The Scottish Parliament
Pàrlamaid na h-Alba

HEALTH AND SPORT COMMITTEE

AGENDA

24th Meeting, 2012 (Session 4)

Tuesday 18 September 2012

The Committee will meet at 9.30 am in Committee Room 1.

1. **Decision on taking business in private:** The Committee will decide whether to take items 3 and 4 in private.

2. **Access to new medicines** The Committee will take evidence from—

   Professor Angela Timoney, Chairman, and Dr Jonathan Fox, Chairman, New Drugs Committee, Scottish Medicines Consortium;

   Professor David Webb, Consultant Physician, Royal Infirmary, Edinburgh and Christison Professor of Therapeutics and Clinical Pharmacology, University of Edinburgh, Royal College of Physicians of Edinburgh;

   Dr Rachel Green, Associate Medical Director - Diagnostic Directorate, NHS Greater Glasgow and Clyde;

   David Pfleger, Director of Pharmacy & Medicines Management, NHS Grampian;

   Melinda Cuthbert, Lead Pharmacist Lothian Medicines Information Service/Yellow Card Centre Scotland, NHS Lothian;

   Andy Powrie-Smith, Director, Sandra Auld, Operations Director, and Dr Frances Macdonald, Pharmaceutical Industry Representative on SMC and Chairman of SMC User Group Forum, Association of the British Pharmaceutical Industry.

3. **Draft Budget Scrutiny 2013-14:** The Committee will consider its approach to the scrutiny of the Scottish Government's Draft Budget 2013-14.

4. **Work programme:** The Committee will consider its work programme.

HS/S4/12/24/A

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The papers for this meeting are as follows—

Agenda Item 2

Submission from ABPI Scotland	HS/S4/12/24/1
Submission from Scottish Medicines Consortium	HS/S4/12/24/2
Submission from The Royal College of Physicians of Edinburgh	HS/S4/12/24/3
PRIVATE PAPER	HS/S4/12/24/4 (P)
Responses from NHS Boards to IPTRs questionnaire	HS/S4/12/24/5
SPICe Briefing	HS/S4/12/24/6

Agenda Item 3

PRIVATE PAPER	HS/S4/12/24/7 (P)

Agenda Item 4

PRIVATE PAPER	HS/S4/12/24/8 (P)

HEALTH AND SPORT COMMITTEE

ACCESS TO NEWLY LICENSED MEDICINES

Submission from ABPI Scotland

The ABPI (Association for the British Pharmaceutical Industry) represents innovative research-based biopharmaceutical companies, large, medium and small, leading an exciting new era of biosciences in the UK.

Our industry, a major contributor to the economy of the UK, brings life-saving and life-enhancing medicines to patients. Our members supply 90 per cent of all medicines used by the NHS, and are researching and developing over two-thirds of the current medicines pipeline, ensuring that the UK remains at the forefront of helping patients prevent and overcome diseases.

The ABPI is recognised by government as the industry body negotiating on behalf of the branded pharmaceutical industry, for statutory consultation requirements including the pricing scheme for medicines in the UK.

ABPI Scotland welcomes the opportunity to submit this evidence to the Committee.

Introduction

ABPI Scotland is pleased to submit this written evidence ahead of the September 18 2012 hearing by the Health and Sport Committee into Access to Medicines. The public, clinicians and the industry want to see access to new medicines for patients in Scotland. Aligned with this desire, in June 2012 the Scottish Government published *Health and Wealth in Scotland: A Statement of Intent for Innovation in Health*[i], which recognises that access to new medicines is an important element in delivering innovation into the heart of NHSScotland. The 2012 Scotland Against Cancer Conference, run by Cancer Research UK, also highlighted that the future of cancer research "needs access to the latest medicines so that they can provide standard care to encourage new drug research to be located in Scotland".[ii] The challenge for the NHS and the Scottish Government Health Department is to realise the benefits for patients from these treatments within constrained budgets. The evidence suggests that access to new medicines for patients in Scotland is problematic and variable and the uptake of new medicines in Scotland is low and slow in comparison with other countries.

Section 1: Context

1. The discovery, research, development and clinical trials of a medicine takes on average over a decade and costs over £1 billion.[iii] As the public purse does not fund this process, companies shoulder the risk so the price reflects both the investment made to bring the successful medicine to patients and the costs of those which failed in development. In the UK, under the Pharmaceutical Price Regulation Scheme, and unlike the situation for any other supplier to the NHS, government caps the profits that can be made by the medicines industry. UK Government Department of Health (DoH) figures show that the price of medicines in the UK is

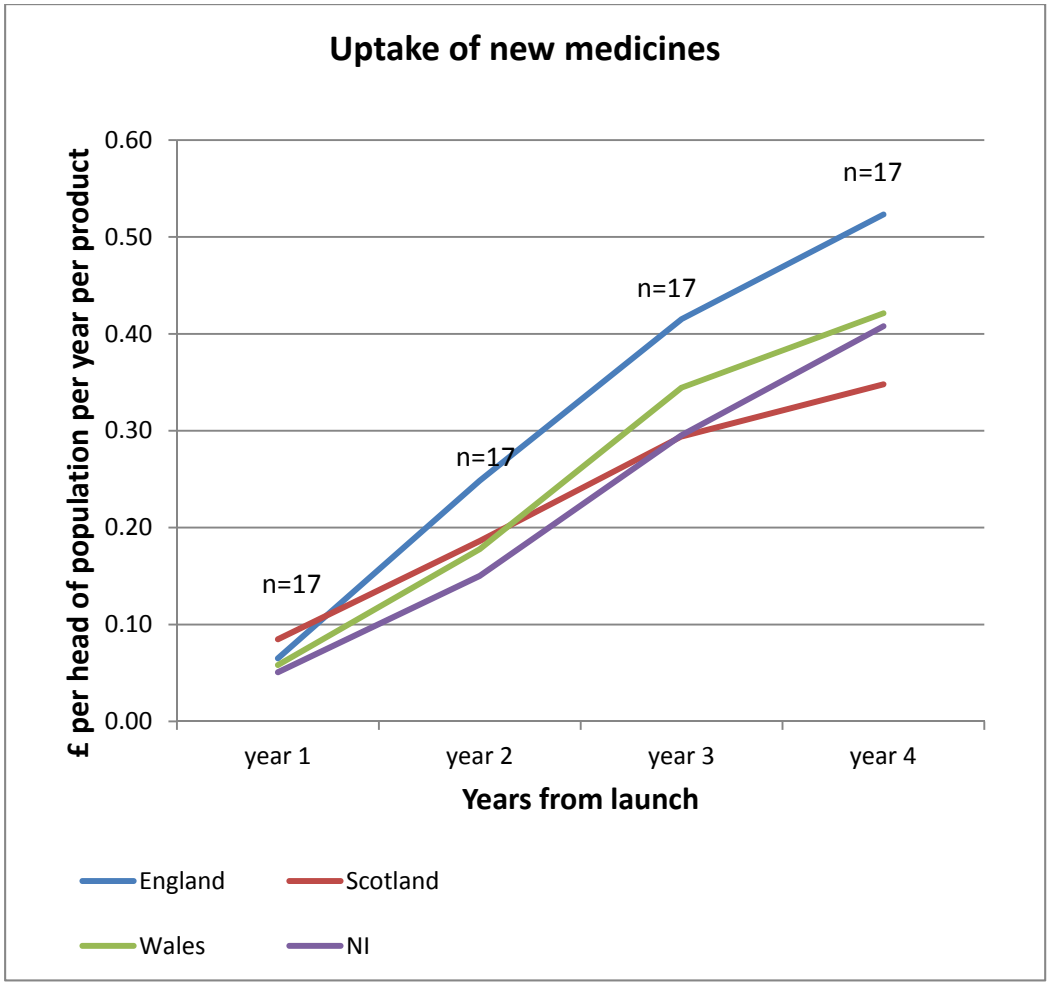
amongst the lowest in Europe; lower than Germany, Ireland, Belgium, Finland, Netherlands, Austria, France, Sweden, Spain and Italy.[iv]

2. The NHS in Scotland has an unparalleled opportunity to invest in new medicines due to the significant sums of money being saved as a consequence of several high use medicines becoming available at lower prices after losing their patent exclusivity {see Section 4.5 below).

3. Unlike any other area of NHS spending, at present all new medicines undergo Health Technology Assessment (HTA) by the Scottish Medicines Consortium (SMC) to establish whether they are value for money ("cost effective") in the context of their clinical benefit to patients[v]. Thereafter a medicine that is "accepted for use" by SMC (either with or without a restriction on that use) undergoes further local examination at NHS Board level before a subsequent decision is made whether or not to place the medicine on the local prescribing formulary. Clinicians cannot generally prescribe any item that is not on their local formulary.

4. Given the requirement to manage NHS resources carefully, the industry accepts the need for HTA and acknowledges the high level of expertise and professionalism of the SMC. However, we believe that there are some disease areas which are not served well by current HTA methods and therefore patients with these diseases risk being further disadvantaged. Methodological and societal discussions are required, with the potential to lead to further evolution of HTA methods.

5. Subsequent to SMC decisions, the industry has deep concerns that medicines assessed as cost effective by SMC face further hurdles before they reach patients. These additional hurdles generally lack transparency, clear processes and clear timelines, and vary across Health Boards, leading to slow decisions and delayed uptake in Scotland. The graph below indicates the opportunity to give access to new medicines for patients by the rapid HTA assessment performed by SMC. It goes on to show a systematic failure to deliver access for patients in comparison to other UK countries.



Explanatory note: Average uptake per capita per new medicine (numbers shown above lines) since 2007/08 which have received a recommended or restricted use decision in NICE and also an approved or restricted decision by SMC. For some medicines a positive decision was reached following resubmission to SMC, these have been included in the analysis. First four years since launch in any UK country, only medicines with at least four years of uptake data are included.[vi]

6. Sharing the industry's concern at the slow rate of uptake of new medicines, the Scottish Government issued guidance on the introduction of new medicines to NHS Boards in 2010 (CEL 17)[vii], clarified in 2011 (CMO 3)[viii] . Further guidance was required in 2012 (CMO 1)[ix] setting new defined timescales for the processes. This latest Guidance was issued in February 2012, for implementation by April 2012.

1. While clearly these guidance letters are welcomed and clarify the aims of Scottish Government, industry has a concern that in general Health Boards have not reviewed their internal processes sufficiently to ensure that the defined timelines can be met. As formal systems are not in place to measure compliance with this guidance, the ABPI have been tracking the on-line publication of formulary decisions by NHS Boards' Area Drug and Therapeutic Committees over the past 6 months i.e. since the latest

guidance was implemented. The analysis presented later in this document (Section 3, paragraph 5) suggests that these guidelines are not being met by many NHS Boards.

Section 2: SMC and HTA in Scotland

1. The SMC is a widely respected HTA body that operates as a consortium of Scottish NHS Boards and in partnership with clinicians, patient representatives and the medicines industry, to high standards of openness and transparency.

2. The SMC's methodology uses the Quality Adjusted Life Year measure (The QALY combines two factors into one measure - the quantity of life (how long you live) and the quality of that life.) which it states is "a widely used economic indicator, or tool, that allows a consistent approach to comparing the value of different medicines."[x]

3. While clearly bringing consistency and transparency to the HTA process, there is increasing recognition that the QALY may not be sensitive enough to take account of the changes in the health status of patients in some disease areas. In cancer, for example, there is evidence of limitations[xi], such as the lack of a measure of vitality in the EQ-5D which is one of the most commonly used health related quality of life measurement systems. Similarly, in other long-term conditions such as central nervous diseases, the QALY is frequently relatively insensitive to clinical changes.

4. In addition the value of some medicines is beyond just the patient, but also to their carer and possibly social services. Methodologies that take a wider perspective on 'value' will be of increasing importance to Scotland as health and social care come together.

5. There is also recognition that when treating patients with very rare diseases, given the significant cost of developing the medicine and the small number of patients likely to receive the medicine, in many cases it is highly unlikely that the medicine will be able to meet the indicative cost per QALY threshold generally required to be categorised as 'cost-effective'; the small number of people requiring these medicines means that they are likely to cost more per patient than more commonly prescribed medicines. While the SMC has modifiers which can be applied in such circumstances, industry questions whether these are sufficient in all cases. Patients living with rare conditions, in particular those with severe conditions should not be further disadvantaged because of the suitability of the HTA process in assessing medicines for their condition. Other jurisdictions have acknowledged that different assessment models may be needed in such circumstances. In England for example, historically very few medicines for rare diseases were assessed (with the relatively recent exception for cancer therapies). More recently, the Advisory Group for National Specialist Services (AGNSS) was set-up to pilot new methods for assessing high-cost drugs for patients with rare conditions, specifically including measures reflecting a wider perspective of 'value' than

traditionally used in most HTA assessments,. The UK Government has shown support for this initiative and from April 2013, NICE will take over this role, building on the AGNSS methodologies. A consultation on new methodologies will taken place in 2013/14 [xii]

6. Research commissioned by ABPI Scotland shows that there have been 96 SMC decisions, on medicines for very rare conditions between 2001 and July 2012. While the SMC accepted (or accepted with restrictions) the majority of these medicines (51%), a large minority (49%) were not recommended (NR). [xiii] This compares to 29 % not recommended when all medicines are considered. It should be noted that for 18 of these orphan indications the pharmaceutical companies decided not to submit their new medicine to the SMC for assessment, on the understanding that this would result in an automatic 'not recommended'. In many cases this lack of a submission reflected that companies' belief that the current system is ill-equipped to assess these medicines.

- **<u>Recommendation 1:</u> The SMC should establish a Short Life Working Group with representation from NHS Boards, ABPI, academia, patients and potentially the public to examine ways to approach HTA for medicines that fall into those categories where the current approach is not fully effective, as summarised above. This Group should make recommendations to the Scottish Government and SMC for changes in the way these medicines are appraised**

Section 3: Post SMC Processes

1. The patient perspective, as evidenced by every MSP's postbag, is still that there is inequity of access and availability of new medicines in comparison i) between Scotland and the rest of the UK and Europe, and ii) between NHS Boards in Scotland. There is also a political debate on the use in England of a Cancer Drug Fund (CDF) to pay for the provision of particular medicines. This highlights the point that for patients in England, a 'no' from NICE doesn't automatically mean that they won't get the treatment they need, whereas for the same patient in Scotland a no from SMC means that they won't receive that treatment. The point of this is not talk about the CDF, or to call for a Scottish equivalent, but simply to point out the inequality that exists; it is less about what the mechanism is or what it is called, but simply the fact that there is a mechanism in England that is ultimately benefiting patients.

2. CMO (2012) 1 requires NHS Boards to make decisions within 90 days of the SMC advice to the Board, confirming whether the medicine is available as a treatment option within the NHS Board formulary in accordance with the agreed treatment protocol(s). The 90 day target is based on the EU Transparency Directive recommendation on acceptable timescales for decision making[xiv] and, as such, NHS Boards' processes should be adapted to achieve local decisions on medicines within this period. NHS Boards must then publish that advice within 14 days of the Board's decision.[xv]

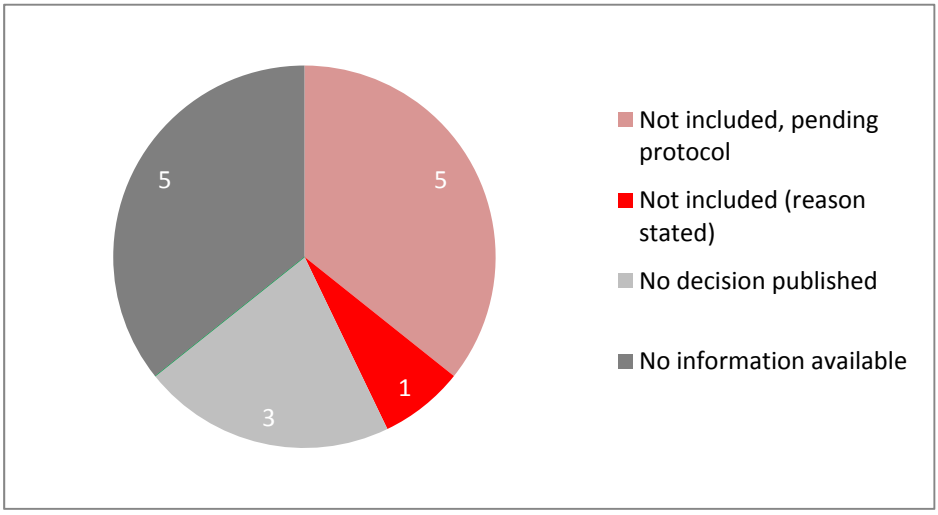
3. CMO (2012) 1 also requires NHS Boards "to present formulary decisions in a consistent and transparent way. As a minimum, NHS Boards are expected to maintain on their website, an up to date list of SMC accepted medicines with standard advice to confirm whether these medicines are included or not included within the NHS Board formulary."[xvi]

4. The current experience of clinicians and patients is that the high expectations of the guidance documents above in supporting the timely uptake of new medicines are not being met, with considerable variation between NHS Boards on the introduction of new SMC-accepted medicines onto local formularies. ABPI Scotland has been tracking whether local formulary decisions have been published for SMC 'accepted' medicines since CMO(2012)1 came into force.

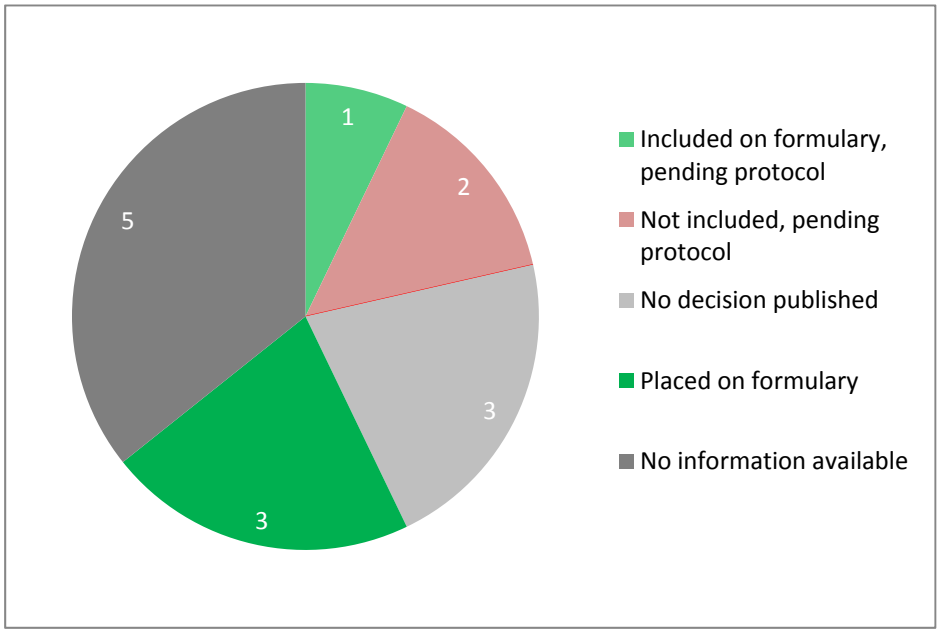
5. Medicines with an SMC notification of acceptance made at the first meeting after CMO (2012) 1 came in to force on 1 April 2012, had 90 days for a decision to be made by Boards and a further 14 days to publish that decision. Medicines notified to Boards in mid-April should therefore have had a formulary decision in mid July with publication by the end of July. SMC made five decisions to accept medicines – three medicines for one indication each and a fourth medicine for two indications. N.B. In 5 Health Boards there is no information for patients on the formulary status of any new medicines available on the Health Board website.

    a. A snapshot review of formulary decisions published online in early August shows:

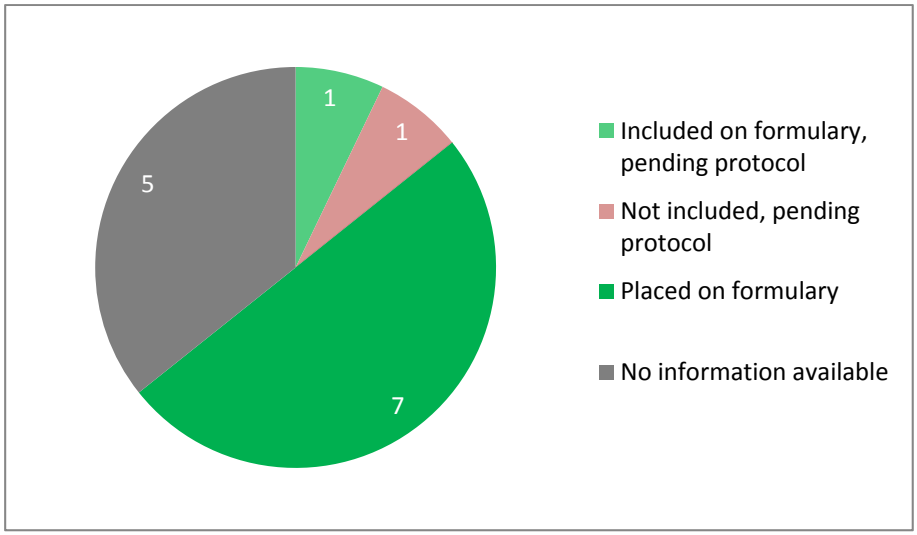
        i. **Medicine 1:**



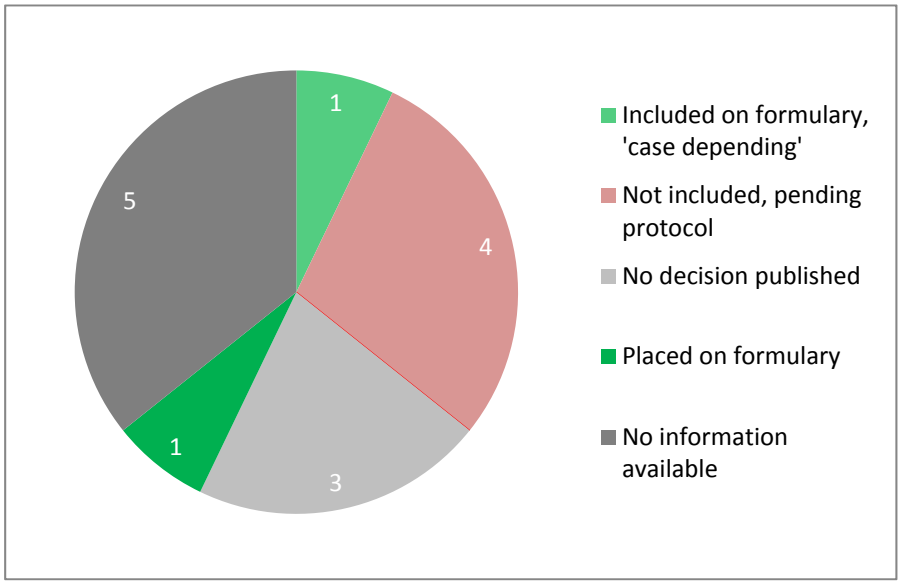
ii. **Medicine 2 (First indication):**



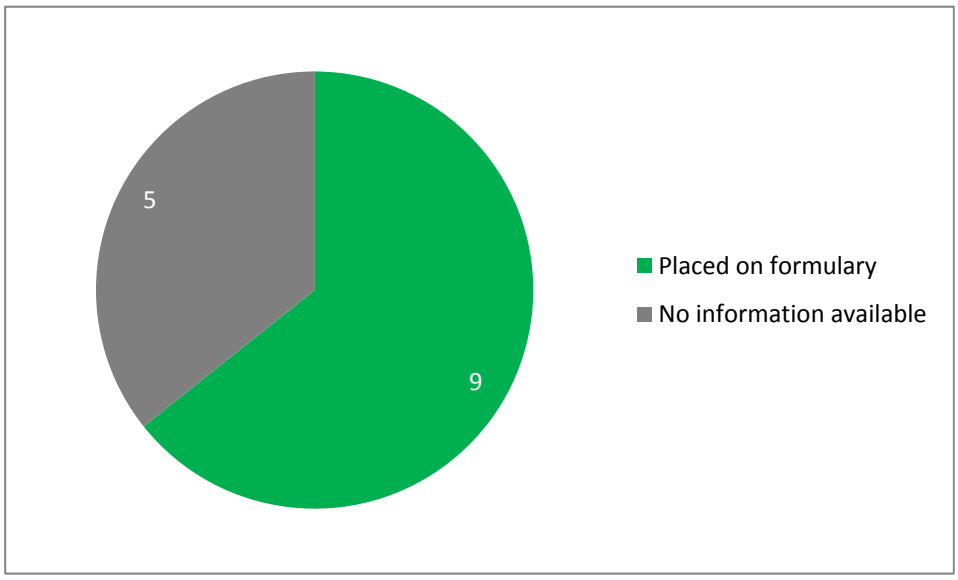
iii. **Medicine 2 (second indication):**



iv. **Medicine 3:**



v. **Medicine 4:**



6. Another factor contributing to delays in patients access to SMC accepted medicines is the interpretation of the wording in CEL(2012)1 stating that medicines can be included or not included on formulary "subject to protocol".[xvii] ABPI Scotland's own research shows that this exception option may be being used on a more regular basis. The 90 day limit should include any redrafting of protocols as patients are not served by the option frequently used by Boards of delaying inclusion of an SMC accepted medicine "subject to protocol", which effectively stops the clock. There is limited evidence that NHS Boards are making the necessary process changes. The above charts demonstrate that all SMC medicines are not

being made available within 90 days, and that some NHS Boards are not doing so 'pending protocol' reviews, but to patients that equates to the same thing; that they do not have access to medicines that they should have access to.

7. In Appendix 1 we include a flowchart, based on one published by the Scottish Government in CMO(2012) 1 explaining the steps that can be taken following the SMC's acceptance of a medicine for use. We have marked the chart to show the extra stages that are carried out at Health Board level. While some of these activities, such as modifying local protocols, may well be required, it is hypothesised that they are contributing to the delays in decision making in some Health Boards. Industry questions whether these delays are reasonable.

8. It is worth noting that the recent inclusion of national consensus meetings to discuss selected SMC-accepted medicines (for example on Dabigatran, which took place on September 21st 2011[xviii]) has added one of the extra steps referenced above. Despite the outputs being "national" statements, individual Boards have still chosen to then develop their own individual protocols. ABPI Scotland does not see how this consensus review, using a process that does not meet the SMC's standards of evidence and transparency, can have added value. Many Health Boards have still not made decisions on these medicines publically available.

    - **Recommendation 2: The Scottish Government should reinforce to Health Boards that they must make formulary decisions publically available, as per CMO 21012(1). The 90 day limit for decisions on inclusion of SMC accepted medicines onto local formularies should also include the introduction or amendment of local protocols.**

9. The Health and Sport Committee is, we understand, considering the Individual Patient Treatment Request process in the context of a series of petitions to the Scottish Parliament. ABPI Scotland will be pleased to support this specific investigation and would only wish to state the following in the context of the Committee's review on access to medicines:

    a. We fully acknowledged that the IPTR process is not meant to be a means of gaining access for all patients to medicines which are 'not accepted' by the SMC. However it is the ABPI view that patients and clinicians are not being well served by IPTR. Recent surveying of leading Scottish oncologists suggests considerable difficulties and dissatisfaction [see appendix 2].

    b. As a consequence of these concerns, a working group bringing together representatives of clinicians, patients and the medicines industry has reported to the Cabinet Secretary for Health and the Health and Sport Committee on ways to make the IPTR process more effective for clinicians and patients. A summary of their recommendations is in Appendix 3, but 2 of the key points are as follows:

i. The view of what the IPTR process is for, and in what circumstances it should be used, differs between government, clinicians and NHS Boards.[xix]

ii. The process, eligibility criteria and usability of the IPTR process continues to vary between NHS Boards such that there are very different rates of IPTR submissions and acceptances.[xx]

Section 4: Affordability

1. ABPI Scotland believes that the biggest barrier to patients being able to access new medicines while in the care of NHSScotland is the increasing efforts to contain spending on medicines, justified on the basis of affordability.

2. Medicines inflation is not out of control – indeed in the most recent year, the medicines bill increased by less than 2%, this rate being below the national rate of inflation. The proportion of the overall NHS Boards' revenue budgets spent on medicines has increased from 17.93% to 18.30% over the past five years [See Appendix 4].

3. Branded (or proprietary) medicines are initially priced to take account of their development costs. However, once they lose patent protection, significantly cheaper 'generic' copies are allowed on the market, providing the opportunity for savings to the payer and possibly wider usage at this lower price. The increase in spending on medicines has been driven almost entirely through more items being dispensed rather than being driven by the cost of new medicines. Over 80% of prescription items are generic rather than branded.[xxi]

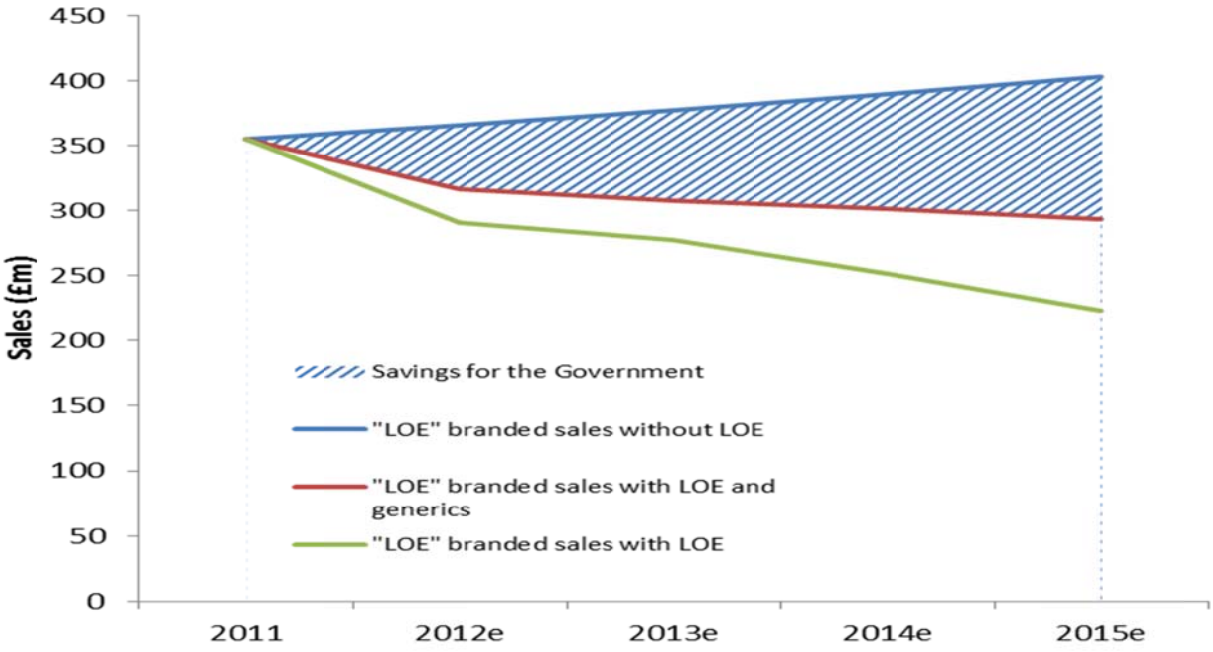
    a. Prescribing volumes have increased from 69.5 million items in 2002/03 to 94.6 million items in 2011/12. NHS ISD states that this growth "reflects not only the availability of new or more effective medicines, but also increasing patient expectation and demographic changes and latterly the implementation of clinical guidelines and recommendations". The trend continues: the rate of increase in prescribing volumes between 2010/11 and 2011/12 was 3.8% compared to 2.4% between 2009/10 and 2010/11."[xxii] [xxiii] Prescription charges were reduced for three years before being abolished in 2011.

    b. Of the top ten items prescribed in NHSScotland by volume, only one is branded. Of the top ten prescribed by cost, 4 are branded[xxiv] [See Appendix 5].

4. Meeting the increased demand of patients for medicines is costing NHSScotland progressively less in real terms given i) the current dominant use of generic medicines and ii) the fact that several commonly prescribed medicines which have collectively been responsible for preventing tens of

thousands of deaths in Scotland are losing their patent protection over the period 2012 to 2015 allowing generic versions to replace them and thus add further potential for savings.

5. Future medicines spending predictions made by the Office of Health Economics show that loss of exclusivity (LOE) will save NHSScotland an estimated £316m in the years 2012-2015[xxv]. The graph below illustrates how we estimate the loss of exclusivity will impact upon sales. We are keen to know how these savings are being taken account of by NHSScotland and the individual NHS Boards, and to know where these savings are going, and whether or not they will be reinvested in medicines spending, or allocated to another part of the budget.



6. In recent years NHS Board policy towards medicines has been one of cost containment with increasing emphasis on "medicines management" whereby prescribing decisions by clinicians are monitored and policed in a way that is not replicated for other interventions. While ABPI Scotland welcomes the scrutiny placed on medicines to prove their effectiveness, clinically and in terms of cost/benefit, we believe that patients are best served when this is driven by quality and patient outcomes rather than a narrow expectation that the medicines budget is always the best place to cut costs.

- **<u>Recommendation 3:</u> A forum should be set up, that includes ABPI and The Office of Health Economics, to share information on significant new product launches, patent losses and other factors affecting medicines budgets to facilitate long term projection and planning by the Scottish Government and NHS Scotland.**

- **<u>Recommendation 4:</u> The Health and Sport Committee of the Scottish Parliament examine the best use of the estimated £316 million of savings being made in the cost of medicines to NHSScotland between 2012-2015, with a view to identifying what proportion can and should be reinvested in meeting patient expectations of access to the latest medicines**

Section 5: Conclusions

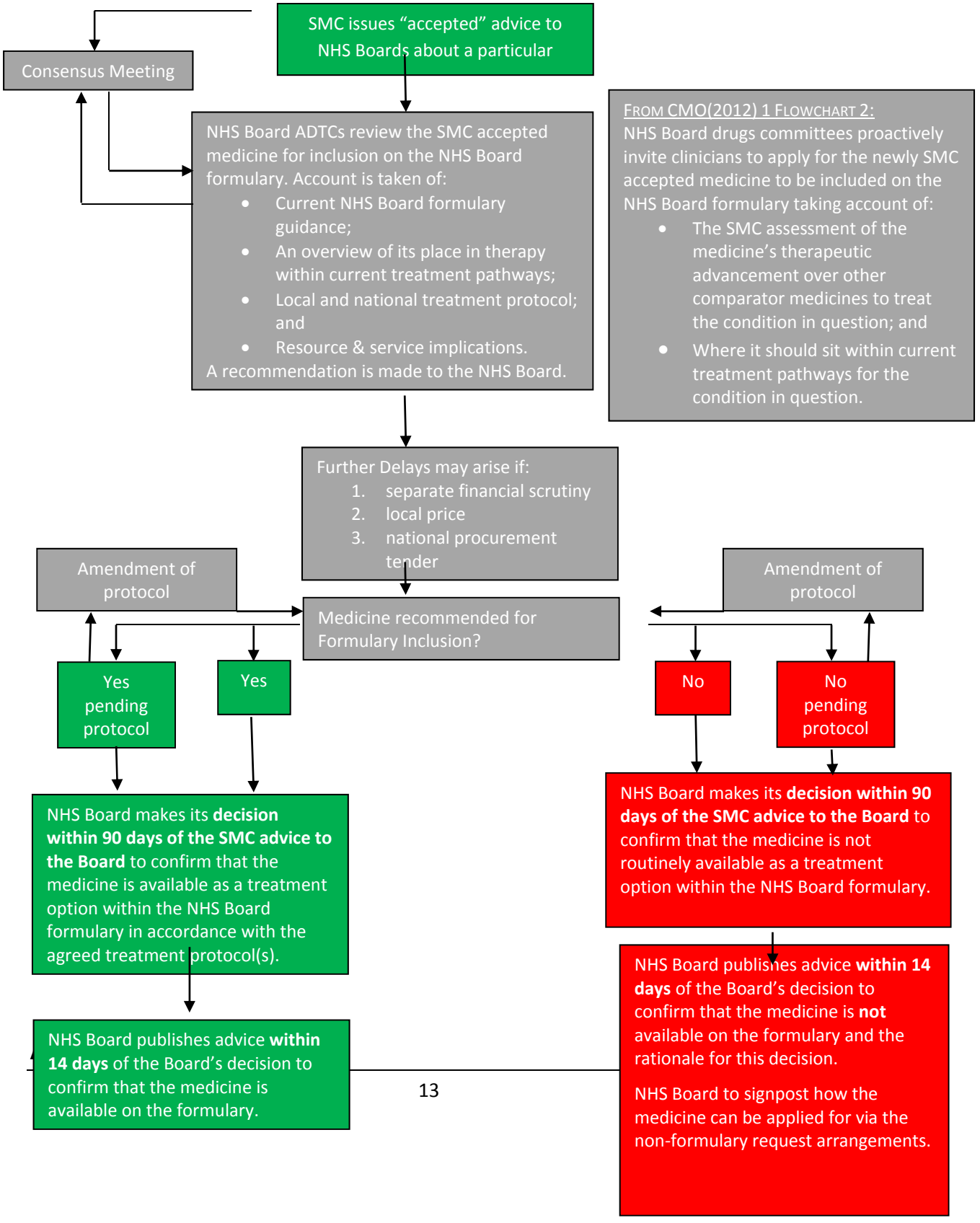
1. ABPI Scotland is committed to working as a partner in the delivery of a world class NHSScotland that is driven by the need to achieve the best outcome for patients through support for innovation, decision making processes that are transparent, as rapid as the evidence allows, consistent and person-centred.

2. The future of medicines is one of increasing sophistication where a patient's illness can be targeted by compounds that are effectively tailored to their specific condition. This offers a much higher level of efficiency and effectiveness of treatment. It also creates a challenge for the NHS to meet the expectations of patients/taxpayers from finite resources.

3. Over the last 50 years the majority of significant improvements in patient outcomes have been down to the development of new, innovative treatments, or example ending the need for ulcer surgery due to oral medication. Despite these and many other examples, we remain focused on a discussion about "if" patients in Scotland should get access to the latest most innovative treatments. At a time when Scotland faces unprecedented healthcare challenges, an ageing population and intense fiscal pressures surely now, more than ever, we need to radically rethink attitudes to introducing medicines in Scotland to move away from simply driving down costs to one of critically appraising where the investment in patients' health through medicines can help Scotland address some of the health and social care challenges of the future. To continue to improve patient outcomes for the same or less resource we may need to change funding priorities in the NHS to ensure – and prove – that patients get the maximum benefit for every pound spent. This may mean looking at reducing spending in some areas to continue to support innovation in others.

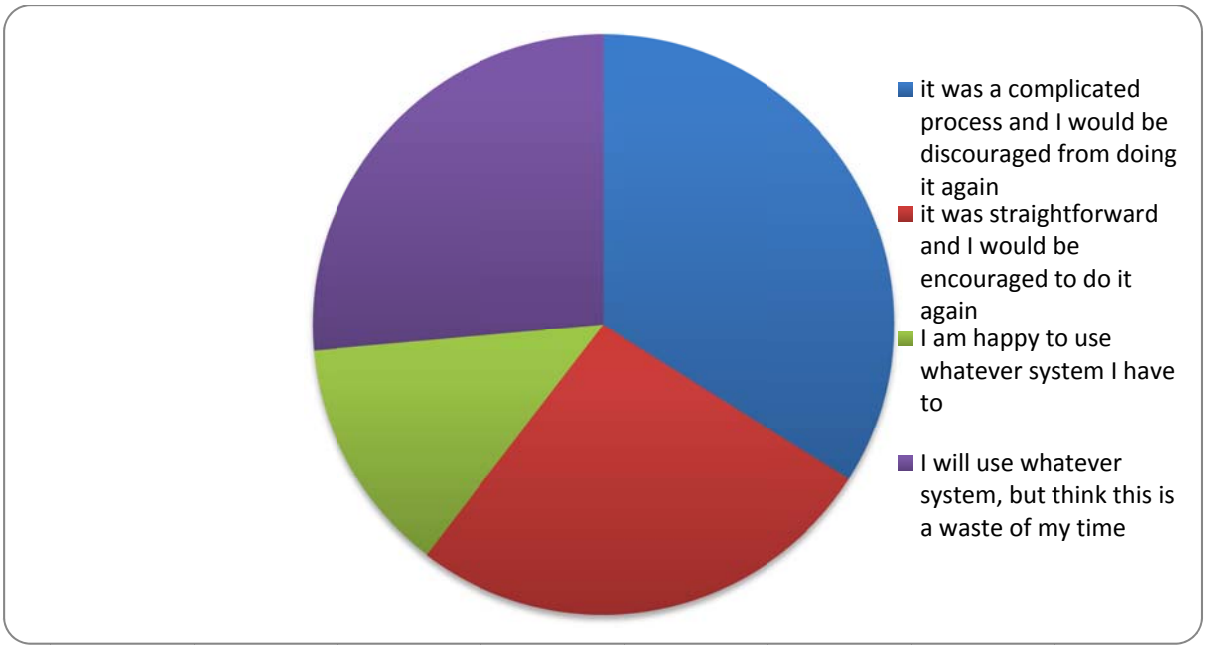
APPENDIX 1

Flowchart from CMO (2012) Guidance (white boxes) with further annotation by ABPI Scotland (shaded boxes). The dark shaded elements represent additional steps not envisaged in the guidance, each with the potential to delay formulary inclusion and patient access to medicines. It is possible for a medicine accepted for use by SMC to undergo six further stages before being included on a local formulary.



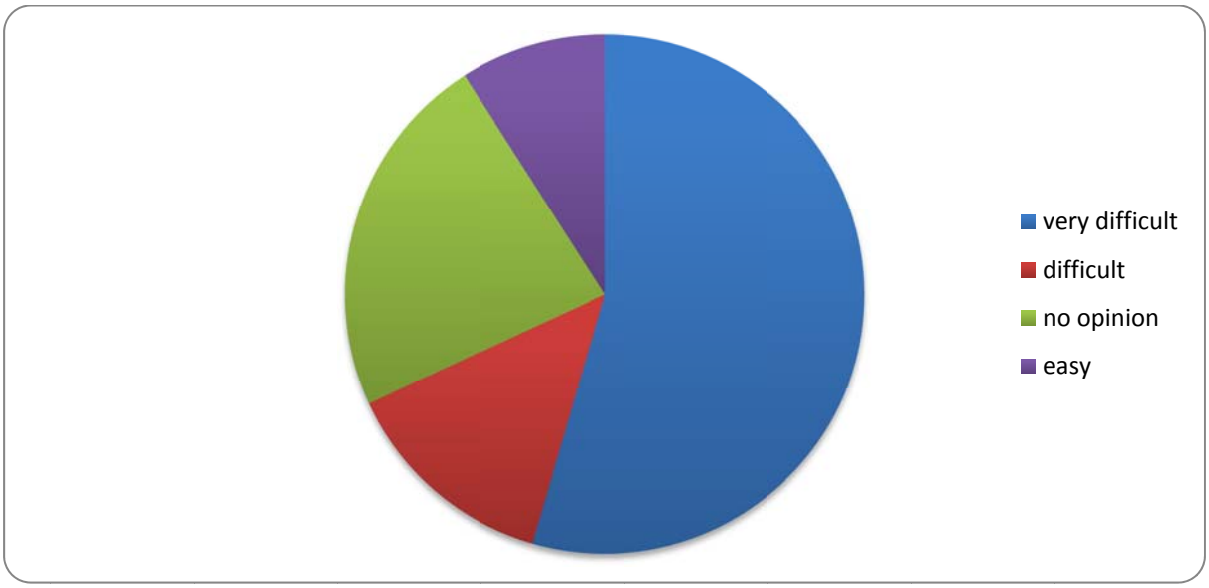
These are findings from a survey of oncologists undertaken by on-line survey and telephone in February and March 2012. This work was undertaken by Morhamburn on behalf of the Scottish Cancer Industry Group. From a database of 168 oncologists across Scotland approached, 44 responded (26% response rate).

When asked how they would characterise their experience of using the IPTR process:



More than nine out of ten clinicians said that they were required to prove exceptionality of their patient, even though this word has been removed from guidance. 8.3% said they did not have to demonstrate exceptionality.

Of those who expected to demonstrate exceptionality of their patient:



APPENDIX 3

Recommendations of IPTR Review Group (May 2012)

There needs to be:

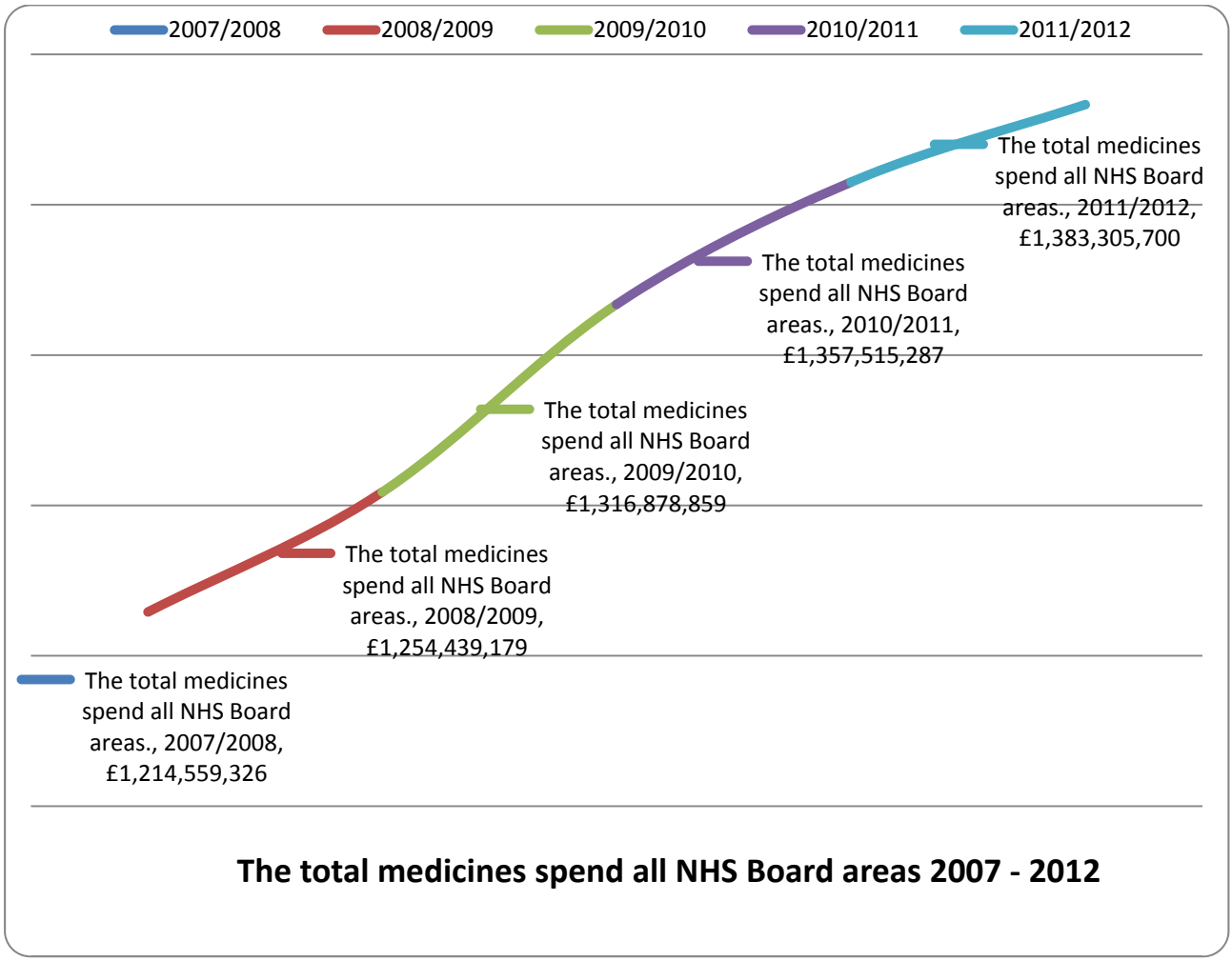
- Additional clarity on access to medicines other than via the IPTR route; i.e. out-of-license use for rare diseases or off-label use (where a medicine is used for a purpose not included in its original license).
- Clarity on the wording of guidance on IPTRs - It needs to be clear exactly what situations IPTRs are for and what they are not for; and also how they are assessed.
- A national quality review panel, not to review individual IPTRs, but as a way to review how well the processes are working and to keep check on regional variation. This group should have a transparent membership, a patient representative and should publish top-level data as a means of driving-up standards. It should look for equitable processes and decisions across both approved and non-approved requests.
- An objective, transparent scoring system as a means of assessing IPTRs and their validity, to ensure uniformity and fairness across illness areas and geographically.
- The establishment of benchmarks from across Scotland of where we are with IPTRs; what is working and what is not. This would help to identify areas of good and bad practice and create a baseline.
- The sharing of best practice across Scotland.
- Information and training on the system – for all participants in the system, which should also be available to patient groups, MSPs and the pharma industry.
- Clarity on who sits on IPTR panels.
- More engagement with patient groups and decision makers. The justification for a decision is an important factor in that decision being accepted by patients, and for that decision to be seen as fair and consistent.
-

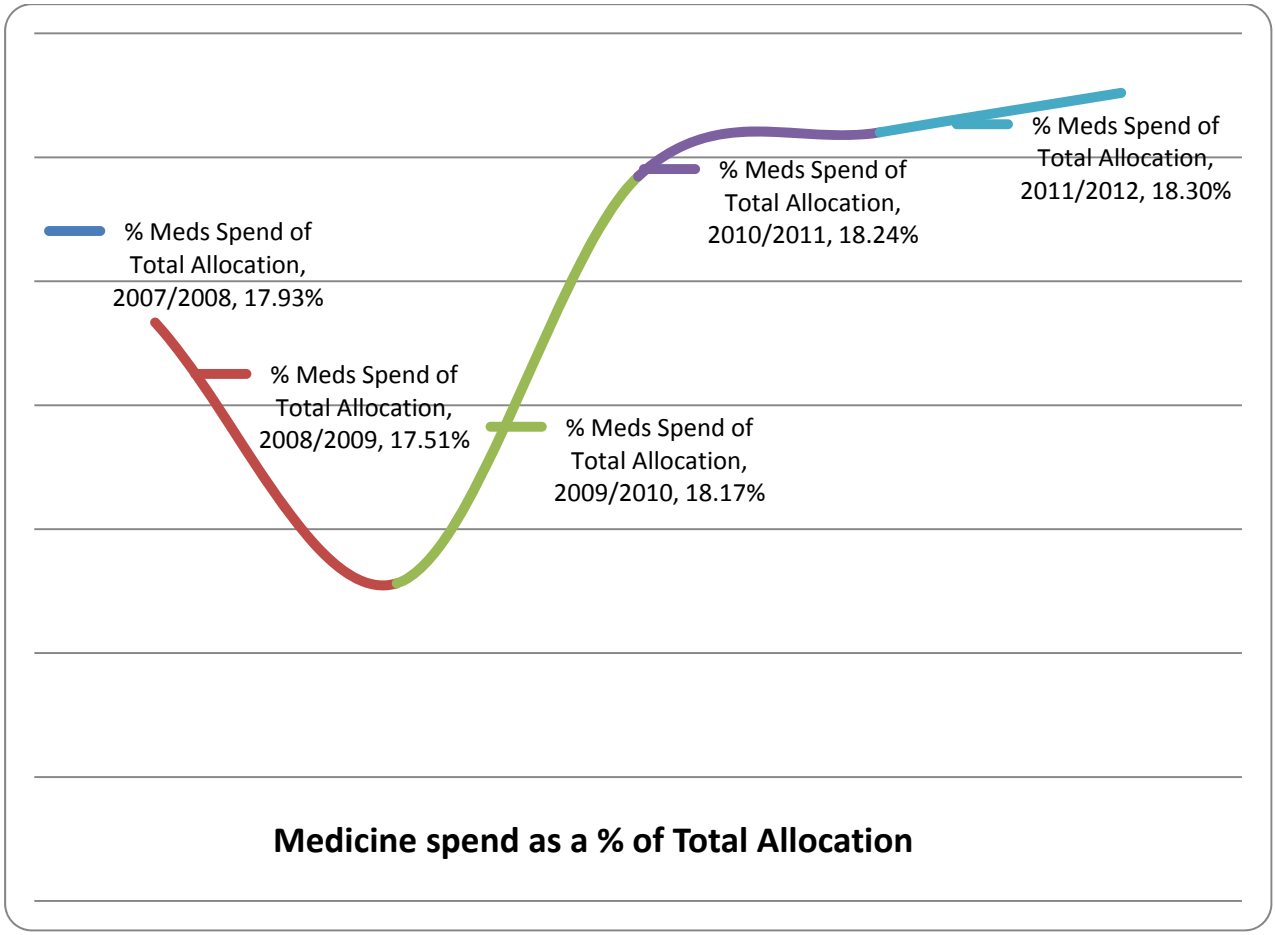
APPENDIX 4

Research was undertaken for ABPI Scotland by Morhamburn Limited. Each NHS Board was asked under FOI to state the budget and spend for medicines for the last three years. This information was compiled alongside the same information for the two previous financial years. The figures for spend by each NHS Board on medicines were presented alongside the revenue budget allocations to each of the 14 territorial NHS Boards as announced to Parliament.

The total medicines spending by NHS Boards increased from £1,214 million in 2007/2008 to £1,383 million in 2011/12. This equates to a rise of 13.89%. The

medicines spend as a proportion of overall spend increased from 17.9% to 18.3% in this period.




% Meds Spend of Total Allocation, 2007/2008, 17.93%
% Meds Spend of Total Allocation, 2008/2009, 17.51%
% Meds Spend of Total Allocation, 2009/2010, 18.17%
% Meds Spend of Total Allocation, 2010/2011, 18.24%
% Meds Spend of Total Allocation, 2011/2012, 18.30%
Medicine spend as a % of Total Allocation

APPENDIX 5

Source: ISD Prescribing Cost Analysis 2012

Top ten medicines in NHS Scotland – Branded medicines in **BOLD;** all other items are generic

	Top 10 by Volume Prescribed		Top 10 by Gross Ingredient Cost	
	Item	Usage	Item	Usage
1	simvastatin	For controlling cholesterol	**salmeterol with fluticasone proprionate**	**For respiratory conditions**
2	omeprazole	For reducing stomach acid	atorvastatin	For controlling cholesterol
3	aspirin	As a blood thinning agent	**tiotropium**	**For respiratory conditions**
4	co-codamol	As a painkiller	**budesonide with formoterol fumarate**	**For respiratory conditions**
5	paracetamol	As a painkiller	**pregabalin**	**For epilepsy**
6	levothyroxine sodium	Sodium for thyroid hormone replacement	blood glucose testing strips	For home blood glucose monitoring
7	salbutamol	For respiratory conditions	wound management dressings	For dressing wounds
8	bendroflumethiazide	For lowering blood pressure	quentiapine	For schizophrenia/mania
9	amlodipine	For angina and lowering blood pressure	co-codamol	As a painkiller
10	emollients	For skin conditions	enteral nutrition	As nutritional supplements

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ABPI Scotland
11 September 2012

HEALTH AND SPORT COMMITTEE
ACCESS TO NEWLY LICENSED MEDICINES
Submission from Scottish Medicines Consortium

Modifiers

The Committee has previously been provided with information about why the SMC uses "modifiers" in its appraisal process and also examples of these modifiers.

1. **Can you clarify under what circumstances the SMC will use modifiers in appraising a medicine, whether or not it is for an orphan disease?**

The Scottish Medicines Consortium (SMC) statement on modifiers is attached in full for information. The following description of the new medicines assessment process may assist the Health & Sport Committee in understanding how the process works and where the modifiers fit in to that process.

When a new medicine is licensed for use by the regulatory authority SMC contacts the pharmaceutical company to request a submission on the product, including results of clinical trials and cost effectiveness data.

SMC has a two stage assessment process. The New Drugs Committee (NDC) is the scientific committee of SMC. Its purpose is to appraise all the evidence that is included in the pharmaceutical company submission and reach an initial position on whether the medicine is clinically and cost-effective. It evaluates the submission with the support of medical, pharmaceutical, and health economics experts. There is also written input from clinical experts in NHS Boards at this stage. The assessment on the medicine is discussed in detail at the New Drugs Committee meeting and NDC then makes a provisional recommendation that is shared with the pharmaceutical company.

The SMC Committee takes a broader perspective in reaching a decision on whether the medicine can be accepted for use in NHS Scotland. As well as reviewing the provisional recommendation from NDC and the response from the sponsor company, SMC also considers submissions from Patient Interest Groups. The Patient Interest Group submissions are an important part of the assessment process; they focus on the difficulties the condition presents for patients and the place of the medicine in addressing patient needs. These often supply useful additional perspectives on new medicines and they are very helpful in guiding SMC's conclusions. SMC also considers special issues related to health care provision in Scotland (such as those related to the highland and island communities), and any relevant societal issues.

SMC uses the cost per Quality Adjusted Life Year (QALY) as a measure to assess the clinical and cost-effectiveness of medicines. The QALY allows comparisons to be made between different medicines for different conditions. Some medicines have a low cost per QALY and these are considered to offer good value for money. Medicines with a high cost per QALY would not be considered good value for money. A cost per QALY of

under £20,000 is generally considered acceptable value for money. For a medicine with a cost per QALY between £20,000 and £30,000 SMC might accept this if the medicine gives significant benefits over existing treatments.

The SMC Committee can consider the application of modifiers for any medicine under assessment where the estimated cost per QALY is relatively high. If a medicine has an estimated cost per QALY
> £30,000, and the Committee is confident that the company's clinical and health economic case is robust, then the Committee will consider whether one or more of the modifiers would allow it to be accepted.

2. **What is the decision-making process for determining what modifiers will be used?**
3. **When a decision has been taken to use modifiers, can you provide more detail as to how they are used and the methodology used to factor them into the appraisal process?**

The following description on how modifiers are used within the process covers both questions 2 and 3 above.

The SMC Committee sees the application of modifiers as an important part of the process and there is a proactive approach to considering whether or not any modifying factor will have a bearing on the decision. In addition, companies often state in their submission or their response to the NDC provisional recommendation whether they believe modifiers are relevant. All SMC members are provided with the full company submission and the company response to the NDC provisional recommendation in their meeting papers. This prompts the Committee to consider whether one or more of the modifiers might be relevant to the medicine being assessed. Patient interest groups are asked to highlight in their submissions any patient / carer and family needs that are not being met by existing treatments or medicines. These patient group submissions may also refer specifically to modifiers or describe special factors showing benefits or health gain that might allow SMC to accept a higher cost per QALY. If the company does not make reference to modifiers this does not preclude SMC members from bringing them into the discussion and decision-making. There is a brief presentation on each medicine being considered at SMC and this makes reference to modifiers where relevant.

As the clinical efficacy data in an orphan drug submission is often limited, SMC will accept a greater level of uncertainty in the economic case. In the event that the clinical and economic case for a medicine is robust but the cost per QALY is beyond the level that would normally be considered acceptable, or for an orphan where there is a high level of uncertainty, the Chair will ask the membership to discuss whether the modifying factors should be considered. The Chair will remind the committee of the stated modifiers (e.g. whether the drug treats a life threatening disease; substantially increases life expectancy and/or quality of life; can reverse, rather than stabilise, the condition; or bridges a gap to a "definitive" therapy e.g. organ transplant) and these will be considered in assessing both the level of uncertainty and cost per QALY which is

acceptable. For example, in considering whether a medicine gives a 'substantial improvement in quality of life' the committee will have a detailed discussion on whether this is indeed supported by the clinical evidence in the submission.

Appraising orphan medicines

The Committee notes the information already provided to the Public Petitions Committee concerning the appraisal process for orphan medicines. It also notes the views expressed by the SMC and the Scottish Government concerning the term "ultra orphan medicine".

4. **Has the SMC Committee itself reviewed or considered its own processes for approving orphan medicines?**

Yes SMC has reviewed its processes for approving orphan medicines. Between 2002 and 2004 SMC had considered only five orphan medicines but the committee recognised that there was an increasing number of orphan medicines in clinical development. A Short Life Working Group on Orphan Drugs was established to consider whether other approaches to decision making on orphan medicines could be applied. The range of options considered included: a multiplier for the threshold for cost per QALY, whether some QALYs may be worth more than others, the use of modifiers, whether all orphan medicines could be provisionally accepted for a time limited period. The group concluded that the best way forward was to allow SMC to accept greater uncertainty in the economic case for orphan medicines and the output of this group was the orphan medicines policy statement that was introduced in 2007.

In 2008 the National Institute for Health and Clinical Excellence (NICE) in England consulted on whether additional weight should be placed on the survival benefits of drugs and technologies for patients with terminal illness and short life expectancy subject to meeting certain criteria. The Scottish Government Health Department asked SMC to consider the feasibility of such an arrangement in processes for NHS Scotland. The SMC view was that the existing decision making process supported a pragmatic approach that would allow medicines with a relatively high cost per QALY to be accepted in some circumstances. The committee recognized that it would be helpful for transparency of process if these modifying factors could be described. As a consequence the SMC revised statement on modifiers was published in 2010 (copy attached) and this subsumed the previous policy statement that related to orphan medicines only.

5. **Has the SMC undertaken any research into the different processes that exist in the UK and beyond for appraising orphan medicines?**

SMC does not have a research function therefore we have not carried out formal research into the health technology appraisal processes for orphan medicines in the UK and beyond. There are informal mechanisms, however, to maintain awareness of how other health technology appraisal bodies in the UK, Europe and internationally consider

orphan medicines. The SMC Chair and members of the Executive Team have attended and contributed to international meetings and symposia on orphan medicines. For example, the SMC Chair attended the World Orphan Drug Summit in Frankfurt in June 2011 and presented on the SMC process. This allows SMC to gain a deeper insight into the issue of rare diseases and the European / international policy context.

6. **When appraising an orphan medicine what steps does the SMC take to ensure it obtains an expert opinion from a specialist in the disease that the medicine seeks to treat?**

The SMC assessment process involves seeking the views and opinions of a range of clinical experts for each new medicine submission. Where possible SMC obtains this input from clinicians in NHS Scotland but when there is difficulty in obtaining responses from clinicians who treat the condition in question it is common practice to seek input from specialists in England. SMC accepts that this can be challenging as often there are few very specialists across the UK with experience of the condition in question. In addition, these specialists may have conflicts of interests (such as their department receiving funding to take part in a clinical trial or payments to contribute to advisory board meetings) that mean their views cannot be taken into account. Although this is challenging for orphan medicines, since clinical expert views were introduced as part of the process SMC has been able to obtain clinical expert input for every orphan medicine considered to date.

7. **What is the SMC's view of the petitioners' argument that the process is particularly weighted against ultra orphan medicines i.e. a disease affecting fewer than one in 50,000 people in the general population?**
8. **What is the SMC's view of the proposal that a separate body assess orphan medicines in Scotland as is the case in England?**

We believe that the SMC process is not weighted against any specific type or class of medicine, including those used to treat diseases affecting fewer than one in 50,000 people in the general population.

SMC has followed with interest recent developments in England including the establishment of the Advisory Group on National Specialist Services (AGNSS). As the AGNSS remit includes the assessment of the cost effectiveness of orphan drugs for very rare diseases (i.e. orphan products for a clinically distinct group of patients totalling no more than 500 cases in England per year) it will not assess all medicines with orphan designation. Some, but not all, of the orphan medicines that are outwith the AGNSS remit will be appraised by the National Institute of Health and Clinical Excellence (NICE). In England, therefore, there may be three different approaches to orphan medicines; AGNSS, NICE or no assessment. SMC considers this a less equitable position than currently exists in Scotland.

It was expected that only orphan drugs approved by AGNSS for use in England would be available for specialist clinicians to prescribe for patients within designated national

specialist services. AGNSS has not issued guidance on any medicines to date, however, and although guidance on two medicines is expected during 2012 we understand that new work has been suspended pending the establishment of the new NHS Commissioning Board for England. It therefore remains the case that the majority of medicines for rare diseases are not subject to any cost-effectiveness assessment in England at present so the approach there is fragmented and not comprehensive for all new medicines.

As outlined in our earlier response to the Public Petitions Committee, SMC believes that there are important strengths in a single, comprehensive assessment process that encompasses all new medicines, regardless of severity or whether the condition they treat is common or rare.

SMC looks at clinical evidence, in the context of modifiers where these might apply, as well as cost-effectiveness. The latter is important and fair to consider in the case of orphan medicines because there is an opportunity cost i.e. paying for these medicines means that funding is not available for something else. The evidence SMC makes its decision on is presented as a result for a typical patient i.e. a gain of 3 months survival at a cost per Quality Adjusted Life Year of £40K. These values are independent of the total number of patients with the condition who are expected to benefit. The same process is used for all medicines but the Committee has flexibility to accept some of them despite greater uncertainty.

The principle of trying to ensure that NHS resources are used most effectively, having regard to the premise that the NHS has limited resources which can only be spent once, underpins all our assessments. The members of SMC apply the same decision making framework across all medicines. We believe this is a key strength of the SMC process. SMC believes that its current methodology is robust, objective, transparent and fair and is therefore appropriate for the assessment of orphan medicines.

10 May 2012

Scottish Medicines Consortium response to the Public Petitions Committee on PE1398, PE1399 and PE1401

The Public Petitions Committee has asked the Scottish Medicines Consortium

- What are your views on the issues raised in the petitions?

1. About the Scottish Medicines Consortium (SMC)

The Scottish Medicines Consortium (SMC) welcomes the opportunity provided by the Public Petitions Committee to describe its role and functions in the assessment of new medicines. The purpose of SMC is to assess the comparative clinical- and cost-effectiveness of new medicines and accept for use those that clearly represent good value for money to NHS Scotland. SMC has a remit to advise Health Boards across NHS Scotland and their Area Drug and Therapeutics Committees (ADTCs) on all new prescription medicines, including new formulations and new indications of existing medicines. Advice is issued as soon as practical after a new medicine becomes available for use. Senior NHS managers, representatives of the public and the pharmaceutical industry are involved in the process. The Patient and Public Involvement Group (PAPIG) subgroup of SMC is responsible for ensuring that the patient/carer perspective is always taken into consideration by the SMC.

2. Orphan medicines

Orphan drug legislation was introduced in the EU in 2000 in an attempt to improve the availability of medicines for rare diseases, described as 'orphan medicines'. This created incentives for pharmaceutical companies to develop medicines for rare diseases. The EU criteria for orphan medicines are those defined by the Committee on Orphan Medicinal Products (COMP) and set out on the European Medicines Agency (EMA) website. In terms of the rarity of the disease, in the EU an orphan drug is defined as one for which the frequency of the disease is less than 5 per 10,000 of the EU population. 'Ultra orphan' is a term used by NICE but not, as far as we are aware, formally recognised by relevant regulatory agencies. The number of new treatments for rare disorders has increased over the past 10 years. Over 800 medicines in development have been designated as orphans and there are now 74 orphan medicines with a marketing authorisation from the EMA (i.e. licensed for prescribing in the UK). This reflects the success of the Orphan Drugs Regulation in Europe.

3. SMC methodology

When a new medicine is licensed for use the pharmaceutical company is asked to make a submission on the product, including results of clinical trials and cost effectiveness data, to SMC. SMC has a two stage process. Firstly, the New Drugs Committee (NDC) critically evaluates the submission with the support of medical, pharmaceutical, and health economics experts. The NDC then makes a provisional recommendation that is shared with the pharmaceutical company concerned. The advice from NDC, together with feedback from the company is then considered by the SMC committee. Patient

Interest Group (PIG) submissions, focusing on the difficulties the disease presents for patients and the place of the medicine in addressing patient needs, are also an important part of the SMC's assessment process. They often supply useful additional perspectives on new medicines and they are very helpful in guiding SMC's conclusions.

4. SMC assessment of orphan medicines

For an orphan medicine the submitting company is required to make the case for clinical and cost-effectiveness in the same way as for all new medicine submissions. In reaching a decision on whether the medicine can be accepted for use in NHS Scotland, SMC recognises that efficacy 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. SMC explicitly state that we will accept greater 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 our process. These additional factors, termed 'SMC modifiers', such as 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 will also be considered in assessing both the level of uncertainty and cost per Quality Adjusted Life Year (QALY). These 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)).

These modifiers form part of a global judgement taken by 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.

When a modifier, or any other special issue which may have been highlighted by the sponsor company, by clinical experts and/or by 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.

5. SMC advice to date on orphan medicines

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. These figures illustrate that the acceptance rate for orphan medicines

submitted to SMC (61%) is lower than the acceptance rate for medicines without orphan status (75%) but that this difference is justifiable.

Summary details of the advice relating to all orphan medicines is attached for information. Full details are available on the SMC website.

6. Societal considerations of valuing rarity

Societal considerations are important in relation to medicines for rare diseases. Societal attitudes toward cost effectiveness have been explored in a number of reports produced by NICE's Citizens' Council including one on Ultra-orphan drugs (November 2004). This concluded that the criteria the NHS should take into account when deciding to pay premium prices for ultra orphan drugs are, in descending order of importance:

- The degree of severity of the disease
- If the treatment will provide health gain, rather than just stabilisation of the condition
- If the disease or condition is life-threatening

Key findings were that rarity on its own is an insufficient reason to justify paying a premium for treatment and that the degree of severity and the amount of health gain are the more critical factors. NICE states that: “Decisions about whether to recommend interventions should not be based on evidence of their relative costs and benefits alone. NICE must consider other factors when developing its guidance, including the need to distribute health resources in the fairest way within society as a whole.”

More recent data on the views of the general public on this issue are available from outwith the UK. In a survey of the Norwegian general population, people were asked whether society should pay more to treat rare diseases than it does for common diseases. The results showed that although respondents supported equity of access to healthcare for people with rare diseases, they did not support providing care for people with rare diseases when the cost of that care was at the expense of people with common conditions. Two citizen’s juries held in Canada had similar findings; opting for health policy that would ensure that effective interventions are made available to the largest number of patients. A preference for treating small numbers of patients was expressed only if the patients were severely ill and the treatment could produce substantial health gain to all of them, bringing them back to normal functioning.

There may also be an issue in relation to how rarity is defined. Globally there are over 6000 identified rare diseases, so collectively the number of patients affected by rare diseases is considerable. To illustrate this, the Rarer Cancers Forum states that between 30% and 50% of all cancers are classified as rarer and an estimate recently quoted in the Scottish media is that in total more than 350,000 people in Scotland will be affected by a rare disease.

7. SMC views on the issues raised by Petitions PE1398, PE1399, PE1401

SMC fully supports the principle that people with rare conditions should be able to access clinically and cost-effective interventions including medicines through the NHS. We believe that SMC helps to ensure that new medicines with the most significant benefits are available across Scotland and improves consistency in their availability from one NHS Board to another. Difficult decisions have to be made in order to spend available resources wisely and this is increasingly important in the current fiscal climate. If money is spent on medicines that do not offer good value, it means that this money is not available to be spent for other treatments that could provide benefits to patients (termed the 'opportunity cost').

Although the SMC acceptance rate for orphan medicines submitted to SMC (61%) is lower than the acceptance rate for medicines without orphan status (75%), SMC believes that these figures are reassuring because *de facto* the evidence base for orphan medicines is often weaker than for other medicines, the SMC modifiers described above do not always apply to the medicine under review and the prices charged for these drugs can make it impossible for them to meet conventional measures of good value.

SMC believes it is important to highlight the extremely high acquisition costs associated with many orphan medicines. This has attracted recent attention in the medical literature, where it is noted that the pharmaceutical industry already receives incentives to develop medicines for rare diseases, and arguing that an unintended consequence of the orphan drugs legislation may be exploitation of the rules for profit. Within NHS Scotland we have the Patient Access Scheme (PAS) which allows the pharmaceutical industry to reduce the cost of a drug where the drug has been shown not to be cost effective. This was set up in 2009 to try to help enable access and to date overall, 25 medicines with a PAS have been reviewed by SMC with 13 accepted for use or restricted use contingent on the PAS being available in NHS Scotland.

If more value or weight is to be put on the health improvement associated with treatments for rare conditions than for common conditions this raises important equity issues. There is evidence from England and elsewhere that the public's willingness to pay for medicines that treat rare diseases is not unlimited.

SMC considers the clinical and cost-effectiveness of all new prescription medicines, regardless of severity or whether the condition they treat is common or rare. The principle of trying to ensure that NHS resources are used most effectively, having regard to the premise that the NHS has limited resources which can only be spent once, underpins all our assessments. The members of SMC apply the same decision making framework across all medicines. We believe this is a key strength of the SMC process. SMC believes that its current methodology is robust, objective, transparent and fair and is therefore entirely appropriate for the assessment of medicines with orphan status.

Scottish Medicines Consortium, 7 November 2011

HEALTH AND SPORT COMMITTEE

ACCESS TO NEWLY LICENSED MEDICINES

Submission from The Royal College of Physicians of Edinburgh

The College understands that healthcare systems world-wide are unable to do everything that they possibly could in the field of patient care because of resource constraints and that all systems therefore have to make choices amongst possible ways of spending that limited resource. This may mean difficult and possibly unpopular decisions having to be made. The College believes that such decisions are best made within a formal, objective, evidence-based process.

Access to new medicines

Inevitably, new medicines are of putative real-world effectiveness at the time of launch into the marketplace and have limited safety data. Older medicines, often now available in generic versions at very modest cost, are of proven real-world effectiveness and have substantially greater evidence of safety in clinical use. While the College understands the demand to access new medicines, it would have major concerns if this threatens the use of highly effective, cheap, existing medicines. In a cash limited system it is important to take a broad perspective on the rational use of all medicines to deliver best value for patients.

The College supports the Health Technology Assessment (HTA) approach to new medicines adopted by the Scottish Medicines Consortium (SMC) (and the National Institute for Health and Clinical Excellence (NICE) in England) as an objective, structured, evidence-based method to assess and compare the relative effectiveness and cost-effectiveness of medicines (and, in NICE's case, other healthcare interventions). The assessments are undertaken using internationally-accepted methodologies and, the College believes, are performed to high standards by both SMC and NICE.

College Fellows and Members are active participants in the SMC process, contributing on the Committee itself as well as providing expert clinical input to the SMC process. This engagement between SMC and clinicians is, the College believes, a significant strength of the Scottish process.

The College believes that the SMC outputs, which are available to view on the SMC website, show that the medicine reviews are undertaken thoroughly and expertly in a transparent process. It is vital that SMC members (and the members of the New Drugs Committee and expert clinical and economic reviewers) have the skills and experience necessary to ensure that the clinical value of new medicines is appropriately identified and then assessed alongside the additional costs of new medicines. The College notes that the quality of the SMC value assessment is not doubted by any of the petitioners.

The College notes that, as a consequence of the robust decision-making processes adopted by SMC, many new medicines in Scotland are now being

offered to the NHS with a Patient Access Scheme (PAS). Most of these schemes represent forms of discount on the list price of the medicine in question - details of the individual schemes and their magnitudes are commercially confidential but it is believed that discounts of up to 50% on the list price have been offered. The SMC process is, in these instances, improving access to new medicines by reducing the acquisition costs, allowing even more patients in Scotland to be treated for the same NHS outlay.

As noted above, concern from petitioners to the Committee is largely around the SMC's decisions (and subsequent implementation within NHS Scotland). The College believes that, if the SMC evaluation of the relative effectiveness and cost-effectiveness of new medicines is accepted as being of high quality but the decisions regarding access in Scotland are being questioned, then it is the context and framework of SMC decision-making that must be called into question. The College is not aware that SMC has outlined any formal framework for its decisions but thinks that it has adopted a similar utilitarian approach to that adopted by the rest of the NHS in Scotland, seeking to achieve the greatest health benefits possible for the population of Scotland without wishing to discriminate positively or negatively around issues such as age, gender, disease type, rarity of disease etc.

SMC's expertise is in the area of medicines evaluation – if the framework in which it makes its decisions is to be changed, then that is not a decision for SMC alone but for the wider NHS or potentially for wider society in Scotland. If it were considered appropriate for SMC to operate within a decision-making framework which chose to discriminate in favour of children, or cancer, or end-of-life medicines, or rare diseases (for example) – the College would ask the Committee to remember that discrimination in favour of any (or all) of these groups also implies discrimination against those not belonging to such a group, and also that Scotland could see a situation develop in which some overall level of heath improvement was being sacrificed in order to favour individual patient groups.

The College believes that research into societal preferences around these difficult issues has not been examined to a great extent in Scotland and the Committee may wish to consider whether this should be explored before any change of decision-making framework is considered. Experience from elsewhere (including the NICE Citizens Council in England[i]) should be noted.

The College thinks that the Health and Sport Committee may be well placed to initiate debate around the framework for decision-making in NHS Scotland – ultimately there may well be a role for debate and decision by the Scottish Parliament itself. The College would welcome the opportunity to be part of that debate (and indeed to facilitate such a debate if that would be helpful) but recognises that these decisions are not for healthcare professionals alone to make but for wider Scottish society.

The College believes that a considered approach to the principles underlying these difficult issues is much preferable to departing from the current process

for individual medicines on a 'case-by-case' basis as the latter could lead to unstructured decision-making and set precedents which could distort later decisions.

The College is aware of the issues surrounding 'Orphan Medicines' (medicines for orphan diseases). Such medicines receive special treatment during the European Medicines Agency (EMA) registration process and are granted longer periods of exclusivity and patent protection than 'normal' medicines. They are often very costly. This poses the question as to whether the use of scarce resource on very expensive medicines is appropriate when this could result in a considerable loss of overall health gain to the Scottish population.

Individual Patient Treatment Request (IPTR)

Concern has been expressed about the Individual Patient Treatment Request (IPTR) process within NHS Scotland. The College supports the process in principle, recognising that all patients are individuals and that SMC (and other) guidance may not always apply to the situation of an individual patient. Clinicians are well-used to individualising patient treatment and so identifying those patients to whom prevailing SMC advice should not apply is an extension of routine practice. The issue at stake is whether the individual patient in question is likely to derive substantially more benefit from a treatment than the average patient (the basis on which SMC advice is founded). If the answer is 'Yes' then use of a 'not recommended' medicine may be appropriate – if the answer is 'No' then the question would be why national advice should not apply in the particular case. It is right that the issue should be confined to additional clinical benefit anticipated from the medicine and that other possible reasons to specially favour an individual patient should generally not apply.

IPTR panels have a responsibility not simply to consider the special interests of the patient whose case is being made but also the interests of other patients whose care or treatment would be adversely affected if resources were used to treat the individual case. The College believes that this balance is best achieved by having the relevant specialist (± patient) make the case to a panel of non-specialists, who can consider the case and the wider ramifications.

The IPTR process will inevitably lead to different decisions being made by different panels as the particular circumstances of individual patients will vary – this is as it should be in a flexible and responsive system. The College believes it would be good practice for Health Boards to share the decisions made by IPTR panels to minimise differences in decision-making processes and criteria between different Boards, accepting, as above, that individual decisions will differ.

Summary

Overall, the College believes that Scotland is well served by the processes in place to control access to new medicines. Unregulated access could distort prescribing and actually reduce overall health benefits. If there is ongoing concern that some decisions about new medicines are not what Scottish society would wish to see, then the College believes that it is the underpinning decision-making framework, rather than the technology assessment processes of SMC, which needs to be discussed, debated, researched and, perhaps, modified.

The Royal College of Physicians of Edinbugrh
30 August 2012

[i] NICE Citizens Council: The Citizens Council provides NICE with a public perspective on overarching moral and ethical issues that NICE has to take account of when producing guidance.

NHS Health Board responses to Questionnaire on Access to newly licensed medicines and Individual Patient Treatment Requests (IPTRs)

NHS Ayrshire and Arran

1.	How many individual patient treatment requests did the board receive in 2011/2012?	In 2011/12 NHS Ayrshire & Arran received 39 individual patient treatment requests.
2.	How many individual patient treatment requests has the board received to date in 2012/2013?	From **1st April 2012 until 30th June 2012** NHS Ayrshire & Arran received **19 i**ndividual patient treatment requests. (Total amended from 16. 12/09/12)
3.	How many of the individual patient treatment requests received by the board were approved in 2011/2012?	22 (56%) individual patient treatment requests in 2011/12 were approved.
4.	How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013?	13 (68%) of individual patient treatment requests have been approved between **1st April 2012 and 30th June 2012.**
5 (a)	How many of the individual patient treatment requests received by the board were rejected in 2011/2012?	17 (44%) individual patient treatment requests considered in 2011/12 were rejected.
5 (b)	What reason was recorded for rejecting these requests?	The patient's circumstances were not considered to be significantly different from the general population of patients covered by the medicines licence/population from clinical trials appraised by SMC/NHS HIS.
6 (a)	How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected?	6 (32%) individual patient treatment requests between **1st April 2012 and 30th June 2012** have been rejected.
6 (b)	What reason has been recorded for rejecting these requests	The patient's circumstances were not considered to be significantly different from the general population of patients covered by the medicines licence/population from clinical trials appraised by SMC/NHS HIS

NHS Ayrshire and Arran
27 August 2012

NHS Borders

1. How many individual patient treatment requests did the board receive in 2011/2012?	13
2. How many individual patient treatment requests has the board received to date in 2012/2013?	6
3. How many of the individual patient treatment requests received by the board were approved in 2011/2012?	11
4. How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013?	6
5. a) How many of the individual patient treatment requests received by the board were rejected in 2011/2012? b) What reason was recorded for rejecting these requests?	2 1. Applicant to assess whether they are able the level of impact the additional treatment they recommended would have on the patient and if their health has still not improved then to come back to the group. 2. More information requested from applicant on number of hospital admissions, the impact on quality of ife and what other medical options have been tried.
6. a) How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected? b) What reason has been recorded for rejecting these requests?	0

NHS Borders
6 September 2012

NHS Dumfries and Galloway

1. How many individual patient treatment requests did the board receive in 2011/2012? **24**

2. How many individual patient treatment requests has the board received to date in 2012/2013? **8**

3. How many of the individual patient treatment requests received by the board were approved in 2011/2012? **15**

4. How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013? **7**

5. a) How many of the individual patient treatment requests received by the board were rejected in 2011/2012? **9**

b) What reason was recorded for rejecting these requests?
i) 4 rejected because other treatment options were available
ii) 5 rejected because application failed to demonstrate exceptionality

6. a) How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected? **1**

b) What reason has been recorded for rejecting these requests?

Application failed to demonstrate exceptionality

NHS Dumfries and Galloway
10 August 2012

NHS Fife

The information from NHS Fife is detailed below:

1. How many individual patient treatment requests did the board receive in 2011/2012?

39 IPTRs were received.

2. How many individual patient treatment requests has the board received to date in 2012/2013?

12 IPTRs were received.

3. How many of the individual patient treatment requests received by the board were approved in 2011/2012?

32 cases were approved.

4. How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013?

8 cases were approved.

5. a) How many of the individual patient treatment requests received by the board were rejected in 2011/2012?

6 cases were rejected.

b) What reason was recorded for rejecting these requests?
Case 1 – Case for exceptionality has not been made. Not approved by SMC.
Case 2 – Not approved by SMC. Not clear that recommended alternatives have been tried.
Case 3 – Case for exceptionality not established. Part of suggested rationale seems to be substitute prescribing for illicit drug use.
Case 4 – On the basis of the limited information provided the Panel felt unable to support.
Case 5 – Case for exceptionality not established. SMC approved alternatives available and not yet tried.
Case 6 – Insufficient information / evidence. Requested further information but none received.

6. a) How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected?

1 case was rejected.

b) What reason has been recorded for rejecting these requests?

Case 1 – Exceptionality not established. SMC approved alternatives available in Department Guidelines.

Further additional information:

In 2011 / 2012 1 case was not considered.

In 2012 / 2013 2 cases were withdrawn and 1 case is still awaiting decision

NHS Fife
16 July 2012

NHS Forth Valley

1. How many individual patient treatment requests did the board receive in 2011/2012?

April 2011 – March 2012 - 20 requests

2.How many individual patient treatment requests has the board received to date in 2012/2013?

April 2012 – July 2012 – 5 requests

3.How many of the individual patient treatment requests received by the board were approved in 2011/2012?

April 2011 – March 2012 - 17 approved

4.How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013?

April 2012 – July 2012 – 5 approved

5.a)How many of the individual patient treatment requests received by the board were rejected in 2011/2012
April 2011 – March 2012 - 3
b)What reason was recorded for rejecting these requests?
The patient's circumstances were not considered to be significantly different from the general population of patients covered by the medicines licence/population from clinical trials appraised by SMC/NHS HIS

6. a) How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected?

April 2012 – July 2012 - NONE

b) What reason has been recorded for rejecting these requests?

Not applicable

NHS Forth Valley
12 September 2012

NHS Grampian

1. Many individual patient treatment requests did the board receive in 2011/2012? – 15 (fifteen): NB Number withdrawn has been less than 5.

2. How many individual patient treatment requests has the board received to date in 2012/2013? – 8 (eight)

3. How many of the individual patient treatment requests received by the board were approved in 2011/2012? – 8 (eight)

4. How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013? – Less than 5

5. a) How many of the individual patient treatment requests received by the board were rejected in 2011/2012? – Less than 5.

b)What reason was recorded for rejecting these requests? – in all cases the first part of decision-making was not proven.
- limited published peer reviewed evidence; use would not provide an opportunity for cure, long-term remission, significant extension of life or avoidance of permanent disability.
or
- patients clinical circumstances are not significantly different to that of the patient group in the trials and the patient would not gain more benefit than the trial cohort.

6. a) How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected? – Less than 5.

b) What reason has been recorded for rejecting these requests?

1 - not authorised –First part of decision-making not proven: the patient circumstances **did not allow** the case to be made that Grampian should put aside its general policy not to use a licensed medicine where SMC has yet to provide advice.

Table 1: NHS Grampian IPTR summary

Period	Authorised	Not-authorised	Withdrawn	Total
2011/12	8	<5	<5	15
2012/13 (YTD)	<5	<5	<5	8

NHS Gampian
10 September 2012

NHS Greater Glasgow and Clyde

1. **How many individual patient treatment requests did the board receive in 2011/2012?**

Between April 1st 2011 and March 31st 2012 there were 101 IPTRs submitted for NHSGGC patients

2. **How many individual patient treatment requests has the board received to date in 2012/2013?**

Between April 1st 2012 and July 31st 2012 there were 28 IPTRs submitted for NHSGGC patients and recorded on the relevant databases. However, it should be noted that there may be some IPTRs that either have not completed due process or have not yet been forwarded for addition to the IPTR database at the time of responding to this request for information.

3 **How many of the individual patient treatment requests received by the board were approved in 2011/2012?**

58 of the IPTRs submitted in the 2011-12 financial year were approved (57%)

4. **How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013?**

17 of the IPTRs submitted in the 2012-13 financial year until the end of July 2012 were approved (61%)

5. **How many of the individual patient treatment requests received by the board were rejected in 2011/2012?**

43 of the IPTRs submitted in the 2011-12 financial year were rejected (43%)
Of these, 4 rejected IPTRs were subject to appeal, 2 of which were subsequently approved.

b) What reason was recorded for rejecting these requests?
The reason for rejection was not routinely recorded **against the standardised SG terminology** in the 2011-12 financial year and the following data in table 1 has been obtained by reviewing the actual submitted IPTRs retrospectively

Documented reason for rejection	Number
The patient's circumstances were not considered to be significantly different from the general population of patients covered by the medicines licence/population from clinical trials appraised by SMC/NHS HIS	36
The patient's circumstances were considered to be significantly different, but it was felt this was unlikely to result in a significant benefit gain from this medicine over what would be normally expected	0
Incomplete form and/or insufficient detail to make an appropriate decision	1
Other reasons (e.g. failure to try other alternative medicines that would normally be trialled prior to IPTR submission)	6

Table 1: Documented reasons why IPTRs received in the 2011-12 financial year were rejected

6. How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected?

11 of the IPTRs submitted between April and the end of July of the 2012-13 financial year were rejected (39%)

b) What reason has been recorded for rejecting these requests?

Of the IPTRs rejected between April and the end of July of the 2012-13 financial year, all 11 were rejected because the patient's circumstances were not considered to be significantly different from the general population of patients covered by the medicines licence/population from clinical trials appraised by SMC/NHS HIS

NHS Greater Glasgow and Clyde
6 September 2012

NHS Highland

1. How many individual patient treatment requests did the board receive in 2011/2012? 7

2. How many individual patient treatment requests has the board received to date in 2012/2013? 1

3. How many of the individual patient treatment requests received by the board were approved in 2011/2012? 5

4. How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013? 1

5. a) How many of the individual patient treatment requests received by the board were rejected in 2011/2012? 2

b) What reason was recorded for rejecting these requests? 1. Limited evidence supporting efficacy and safety, 2. limited evidence supporting efficacy and safety and not cost-effective.

6. a) How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected? none

b) What reason has been recorded for rejecting these requests? Not applicable

NHS Highland
8 September 2012

NHS Lanarkshire

		PROCESS LAUNCHED IN JUNE 2011
1.	How many individual patient treatment requests did the board receive in 2011/12?	**From June 2011 to March 2012 = 34**
2.	How many individual patient treatment requests has the board received to date in 2012/13?	**April 2012 to thus far in August 2012 = 16**
3.	How many of the individual patient treatment requests received by the board were approved in 2011/12?	**June 2011 to March 2012 = 17**
4.	How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013?	**April 2012 to thus far in August 2012 = 8 + *1 in progress due for closure 14.9.12***
5. (a)	How many of the individual patient treatment requests received by the board were rejected in 2011/2012?	**From June 2011 to March 2012 = 17**
(b)	What reason was recorded for rejecting these requests?	• **Insufficient information & incomplete detail** • **Other treatment options remain** • **Insufficient information to proceed to panel** • **Medicine not approved for requested use.** • **Clinical benefit of non approved treatment not articulated and applications not demonstrated that patients can be considered as having factors that would make them exceptional to the current guidance.** • **Application failed to demonstrate exceptionality in relation to referral criteria**

6. (a)	How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected?	**April 2012 to thus far in August 2012 = 7**
(b)	What reason has been recorded for rejecting these requests?	• **Other treatment options remain** • **Insufficient information to proceed to panel** • **Insufficient evidence for panel to reach a decision** • **Treatment not approved for the requested use.** • **Application failed to demonstrate exceptionality in relation to referral criteria**

NHS Lanarkshire
5 September 2012

NHS Lothian

1. How many individual patient treatment requests did the board receive in 2011/2012?

   NHS Lothian received 17 individual patient treatment requests in 2011/2012. This includes all new applications, resubmissions, urgent requests and requests to the IPTR from Cancer Medicines Management Committee for ratification.

2. How many individual patient treatment requests has the board received to date in 2012/2013?

   NHS Lothian received 15 individual patient treatment requests in 2012/2013. This includes all new applications, resubmissions, urgent requests and requests to IPTR from Cancer Medicines Management Committee for ratification.

3. How many of the individual patient treatment requests received by the board were approved in 2011/2012?

   13 individual patient treatment requests received by the board were approved in 2011/2012.

4. How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013?

   6 individual patient treatment requests received by the board have been approved to date in 2012/2013

5. a) How many of the individual patient treatment requests received by the board were rejected in 2011/2012?

      4 individual patient treatment requests received by the board were rejected in 2011/2012.

   b) What reason was recorded for rejecting these requests?

      Please see enclosed reasons recorded for rejecting these requests.

6. a) How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected?

      9 individual patient treatment requests received by the board to date in 2012/2013 have been rejected

   b) What reason has been recorded for rejecting these requests?

      Please see enclosed reasons recorded for rejecting these requests.

Applications Rejected 2011/2012

11/0001
This application request was for the prescription of Cannabinoid Oromucosal Spray for the management of Multiple Sclerosis (MS) related spasticity which was not responding to other available treatment options or there were other intolerable side effects. The Panel were advised that Cannabinoid Oromucosal Spray had not been SMC approved as there had not been an application to SMC – the medicine had therefore not been considered by the SMC.
Because this medicine had not been considered by the SMC, the Panel noted that there was a lack of evidence and information on this treatment.
The Panel noted that this medicine could be prescribed to 6% of MS patients and therefore did not meet the IPTR criteria – that the patient's clinical circumstances (condition and characteristics) and potential response to treatment were significantly different to the general population of patients covered by the medicines license or the population of patients includedin clinical trials for the medicine's licensed indication appraised by the SMC.
Panel members also commented that the patient's employer could consider making adjustment to reduce pain and fatigue for the employee.

Decision The Panel was unable to support this application for the following reasons. The patient's clinical circumstances (condition and characteristics) and potential response totreatment were not significantly different to the general population of patients.

11/0004
This application was for the use of an unlicensed medicine where a licensed version exists.
The patient had a confirmed diagnosis of Lambert-Eaton Myasthenic Syndrome and had been treated with 3,4-diaminopyridine (3,4-DAP) and pyridostigmine – and had obtained more benefit from 3,4-diaminopyridine (3,4-DAP). The Panel agreed that the clinician could consider submitting an application to the Formulary Committee [FAF3, unlicensed medicines], as it applied to a particular group of patients.

Decision The Panel was unable to support this application but recommended that this treatment for this group of patients, be submitted to the Formulary Committee as a FAF3.

12/002
The Panel discussed the application for the Ross Procedure. The Panel agreed that this application could not be approved as more information was required on how this patient had factors different than the population and would likely gain significantly more benefit from this treatment.

Decision The Panel did not approve the above application as more information was required.
The consultant would be invited to resubmit the application and would also be encouraged to attend the meeting to speak to the application. Panel members also suggested inviting a surgeon to attend the meeting as this was for a surgical procedure. The application should also be signed by the clinical director.

12/004
The Chair explained that this was a resubmission of a previous application. The application for the Ross Procedure that had been discussed at the January meeting had not been approved for the following reasons - more information was required on how this patient had factors different than the population and would likely gain significantly more benefit from this treatment.
The Panel agreed that the resubmission could not be approved. The Panel felt that it would not be equitable to approve this application. The Panel agreed that there was not enough information on the individual clinical circumstances and why this patient had factors different than the population and would likely gain significantly more benefit from this treatment.
The Panel commented that if this treatment would benefit a group of patients then this should be considered as a service development rather than an individual treatment request.

Decision The Panel did not approve the above application as more information was required on why the patient's clinical circumstances were different from the population.

Applications Rejected 2012/2013
12/014
The Panel noted the SMC Report No. 490/08 (11.08.08). Teriparatide (Forsteo®) – not recommended for use within NHS Scotland for the treatment of osteoporosis in men at increased risk of fracture. The Chair explained that this treatment had been approved twice before at previous meetings
.
Decision Before a decision was made, the Panel requested clarification on whether the patient was male or female and how this patient had factors different than the population and would likely gain significantly more benefit from this treatment than the general population.

12/016
The Panel noted the second application for Tocilizumab. The panel noted that only one other treatment had been tried before requesting Tocilizumab.

Decision Before a decision was made, the Panel requested further information on why other treatment options had not been tried.

12/017
The Panel noted the *SMC Report No. 653/10.* prucalopride (Resolor) – not recommended for use within NHS Scotland. Indication under review: for symptomatic treatment of chronic constipation in women in whom laxatives fail to provide adequate relief. The Panel noted that there had been a number of other applications to previous IPTR meetings for prucalopride. At the March meeting it had been agreed that if there were any further applications for this treatment, the Panel would request an explanation of the pathway for patients with this condition.

Decision Before a decision was made, the Panel requested: _ Feedback from the clinicians on the previous applications forprucalopride

_ Clarification on whether the patient was female or male.
_ The patient should be made aware that the treatment was being used off label for an unapproved indication.
_ Provide information on the pathway for patients with this condition

12/019
[----] gave some background information on this application. He explained that the patient had developed metastatic renal cell carcinoma. [----] The panel noted that Nanoknife Electroporation treatment was not available in Scotland and only available at one private clinic in London. [----] advised that there was no clinical evidence to suggest that this treatment would be effective. The Panel asked about other treatment options, including Radiofrequency Ablation (RFA). He explained that this had been considered but it was unclear whether it would be more effective than Nanoknife Electroporation.

Decision The Panel did not approve this application and felt that other options, including RFA,should be considered in further detail.

12/023
The Panel ratified the decision made by the Cancer Medicines Management Committee
(CMMC).
12/024
The Panel ratified the decision made by the CMMC.
12/025
The Panel ratified the decision made by the CMMC.
12/026
The Panel ratified the decision made by the CMMC.
12/027
The Panel ratified the decision made by the CMMC.

NHS Lothian
22 August 2012

NHS Orkney

1. How many individual patient treatment requests did the board receive in 2011/2012?
Two

2. How many individual patient treatment requests has the board received to date in 2012/2013?
None

3. How many of the individual patient treatment requests received by the board were approved in 2011/2012?
One

4. How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013?
N/A

5. a) How many of the individual patient treatment requests received by the board were rejected in 2011/2012?
One

b) What reason was recorded for rejecting these requests?
The medicine which was requested was rejected as there was no additional evidence to that submitted to the SMC of clinical benefit. Additionally there was no evidence submitted that the this particular patients quality of life would be improved - the medicine was not likely to increase the symptom free period of the illness and would potentially cause additional problems for the patient.

a) How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected?
N/A

b) What reason has been recorded for rejecting these requests?
N/A

NHS Orkney
17 July 2012

NHS Shetland

1. How many individual patient treatment requests did the board receive in 2011/2012? **ONE**

2. How many individual patient treatment requests has the board received to date in 2012/2013? **ONE**

3. How many of the individual patient treatment requests received by the board were approved in 2011/2012? **ONE**

4. How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013? **ONE**

5. a) How many of the individual patient treatment requests received by the board were rejected in 2011/2012? **NONE**

b) What reason was recorded for rejecting these requests? **NA**

6. a) How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected? **NONE**

b) What reason has been recorded for rejecting these requests?**NA**

NHS Shetland
12 September 2012

NHS Tayside

1. How many individual patient treatment requests did the board receive in 2011/2012?
47

2. How many individual patient treatment requests has the board received to date in 2012/2013?
17

3. How many of the individual patient treatment requests received by the board were approved in 2011/2012?
35

4. How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013?
14

5. a) How many of the individual patient treatment requests received by the board were rejected in 2011/2012?
12

b) What reason was recorded for rejecting these requests?
The patient's clinical circumstances did not imply that they were likely to gain significantly more benefit from the medicine than would be expected in the general population of patients covered by the medicine's licence or in the population of patients included in the clinical trials appraised by SMC.

6. a) How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected?
3

b) What reason has been recorded for rejecting these requests?
As above

NHS Tayside
10 September 2012

NHS Western Isles

1. How many individual patient treatment requests did the board receive in 2011/2012?
None

2. How many individual patient treatment requests has the board received to date in 2012/2013?
None

3. How many of the individual patient treatment requests received by the board were approved in 2011/2012?
None

4. How many of the individual patient treatment requests received by the board have been approved to date in 2012/2013?
None

5. a) How many of the individual patient treatment requests received by the board were rejected in 2011/2012?
None

b) **N/A**

6. a) How many of the individual patient treatment requests received by the board to date in 2012/2013 have been rejected?
None

b) **N/A**

NHS Western Isles
6 September 2012

Agenda item 2 5 March 2012		HS/S4/12/24/ 6

HEALTH AND SPORT COMMITTEE

INDIVIDUAL PATIENT TREATMENT REQUESTS – RESPONSE FROM NHS BOARDS

Background

In June 2012, the Committee wrote to all NHS boards requesting data related to the numbers of Individual Patient Treatment Requests (IPTRs) that their IPTR panels had received in 2011-12 and 2012-13, how many of these had been approved, how many rejected and the reasons for that rejection. This followed on from data that the ABPI had obtained for the years 2009-10 and 2010-11 through Freedom of Information requests, on which a report was forwarded to the Committee in June 2012.

IPTR Application data

The ABPI results can be found in Table 2 (p 3), whilst the findings from the Committee's request for data can be found in Table 3 (p 4). The ABPI data had been incomplete, presumably as a result of not all boards having responded. However, the Committee has received data from all NHS boards.

When reviewing the data in Table 2 it is important to note that the vast majority of boards did not provide any information on how many applications were new, or related to a resubmission or an appeal. It has not, therefore, been possible to compare these factors.

Overall, a total of 359 IPTR requests were received by NHS boards in 2011-12 and 135 were reported as having been received in 2012-13 (some of these were in specific time periods – please see Table 2). Of these, 65% were approved in 2011-12 and 64% so far in 2012-13. However, as Table 2 shows, there is a significant difference in the numbers received. For example in 2011-12 NHS Greater Glasgow and Clyde received 101 requests, whilst NHS Western Isles received none. Perhaps unsurprisingly, there appears to be a correlation between the numbers received and the population size of the NHS Board area, though this is variable. For example, NHS Tayside had 47 requests in 2011-12 whilst the larger NHS Lothian had 17. It is difficult to make any assessment concerning the rate of approval given the IPTRs are being submitted on the basis of the individual circumstances of the patient. However, it is important to take account of the variation in the actual numbers of applications when analysing the percentage approved. For example, NHS Orkney has an approval rating of 50% in 2011-12 but it received only two applications.

Reasons for not approving IPTRs

The information provided by boards varied considerably. Some provided a more general statement of the reasons as to why IPTR requests were not approved. Others, because of the numbers involved (e.g. NHS Greater Glasgow and Clyde) provided information by category of refusal. The remainder provided information on each case (this tended to be where the number had been very small). NHS Lothian provided the most detailed information, providing the Committee with a summary of the background to each request and the reason for not approving it.

A table setting out all the reasons provided by NHS boards is available from SPICe. However, many of the reasons for refusal are summed up by the statement given by NHS Ayrshire and Arran:

> "The patient's circumstances were not considered to be significantly different from the general population of patients covered by the medicines licence/population from clinical trials appraised by SMC/NHS HIS."

Due to the very different levels of information provided, it is difficult to analyse the information quantitatively. However, when NHS boards were able to state why specific individual IPTRs had been turned down, a number of categories began to emerge, including:

- There was insufficient information provided and / or a lack of detail which meant the request could not progress
- The application did not consider the level of impact that the treatment would have on the patient, including on their quality of life
- Other treatment options were available and / or these had not been tried in the first instance
- The medicine was not approved for the requested use
- The application failed to demonstrate exceptionality in relation to referral criteria

Jude Payne
SPICe Research
13 September 2012

Note: Committee briefing papers are provided by SPICe for the use of Scottish Parliament committees and clerking staff. They provide focused information or respond to specific questions or areas of interest to committees and are not intended to offer comprehensive coverage of a subject area.

Table 1: Numbers of IPTR applications and approvals for 2009-10 to 2010-11, by NHS board, according to ABPI report

	Applications 09/10	Applications 10/11	Approved 09/10	Approved 10/11	% Approved 09/10	% Approved 10/11
NHS Ayrshire and Arran	117	101	Majority	Majority	Majority	Majority
NHS Borders	4	5	2	0	50%	0%
NHS Dumfries and Galloway	50	56	33	47	66%	84%
NHS Fife	7	8	7	8	100%	100%
NHS Forth Valley	17	14	16	10	94%	71%
NHS Grampian	2	6	0	3	0%	50%
NHS Greater Glasgow and Clyde	39 (2010 to March 2011)		32		82%	
NHS Highland	NR	NR	NR	NR	NR	NR
NHS Lanarkshire	14	<5	9-13	<5	>64%	-
NHS Lothian	204	186	188	166	92%	89%
NHS Orkney	NR	NR	NR	NR	NR	NR
NHS Shetland	NR	NR	NR	NR	NR	NR
NHS Tayside	212	146	202	137	95%	93%
NHS Western Isles	0	0	-	-	-	-

NR = Nil response. In the case of the data from APBI for 2009/10 and 2010/11, it is assumed these were nil responses as the relevant parts of the table have been left blank.

Table 2: Numbers of IPTR applications and approvals for 2011-12 and 2012-13, by NHS board, following request for data by the Health and Sport Committee

	Applications 11/12	Applications 12/13	Approved 11/12	Approved 12/13	% Approved 11/12	% Approved 12/13
NHS Ayrshire and Arran [1]	39	19	22	13	56	68
NHS Borders	13	6	11	6	85	100
NHS Dumfries and Galloway	24	8	15	7	63	88
NHS Fife	39	12	32	8	82	67
NHS Forth Valley [2]	20	5	17	5	85	100
NHS Grampian	15	7	8	<5 [5]	53	-
NHS Greater Glasgow and Clyde [3]	101	28	58	17	57	61
NHS Highland	7	1	5	1	71	100
NHS Lanarkshire	34	16	17	8	50	50
NHS Lothian	17	15	13	6	76	40
NHS Orkney	2	0	1	-	50	-
NHS Shetland	1	1	1	1	100	100
NHS Tayside [4]	47	17	35	14	74	82
NHS Western Isles	0	0	-	-	-	-
Scotland	359	135	235	86	65	64

NB It should be assumed that the data shown for 2012-13 relates to the period 1 April 2012 until the submission was made by the relevant board, unless otherwise stated

1 - data relates to the period 1st April 2012 to 30th June 2012

2 - data relates to the period April 2012 to July 2012

3 - data relates to the period 1st April 2012 to 31st July 2012

4 - data relates to the period 1st April 2012 to 31st July 2012

5 – given the small number of patients involved NHS Grampian did not provide figures below five in line with its data protection procedures.