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The Scottish Government

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DELIVERING
A GAMES LEGACY FOR SCOTLAND

Our Ref: PE1398/PE1399/PE1401
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Dear Anne,

Thank you for your letter dated 20 December 2011 addressed to Anne Lillico/Denise McLister regarding petitions PE1398, PE1399 and PE1401. Your letter set out a number of questions that were raised at the Committee meeting held on 13 December 2011 in relation to the petitions.

I am grateful to the Committee for providing the opportunity to answer these questions and the Scottish Government's response is attached.

Yours sincerely

VERONICA MOFFAT

PE1399/R

PUBLIC PETITIONS COMMITTEE CONSIDERATION OF PE1398, PE1399 AND PE1401 - QUESTIONS ARISING FROM COMMITTEE MEETINGS TUESDAY 4 OCTOBER 2011

The Scottish Government—

- **In the response of 8 November 2011, the Scottish Government states that it will —give consideration to the extant arrangements for appraisal of medicines to treat rare diseases". What form will this consideration take, who will be involved and what is the intended timeframe?**

1. This matter is still under consideration by the Scottish Government Health Directorates.

- **What is the timeframe for the review being carried out by the Chief Medical Officer and Chief Pharmaceutical Officer of the IPTR arrangements?**

2. The clinical Short Life Working Group (SLWG) to consider what actions were needed to further strengthen the safe and effective use of new medicines across the NHS in Scotland met twice in December 2011. The SLWG concluded that there were some improvements that could be made in relation to NHS Board management of formulary arrangements and that further guidance would be issued in early 2012. This is work in progress.

3. In relation to the Good Practice Guidance for NHS Boards management of Individual Patient Treatment Requests (IPTRs) published in March 2011, the SLWG concluded that the guidance had yielded benefits for NHS Boards and should be allowed to "bed in" before any further changes should be considered.

4. However, it was agreed that a number of key messages to underpin the extant guidance would be published in due course together with anonymised examples of IPTR decisions.

5. It was further agreed that the IPTR Good Practice guidance should be kept under review and that any recommendations for refinement to the processes to emerge from the Petitions Committee deliberations of petitions PE1398; PE1399 and PE1401 would be taken forward as appropriate within timescales to be agreed.

- **It is noted that the National Planning Forum is currently reviewing national commissioning for highly specialised services. What is the timeframe for this work?**

6. The National Planning Forum (NPF) commissioned a subgroup in October 2010 to undertake a "review of commissioning arrangements and efficiency of national specialist services and make recommendations to the National Planning forum".

7. The Review Group was asked to make recommendations on:

  - the ongoing management of performance; quality and efficiency of national specialist services by National Services Division (NSD); and

- the governance structure for overseeing the portfolio of national specialist services (including the roles and relationships of National Services Advisory Group (NSAG); the NPF; and the Board Chief Executives Group).

8. The Review Group looked only at commissioned specialist services provided in Scotland and did not consider those services commissioned outwith Scotland, or other Risk Share arrangements managed by NSD. The Review Group"s report was considered by the National Planning Forum on 13 December 2011 when it was agreed that the recommendations should be further developed before being presented to the Board Chief Executives and the Cabinet Secretary for Health, Wellbeing and Cities Strategy.

9. We expect that the work of the National Planning Forum Review Group on the commissioning of national specialist services will be completed by summer 2012.

- **In the earlier response, the Scottish Government states –Within the context of PPRS and procurement legislation the NHS in Scotland can improve the procurement of orphan drugs…" What improvements can be made and what legislation is being referred to?**

Pharmaceutical Price Regulation Scheme (PPRS)

10. In the UK, prices of branded prescription medicines are regulated by the Pharmaceutical Price Regulation Scheme (PPRS). The PPRS has existed since 1957 and is usually negotiated every five years. It is a voluntary scheme agreed between the Department of Health in England and the branded pharmaceutical industry through the Association of the British Pharmaceutical Industry (ABPI). It is underpinned by statutory powers[1].

11. The PPRS has sought to achieve a balance between reasonable prices for the NHS and a fair return for the pharmaceutical industry to enable it to research, develop and market new and improved medicines. Under the PPRS, pharmaceutical companies have freedom of pricing for new active substances. However, the PPRS controls the prices of branded medicines through regulating the profits that pharmaceutical companies are allowed to make on their sales to the NHS.

12. The UK Health Departments do not support additional or alternative initiatives by health authorities in respect of pricing of such supplies in primary care. Individual pharmaceutical companies do occasionally offer a discount on their branded medicines to the hospital sector within NHSScotland. National Procurement within NHS National Services Scotland is recognised as the organisation which procures medicines for the hospital sector and as such works with any individual companies offering a discount to ensure that it is applied to all NHS Boards where applicable. Under these circumstances, there is no need for any OJEU[2] tender provided the branded product is covered by the PPRS agreement and there is only one provider of the specific medicine on which the discount is being offered.

[1] Schedule 5, part 2, Head J, Section J4 of the Scotland Act 1999 reserves to Westminster the regulation of prices charged for medicinal supplies or medicinal products which are supplied for the purposes of the Health Service established under Section 1 of the National Health Services (Scotland) Act 1978.

[2] The Official Journal of the European Union (OJEU) is the central database for European Public Sector tender notices.

Medicines Not Covered under the PPRS

13. For those medicines not covered under PPRS, the Pharmacy Team within National Procurement (NP) manage national contracts for a wide range of medicines used in secondary care within NHSScotland.

Patient Access Schemes

14. A key feature of the 2009 PPRS was the introduction of more flexible pricing options which enable drug companies to improve the value of specific drugs to the NHS. Patient Access Schemes (PAS), which offer discounts or rebates to reduce the cost of a drug to the NHS, have played an important role in helping more patients to access drugs that would not otherwise be assessed as cost-effective by the SMC.

15. The Patient Access Scheme Assessment Group (PASAG) has been established under the auspices of NHS National Services Scotland to deliver a national service to conduct an objective and independent assessment of PAS submitted by pharmaceutical companies on behalf of NHSScotland and advise on their feasibility for implementation by NHS Boards in Scotland.

16. Where a PAS is considered feasible, the SMC is able to take account of the discount offered under the terms of the PAS. Where a PAS is not considered feasible, SMC appraises the drug on its standard costs – i.e. without taking account of the discount offered under the terms of the PAS.

17. The PASAG recently updated their arrangements for PAS. Simple PAS (i.e. those PAS which offer a straight discount from the list price, applied at the point of invoice) are now signed off nationally by National Procurement on behalf of all NHS Boards. Individual NHS Boards still have responsibility for signing off more complex PAS (such as those which require patient tracking or for the Board to provide more detailed data).

- **Will the reviews give consideration to the specific issues raised in recent submissions by Rare Disease UK (PE1398/N), the British Pharmaceutical Industry (PE1398/K), the Association of Glycogen Storage Disorders (UK) (PE1399/M) and PNH Alliance/PNH Scotland (PE1401/N)?**

Rare Disease UK (PE1398/N)

The Scottish Government -

- **The new medicines appraisal process employed by Scotland does not adequately capture the unique nature of rare diseases and the inherent problems in developing medicines for rare diseases. The SMC evaluation should be based on an appraisal of the technology against multiple criteria and not simply a cost-utility analysis.**

18. The Scottish Government believes that the SMC arrangements for the appraisal of orphan medicines are robust and comprehensive. However, as stated previously, Scottish Government policy regarding arrangements for appraisal of new medicines to treat rare diseases is under consideration.

19. The SMC fully recognises that the efficacy data are very often limited due to the rarity of the condition and may therefore accept a greater level of uncertainty in the health economic case when assessing a medicine with an orphan indication. There are also situations when a higher cost per Quality Adjusted Life Year (QALY) may be acceptable and this is factored into their process.

20. SMC would argue that the decision *is* based on an appraisal of the medicine against multiple criteria and not simply based on the results of a cost-utility analysis. How the health benefits to patients are captured in the economic analysis is an important part of a global judgement taken by SMC but other equally important elements influencing the decision are the views of clinical experts, submissions from Patient Interest Groups and whether or not the additional factors termed "SMC modifiers" are considered to apply in the case of the medicine under review.

21. The "SMC Modifiers" include whether the medicine: treats a life-threatening disease; substantially increases life expectancy and/or quality of life; can reverse, rather than stabilise the condition; bridges a gap to a "definitive" therapy; or provides a licensed alternative to a previously unlicensed medicine. The SMC modifiers are always actively considered when reaching a decision on a medicine with orphan status (according to the EMA Committee on Orphan Medicinal Products (COMP)).

22. Where a modifier, or any special issue which may have been highlighted by the sponsor company, by clinical experts and/or Patient Interest Groups, is a factor in SMC acceptance of an orphan medicine, this is stated in the health economics section of the SMC Detailed Advice Document (DAD).

23. In summary, although the findings of the economic analysis are key to the SMC decision, other important factors taken into account and integral to the decision include:

- the needs of patients
- the views of doctors
- the number of people affected by the condition
- what other treatments are available for that condition

- **How policy decisions regarding the SMC are made.**

24. The Scottish Medicines Consortium (SMC) is a consortium of NHS Board Area Drug and Therapeutics Committees (ADTCs). It was established in 2002, initially to provide advice to NHSScotland on the clinical and cost-effectiveness of newly licensed medicines. Over the last 10 years however, its remit has expanded to include annual horizon scanning intelligence on medicines to support territorial Board service/financial planning and the work of the Scottish Antimicrobial Prescribing Group (part of Healthcare Acquired Infection strategy). The work of the SMC is now broader than Health Technology Assessment.

25. Healthcare Improvement Scotland (HIS) has a statutory authority for Health Technology Appraisal (HTA). It has four separate constituent groups within its umbrella, including the SMC, which together lead and deliver a programme of work for NHSScotland on clinical and cost-effectiveness of health technologies, and guidelines development. HIS is the lead organisation for collaboration with the National Institute for Health and Clinical Excellence (NICE).

26. The SMC has had an NHS Policy Group in place since 2004. This group includes NHS Board Chief Executive Officers, Directors of Finance and HIS members of SMC and SGHD observers of SMC, as well as SMC chairs, vice chairs and senior officers. The group meets periodically after SMC meetings to discuss and agree policy issues.

27. Any proposed policy changes to the extant SMC processes as agreed by the SMC NHS Policy Group would be subject to further discussion via Healthcare Improvement Scotland"s Overarching Medicines and Technologies Group (OMTG). The work of OMTG is monitored by the Evidence and Scrutiny Committee chaired by the HIS non-executive board member Professor Lewis Ritchie.

28. The Healthcare Quality Strategy for NHSScotland[3] published in May 2010 provides the focus for all our activity to support our aim of delivering the best quality healthcare to the people of Scotland.

- **The analysis presented by the ABPI provides evidence that the current appraisal process being used by the SMC in relation to orphan medicines are inadequate. Suggest that due consideration is given to the approaches taken by AGNSS in England (for medicines used to treat 500 or fewer patients); the All Wales Medicines Strategy Group (AWMSG) policy (for ultra-orphan medicines with a UK prevalence of 1:50,000); and the Netherlands where orphan drug developers are exempted from providing a full pharmacoeconomic evaluation.**

29. The Scottish Government would question the assertion that the analysis provided by ABPI can be taken as evidence that the SMC appraisal process is inadequate for the consideration of orphan medicines. The Office of Health Economics (OHE) is not independent of the pharmaceutical industry. Furthermore, SMC has reviewed the data used by OHE in their analysis of the SMC decisions on orphan medicines and found that it has some limitations.

30. SMC provided data to the Public Petitions Committee on its decisions on orphan medicines in its response in November 2011.

31. Up to and including October 2011, SMC has assessed 51 full submissions for orphan medicines of which 10 (20%) have been accepted for use and 21 (41%) accepted for restricted use. The remaining 20 (39%) were not recommended. For a further 12 medicines the manufacturer did not make a submission to SMC so these were not recommended. Three orphan medicines have been accepted for use after assessment through the SMC abbreviated submission process. The corresponding figures for medicines without orphan status assessed by SMC are: up to and including October 2011, 422 full submissions have been assessed of which 127 (30%) have been accepted for use, 189 (45%) accepted for restricted use, and 106 (25%) not recommended.

32. These figures illustrate that the SMC acceptance rate for orphan medicines (61%) is lower than the acceptance rate for medicines without orphan status (75%) but SMC maintains that this difference is justifiable.

[3] http://www.scotland.gov.uk/Publications/2010/05/10102307/0

33. The European Union (EU) introduced legislation, REGULATION (EC) No 141/2000, in which pharmaceuticals developed to treat rare diseases are referred to as "orphan medicinal products." The EU's definition of an orphan medicine is defined as one for which the frequency of the disease is less than 5 per 10,000 of the EU population.

34. The purpose of the legislation is to encourage pharmaceutical companies to develop drugs for disease that have a small market. Orphan drug status gives marketing exclusivity in the EU for 10 years after approval. The EU's legislation is administered by the Committee on Orphan Medicinal Products of the European Medicines Agency (EMA).

35. Whilst the Scottish Government is aware of the term “ultra orphan” used by NICE, we are not aware of any formal recognition of this term by relevant regulatory agencies and therefore do not believe it necessary for the SMC to develop a policy for medicines that would fall within this description. The SMC's arrangements for appraising orphan medicines including its modifiers will capture those medicines which would be deemed by NICE to come under the description of “ultra orphan”.

36. As stated previously, Scottish Government policy regarding arrangements for appraisal of new medicines to treat rare diseases is under consideration.

- **The principle of clinicians having to demonstrate the IPTR criteria is extremely difficult and where the IPTR is for an orphan medicine, it is more likely to lead to patients being refused access to the therapy.**

37. The IPTR process is designed to provide an opportunity for clinicians to pursue, on a “case by case” basis for individual patients, a medicine that has not been accepted for routine use by the Scottish Medicines Consortium (SMC) or Healthcare Improvement Scotland. As such medicines have not been considered clinically and cost-effective, they are not expected to be used routinely for patients with the condition in question. The IPTR process is designed for situations where the clinician believes that a patient is likely to gain significantly more benefit from the medicine in question (which has not been recommended by the SMC) than would normally be expected from the group of patients with the condition covered by the medicine's license.

38. It is for the clinician to demonstrate the clinical case for the IPTR request and the means by which to do so are that the patient's clinical circumstances differ from the general group of patients with the condition; or differ from the population of patients who were included in the clinical trial – i.e. the evidence base on which the SMC made their decision.

ABPI (PE1398K)

The Scottish Government -

- **Cost utility, QALY based modelling as employed by the SMC fails to recognise the value orphan medicines bring to patients. The lack of any suitable comparator medicines and the relatively small numbers of patients enrolled in trials can lead to high degrees of uncertainty resulting in unreliable QALY estimates.**

39. Cost-utility analysis uses a measuring instrument or „yardstick" called the Quality Adjusted Life Year (QALY). The QALY approach takes into account that both quality of life and length of life are important. A QALY is based on credible evidence of life years gained through clinical trials and views on the quality associated with that gain. The use of QALYs is currently the accepted method in health economics used by both the Scottish Medicines Consortium (SMC); and the National Institute for Health and Clinical Excellence (NICE). The

QALY has the advantage that it allows treatments for very different conditions and patient groups to be directly compared.

40. Although QALYs provide the basis for discussion within the SMC appraisal of new medicines, the QALY alone does not determine the decision. As stated above, the SMC has developed modifiers which are always actively considered when reaching a decision on a medicine with orphan status (according to the EMA Committee on Orphan Medicinal Products (COMP)). The use of these modifiers in the decision making process compensates for the high degree of uncertainty in the QALY estimates.

41. These modifiers form part of the global judgement by the SMC, which is also influenced by input from clinical experts and Patient Interest Groups as well as the clinical and cost-effectiveness data on the new medicine submitted by the manufacturer. This process allows due consideration to be given to the value orphan medicines bring to patients. SMC has accepted a number of orphan medicines where there has been a high degree of uncertainty in the clinical evidence but the committee has been satisfied of the substantial health benefits that the treatment would provide.

42. A link to an explanation of how QALYs are used in the SMC processes is attached: http://www.scottishmedicines.org.uk/About_SMC/Policy_Statements/A_Guide_to_Quality_Adjusted_Life_Years

43. A link to the NICE website which sets out their use of QALYs is attached; http://www.nice.org.uk/newsroom/features/measuringeffectivenessandcosteffectivenesstheqaly.jsp

- **The specialist commissioning body the Advisory Group for National Specialised Services (AGNSS) to provide access to certain orphan medicines on a national basis.**

National Specialised Services - Scotland

44. For rare diseases and conditions that require highly specialised services that (due to their rareness and specialist nature) are not provided in Scotland because it is impractical and/or not economical to do so, there are long-standing arrangements whereby access is provided to services in England which are nationally designated on behalf of the UK.

45. Such referrals may be arranged via National Services Division within NHS National Services Scotland (NHS NSS) who may hold specific top sliced funds agreed by NHS Boards to fund access. Alternatively, NHS Boards may refer directly on a case by case basis to selected highly specialised services as agreed locally on clinical grounds.

Treatment of Patients through National Specialised Services – England

46. In England, medicines to treat the rare condition are provided as part of the designated service for all patients fitting the clinical criteria.

Treatment of Patients with PNH through National Specialised Services - Scotland

47. Where a Scottish patient is referred to a UK National Specialised Service, a medicine to treat the condition can only be prescribed where the medicine has been accepted for routine use by the SMC unless their local NHS Board decides to fund the medicine following a successful Individual Patient Treatment Request (IPTR).

48. As stated previously at para 18, Scottish Government policy regarding arrangements for appraisal of new medicines to treat rare diseases is under consideration.

Association of Glycogen Storage Disorders (UK) (PE1399/M)

The Scottish Government -

- **Due to the small numbers of patients suffering from Pompe disease, the extant IPTR criteria will not be met.**

49. See comments under paras 37 and 38.

- **What consideration has the Scottish Government given to adopting a similar approach to that of AGNSS for Scotland?**

50. See comments under paras 44 – 48.

- **Within the context of PPRS and procurement legislation, what opportunities are there for the NHS in Scotland to improve the procurement of orphan drugs in order to mitigate against the high cost of these medicines and improve availability?**

51. See comments under paras 10 – 12.

- **The Mackie Report published in September 2010 called upon the Scottish Government to review the situation regarding the unequal treatment of the small number of patients with Pompe disease living in Scotland. Whilst some patients are receiving enzyme replacement therapy, others are being refused this treatment. In England, all patients are able to access this treatment. Has the Scottish Government reviewed this situation, and what is their response?**

52. See comments under paras 37 and 38 and paras 44 -48. Specific advice on the Scottish Government‟s response to the Mackie Report is set out below.

Mackie Report

53. The report *Access to specialist neuromuscular care and social care in Scotland* (known as the Mackie Report) contained the following recommendation (Recommendation 11):

> *11. The Scottish Government reviews the situation regarding the unequal treatment of the small number of patients with Pompe disease living in Scotland. While some patients are currently receiving enzyme replacement therapy, others are being refused this treatment. In England, all patients are able to access this treatment.*

54. The Scottish Muscle Network has been undertaking a valuable programme of work to improve services for people with neuromuscular conditions, including Pompe disease. Members of the Network are meeting with members of the inherited Metabolic Disease Network to discuss the progress that has been made with services and therapy for people with Pompe disease. This meeting is scheduled to take place in March 2012. Healthcare professionals from England will be attending the meeting to share good practice. However,

whilst this work will consider improvement in services, it will not seek to address issues around access to specific medicines which are already the subject of national advice and guidance.

UK Plan for Rare Diseases (EU Council Recommendation)

55. Led by the Department of Health (London), officials from the four UK Health Departments are finalising work on a UK plan for rare diseases in line with the EU Council Recommendation"s request for a report on the implementation of its actions by 2013. The areas under consideration are those contained in the EU Recommendation, and are as follows:

- Plans and strategies in the field of rare diseases;
- Adequate definition, codification and inventorying of rare diseases;
- Research on rare diseases;
- Centres of expertise and European Reference Networks for rare diseases;
- Gathering the expertise on rare diseases at a European level;
- Empowerment of patient organisations; and
- Sustainability.

56. On completion, the draft plan will be the subject of a UK wide public consultation. A single UK consultation document will be produced as soon as possible.

PNH Alliance/PNH Scotland (PE1401/N)

The Scottish Government -

- **The PNH Alliance and PNH Scotland believe it is the responsibility of the Scottish Government to set the criteria and framework within which the SMC operates in order to improve access.**

57. The Scottish Medicines Consortium is a consortium of NHS Board Area Drug and Therapeutic Committees (ADTCs) which was introduced to avoid duplication of new medicines assessment by individual ADTCs, to avoid geographical inequity in decision making and to make the best use of the expertise across the NHS in Scotland. It operates independently from the Scottish Government as a constituent group within Healthcare Improvement Scotland (HIS) which has a statutory authority for Health Technology Appraisal (HTA). The Scottish Government is represented on the SMC as an observer with no voting rights.

- **The PNH Alliance and PNH Scotland welcomes the work of the Committee associated with PE1108 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.**

58. See para 37 regarding medicines which have not been assessed as clinically and cost-effective by the SMC. Eculizumab was launched in the UK for the treatment of Paroxysmal Nocturnal Haemoglobinuria (PNH) in June 2007. In December 2007 SMC issued “not recommended” advice for this medicine as the manufacturer, Alexion, had been unable to make a submission. The company intimated, however, that it was their intention to make a full submission once further data were available to support a health economic case. Over the following two years SMC contacted Alexion on six occasions to request an update

on their submission plans. Up until October 2009 Alexion advised that they were not in a position to make a submission.

59. It is important to note that pharmaceutical companies who wish to have their medicines used within the NHS in Scotland, are required to comply with the submissions process which includes the need for manufacturers to submit clinical and economic evidence according to the principles and standard outlined in the guidance documents. A link to the SMC templates for Industry is attached:
http://www.scottishmedicines.org.uk/Submission_Process/Submission_Guidance_and_Templates_for_Industry/Templates-Guidance-for-Submission/Templates-Guidance-for-Submission

60. As the manufacturer did not supply any health economic analysis and cost-effectiveness was not demonstrated in an independent economic analysis commissioned by the SMC, the SMC was unable to accept eculizumab for use within NHS Scotland. However, it remains open to the pharmaceutical company that manufactures eculizumab to resubmit to the SMC at any time or to submit a Patient Access Scheme to offer a discount or rebate to reduce the cost of the drug to the NHS which may improve its cost-effectiveness. As stated previously at para 18, the extant policy on appraisal of new medicines to treat rare diseases is under consideration by the Scottish Government.

- **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.**

61. The Scottish Government is monitoring the implementation of CEL 17 (2010) and will consider any issues emerging from that process in relation to consistency of approach across NHS Boards, including consistency of approach in relation to IPTRs.

- **The SMC has comprehensively failed PNH community for failing to recommend eculizumab despite the use of modifiers. As eculizumab has been demonstrated to be extremely effective in the treatment of PNH it would suggest that the SMC modifiers do not work in that they fail to take into account the —evidence of substantial improvement in life expectancy and quality of life".**

62. The Scottish Medicines Consortium appraisal arrangements are widely considered to be robust. Any particular queries regarding the methodology applied during the appraisal of eculizumab or conclusions reached should be directed to the SMC.

- **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 IPTRs. We would however, urge the Government to ensure that both of these processes are undertaken in a publically transparent nature, potentially via a public consultation.**

- **(Recommendation 1) The Scottish Government should undertake a public consultation on new means for appraising ultra-orphan medicines in line with arrangements in England and Wales.**

63. As stated previously at para 5, the SLWG agreed that the IPTR Good Practice guidance should be kept under review and that any recommendations for refinement to the processes to emerge from the Petitions Committee deliberations of petitions PE1398; PE1399 and PE1401 would be taken forward as appropriate within timescales to be agreed.

64. As stated at para 18 Scottish Government policy regarding arrangements for appraisal of new medicines to treat rare diseases is under consideration. Any changes to the extant policy would require consultation with appropriate stakeholders.

- **(Recommendation 2) In the interim, the Scottish Government should designate funding for PNH patients to prevent putting further lives at risk.**

65. The extant arrangements for appraisal of new medicines will remain in place until such times as any changes are agreed following due process – i.e. access to eculizumab to treat PNH will continue to be considered via the IPTR process for individual patients.

- **(Recommendation 3) NHS Greater Glasgow and Clyde should make publicly available their –expert clinical opinion" on the use of eculizumab and that this is compared with clinical evidence developed by recognised clinical leaders in the treatment of PNH.**

66. NHS Boards are responsible for the planning and provision of NHS services for their patient populations. NHS Board consideration of IPTRs are taken on a "case by case" basis reflecting clinical opinion on each individual patient"s clinical circumstances. As such, these cannot be generalised.

Scottish Medicines Consortium –

- **PNH is an ultra-orphan condition and the PNH Alliance and PNH Scotland recommend that the SMC formally recognise it in line with the definitions stipulated in England by NICE and in Wales by AWMSG.**

67. See comments under para 35.

- **The PNH Alliance and PNH Scotland note from their own analysis that of the 12 medicines licensed by the EMA for ultra-orphan conditions, the SMC only recommends one for restricted use.**

- **It is inappropriate to apply conventional cost effectiveness analyses and thresholds to ultra-orphan therapies and the SMC processes should reflect this.**

- **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.**

68. As stated at para 18, Scottish Government policy regarding arrangements for appraisal of new medicines to treat rare diseases is under consideration. Any changes to the extant policy would require consultation with appropriate stakeholders.