

Health Select Committee Inquiry into Commissioning Response Submission from the PNH Alliance

1. Overview:

1.1 The PNH Alliance is a campaign group for Paroxysmal Nocturnal Haemoglobinuria (PNH), a very rare acquired bone marrow disease which frequently leads to transfusion dependence and life threatening thrombosis. Services for PNH are currently commissioned nationally. We are pleased to provide this submission to the committee's questions regarding specialist services.

1.2 Since 2008 the National Commissioning Group (NCG) has designated 2 PNH sites in England – St James' University Hospital in Leeds and King's College Hospital in London. The principles of the NCG PNH services are:

- To provide a national, clinical and laboratory referral service to all patients with PNH in England.
- To provide funding for eculizumab (Soliris®) treatment for all appropriate patients.
- To provide comprehensive information and support.
- To oversee the administration of eculizumab (Soliris®) infusions to patients at their home where possible and supervise the home service team's work.
- To continue to share the care of referred patients with the referring haematology units.
- To provide ongoing education about PNH to other healthcare professionals.

2. Summary:

- The commissioning of specialist services will be under the remit of the NHS Commissioning Board, but will require the engagement of GP Consortia to meet the needs of patients throughout their care pathway.
- The National Strategy for Rare Diseases will be fundamental to the successful commissioning of specialist services.
- There should be no regional disparities in treatment and an end to 'post-code lottery' prescribing.
- Central leadership will be crucial, specifically the creation of a National Clinical Director for Rare Diseases.
- Treatments for rare diseases should be organised centrally and a budget should be given directly to the planning body for specialist services.
- Best practice commissioning guidelines should be developed and shared, and patients should be notified of their legal position when refused treatment for their condition.
- The commissioning process should be as transparent as possible.
- Consistency of approach is important in that constant reorganisation can cause confusion and inefficiency through poor commissioning arrangements and decisions.

3. Response:

3.1 Question: What arrangements are proposed for commissioning of specialist services?

3.1.1 The PNH Alliance welcomes the commitment of the proposed commissioning arrangements to ensure that national and regional services are the responsibility of the NHS Commissioning Board with the support and engagement of GP Consortia.

3.1.2 In relation to existing policy, the PNH Alliance suggests that the National Strategy for Rare Diseases, agreed to at a Council of Ministers session in 2009, is fundamental to the future of commissioning services nationally and regionally and will act as a significant lever to ensure GP Consortia are able to effectively support the commissioning of specialist services. Rare Disease UK (RDUK) is in the process of formulating this strategy and has set up five working groups of expert stakeholders to look into the issues involved. The final report will be launched on Rare Disease Day 2011 (February 28th 2011). One of the five working groups is currently focusing on commissioning and planning and is collating consultation responses until 29th October 2010 on the issue of commissioning specialist services.

3.1.3 The PNH Alliance supports the findings of the RDUK working group on commissioning and planning, and would urge the Health Select Committee to ensure that there is an end to 'post-code lottery' prescribing which resulted from the ineffective workings of the SSNDS (Specialised Services National Definitions Set) and the ten regional SCGs (Specialised Commissioning Groups).

3.1.5 Also as stated by RDUK, the PNH Alliance proposes that all treatments for rare diseases should be organised centrally in order to avoid regional inconsistencies and complex patient pathways. In parallel with this, the specialist commissioning/planning body for rare diseases should be allocated funding directly in order to safeguard specialist services against the current economic climate.

3.1.6 In addition, the PNH Alliance recommends that best practice guidelines for the commissioning specialist services be developed and shared nationally. Also patient guidelines should be developed to inform people of their legal position if refused treatment for their particular disease. These recommendations have also been made by the RDUK consultation.

3.1.4 PNH Alliance believes that central leadership will be key to GP Consortia and the NHS Commissioning Board working practically and effectively together for the organisation and delivery of specialist services. Therefore the PNH Alliance supports the creation of a National Clinical Director for Rare Diseases, as previously suggested by the Chief Medical Officer.

3.2 Question: How will these arrangements interface with the rest of the system?

3.2.1 The PNH Alliance suggests that GP Consortia engagement is an essential requirement for these services to be commissioned and managed appropriately. For many patients their care will not be siloed into national, regional or local services. Treatment and care pathways will more often than not begin at a local level, even if nationally commissioned services are required. Indeed, patients with rare or complex conditions are likely to experience a mixture of both specialist and locally commissioned care. There must therefore be clear understanding and co-ordination between all services commissioned by the NHS. An infrastructure that allows for this is essential.

4. Additional Comment

4.1 From the experience of securing national funding for PNH services, the PNH Alliance would advocate increased transparency in all decision-making on what will be commissioned nationally, regionally and locally. The caveat remains that frequent changes to refine this decision-making process must be avoided.