use of Eculizumab and that this is compared with clinical evidence developed by recognized clinical leaders in the treatment of PNH.**

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<sup>i</sup> THE SCOTTISH GOVERNMENT, NHS Quality Strategy, May 2010: <http://www.scotland.gov.uk/Topics/Health/NHS-Scotland/NHSQuality> ; Accessed 8<sup>th</sup> November 2011

<sup>ii</sup> EMA website; accessed November 2011