Dear Mr Windsor

Health and Sport Committee Meeting on 18 September 2012

Please find attached as background information for the above meeting, a briefing paper which reflects the extant Scottish Government policy on the managed introduction of newly licensed medicines within the NHS in Scotland.

Please do not hesitate to contact me should you have any queries.

Yours faithfully

Veronica Moffat
Policy Lead, New Medicines
HEALTH AND SPORT COMMITTEE MEETING ON 18 SEPTEMBER 2012 - SCOTTISH GOVERNMENT BRIEFING PAPER

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Petitions PE1398; PE1399 and PE1401

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<td>Petition calls on the Scottish Parliament to urge the Scottish Government to review the mechanism and methodology used by the Scottish Medicines Consortium to appraise the value of medicines for orphan diseases and to instruct the Chief Medical Officer to revise the criteria for accessing Individual Patient Treatment Requests by removing the term “exceptional” from all Health Board IPTR requests in relation to orphan diseases.</td>
<td>Alastair Kent on behalf of Rare Disease UK on 2 September 2011</td>
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<td>1399</td>
<td>Petition calls on the Scottish Parliament to urge the Scottish Government to instruct the Chief Medical Officer (CMO) to revise the criteria to access IPTRs for Orphan diseases as these criteria are detrimental to patients suffering from Pompe disease.</td>
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<td>1401</td>
<td>Petition calls on the Scottish Parliament to urge the Scottish Government to review the mechanism and methodology used by the Scottish Medicines Consortium to appraise medicines for rare diseases and to instruct the Chief Medical Officer to revise the criteria by which Health Boards assess Individual Patient Treatment Requests in order to improve access to therapy for patients with paroxysmal nocturnal haemoglobinuria (PNH)</td>
<td>Lesley Loeliger on behalf of PNH Scotland and Professor Hillmen on behalf of the PNH Alliance on 13 September 2011</td>
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KEY MESSAGES TO CONVEY

- Medicines are an essential part of clinical care for patients.

- NHS Boards in Scotland are responsible for making sure clinical care is optimised through the most effective and efficient use of the most appropriate medicines.

- The Scottish Government is committed to patients in Scotland receiving medicines of established cost-effectiveness and therapeutic value.

- The drugs bill in Scotland for 2010/11 was in the region of £1.3bn which represents approximately 12% of NHS Board expenditure.

- Scotland has independent, robust and internationally respected arrangements in place for the appraisal newly licensed clinically and cost-effective medicines to treat all conditions, not just those to treat rare diseases.

- We have also been working to improve the system overall by implementing previous Petitions Committee recommendations.

- It is important that any policy proposal in this area does not compromise the independence and equity of our current arrangements.
SECTION C

KEY POINTS - NEW MEDICINES APPRAISAL ARRANGEMENTS

National Appraisal Arrangements

- As health is a devolved issue, decisions regarding the introduction of new medicines and treatments are taken by each of the UK countries through their own appraisal and assessment arrangements in line with established national priorities.

- The arrangements in place in Scotland for appraising newly licensed medicines and treatments are through bodies such as the Scottish Medicines Consortium and Healthcare Improvement Scotland.

- The Scottish Medicines Consortium (SMC) is a consortium of NHS Board Area Drug and Therapeutics Committees (ADTCs).

- The SMC was introduced to make the best use of expertise available across Scotland in the assessment of new medicines.

- The Scottish Medicines Consortium and Healthcare Improvement Scotland processes for providing advice on new medicines are independent of the Scottish Government. These processes are widely acknowledged to be robust.

- SMC appraises new medicines submitted by the manufacturer. It is the responsibility of the manufacturer to submit to SMC to have their medicine appraised for use in NHS Scotland.

- The SMC encourages members of the public to get involved in its work. The Patient and Public Involvement Group (PAPiG) is responsible for ensuring that the patient/carer perspective is always taken into consideration by the SMC.

- The SMC appraises all new medicines as soon as possible following licensing and aims to issue advice to NHS Boards on their clinical and cost-effectiveness within 18 weeks of receipt of the company submission – these timelines being among the fastest of any country in the world undertaking medicines appraisals.

- Healthcare Improvement Scotland considers the applicability of Multiple Technology Appraisal (MTA) recommendations from the National Institute for Health and Clinical Excellence (NICE) within the Scottish context and issues advice to NHS Boards in Scotland at the same time as NICE does in England.

- When the SMC has published “not recommended” advice in relation to a new medicine, they encourage the company to resubmit based on new clinical or health economic data and in many cases (40%), the pharmaceutical company chooses to do so.
The SMC employs open and consultative working procedures and methodology by maintaining dialogue between the SMC and sponsor pharmaceutical companies. This helps to minimise the number of occasions on which serious disagreements in relation to process or scientific assessment can occur. A comprehensive description of these arrangements can be accessed from the SMC website via the following link:
http://www.scottishmedicines.org.uk

**Health Economics Tool to Measure Benefits of Medicines**

- Medicines can prolong life, improve the quality of life, or both. Because the SMC appraises all newly licensed medicines for different purposes, there needs to be a means to compare the value of different medicines in a meaningful way.

- The SMC uses the Quality Adjusted Life Year (QALY). The QALY is a widely used economic indicator or tool which takes into account how a medicine affects a patient’s:
  - quantity of life (how long you live for); and
  - quality of life (the quality of your remaining years of life).

- The QALY combines both these factors into a single measure that puts a figure on the health benefits for any medical treatment including medicines.

- QALYs provide a benchmark that can be used to measure and compare the benefits that each medicine is likely to offer.

- In the SMC appraisal, QALYs provide the basis for discussion, the QALY alone does not determine the decision.

**SMC Modifiers**

- The SMC has developed modifiers to be used when appraising medicines in particular categories where the cost per QALY is in excess of the normal parameters.

- Subject to meeting certain criteria, application of modifiers allows the SMC to take into account additional factors and for the cost per QALY to be viewed flexibly with the potential to recommend a medicine notwithstanding the economic evidence provided.

- The list of SMC modifiers has been published on the SMC website.

**Patient Access Schemes**

- Scotland has introduced national arrangements to consider Patient Access Schemes (PAS) as a way of potentially reducing the overall cost of new medicines to the NHS and improve their cost-effectiveness.

- Scotland has established a national group to assess proposed schemes against standard objective criteria and provide advice to the Scottish Medicines Consortium on their operational feasibility.
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 medicine on its standard costs – i.e. without taking account of the discount offered under the terms of the PAS.

To date, the SMC has accepted or accepted for restricted use 18 medicines which have included a Patient Access Scheme. Of these, 5 were orphan medicines.

It is the responsibility of the manufacturer to submit a Patient Access Scheme as part of the SMC appraisal of the new medicine, where they want to use this as an option to making their medicine available in NHS Scotland.
KEY POINTS – NHS BOARD IMPLEMENTATION OF SMC ADVICE

General

- NHS Boards and clinicians are expected to take full account of the advice provided by the SMC and Healthcare Improvement Scotland in the planning and provision of NHS services.

- The implementation of these are a matter for NHS Boards and Area Drugs and Therapeutics Committees (ADTCs) which operate within NHS Board governance arrangements.

- The ADTC is the key clinical group to advise on and ensure safe systems for medicines use and the provision of formularies to support the cost effective use of medicines.

- The development of formularies is an iterative process. The ADTC considers the SMC recommended medicines against their existing treatment options. It is for the clinical community to determine which medicines should be available for routine use in the NHS Board.

SMC Accepted Medicines

- Where the SMC has accepted a medicine for routine use, NHS Boards are expected to make it, or its equivalent, available.

- SMC “accepted” medicines are considered locally through the NHS Board Area Drug and Therapeutics Committees through one of two routes:
  
  o through ADTC consideration of the SMC accepted advice as a matter of course by taking full account of the SMC’s assessment of the medicine’s therapeutic advancement over and above comparator medicines to treat the condition in question; considering its place in therapy within current treatment pathways; agreeing the treatment protocol; and assessing its resource and service implications. or
  
  o through a process whereby application for formulary inclusion for the newly licensed medicine is proactively sought from the appropriate NHS clinicians in the Board area.

- The Scottish Government Guidance on the Introduction and Availability of Newly Licensed Medicines in the NHS in Scotland\(^1\) which was published on 17 May 2010 provided a framework within which NHS Boards are expected to align their policies regarding access to newly licensed medicines.

• The Scottish Government Guidance to Further Strengthen the Safe and Effective Use of New Medicines Across the NHS in Scotland\(^2\) which was published on 13 February 2012 included a specific guidance framework for NHS Board formulary decision-making.

• The guidance clarified that NHS Boards are expected to reach a decision on an SMC accepted medicine within 90 days of the issue of the SMC advice to NHS Boards (NB: this advice is confidential for the first 30 days); the guidance also clarified that NHS Boards are expected to publish on the website, the formulary decision within 14 days of the decision being reached.

• NHS Boards were asked to confirm by 1 April 2012 that their policies on formularies had been updated to reflect the additional guidance. All NHS Boards have provided this confirmation.

SMC “Not Recommended” Medicines

• Where the SMC has published advice to confirm a medicine is not recommended, NHS Boards are not expected to make it routinely available, as it has not been assessed as being cost effective.

Individual Patient Treatment Requests (IPTRs)

• However, NHS Boards have arrangements in place for clinically-led consideration of medicines which are “not recommended” by the SMC for individual patients in certain circumstances.

• Such consideration is given on a on a “case by case” basis for individual patients through the Individual Patient Treatment Request (IPTR) arrangements.

• These allow clinicians to put forward a case for an individual patient to receive an SMC “not recommended” medicine if they feel that they can demonstrate that the patient’s clinical circumstances are such that they are likely to gain significantly more benefit from the medicine than would normally be expected.

• Medicines which are “not recommended” due to a non-submission (by the manufacturer), can also be considered under certain clinical circumstances.

• Consensus-based Good Practice Guidance on NHS Board Management of Individual Patient Treatment Requests (IPTRs) was published on 18 March 2011 under cover of SGHD/CMO(2011)3\(^3\). This guidance provided a framework to support local NHS Boards decision-making on IPTRs through a consistent approach.

• As part of our commitment to monitor implementation of CEL 17 (2010) which took effect from 1 April 2011, data on IPTR was collected for the period 1 April 2011 – 31 March 2012. A Summary Report is attached for information at Section D. This indicated that overall 291 IPTR requests for 67 medicines were received during the reporting period of which 194 were accepted (67%).

\(^3\) [http://www.sehd.scot.nhs.uk/cmo/CMO(2011)03.pdf](http://www.sehd.scot.nhs.uk/cmo/CMO(2011)03.pdf)
KEY POINTS - ORPHAN MEDICINES

General

- Medicines to treat rare diseases are described as “orphan” medicines. These are medicines intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than 5 in 10,000 people in the European Union at the time of submission of the designation application.

- There is no formal or internationally agreed definition for an “ultra orphan” medicine although we are aware that this term is used to describe medicines to treat diseases with a UK prevalence of less than one in 50,000 of the population.

- The submitting manufacturer for an orphan medicine is required to make the health economic case for cost-effectiveness to the SMC in the same way as manufacturers do for all new “non-orphan” medicines.

- In reaching a decision on whether an orphan medicine can be accepted for routine use within NHSScotland, the SMC recognises that data are very often limited due to the rarity of the condition and may therefore accept a greater level of uncertainty in the economic case.

- Although the SMC does not formally distinguish between orphan medicines and those that would be regarded by some other national appraisal bodies as “ultra-orphans”, SMC modifiers are always taken into consideration for any medicine with orphan status.

Paroxysmal Nocturnal Haemoglobinopathy Workshop Held on 24 May 2012

- NHS National Services Scotland hosted a workshop on 24 May 2012 to consider how best to achieve a consistent approach on how NHSScotland provides an effective service for patients diagnosed with paroxysmal nocturnal haemoglobinuria (PNH).

- The workshop brought together senior haematologists in Scotland together with pharmacists and senior health professionals within the Scottish Government.

- It was agreed that IPTR requests for eculizumab to treat PNH would be assessed on their own merits by NHS Boards, based on a full and detailed consideration of individual patient circumstances and informed by specialist advice from the PNH specialist service at Monklands.
NHS Board Chief Executive Risk Share Arrangements for Orphan Medicines

- NHS National Services Scotland (National Services Division) administers an Orphan Drugs Risk Share scheme through which NHS Boards can pool funds to share the expense of specific high cost medicines required by very small numbers of patients in Scotland.

- All new medicines included in the risk share arrangements must have been accepted or accepted for restricted use within their licensed indication by the SMC.

- The only exception to this principle relates to three Enzyme Replacement Therapies which have been previously agreed for inclusion in the Orphan Drugs Risk Share (subject to a successful Individual Patient Treatment Request (IPTR) at the patient’s Board of residence.)

- Unless there are special circumstances, medicines which have not (or have not yet) been recommended by the SMC are excluded from the Orphan Drugs Risk Share (including those medicines which have not been recommended as a result of a non-submission by the manufacturer).

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4 An example of special circumstances would be where there was a shortage of an SMC accepted medicine which would allow consideration of temporary inclusion on the Orphan Drugs Risk Share of a medicine which had not (or not yet) been accepted for use by the SMC.
Pharmaceutical Price Regulation Scheme (PPRS)

- In the UK, prices of branded prescription medicines are regulated by the Pharmaceutical Price Regulation Scheme (PPRS).

- The PPRS is a voluntary scheme agreed between the Department of Health (DH) and the branded pharmaceutical industry. It is underpinned by statutory powers.

- The PPRS has existed since 1957 and is usually renegotiated every five years.

- The PPRS has sought to achieve a balance between reasonable prices for the NHS and a fair return from 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.

- Negotiations between the DH and the Association of the British Pharmaceutical Industry (ABPI) regarding PPRS 2014 are underway. The Scottish Government Health Department are consulted by the DH to contribute to the DH negotiations position.

Value-Based Pricing

- The UK Government set out in the coalition agreement, their intention to introduce a new system of value-based pricing by reforming arrangements for the pricing of branded medicines when the current PPRS expires at the end of 2013.

- The purpose of this approach is to improve NHS patients’ access to effective and innovative drugs by ensuring they are available at a price that reflects the value they bring.

- The Devolved Administrations are working closely with the DH in designing the value based system and the successor to PPRS to ensure a co-ordinated and coherent approach.
KEY POINTS – ABOLITION OF PRESCRIPTION CHARGES

- The SNP Manifesto in 2007 committed to “immediately abolish prescription charges for people with chronic health conditions, with cancer and for people in full time education – and to phase out prescription charges for the rest of the population by 2012”.

- Abolishing prescription charges is the fairest and simplest way of removing this tax on ill health, it restores the NHS to its founding principles that services should be free at the point of delivery and should be based on clinical need and not ability to pay.

- Wales abolished prescription charges in 2007 and Northern Ireland abolished prescription charges on 1 April 2010.
The Act, which received Royal Assent on 31 March 2011, aims to improve patients’ experiences of using health services and to support people to become more involved in their health and health care.

It will help us to meet our aspiration for an NHS which respects the rights of both patients and staff and from 1 April 2012 gives all patients the right that the health care they receive should:

- consider their needs;
- consider what would be of optimum benefit to them; and
- encourage them to take part in decisions about their health and wellbeing and provide information and support for them to do so.

The Act does not embody a right to access a certain treatment or medicine; rather it deals with general principles about how healthcare should be delivered. It does not give rights to specific treatments or medicines, or for specific conditions.

It also sets out that people who provide NHS care (such as doctors, nurses, dentists) must, from 1 April 2012, take into account a set of Health care Principles when providing services. This includes sections on Patient Focus; Quality Care and Treatment, Patient Participation; Communication; Patient Feedback; and Waste of Resources.

The Act also makes provision for the introduction on 1 October 2012 of:

- a Charter of Patient Rights and Responsibilities which will bring together, in one place, a summary of the rights and responsibilities that patients have when using NHS services;

- the introduction of a 12 Week Treatment Time Guarantee whereby eligible patients who are receiving planned treatment provided on an inpatient or day-case basis will not wait longer than 12 weeks from the date that the treatment is agreed to the start of that treatment. Some examples of treatments include hip or knee replacements or hernia surgery.

Action to deliver the rights and principles should be proportionate and appropriate to the circumstances and should balance the rights of the individual patients with the effects on the rights of other patients. It should also take into account resources available and the responsibility of the Health Board to use resources efficiently and effectively.
KEY POINTS - NHS BOARD FEEDBACK ON SCOTTISH GOVERNMENT GUIDANCE RELATED TO NEW MEDICINES

General

- As part of our commitment to monitoring implementation of CEL 17 (2010), the Scottish Government wrote to NHS Boards seeking feedback on the extent to which that, and subsequent guidance, had assisted NHS Board local decision making – and in particular – IPTRs.

- There was 100% response rate to the questionnaire.

- All Boards were able to confirm that training had been provided for the IPTR panels through either awareness sessions or specific training identified by individuals.

- NHS Boards are working together with neighbouring Boards to achieve consistency of approach and will continue to develop this collaborative working to achieve consistency of approach across Scotland.

- The Scottish Government will give consideration to how best to pursue a national event to support this objective.
Monitoring of NHS Board Data on Individual Patient Treatment Requests received for the period 1 April 2011 – 31 March 2012

Introduction

NHS Boards were asked to return data relating to Individual Patient Treatment Request (IPTR) requests for the periods 1 April to 30 September 2011 and 1 October 2011 to 31 March 2012. This report anonymises the collated data that was returned to ensure that individual patients cannot be identified.

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 by the Scottish Medicines Consortium (SMC) or Healthcare Improvement Scotland (formerly NHS Quality Improvement Scotland) following their appraisal on clinical and cost-effectiveness.

Circumstances under which IPTRs will be Considered

IPTR as described in CEL 17 (2010) relates to newly licensed medicines that have not yet been appraised by the SMC or have been appraised by the SMC and are not recommended for use.

An IPTR application for a new medicine may be made when:

(i) the SMC or HIS have issued “not recommended” advice for the medicine;
(ii) the request relates to the use of the medicine outwith an SMC restriction;
(iii) before SMC or HIS has issued advice on the medicine.

Referral Criteria

The clinician making the application is expected to demonstrate the clinical case for the patient. The patients clinical circumstances (condition and characteristics) are required to be significantly different from either:

(i) the general population of patients covered by the medicines licence; or
(ii) the population of patients included in the clinical trials for the medicines licensed indication as appraised.

Non IPTR Applications for Medicines for Individual Patients

Some NHS Boards also consider applications for unlicensed medicines, off label use of medicines, non-formulary medicines and surgical or interventional procedures at their IPTR

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5 IPTRs should not be used to circumvent established assessment processes. Where no SMC or Healthcare Improvement Scotland advice is available due to a non-submission, the policy position across Scotland is that a medicine would not be expected to be routinely available. However, NHS Boards can consider IPTRs where the clinician responsible for the patient’s care believes a delay in treatment would result in a significant adverse outcome for the patient.

6 Where SMC/HIS advice is awaited, the policy position across Scotland is that a medicine would not be expected to be routinely available. However, NHS Boards can consider IPTRs in such instances where the clinician responsible for the patient’s care believes a delay in treatment pending such advice would result in a significant adverse outcome for the patient.
panels. Such requests do not fall within the IPTR criteria as set out above and therefore for the purposes of reporting on IPTRs (e.g. any periodical requests coming from the Scottish Government or requests submitted under Freedom of Information Legislation), these other requests should not be included. The data returned on these ‘non-IPTR’ requests has not been included in this report.

Summary of IPTR requests

All 14 of the territorial Boards returned data, however 2 of these had no IPTR applications during the period reported.

In total 291 IPTR requests were made, 194 were accepted (66%).

It is important to note that in anonymising the data to preserve patient confidentiality, some of the detail is lost. For example boards vary in size so naturally some have small numbers of IPTR requests, which therefore means comparison between different boards should be interpreted with care and may not yield any useful information.

Acceptance rates for IPTR applications by NHS Board

(NB: Boards C and J had no IPTR requests during this time period)

The 291 IPTR requests were for a total of 67 different drugs. It would be very difficult to interpret data at an individual drug level, as each request relates to a patient. To protect the anonymity of patients no information is included on the number of times a drug was requested or to detail the drugs requested.

It has not been possible to formally analyse for any patterns to the requests, the data can be summarised as follows:

- of the requests approved for use, this related to 57 different drugs; and
- of the requests not approved this related to 33 different drugs, of these 11 different drugs were never accepted for use.