Rare Disease UK (RDUK) is grateful to the Public Petitions Committee for taking forward PE1398 'Access to therapy for orphan diseases' and welcomes the opportunity to comment on the response from the Scottish Government dated 1st February 2012.

Individual Patient Treatment Requests

- RDUK is aware of consideration given to the extant arrangements for appraisal of medicines to treat rare diseases and has been made aware of the document dated 13th February 2012 'Guidance to further strengthen the safe and effective use of medicines across the NHS in Scotland'. RDUK continue to be concerned that the current criteria for accessing orphan medicines through an Individual Patient Treatment Request (IPTR) is too onerous for patients with rare diseases.

  The criteria for IPTRs state - "The patient's clinical circumstances (condition and characteristics) are significantly different from either:
  - the general population of patients covered by the medicine's license; or
  - the population of patients included in the clinical trials for the medicine's licensed indication as appraised.

  In rare diseases it is extremely difficult to demonstrate the above criteria. The small patient numbers who make up the clinical trial populations are those patients with the greatest clinical need for the drug and therefore the license will be based on this group of patients. It is therefore extremely difficult to show that a patient with genuine clinical need will be "More likely to benefit from the medicine than might be expected for other patients with the condition". The patients who are likely to have the greatest need for the treatment will be the same as those patients within the clinical trials upon whom the license is based. Unlike in some of the more common conditions and even certain cancers where there is often more than one licensed treatment available, in the majority of rare diseases there is likely to be only one licensed treatment available, apart from just supportive care. In orphan diseases the above criteria are therefore more likely to lead to those patients with the greatest clinical need being refused access to therapies, which may be life changing and / or life saving.

- Point 37 of the response explains that '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 than would normally be expected from the group of patients with the condition covered by the medicine's license'. RDUK would appreciate an explanation of the term 'significantly more benefit' and asks how it is ascertained whether the clinician involved in this assessment is the best possible clinical expert in that particular condition.

- We welcome the willingness following the reviews by the SLWG to consider further refinement of the IPTR Good Practice guidance in relation to the findings from the results of our petition and PE1399 and PE1401. To ensure appropriate refinements are able to be made, RDUK calls upon the Public Petitions Committee to ask the Scottish Government for a thorough review of the current criteria in relation to the implementation of their IPTR process specifically in regards to orphan medicines. A review should involve all relevant stakeholders, including patient organisations, industry, health boards and clinicians.

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The Scottish Medicines Consortium appraisal process for orphan medicine

- The Scottish Government response states that 'Scottish Government policy regarding arrangements for appraisal of new medicines to treat rare diseases is under consideration'. RDUK asks the Scottish Government to provide the details of this consideration, in particular, a timeline for when a decision on this matter is likely to be made. RDUK would also call upon the Scottish Government to consult with all relevant stakeholders, including patient organisations, industry, health boards and clinicians.

- The Scottish Government response states 'The Scottish Government believes that the SMC arrangements for the appraisal of orphan medicines are robust and comprehensive'. RDUK calls on the Scottish Government to undertake an open and transparent public review to instil confidence in RDUK's members that this is the case. Although there are many positive elements of the way the SMC conducts its appraisals, RDUK remains fundamentally concerned that the process used cannot adequately appraise orphan drugs equitably.

- It is stated that 'the economic analysis is an important part of a global judgement taken by the SMC but other equally important elements influencing the decision are the views of clinical experts…' RDUK asks the SMC to explain the criteria used to identify experts for rare diseases. The nature of rare diseases means that expertise is often scattered. Often, an expert in a particular rare condition may be located out with Scotland or even the UK. Does the SMC take into account the views of an expert in a particular rare disease when that expert is out with Scotland?

- RDUK wishes to thank the Scottish Government for clarifying which body has statutory authority for Health Technology Appraisal. RDUK calls on the Public Petitions Committee to write to Healthcare Improvement Scotland's Overarching Medicines and Technologies Group to ask for its opinion on the calls made by PE1398.

- The Scottish Government highlights that 'The Healthcare Quality Strategy for NHSScotland 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'. Does the Scottish Government acknowledge that the current SMC methodology relating to orphan medicines fails the third quality ambition 'Effective: The most appropriate treatments, interventions, support and services will be provided at the right time to everyone who will benefit, and wasteful or harmful variation will be eradicated'.

- RDUK note the Scottish Government's assertion that the OHE data, used by the ABPI in their response to the Public Petitions Committee, has 'some limitations'. RDUK would be interested in further details of these limitations and asks the Scottish Government to commission their own independent analysis of the SMC decisions on orphan medicines as part of a wider public review into the SMC's arrangements for the appraisal of orphan medicines.

- Point 32 of the Scottish Government response state that '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'. RDUK asks for further elaboration on why the SMC believe this is justifiable?

- Point 39 acknowledges that 'the use of QALYs is currently the accepted method in health economics used by both the Scottish Medicines Consortium and the National Institute for Health and Clinical Excellence (NICE).' However, when appraising medicines for conditions effecting fewer than 500 patients in England, NICE recognise that QALYs are not an appropriate measure and refers such medicines for appraisal by AGNSS which takes into account a broader range of criteria. Would the Scottish Government consider using a separate mechanism for appraising orphan or ultra-orphan medicines in Scotland?

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Summary of requests

Strategy for Rare Diseases

- RDUK calls upon the Public Petitions Committee to ask the Scottish Government what plans it has in place for the implementation of a National Plan for rare diseases, in particular, when will a consultation on the strategy for rare diseases will be launched?

Individual Patient Treatment Requests

- RDUK would appreciate an explanation of the term 'significantly more benefit' and asks how it is ascertained whether the clinician involved in this assessment is the best possible clinical expert in that particular condition.

- RDUK calls upon the Public Petitions Committee to ask the Scottish Government for a thorough review of the current criteria in relation to the implementation of their IPTR process specifically in regards to orphan medicines, involving all relevant stakeholders, including patient organisations, industry, health boards and clinicians.

The Scottish Medicines Consortium appraisal process for orphan medicine

- RDUK asks the Scottish Government to provide the details of it's consideration of the arrangements for appraisal of new medicines, in particular, a timeline for when a decision on this matter is likely to be made. RDUK would also call upon the Scottish Government to consult with all relevant stakeholders, including patient organisations, industry, health boards and clinician.

- RDUK calls upon the Public Petitions Committee on the Scottish Government to undertake and open and transparent public review into the SMC's arrangements for the appraisal of orphan medicines.

- RDUK asks the SMC whether it takes into account the views of an expert in a particular rare disease when that expert is out with Scotland.

- RDUK calls on the Public Petitions Committee to write to Healthcare Improvement Scotland's Overarching Medicines and Technologies Group to ask for its opinion on the calls made by PE1398.

- Does the Scottish Government acknowledge that the current SMC methodology relating to orphan medicines fails the third quality ambition 'Effective: The most appropriate treatments, interventions, support and services will be provided at the right time to everyone who will benefit, and wasteful or harmful variation will be eradicated'.

- RDUK asks the Scottish Government, to commission their own independent analysis of the SMC decisions on orphan medicines.

- RDUK asks for further elaboration on why the SMC believe the difference between the acceptance rate for orphan medicines and those medicines without orphan status is justifiable.

- Will the Scottish Government consider using a separate mechanism for appraising orphan or ultra-orphan medicines in Scotland?

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