Health and Sport Committee
8th Report, 2013 (Session 4)
Access to New Medicines

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# Health and Sport Committee

## 8th Report, 2014 (Session 4)

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### Annexe A: GLOSSARY

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Health and Sport Committee

Remit and membership

Remit:

To consider and report on health policy, the NHS in Scotland, anti poverty measures, equalities, sport and other matters falling within the responsibility of the Cabinet Secretary for Health, Wellbeing and Cities Strategy apart from those covered by the remit of the Economy, Energy and Tourism Committee.

Membership:

Bob Doris (Deputy Convener)
Richard Lyle
Aileen McLeod
Duncan McNeil (Convener)
Nanette Milne
Gil Paterson
Dr Richard Simpson
Drew Smith
David Torrance

Committee Clerking Team:

Clerk to the Committee
Eugene Windsor

Senior Assistant Clerk
Rodger Evans

Committee Assistant
Bryan McConachie
INTRODUCTION

1. The Committee’s work began (as described below in more detail) in response to three petitions (PE 1398, brought by Alastair Kent on behalf of Rare Disease UK, PE 1399, brought by Allan Muir on behalf of the Association for Glycogen Storage Disease (UK) Ltd and PE 1401, brought by Lesley Loeliger and Professor Peter Hillmen on behalf of PNH Scotland and the PNH Alliance) to the Parliament on behalf of patients suffering from rare and orphan diseases.

2. These petitions acknowledged work that had previously been carried out by the Public Petitions Committee in January 2008 on PE 1108, by Tina McGeever, on behalf of the late Mike Gray. The petition had called on the Scottish Government to consider the provision, on the NHS, of cancer treatment drugs, in particular cetuximab, to ensure equity across NHS boards on the appropriateness, effectiveness and availability of such treatments. This petition had led to revised guidelines being issued by the Scottish Government on the “end to end” process from licensing of medicines through to individual patient treatment requests (IPTR). However, the petitioners argued that this had not improved access to orphan medicines for patients with a rare disease because the clinical case made by the requesting physician continued to rely on the principle that the patient is in some way ‘exceptional’ from the general population of sufferers. The petitioners argued that the criteria that required to be met as part of this process were proving to be a particular challenge for patients with rare diseases.

3. Parliamentary committees have been involved with these issues over a long period, and there has generally been broad cross-party consensus on the need for change. However, the bringing of further petitions on the topic seemed to show that, at least in respect of rare and very rare conditions, the revised arrangements for IPTRs could not be sustained, in the sense that they did not address previous shortcomings, and there continued to be a need for further improvement in the system.
4. No formal inquiry was planned by the Committee – its original intention was simply to gain an understanding of the issues surrounding access to new medicines in order to enable it to deal with the petitions appropriately. However, it soon became clear that the issue of access to new medicines was not only complex and multi-faceted, but it was also one that attracted wide interest and varying opinion across stakeholders – patients, NHS boards, clinicians, the pharmaceutical industry and others.

5. It is perhaps not surprising, therefore, that the Committee’s work on this topic, despite not being established formally as an inquiry, rapidly attracted wide interest, and became easily the Committee’s most high-profile activity so far this session.

6. In introducing this report, the Committee expresses its thanks to all those who have contributed their views and experiences over the last year, but none more so than the original petitioners, who were responsible for initiating all the work that the Committee has carried out on this topic since.

7. As a number of the Committee’s witnesses have stated in evidence, NHS resources are finite and new or innovative medicines can be expensive. Thus, decisions need to be made about the value of treatments in relation to their effectiveness, cost and wider societal benefits, but within the context of a public sector under increasing budgetary pressures. When these issues combine with personal circumstances and experiences of individual patients and their loved ones and the impact that decisions can have on their length and quality of life, it is probably not surprising that the answers to the questions posed by this issue are not easily found.

8. Nevertheless, the Committee is pleased to have been able to contribute to and influence the debate on access to new medicines over the last year, and it submits this report to the Parliament in a spirit of genuine cross-party consensus over the issues and the way ahead, but in recognition that there are no easy answers.

9. Clearly, this issue has wider political resonance than the Committee. Many MSPs will have had contact from individual patients within their constituency or region, with a tale to tell of their patient journey and the impact that decisions on access to medicines has had on that journey. It is hoped, therefore, that there will be an opportunity, following the summer recess 2013, for a parliamentary debate led by the Health and Sport Committee that will enable the wider political interest in this topic to be explored and debated more fully.

**STRUCTURE OF THE REPORT**

10. The Committee has agreed to make this report relatively brief. There is a short chronology of the Committee’s activities on this topic over the last year, set in the context of wider developments. Following that there is a very brief explanation of the systems governing access to new medicines. The main body of the report will set out the Committee’s general views on the current situation, in the light of the Routledge and Swainson reviews and other recent developments, together with the Committee’s recommendations.
11. Finally, in Annexe B, the Committee will summarise the evidence it received on each of the specific recommendations made in the Routledge and Swainson reviews.

**CHRONOLOGY**

12. The Committee began to look into this issue in March 2012, following three petitions (petitions PE 1398, 1399 and 1401) being referred to it by the Public Petitions Committee, on the subject of “orphan” medicines, which may be used in treatment of very rare diseases.

13. After taking evidence from the petitioners, the Committee agreed to seek an informal briefing from the Scottish Medicines Consortium (SMC). This took place during the Committee’s business planning day on 28 August 2012.

14. On 18 September 2012¹, the Committee took evidence from the SMC, a number of health boards and the Association of the British Pharmaceutical Industry (ABPI).

15. Following that meeting, the Committee agreed to hold two further sessions on this topic, in order to hear the views of clinicians and patient representative organisations. The first of these sessions took place on 4 December 2012², when the Committee took evidence from oncologists and patient organisations and charities representing patients suffering from different types of cancer. A second session, concerned with other conditions (including rare diseases) took place on 29 January 2013³.

16. On 14 November 2012, the Cabinet Secretary announced⁴ a review of access to medicines, to be led by Professor Philip Routledge, Professor of Clinical Pharmacology at Cardiff University and Clinical Director and Chairman of the Board of the All Wales Therapeutics and Toxicology Centre and a review of the operation of Area Drug and Therapeutic Committees (ADTC) and the Individual Patient Treatment Request system, to be led by Professor Charles Swainson, former medical director of NHS Lothian. During the reviews, following interim advice from Professor Swainson, the Cabinet Secretary announced⁵ the establishment of a £21m fund to provide access to medicines for orphan diseases, specifically for those drugs not approved by SMC for use in the NHS in Scotland. The interim fund was to run from March 2013 to March 2014.

17. Following the publication of the Routledge and Swainson reviews on 3 May 2013, the Committee took evidence from the two review report authors, the

Cabinet Secretary and the Scottish Government’s Chief Pharmaceutical Officer, at its meeting on 7 May 2013\textsuperscript{6}.

18. The Committee also agreed to hold a further roundtable evidence session, involving all the stakeholders who had previously engaged with it on this topic, to gauge wider reaction to the findings of the two reviews. This session was held on 21 May 2013\textsuperscript{7}.

INTRODUCTION TO ACCESS TO NEW MEDICINES

19. The information in this section of the report is not intended to be a comprehensive overview of the relevant policy and legislative framework – it is, rather, intended to offer readers an outline of the policy context. It is adapted from the SPICE\textsuperscript{8} briefing on the subject, to which readers who require further information are addressed.

Overview of licensing

20. Licensing of medicines is a matter reserved to the UK government. Pharmaceutical companies that have developed new medicines may apply to one of two licensing authorities: the European Medicines Agency, which, in the case of successful applications, will grant a licence that applies in all EU member states, or to the UK Medicines and Healthcare products Regulatory Authority, which may grant a licence for UK use only. Both agencies carry out ongoing monitoring of medicines that have been granted licences.

After a medicine has received a licence

21. Once a medicine has been licensed in the UK it is available for general use by prescribers in the NHS, though many NHS bodies and prescribers will prefer to await official guidance on its use. The purpose of the guidance for the NHS is different to that of licensing, which considers efficacy and safety. Whilst NHS guidance does consider the efficacy of the medicine, it also reflects on its clinical effectiveness (i.e. how the medicine fits with what is currently being used) and its cost effectiveness (i.e. whether or not it is good value for money).

22. There are different arrangements across the UK for producing this guidance, and this can cause confusion and variation. Guidance over the use of licensed drugs in the NHS in Scotland is a devolved matter. However, there is a level of joint working that takes place between the organisations responsible for issuing guidance across the UK.

Scotland: role of Healthcare Improvement Scotland and the Scottish Medicines Consortium

23. In Scotland, Healthcare Improvement Scotland (HIS) is the body with statutory responsibility for producing guidance on all technologies, including medicines. However, for new medicines, the Scottish Medicines Consortium (SMC) is responsible for issuing advice. In England and Wales the equivalent body for both functions is the National Institute for Health and Clinical Excellence (NICE). However, there are circumstances where NICE’s “Multiple Technology Appraisal” guidance can be validated in Scotland, by Healthcare Improvement Scotland.

24. The SMC was established in October 2001. Its role is to advise NHS boards and their Area Drug and Therapeutic Committees (ADTCs) on the use of new medicines as soon as they are licensed by MHRA or EMA. The SMC is made up of representatives of all ADTCs, other health professionals, the pharmaceutical industry and patient representatives. HIS, is accountable for the governance and internal controls which support SMC to fulfil its responsibilities. Responsibility for provision of the health technology assessment of new medicines to the health service is delegated to SMC, which undertakes this on behalf of HIS. The SMC committee, however, as the decision-making body, makes autonomous, independent clinical decisions about the clinical and cost-effectiveness of new medicines.9

25. The SMC monitors when manufacturers launch new medicines and proactively asks them to make a submission on the product, including results of clinical trials and cost effectiveness data. The SMC’s role is to undertake an evaluation of the medicine’s clinical efficacy and cost effectiveness, relative to other available medicines, if applicable, and then to determine whether the medicine should be recommended for use in the NHS in Scotland. Additionally, it carries out pro-active horizon scanning in relation to forthcoming medicine developments.

England and Wales: role of NICE

26. NICE is responsible for providing national guidance on the promotion of good health and the prevention and treatment of ill health in England and Wales. As part of its role it produces technology appraisals, which offer guidance on the use of new and existing medicines and treatments within the NHS in England and Wales. It generally only reviews medicines referred to it by Ministers (which is unlike the situation with the SMC which appraises all new medicines when they receive a licence). The NHS in England and Wales is obliged to adhere to NICE guidance.

27. In Wales, there is also the All Wales Medicines Strategy Group (AWMSG), which takes decisions about which medicines should be available within the NHS in Wales. It aligns its work to NICE and will not normally review a medicine if NICE are planning to look at the medicine within the following 12 months. AWMSG issues interim guidance to the NHS in Wales, which is then superseded by any

9 Healthcare Improvement Scotland Code of Corporate Governance (still to be published at time of writing).
NICE guidance. Thus, if AWMSG says a medicine should be made available but then NICE issues guidance to say that the medicine should not be made available, then the NICE guidance stands.

Measuring the benefits of a medicine: the quality-adjusted life year

28. The health economics tool used to measure the benefit of a medicine is the quality-adjusted life year (QALY). This takes into account how a treatment affects a patient’s quantity of life (how long they live for) and the quality of life (the quality of their remaining years of life). These factors are then combined into a single measure that puts a figure on the health benefits for a medicine. The resulting QALY can then be used to benchmark the benefits each medicine is likely to offer. Then, to consider the cost effectiveness of the medicine, the QALY is combined with the cost of the medicine to produce a ratio called the cost per QALY.

29. Some medicines have a lower cost per QALY and these are normally considered to offer good value for money. Medicines with a higher cost per QALY may be less likely to be considered good value for money. The SMC has stated that a cost per QALY of under £20,000 is generally considered acceptable value for money. SMC might also accept a cost per QALY of between £20,000 and £30,000 if the medicine gives significant benefits over existing treatments. In addition, the SMC can consider using “modifiers” for any medicine under assessment where the estimated cost per QALY is relatively high. This means that, in some specific situations, SMC may exercise greater flexibility in its decision making to allow consideration of additional factors. Thus, if a medicine has an estimated cost per QALY of more than £30,000, and the SMC Committee is confident that the company’s clinical and health economic case is robust, it can consider whether one or more of the modifiers would allow it to be accepted. This could mean, for example, that medicines with a cost per QALY above £30,000 might be considered to offer good value, in certain circumstances.

30. However the Committee notes that in his review Professor Routledge found that SMC “does not have formal cost effectiveness threshold because the QUALY is only one (albeit important) factor informing the process of arriving at a recommendation on a specific medicine”. 10

After SMC assessment

31. On completion of the SMC assessment process, its advice for NHS Scotland is published. NHS boards are required to consider this advice. It is important to note that NHS boards will consider all SMC accepted advice as a matter of course but can still decide not to include such medicines on their own local formulary i.e. where the medicine does not represent sufficient added benefit to other medicines already on the formulary for the same indication. However, the Committee notes that clinicians are still free to prescribe medicines not on a local formulary. The Scottish Government recently issued guidance to boards on this issue.

10 https://www.scotland.gov.uk/Publications/2013/05/2542/0
32. NHS boards are expected to fund the cost of SMC accepted medicines from within their resource allocations, according to their local formulary/approved lists.

33. SMC advice may be superseded when, in the case of “not recommended” advice, a pharmaceutical company makes a resubmission which subsequently leads to “recommended” advice, or, when NICE publishes a Multiple Technology Appraisal which is then adopted by Healthcare Improvement Scotland.

Accessing medicines not recommended for use

34. Prior to April 2011, there was no formal guidance for NHS boards to make decisions on requests by patients to be treated with a medicine not recommended for use within NHS Scotland. However, it was generally the case that, in order for a board to agree to such a request, the patient would need to be significantly different to the general population of patients with the condition in question and, additionally, likely to gain more benefit from the medicine than would the average patient. These criteria were referred to as “exceptional circumstances”. NHS boards had their own procedures for dealing with such decisions.

35. Following from the issues raised by petition PE1108, concerning the provision on the NHS of cancer treatment medicines, the Scottish Government published guidance in May 2010, which set out a framework for the development of NHS board written policies for dealing with such requests, now known as Individual Patient Treatment Requests (IPTRs). Further guidance has been subsequently published. The Scottish Government expects these processes to be used though accepts there will need to be a degree of flexibility.

36. Each board’s written policy should have been in place and available on the board’s website by 1 April 2011.

Pharmaceutical Price Regulation Scheme

37. The regulation of pricing of branded medicines is listed as a reserved matter and is currently taken forward through the Pharmaceutical Price Regulation Scheme (PPRS), which is a voluntary scheme used to regulate the price of branded medicines. The current PPRS arrangements are in place until 31 December 2013. It seeks to achieve a balance between reasonable prices for the NHS and a fair return for the industry to enable it to research, develop and market new and improved medicines.

Patient access schemes

38. The 2009 PPRS introduced Patient Access Schemes (PAS) – an agreement reached with a pharmaceutical company where discounts or rebates are offered to reduce the costs of a medicine to the NHS. This then improves its cost-effectiveness, and thus enhances the likelihood of availability. In Scotland, the manufacturer can propose a PAS when making a submission for a new medicine to the SMC. The PAS Assessment Group (established under the auspices of NHS National Services Scotland) carries out an assessment of the PAS in a process independent of the SMC. Where a PAS is considered feasible, the SMC is able to
take account of the discount offered under the terms of the PAS. Where a PAS is not considered feasible, SMC would appraise the drug on its standard costs.

**Value-based pricing**

39. The UK Government stated, in its programme for government, its intention to reform arrangements for the pricing of branded medicines and to introduce a new system of value-based pricing (VBP). It is argued this would create a closer link between the price the NHS pays and the value that a medicine delivers. As part of this inquiry, the Committee has received briefings on value-based pricing from the UK Department of Health and the Scottish Government. Clarity has yet to be provided by both the Scottish and UK governments regarding which parts of value-based pricing are reserved and devolved.

**The cancer drugs fund in England**

40. The cancer drugs fund (CDF) provides £200m each year to enable patients in England to access drugs that are not routinely funded by the NHS as NICE has assessed them not to be cost-effective.\(^{11}\) It was established in 2010 and is planned to run until the end of March 2014, when the value-based pricing regime is due to come into effect. Much of the media coverage of this issue, particularly in relation to certain cancer drugs being available on the NHS in England, but not in Scotland, has related to the provision of these drugs through the CDF.

**THE COMMITTEE’S COMMENTARY ON THE ROUTLEDGE AND SWAINSON REVIEWS**

41. The Committee took evidence from Professors Routledge and Swainson, and from the Cabinet Secretary, immediately following the publication of the review reports.

42. Both review reports contain specific recommendations, which are set out in annexes to this report, together with a bullet point summary of the reactions. In this section, the Committee offers its general commentary on the overall conclusions and recommendations of the review and their likely impact, should they be implemented in full.

**Operation of the SMC and ADTCs**

43. There is widespread acknowledgement in both reviews that the work of SMC is highly regarded and internationally renowned. Professor Routledge told the Committee that he had concluded that the process used in Scotland to appraise new drugs was “very good and one of which it should be proud”\(^ {12}\). He went on to say that his recommendations related “largely to trying to increase the transparency of the process, so that all those who are involved in it and who have

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\(^{11}\) Funding is only considered for cancer medicines that meet one or more of the following: the medicine has not been recommended for use by NICE; NICE has not, or not yet, issued guidance on the medicine; NICE is not going to consider the medicine; or, the medicine not been not been prioritised through the NHS Commissioning Board Cancer Drugs Prioritisation Process.

an interest in the outcome can see the qualities of the process that Scotland uses"\textsuperscript{13}.

44. Similarly, Professor Swainson told the Committee that, overall, he found the systems of the Area Drug and Therapeutics Committees (ADTCs) and individual patient treatment requests to be “reasonably sound”\textsuperscript{14}. He did add, however, that he was struck by the evidence he received that “the quality and consistency of the arrangements left something to be desired”\textsuperscript{15}. Many of his recommendations, therefore, were about tightening up and improving on the arrangements that were already in place. In essence, he said, he was “asking for more transparency, public reporting and, in some cases, involvement in these important systems”\textsuperscript{16}.

45. Recommendations in the reviews, therefore, it seems to the Committee, are about making incremental improvements to a set of systems and processes that, while regarded as functioning adequately, require improvement. Many of the suggestions – for example for SMC to meet in public, for the creation of citizen’s juries and the increased role of patient participation, the standardisation of IPTR paperwork and regular audit of IPTR decisions – are about relatively minor enhancements intended to improve the transparency of the systems and processes, rather than radical changes to the systems themselves. It was noted that the changes suggested by both reviews would do very little to improve access to new medicines in any meaningful way.

**Stakeholder views**

46. The review recommendations were generally welcomed by the Committee’s witnesses during its stakeholder session and there was broad consensus that the proposed changes intended to increase openness, transparency and the provision of information and support were to be welcomed. There were, however, some questions raised about, amongst other things, the extent to which the proposals would improve access to new medicines, and the resource implications that the increase transparency and scrutiny could bring.

**Petitioners**

47. For the petitioners and other representatives of patients suffering rare diseases, questions remained about whether the proposals went far enough in relation to rare conditions, and the extent to which they would address the concerns of the petitioners. Lesley Loeliger, for example, a patient with the ultra-orphan bone marrow disease paroxysmal nocturnal haemoglobinuria, or PNH, welcomed the Routledge report and was grateful that it acknowledged the term “ultra-orphan”. However, she went on to say that issues that still surround the IPTR system and the rare conditions medicine fund “were not necessarily brought out in the report”\textsuperscript{17}.

48. Ian Mackersie of aHUSUK, a charity and support group for patients in the UK with atypical haemolytic-uraemic syndrome – an ultra-rare disease that is thought

to be suffered by about 20 to 25 patients with aHUS in Scotland. Particularly welcomed Routledge’s recommendation 5, that the “SMC should develop a policy … to guide the process of consideration of all available evidence relevant to its advice on” ultra-orphan medicines. No such thing had existed before, he said, and this meant that ultra-orphan medicines had “struggled to get past the SMC”. aHSUK also welcomed Swainson’s recommendation 12, that the rare conditions medicines fund should concentrate on funding access to medicines for ultra-orphan diseases and the proposal that a group PTR could contribute to the funding of drugs that had not got past the SMC.

49. Joan Fletcher of the Association for Glycogen Storage Disease, a patient support group that also covers Pompe disease (one of the original petitioners, along with PNH Scotland and Rare Disease UK) while welcoming the general endorsement of the work of the SMC and the review proposals for increasing transparency, told the Committee that the charity believed that the requirement to prove exceptionality in order to achieve a successful IPTR remained “a stumbling block”. She continued—

“We believe that the drug that is used for Pompe disease—the enzyme replacement therapy—is effective. We are aware that it is very expensive, but it has been proven to be effective. How will proving exceptionality—saying that patients are different from those who were on the trial—make any difference? Why should we prove exceptionality when we say that the drug works? Why should the patients whom we want to use it for be any different from the patients who were used for the trial?”

50. The Committee also received a written submission from Rare Disease UK and AGSD UK, on behalf of the original petitioners PE 1398 and 1399. Like the oral evidence above, the submission welcomed many of the proposals in the Routledge and Swainson reviews, including proposals for improving the transparency of the SMC, ADTC and IPTR processes, which they said would “improve public and patient confidence and improve understanding of how each process works”. However, the submission also stated that—

“The Petitioners remain concerned however, that the reviews have failed to demonstrate any meaningful improvement to the process for accessing new medicines. For example, whilst Recommendation 5 of Professor Routledge’s review recommends that the SMC adopt a policy for ultra-orphan medicines, the review falls short of making any meaningful recommendations as to what this policy should look like in practice. The petitioners are also disappointed that Professor Swainson’s review fails to address the difficulties faced by rare disease patients in achieving success through the IPTR process.”

NHS boards and clinicians
51. Other witnesses at the Committee’s roundtable event on 21 May 2013, echoed the general welcome to the two reviews. Professor Charlie Gourley, for example, told the Committee that many of the recommendations in the reports were “very welcome”. Dr Stephen Harrow of the Beatson West of Scotland Cancer Centre said that the Routledge report was “fair” noting that all the consultants with

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whom he worked understood the complexity of the situation and were sensitive to
the fact that all the drugs were expensive and that they must consider cost
effectiveness. He concluded that transparency was “always a good thing”\(^{19}\).

52. Clinicians and boards did, however, like the petitioners and other patient
groups, have concerns that the review of the IPTR process did not contain
proposals that would make much difference in terms of access to new medicines.
Dr Harrow said that the Swainson report was “really disappointing”—

“It does not change my practice at all. We are still in the situation that we
were in before the report was published, in that we have a difficult system to
navigate and the recommendations will not provide any greater access to
medicines that have not been through the SMC or which it has turned
down.”\(^{20}\)

53. There were also concerns amongst the boards and clinicians over the
resource demands that some of the recommendations in the reviews would bring.
Melinda Cuthbert of NHS Lothian explained that some of the things that needed to
be achieved related to resource issues. She noted that if boards were to deliver
training to all doctors on IPTR processes or provide greater support to patients so
that they could engage the IPTR process, they would “need to look at
resources”\(^{21}\).

54. This position was echoed by David Pfleger of NHS Grampian, who noted that
scrutiny comes with resource implications and that resource comes from
elsewhere, so there is an opportunity cost—

“I accept that we need to rebuild public and patient trust with our processes
and systems. That resource may therefore be well used, but we must
remember that it comes from somewhere else in the system.”\(^{22}\)

55. The Committee, too, welcomes the review proposals in regard to the
operation of the SMC and the IPTR process, which will have some benefits,
including improving the transparency of the process, make the system less
complex and should promote better access to specialist clinicians within the
decision making process.

56. However, the Committee remains concerned that, welcome though the
changes proposed in the reviews are, they do very little to address the clear
barriers to access to new medicines experienced by patients. The challenge
is not only to improve transparency and consistency within the IPTR
process but to ensure that the SMC process in the first instance better
assesses the cost effectiveness of medicines.

57. One of the central difficulties with the IPTR system expressed in the
evidence given to the Committee was proving the exceptionality of patients’
circumstances, particularly with orphan and ultra-orphan conditions. It is

believed that, in its current form, this approach acts as a barrier to accessing drugs clinicians believe their patients need. The Scottish Government must outline steps it plans to take that will improve the process.

58. The Committee also believes that, in the interests of improving transparency of the IPTR system, the Scottish Government should review and analyse previous IPTR decisions and look to improve monitoring of applications and consistency of future IPTR decisions. The number of applications, negative and positive decisions, among other relevant details, should be published regularly.

59. The recommendation that decisions on whether to include a medicine on the local formulary be made and announced within three months on an SMC recommendation should help to promote consistency and transparency. There may also be a further case to be argued that all ADTCs should put new SMC approved medicines on their formulary within three months, whether or not the prescribing and clinical guidelines had been fully completed by that time. Clinicians can therefore use their professional judgement whether to use medicines on a nationally available formulary or to await guidance locally.

Area drug and therapeutic committees

60. The Swainson report considered the question of whether there was a need for 14 ADTCs across Scotland. In relation to the operation of formularies, he commented—

“In practice, the Western Isles, Orkney and Shetland share a formulary with Highland and Grampian respectively, Lothian and Borders work closely together, and the regional cancer, diabetes and cardiac networks deliver co-ordinated and agreed treatment clinical pathways, each covering several health boards. Thus many decisions are taken collectively now while retaining local ownership and governance. The HIS audit demonstrates also considerable convergence of Board ADTC decision making.”

61. Professor Swainson also considered in detail the advantages and disadvantages of the current system, a move to a regionalised system or to a single national ADTC. He concluded—

“ADTC do more than maintain a local formulary of medicines based on SMC recommendations. They act as a catalyst for education in the correct use of medicines and therapeutic developments, and agree many other aspects of medicines management including intravenous medicines, special medicines for skin disease and abdominal illness, feeding supplements, antimicrobial policies based on local laboratory data as well as national policy, medicines surveillance and post marketing identification of side effects. They undertake clinical audit and monitor the compliance with local formulary. These functions are not likely to be replaced by a national body. In my view, the gains from a national ADTC are small in relation to the costs needed and the associated opportunity costs generated. Much more can be done to
encourage ADTC to address the issues of variation, delay and lack of transparency as I have recommended. However the ADTC need to work smarter and harder to demonstrate that the local advantages of retaining ADTC are matched by improvements in access to new medicines and public reporting of their work.

62. The Committee notes Professor Swainson’s findings in relations to ADTCs and heard arguments put forward for the retention of all 14 ADTCs in their current form. However, there may well also be arguments that have not, so far, been fully explored, in favour of a smaller number, or even a single national body, particularly in relation to patient treatment requests. Moving towards a national patient treatment request body may be the most effective way to ensure both a consistent application of IPTR and GPTR criteria as well as consistency in decision making. This point is particularly important in terms of orphan and ultra-orphan conditions, which, given their rarity, are less likely to have sufficient clinical expertise at individual ADTC level. Indeed, the necessary expertise might not exist within Scotland at all, for some conditions.

Citizens’ juries

63. Professor Routledge recommended that citizens’ juries or councils be established to explore views around specific societal issues of importance to the people of Scotland in relation to the availability of new medicines, and the impact of these views on the existing processes for ensuring access to medicines.

64. This proposal was welcomed by those stakeholders who gave evidence to the Committee. David Pfleger, for example, told the Committee—

“...the citizens’ panel may provide a useful forum to have societal discussions about how much and what we are willing to pay for medicines and about some of the modifiers that the SMC is perhaps able to use in its discussions on cost per quality-adjusted life year. That societal debate is absolutely needed, so a forum in which that can take place would be very welcome.”

65. There were, however, some notes of caution, with some witnesses voicing concern about asking members of the public to deliberate on such complex issues, suggestions that a lot of work would be necessary to make what was a good idea work in reality and an acknowledgement that while engagement with the public was a good thing, the pathways were “extremely complicated”.

66. The Committee accepts that this proposal would be likely to be helpful, although it notes the caveats that were mentioned by witnesses, and therefore recommends that developments in regard to citizens’ juries be taken forward cautiously.

67. **The Committee calls on the Scottish Government to consider plans for citizens’ juries and provide more detail on how it expects them to work in practice to ensure that they improve the process.**

Do these proposals go far enough?

**Process improvements but QALYs and modifiers remain basis of decisions**

68. Although, as mentioned, there was a general welcome from witnesses for the review proposals, there was also recognition from many that the reviews would not necessarily address all the problems that had been identified with the system as it currently operates. David Pfleger of NHS Grampian, for example, told the Committee—

“At the end of the day, we have a triangle that is made up of the cost of a medicine, its clinical effectiveness and the health system’s willingness to purchase it at a cost-effective level. None of the discussions that we have had change that at all. The only out to that is the rare conditions medicines fund and the group patient treatment request that is included in that. An example is the ivacaftor group.

We hope that we will have much more responsiveness, transparency and openness, and much more person-centredness in the IPTR process. Will the national level of access change? If the SMC still uses the costs per QALY and the modifiers that it currently uses, the rates of yeses and noes will remain unchanged.”

69. Others echoed this view. Professor Angela Timoney, the SMC Chairman told the Committee that the recommendations in the report were good but were “about process.” She stated that the SMC was concerned that “all we are doing is putting in more processes” which “might slow us down a bit, but would “probably not change our decisions—possibly a little around the ultra-orphans but not necessarily”. Eric Low of Meloma UK said that “we have not had the right debate; we have been looking under the wrong stone for the solution”.

70. **The Committee, as already stated, welcomes the moves signalled in the review reports towards higher degrees of openness and transparency. However, it remains concerned that these recommendations will not entirely address the issues that have given rise to most concern over the last few years. SMC’s methodologies will remain largely unchanged, even though it may meet, at least partially, in public. But decisions on whether to recommend a medicine for use in NHS Scotland will still be based on the cost of the additional quality adjusted life years (QALY) that a drug treatment might provide.**

71. **No one argued that there was a better system than the QALY for assessing the value offered by competing treatments, despite its limitations. The committee has already considered the complexities around the cost per**

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QALY. It is clear that the way in which modifiers are applied is a crucial element in determining the cost effectiveness of medicines by SMC. The committee therefore calls on the SMC and Scottish Government to review as a matter of priority how modifiers and thresholds are applied to better take account of orphan and ultra-orphan conditions, end of life and innovation and to bring a higher degree of transparency.

**Orphan and ultra-orphan conditions**

72. As part of the current SMC appraisal process it is accepted that: “[in] some specific situations, the SMC may exercise greater flexibility in its decision making to allow consideration of additional factors.”

One of these situations relates to orphan medicines – those for the prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than 5 in 10,000 people. Whilst the SMC requires orphan medicines to go through the same assessment process as with any other medicine, it recognises that such medicines have more limited data on efficacy. Therefore, the SMC takes into account additional factors termed “modifiers” to be used in the assessment of orphan medicines, which allow it to accept greater uncertainty in the health economic case. In some cases, this allows it to accept a higher cost per QALY.

73. The original petitioners were particularly concerned that this process, even with the modifiers, was inappropriate for medicines intended for use in the treatment of “ultra-orphan” or “ultra-rare” conditions—those licensed for the treatment of diseases with a prevalence of less than 1 in 50,000. In evidence Stephen Nutt (Rare Diseases UK) noted a particular problem in taking this forward, in that—

“[the] term “ultra-orphan” is generally accepted among the rare disease community, but there is no formal mechanism for recognising an ultra-orphan disease in Scotland.”

74. However, it is a term recognised by NICE and the All-Wales Medicines Strategy Group. In his report, Professor Routledge considered this issue and recommended that—

“In order to further address the specific challenges associated with “ultra-orphan” medicines (those medicines licensed for the treatment of diseases with a UK prevalence of less than 1 in 50,000) SMC should develop a policy specifically relating to ultra-orphan medicines to guide the process of consideration of all available evidence relevant to its advice on these medicines.”

75. The Committee welcomes this recommendation and, as mentioned in the previous section, recommends that SMC and Scottish Government urgently address the recommendation, with particular effort to review and

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29 Scottish Medicines Consortium (2012). ‘SMC Modifiers used in Appraising New Medicines’


consolidate the system of modifiers to take account of the factors that are specific to orphan and ultra-orphan medicines and to increase transparency and clarity in relation to the use of modifiers.

76. However, the Committee is conscious that there is a wider debate on the effectiveness of current modifiers, not just for ultra-orphan medicines but for other high cost medicines. Even after the publication of Professor Routledge’s report, concerns were raised that the recommendations in themselves would not necessarily result in any greater number of medicines being recommended for use in the NHS. Eric Low (Myeloma UK) argued—

“The issue, which was not part of the review, is why we get noes from the SMC. We need to focus on that, because ultimately what we want is to get more yeses and reduce the reliance on individual patient treatment requests, which are not the way in which to make decisions on access to medicines. We need to work with the SMC, which has a willingness to engage, and focus on its mechanisms and modifiers, to ensure that we can get more yeses.”

77. The Committee is conscious that its work on this issue started with a number of petitions relating to orphan and ultra-orphan diseases. The Committee therefore welcomes the decision of the Cabinet Secretary, based on advice from Professor Swainson, to establish the £21m rare conditions medicines fund (RCMF), which should provide increased access for some patients suffering from orphan and ultra-orphan conditions, to medicines not approved by the SMC.

78. However, questions remain about the extent to which this fund will, in practice, result in improving access to these medicines, given that it will still be necessary for the patient’s clinician to have made a successful IPTR or been part of a Group Patient Transfer Request (GPTR) in order to access this fund. The Committee notes that guidance is to be published and hopes that this will lead to greater understanding of how the fund works.

79. The Committee accepts that there needs to be some objective measure that determines the likely benefit to the patient from gaining access to the medicines in question, but in view of the extremely small number of patients in Scotland who suffer from these rare conditions, the Committee expects that it would be very difficult for clinicians to be able to demonstrate the degree of exceptionality required for a successful IPTR.

80. The Committee also notes that the RCMF, as announced by the Cabinet Secretary, is to be available for a period of only thirteen months. The Committee understands that this is intended to cover the period before the introduction of value-based pricing, but, so far, it has not been possible to establish this clearly. The Committee therefore calls on the Cabinet

34 A GPTR is one of the proposals contained in the Swainson report. What is proposed is a system whereby the relevant specialists get together on behalf of the group of patients whom they look after, if they share common characteristics, and make a single application on behalf of that group, rather than each patient having to go through an individual application.
The cancer question

81. Much of the media coverage of this issue has been focused on cancer treatments and the differences in the routine availability on the NHS of certain expensive medicines in Scotland and in England. The Committee accepts that the establishment of the RCMF will be helpful for some patients suffering from the rarer forms of cancer. However, it is not immediately obvious that the review proposals will, in the cases of patients suffering from the commoner cancers, result either in more expensive and innovative cancer drug treatments being recommended by SMC, or in higher numbers of IPTRs for these treatments being successful.

82. The Committee is also aware that the existence of the cancer drugs fund in England has helped to provide access there to drug treatments not assessed as offering good value by the NICE appraisal. No such fund currently exists in Scotland and the Committee has heard no evidence to suggest that there should be such a fund. The cancer drugs fund however is a factor in increasing availability of cancer drugs in England. Professor Routledge told the Committee that, although rarer cancers that fell within the ultra-orphan or orphan category would be part of the RCMF provisions, he “would be loth to single out cancer from other conditions that shorten life or reduce the quality of life significantly”. Professor Swainson agreed, telling the Committee that no evidence was presented to him that drugs for cancer were treated any differently from other drugs in SMC decision making or in decisions on IPTRs. Roughly two thirds of IPTRs were successful, he said, and the same proportion applied to IPTRs for drugs for patients with cancer. Singling out a particular condition, he said, “would lead us down a very different road” suggesting that “many patients with different conditions would argue that the same should apply to their condition”.

83. None of the clinicians or patient organisations who took part in the Committee’s stakeholder consultation event argued for an equivalent to the cancer drugs fund for Scotland. Eric Low, of Myeloma UK, for example, told the Committee—

“we need to come up with our own solution that is not the CDF but is another mechanism. I think that we are all saying the same thing. We just need to get to the point. Let us get round the table and have the discussion, because we cannot have a widening of the disparity in access between Scotland and England. That would be terrible. Let us get on and have the discussion.”

84. This view attracted a significant degree of consensus. For example Professor Charlie Gourley said—

“I back that up. Clearly, there is a big disparity, particularly with regard to cancer medicines, but I do not think that the creation of a cancer drugs fund is necessarily the best solution. The idea is to find a Scotland-specific solution that is fair for all conditions, but flexibility will be needed by the SMC or any organisation that sits outside it that is going to do the negotiation and try to get the best possible value…Rather than having a fund that sits outside
everything, such as the cancer drugs fund, which patients with non-cancer conditions could consider to be unfair, we should have a more global solution. We need to start working for that now. At present, there is a big disparity and patients who have diseases that they want to get drugs for are talking about moving south of the border.”

85. The Committee notes that its evidence found little support for the establishment of a cancer drugs fund in Scotland. The Committee also notes that criticism of the Cancer Drugs Fund was to an extent its focus on one particular disease category; bypassing the cost effectiveness and regular NICE procedures.

86. Indeed, the Committee notes that Professor Swainson’s review recommended the creation of a fund specifically for orphan and ultra-orphan conditions. The Committee therefore notes that evidence and recommendations given to it does not lead it to believe that the Scottish Government is opposed to the creation of funds to improve access to drugs for certain conditions.

87. The Committee therefore agrees that, although SMC decisions about specific cancer medicines may be unpopular, they are soundly made and should continue to be made on the same basis as they are for medicines for other conditions, and that cancer should not be singled out for special treatment in comparison to other life-shortening conditions. The Committee accepts that there are difficult issues in relation to individual patient’s treatments, and that it can be difficult for patients and their families to accept that medicines may not be available on the same basis in all parts of the UK. Nevertheless, the establishment of a cancer drugs fund in Scotland would not be the answer to these problems.

88. The Committee received evidence from some clinicians expressing frustration at an unsuccessful IPTR despite them submitting clinical and expert evidence as part of the request. For example, Dr Stephen Harrow of Beatson West of Scotland Cancer Centre said—

“We, as a team of colorectal cancer consultants, all tried to sign the IPTR request to show that we were united behind the drug. I also got the surgeons to agree to the proposal for the patient. However, the request was turned down at the IPTR level. In the appeals process, we sought advice from 10 specialists across the UK who were using the drug and I presented that advice at the appeal. However, that did not carry any weight although they all agreed that we had met the criteria in the IPTR process. In addition, I had supporting documentation from the professor of medical oncology and the professor of translational research saying that I had met the criteria in the IPTR process. When that was turned down, I appealed the appeal decision with another letter from the professor of medical oncology, but again that was not deemed to be sufficient.”35

89. The Committee believes that urgent consideration should be given to encouraging greater flexibility in the IPTR process to approve drugs where there is clear, clinical evidence that a particular patient would derive material benefit from such a drug even if existing IPTR criteria had not been met fully.

Clinical research trials

90. During the Committee’s inquiry, the question of clinical research trials had been raised, mainly by clinicians, on a number of occasions. Professor Charlie Gourley, for example, had told the Committee\(^{36}\) on 4 December 2012 that he and others were “proud of our history of conducting good clinical trials and being at the forefront of medical research”. Historically, he said, clinicians had been involved in proving that the new drugs were beneficial to cancer patients, but we were now “moving into an era in which we are not being allowed to give the drugs that we have proved are beneficial”. He said that this had a knock-on effect for the next generation of clinical trials because, “people will assume that we can access the drugs—as the standard of care—through our normal healthcare system. Explaining that all trials compare a new combination of drugs with the standard of care, he said that new drugs were now considered to be the standard of care but, because they are not generally available in Scotland, the next generation of patients is denied access to trials of the next line of drugs. He said that Scotland was “moving to being a generation behind because of that”. This view was echoed by the other clinicians present.

91. In the Swainson review, it was noted that the Chief Scientist Office had indicated that in recent years there had been a decline in traditional, large phase III commercial trials. This was said to be “in line with expectations” as the UK was “not able to compete on price with emerging markets in Eastern Europe, the Far East and South America”.\(^{37}\) However, the review went on to state that Scotland had emerged as a country where smaller phase II studies are placed, frequently involving fewer than five patients, and for studies that were proving difficult to recruit in other countries. As a consequence, the number of commercial trials undertaken at sites in NHS boards in Scotland had held up at over 600 since 2010. Data on contract value from NHS boards in Scotland had suggested there has been no fall off in income for commercial studies.

92. At the evidence session on 7 May 2013, Professor Swainson added that “we no longer do the same larger-scale trials in Scotland or indeed in England and much of Western Europe”.\(^{38}\) The emphasis of research had shifted towards smaller-volume, higher-value studies, often of proof-of-concept or very early investigations on the drugs concerned. The larger-scale work for licensing, he said, was done elsewhere in the world.

93. In response to that section of the Swainson report, Professor Gourlay reiterated that there was “absolutely no doubt that a number of examples of clinical studies cannot be done in Scotland because we cannot provide what is regarded internationally as the standard of care—we cannot provide the standard drugs”. He

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\(^{37}\) [http://www.scotland.gov.uk/Publications/2013/05/2542/0](http://www.scotland.gov.uk/Publications/2013/05/2542/0)

said that by the time that shows up in a clinical trial activity data, Scotland would have lost its “position at the forefront of clinical research”. The clinical research and trials issue, he said, was “a massive one” and was “also a massive one for patients because patients who get on to clinical trials benefit from those clinical trials”.

94. Leigh Smith of Melanoma Action and Support Scotland told the Committee that she was concerned about Scotland “losing out on phase 3 trials”. She said she found it “almost objectionable” that patients were “being used as guinea pigs for fairly untried stuff, but are not getting the benefit of the phase 3 trials, which is when we know the dose and so much more about the drug”.

**Transparency of clinical research data**

95. A related, but separate, issue that arose during the Committee’s evidence session with Professors Routledge and Swainson, was the question of access to research data held by pharmaceutical companies. Asked by the Committee (in light of campaigns by the British Medical Association and British Pharmacological Society) whether it should be a requirement for companies to publish all clinical trial data at the time of any submissions to SMC Professor Routledge said he firmly believed that all trial data should be made available adding that he was “delighted that many pharmaceutical companies are signing up to that”.

96. The Committee recognises the concerns of clinicians and others about the impact of the latest innovative medicines not being available routinely in Scotland and its possible effect on the number of clinical trials taking place in Scotland. The Committee also notes the general trend within the industry for fewer larger-scale clinical trials to take place in Scotland and the rest of Western Europe, which could not compete on price with eastern European nations. The Committee calls on the Scottish Government to urgently investigate and report back to the Committee on whether there is a decline in the number of phase 3 trials being placed in Scotland and consider any steps that may be needed to increase their number as well as maximising the overall number of clinical trials.

97. A more systematic approach should be taken by health boards collectively to collating and updating data on clinical trials (both national and international) with a view to facilitating greater access to participation in such trials for patients in Scotland where appropriate.

98. The Committee considers, however, that there are also opportunities that Scotland could be exploiting. This is considered in the next section.

99. The Committee also notes the campaigns by BMA and BPS on access to clinical research data.

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Value-based pricing

100. The Committee regrets the confusion that currently exists between the Scottish and UK Governments regarding responsibility for the delivery of value-based-pricing and calls for urgent clarity on the matter.

101. Throughout this inquiry, the Committee has been told it was intended that a value-based pricing regime, led by the UK Department of Health, would be established across the UK in April 2014. However, it has been difficult for the Committee to establish much detail of how this will work in practice and what its implications would be for Scotland and for the SMC and the IPTR process in particular. The Committee took evidence from UK Department of Health officials and received a private briefing on VBP from Scottish Government officials.

102. The Committee understands that the Scottish Government had been planning on the basis that the UK Department of Health would assume responsibility for all aspects of value-based pricing, including the health technology appraisal, through NICE, on behalf of all the devolved administrations in the UK, alongside England. This was based on an interpretation of the Scotland Act 1998, which lists medicine pricing as a reserved matter. Like others, the Committee assumed that, because pricing was reserved, value-based pricing would also be reserved. However, the evidence that the Committee took on 14 May 2013 from UK Department of Health official Katy Peters appeared to suggest an alternative proposition, that, while the price at first marketing of a medicine was indeed reserved, the health technology appraisal aspects of value-based pricing could be devolved. This would mean that HTA would only be carried out by NICE on behalf of NHS England, while this process in Scotland and Wales could be undertaken by the SMC and the All Wales Medicines Strategy Group respectively.

103. The Committee considers that, should it indeed turn out that certain aspects of value-based pricing could, in fact, be undertaken by the devolved administrations, it is unfortunate that this confusion has persisted for such a long time – time that could have been used to establish new Scottish arrangements. It is going to be challenging for such arrangements to be established in time for VBP’s commencement date of January 2014.

104. Nevertheless, the Committee considers that, should the health technology aspects of value-based pricing indeed be devolved, this may present a unique opportunity to develop a Scottish solution to these matters that could be much more flexible than the current arrangements.

105. The Committee welcomes the recommendation by Professor Routledge that SMC should be able to have a temporary pause in the appraisal process at any stage in order to permit further dialogue with manufacturers on issues that would be likely to be central to the subsequent decision-making process. In addition to permitting further dialogue, such a pause could also create an opportunity for discussion on, for example, whether there was scope to develop a reimbursement rate which could take into account

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various factors such as supplying post licensing data or assessed benefit of medicines post approval.

106. It is clear that much remains to be done, both at UK and Scottish government levels, if value-based pricing is to come into effect when intended and to operate in the way that has been envisaged. The Committee also believed it is clear that there are significant potential opportunities that will present themselves with the advent of value-based pricing, both in relation to the possibility of gaining access to innovative medicines at more favourable prices than currently and in securing related clinical trial and post-licensing data benefit.

107. The Committee will be seeking regular updates from the Scottish and UK Governments over the next year and beyond as VBP becomes established.

NEXT STEPS

108. The Committee welcomes the Scottish Government’s intention to consult on these matters, taking into account the two review reports and the recommendations of this Committee.

109. Stakeholders in both written and oral evidence have demonstrated an enthusiasm for all interested groups to work constructively with the Scottish Government to develop the system for accessing new medicines further in a way which is able to take account of a wider assessment of their value; the committee notes the variety of submissions in this area from a wide range of groups including Myeloma UK, ABPI, Beating Bowel Cancer and Cancer Research UK.

110. While it welcomes the recommendations within the Swainson and Routledge reviews, describing the improvements as “incremental”, the Committee makes a number of recommendations that go beyond both reviews. It believes that these recommendations have the potential to significantly improve both the Patient Treatment Request and SMC processes and, in doing so, would ensure a better and more transparent system for the accessing of new medicines.
ANNEXE A: GLOSSARY

Acronyms used in the report—

• ABPI - Association of the British Pharmaceutical Industry (ABPI)
• ADTC - Area Drug and Therapeutic Committees (ADTC)
• AGSD UK - Association for Glycogen Storage Disease (UK)
• aHUSUK - atypical Haemolytic Uraemic Syndrome (UK)
• ALTCSG - Angus Long Term Conditions Support Groups
• AWMSG - All Wales Medicines Strategy Group
• CDF - cancer drugs fund (CDF)
• EMA - European Medicines Agency
• GPTR - Group Patient Transfer Request. (GPTR)
• HIS - Healthcare Improvement Scotland (HIS)
• IPTR - individual patient treatment requests (IPTR)
• NICE - National Institute for Health and Clinical Excellence (NICE)
• PAS - Patient Access Schemes (PAS)
• PNH Alliance and PNH Scotland (Paroxysmal Nocturnal Hemoglobinuria)
• PPRS - Pharmaceutical Price Regulation Scheme (PPRS)
• QALY - quality-adjusted life year (QALY)
• SMC - Scottish Medicines Consortium (SMC)
• VBP - value-based pricing (VBP)
ANNEXE B: BULLET POINT SUMMARY OF EVIDENCE RECEIVED BY THE COMMITTEE IN RELATION TO THE SPECIFIC RECOMMENDATIONS IN THE ROUTLEDGE AND SWAINSON REPORTS

The Routledge report

Recommendation 1. SMC should meet in public so that members of the public, patients, patient group representatives, other health professionals and members of the pharmaceutical industry can attend to observe the appraisal process.

- Professor Webb (RCPE) said that the SMC recommendations were geared towards the approach in Wales but he pointed out that the SMC predated what had been established in Wales and that much of the discussion and all of the decisions there were made in private. If this were the case here, meetings would be much longer, he said, as the public were ushered in and out and this would have an impact on clinicians’ time and perhaps even their commitment to the SMC: “That would be a tragedy”.

- Professor Timoney said that SMC would be happy to meet in public but was wary of criticism of asking people to leave the meeting when it came to commercial issues with more controversial decisions.

Recommendation 2. SMC should invite the manufacturer of the new medicine under consideration to give evidence at their main SMC appraisal meeting, in order to address any outstanding questions that SMC members have and highlight any outstanding issues of which they believe SMC should be aware prior to its advice being published.

Recommendation 3. SMC should be able to appraise any new medicines which the NHS in Scotland considers potentially of major importance to patient care, but which have not been submitted to SMC by the manufacturer within 12 weeks of launch. If necessary this appraisal may be conducted using such data as is already available in the public domain.

Recommendation 4. SMC should be able to have a temporary pause in the appraisal process at any stage in order to permit further dialogue with manufacturers on issues that would be likely to be central to the subsequent decision-making process.

Recommendation 5. SMC should develop a policy specifically relating to ultra-orphan medicines to guide the process of consideration of all available evidence relevant to its advice on these medicines.

- Happy the term “ultra-orphan” recognised (Lesley Loeliger).

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I Mackersie concurred.  

Recommendation 6. SMC, with the appropriate resource and in partnership with other relevant bodies in Scotland, should be encouraged to set up an engagement process such as a “Citizen’s Council” or “Citizen’s Jury” to explore views around specific societal issues of importance to the people of Scotland in relation to the availability of new medicines and the impact of these views on the existing processes for ensuring access to medicines.

“...the citizens’ panel may provide a useful forum to have societal discussions about how much and what we are willing to pay for medicines and about some of the modifiers that the SMC is perhaps able to use in its discussions on cost per quality-adjusted life year. That societal debate is absolutely needed, so a forum in which that can take place would be very welcome.” (David Pfleger)  

ABPI welcomed “a discussion on whether society wants to pay more in certain areas”.  

Eric Low underlined the importance of transparency but suggested it was unlikely to change the decisions significantly.  

Dr MacDonald said the industry was supportive of the idea that more clinical trials be published.  

George Grindlay was supportive of the idea of a citizens’ council or jury.  

Ian Mackersie voiced concern about asking members of the public to deliberate on such complex issues.  

Natalie Frankish echoed that concern and suggested a lot of work would be necessary to make what was a good idea work in reality.  

Engagement with the public was a good thing but the pathways were “extremely complicated” said Dr Harrow.  

Recommendation 7. SMC should explore other innovative approaches to increasing patient and public awareness of its role in ensuring timely access to clinically effective and cost-effective medicines in Scotland. Consideration should also be given to expansion of its role to support other aspects of safe, effective and cost-effective prescribing. SMC should

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produce a publicly available annual report of progress in this regard detailing its important contributions to this objective.

- D Pfleger suggested the challenge was how to implement some of these recommendations “without impeding the responsiveness of the system that we have”.  

- “SMC does a good job but it can only do what it is told to do, which is to make decisions regarding cost effectiveness” (Professor Gourley)

- Even if Scotland adopted VBP, there would still be public expectations to manage – there will be medicines that people want but are not cost effective (Melinda Cuthbert).

Recommendation 8. NHS Scotland should explore ways in which the expertise available within SMC might be used to support the process of Value Based Pricing (VBP).

- “We have been working on the question of VBT and waiting to understand what role it will have in Scotland.” (Eric Low)

- VBP “may not be practical” and that gives Scotland a “wonderful opportunity” to look at the balance between demand-orientated healthcare and achieving a fair and value-based price (Eric Low).

- Could say that SMC already does a form of VBP but chance now to enhance VBP or modify it (Eric Low).

- Still not clear what Scotland’s role is with VBP – pricing reserved but reimbursement devolved (A Timoney).

- VBP part of a health technology value assessment rather than a pricing mechanism linked to PPRS (Eric Low).

- If role of SMC was expanded, extra resourcing would be necessary said Professor Webb.

- Professor Timoney pointed out that it costs NICE £160,000 to do a technology assessment, for example.
Recommendation 9. A register of IPTR decisions in all health boards, suitably anonymised to protect patient confidentiality should be kept, and supporting information related to IPTRs shared between health boards. Recommendation 10. There should be regular sharing of experiences between the IPTR panels and members of IPTR panel members across Scotland should meet at least annually for induction, feedback and training.
The Swainson report

Recommendation 1: Board ADTC should publish their local response to the SMC published advice within 30 days of the SMC advice, on the Board website and in a manner which is accessed easily by the public and patients (as required by CMO 1 2012). The response need not be definitive if further work is required but should indicate clearly the Board’s intentions; the final arrangements should be published within 90 days. Members of the public involved in the work of the ADTC (drawn from the members of the Board Patient and Public Forum (PPF) can assist with describing the processes in a way that is “user-friendly” for the general public, and act as a link with the wider PPF.

- Ought not to be a race, said Melinda Cuthbert. If you try to introduce medicines faster, you may bring in more risk.  

- Useful recommendation regarding need to be more transparent during that 90-day period (D Pfleger).

- Hope that boards will read the report and consider how to get stuff onto the formulary earlier (Dr Green).

Recommendation 2: Board ADTC should publish their decisions and the reasons for their decisions in respect of SMC advice to be compliant with CMO (2012)1. These reasons should include the consequences for the local formulary, even if, in the case of novel medicines, this requires further deliberation and planning. Patients and the public should be signposted from the front page of the Board website to a link which will provide information about recent SMC decisions and subsequent formulary decisions and the overall formulary should be published alongside this information and updated as required.

- “We need to be clear that getting stuff on the formulary is one part of the problem. We need to do that quickly and efficiently to enable clinicians to prescribe the drugs that they think are best of their patients as soon as possible.” (Eric Low, Myeloma UK)

- NHS Lothian said they already published formulary information and were looking at making it accessible via its placing on the website.

Recommendation 3: Board ADTC should demonstrate the engagement of their PPF in the work of the ADTC. For preference, Board ADTC should have at least one member drawn from the PPF or demonstrate the connection between the PPF and the work of the ADTC

Recommendation 4: NHS Scotland should consider a national meeting of all relevant specialists, organised by Healthcare Improvement Scotland (HIS), to agree a national implementation plan for some new medicines accepted by the SMC that meet agreed criteria. These criteria may include the introduction of novel, first in class medicines where there is considerable uncertainty of its place in therapy. The plan will apply to all patients covered by the SMC “accepted” advice and to all Boards to support equity of access. Further, HIS should continue to audit access to new medicines and compliance with CEL 17 (2012) and SGHD/CMO (2012).

- Finding time to have meetings away from seeing patients another resource issue according to NHS Lothian.\(^{70}\)
- Wider point made about “opportunity cost” of putting resources into enhancing scrutiny – welcome though that was. Resources had to come from somewhere else (NHS Grampian).\(^ {71}\)

Recommendation 5: NHS Scotland should retain the existing ADTC to maintain alignment of patient and GP interests, safe prescribing and enable Boards to manage their costs. Regional clinical networks could have a role in agreeing equitable access to new medicines in relation to their populations.

- Melinda Cuthbert of NHS Lothian supported what Swainson said about ADTCs. Local clinicians need ownership of the formularies. \(^{72}\)
- Swainson clear about the advantages of having the 14 ADTCs over other approaches, said D Pfleger. \(^{73}\)
- Maintaining the local structure is valuable (Professor D Webb, RCPE). \(^{74}\)

Recommendation 6: All Boards should adopt the same IPTR paperwork and process, based on the examples from Greater Glasgow and Clyde, Lothian or Grampian. The application should contrast the clinical criteria appraised by the SMC where “not recommended” advice has been published with the patient’s disease and personal clinical characteristics so that the reasons for the IPTR are more easily assessed, and can be audited.

- He wasn’t sure about the word “integration”, but D Pfleger gave example of north eastern boards working together around the IPTR process. This is about how resources are shared. \(^ {75}\)

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\(^{74}\) Scottish Parliament Health and Sport Committee. Official Report, 21 May 2013, Col 3797
• Natalie Frankish wanted to ensure the IPTR process was addressed as she felt the report changed nothing and would still impact negatively on rare disease patients.  

**Recommendation 7:** The IPTR arrangements in Boards should be audited by HIS to assess compliance with guidance and its consistency of application, and to publish the results.

• Need to understand there will be differences locally but Swainson didn’t find this an issue re accessing SMC-approved medicines and where alternatives where in place. Pro-transparency but not convinced that desired outcome was “absolute uniformity” of SMC medicines via the formularies.

**Recommendation 8:** Clinicians should be provided with basic training and guidance in the IPTR process locally. Clinicians who are uncertain or inexperienced should be able to access specialist advice and support (see recommendation 10).

• NHS Grampian’s David Pfleger suggested such a register would be a useful addition to the process.

• It would depend on context, said Professor Gourley of Cancer Research Network – if a non-specialist and not familiar with the process, then yes.

• Useful where clinician needed extra support – David Pfleger.

• To deliver training in IPTR to all doctors, NHS Lothian said they would look at resources (Melinda Cuthbert).

• Lesley Loeliger said the process was in need of a body of experts.

**Recommendation 9:** Boards should consider whether IPTR panels should include a member of the public drawn from the Board’s patient and public forum. Member(s) will require training and support.

**Recommendation 10:** All doctors considering an IPTR must be able to access consistent, knowledgeable support for their patients. National Services Division (NSD) should establish and maintain a register of approved specialists to support IPTR. One specialist may be sufficient for orphan and ultra-orphan diseases, but more than one specialist may need to be available for more common diseases, or variants, and on a regional basis. The model of the cancer networks is an example.

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• Rachel Green of NHS GG&C supported the access to specialist advice on basis submissions are improved if supported by expert peers. She did however voice concern if that advice was sought from England, where clinicians were unaware of the system.\footnote{Scottish Parliament Health and Sport Committee. \textit{Official Report}, 21 May 2013, Col 3795}

• Melanoma Action and Support’s Leigh Smith also suggested an issue for them in that there was only one specialist in Scotland.\footnote{Scottish Parliament Health and Sport Committee. \textit{Official Report}, 21 May 2013, Col 3797}

• Not a massive disparity in the formularies and choices in different areas.\footnote{Scottish Parliament Health and Sport Committee. \textit{Official Report}, 21 May 2013, Col 3840.}

• Lesley Loeliger supported the register of specialists idea.\footnote{Scottish Parliament Health and Sport Committee. \textit{Official Report}, 21 May 2013, Col 3833.}

Recommendation 11: The Scottish Government and Boards should produce clear and concise documentation, available on national and local websites, that explains the roles of ADTC and IPTR, how the public and patients can be involved, and provide links to the reports recommended above and for ADTC.

• “The issue is that, if IPTR rules are applied properly, they are not a mechanism to access SMC not-approved drugs...If we are going to improve access to drugs, we need to find another way.” (Professor Gourley)\footnote{Scottish Parliament Health and Sport Committee. \textit{Official Report}, 21 May 2013, Col 3805.}

• Dr Harrow said the specialists dealing with the patients ought to be involved in the decision making for IPTR.\footnote{Scottish Parliament Health and Sport Committee. \textit{Official Report}, 21 May 2013, Col 3833.}

• Dr Grindlay agreed.\footnote{Scottish Parliament Health and Sport Committee. \textit{Official Report}, 21 May 2013, Col 3848.}

• There was some discussion of whether the SMC should have a more central role in the interests of consistency versus the importance of local responsiveness (Dr Harrow, Joan Fletcher, Dr Casasola, Lesley Loelinger).\footnote{Scottish Parliament Health and Sport Committee. \textit{Official Report}, 21 May 2013, Col 3849.}

• Vicky Crichton suggested there was a need for “parity of process” (in IPTRs) in decision making and if that could not be demonstrated, another approach would be called for.\footnote{Scottish Parliament Health and Sport Committee. \textit{Official Report}, 21 May 2013, Col 3849.}

Recommendation 12: The RCMF should focus on access to medicines for ultra-orphan diseases. Access should be supported where the SMC has published ‘not recommended’ advice after a full submission of the medicine, and after a successful IPTR or GPTR has been agreed.
• I Mackersie welcomed reference to ultra-orphan diseases on basis it might give “fair consideration” to those drugs the SMC does not recommend. 92

• George Grindlay suggested it was difficult for somebody with a long-term condition to go through what was considered the lengthy IPTR process in order to receive a yes or no answer. 93

• Lesley Loeliger questioned the claim that IPTRs are based entirely on clinical effectiveness. 94

• A view echoed by Dr Casasola. 95

• Dr Harrow expressed frustration at his experience of the IPTR process when he had a body of expert advice and support but to no avail. 96
ANNEXE C: EXTRACT FROM MINUTES OF THE HEALTH AND SPORT COMMITTEE

24th Meeting, 2012 (Session 4)
Tuesday 18 September 2012

2. **Access to new medicines** The Committee took 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 and 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.

30th Meeting, 2012 (Session 4)
Tuesday 6 November 2012

1. **Decision on taking business in private**: The Committee agreed to take item 5 in private. The Committee also agreed to take consideration of a draft report on the Draft Budget 2013-14 in private at future meetings.

5. **Access to new medicines**: The Committee agreed to hold two further evidence sessions on access to new medicines. The Committee will take evidence from clinicians and patient representative organisations.
2. **Access to new medicines:** The Committee took evidence from—

- Dr David Dunlop, Clinical Lead, Scottish Cancer Research Network (West of Scotland);
- Dr Russell Petty, Clinical Senior Lecturer in Medical Oncology, Clinical Speciality Lead for Oncology, Consultant Medical Oncologist, University of Aberdeen, Scottish Cancer Research Network (North);
- Professor Charlie Gourley, Clinical Lead, Scottish Cancer Research Network (South East Scotland);
- Dr Richard Casasola, Consultant Clinical Oncologist, Clinical Lead for Oncology, Tayside Cancer Centre, NHS Tayside and University of Dundee, Scottish Cancer Research Network (East of Scotland);
- Dr Tim Crook, Consultant Oncologist, Scottish Melanoma Group;
- Dr Noelle O’Rourke, Chair, Beatson Consultants Committee, Beatson West of Scotland Cancer Centre;
- Dr Stephen Harrow, Consultant Oncologist, Beatson West of Scotland Cancer Centre;
- Vicky Crichton, Public Affairs Manager, Cancer Research UK;
- Karen McNee, Community Development Scotland, James Whale Fund for Kidney Cancer;
- Kate Seymour, External Affairs Manager, Macmillan Cancer Support;
- Eric Low, Chief Executive, Myeloma UK;
- Leigh Smith, Chair, Melanoma Action and Support Scotland;
- Alistair Haw, Head of Media and PR, Prostate Cancer UK.

1. **Decision on taking business in private:** The Committee agreed to consider item 3 in private.
3. **Access to new medicines (in private):** The Committee considered and agreed its approach to the inquiry.

**3rd Meeting, 2013 (Session 4)**

**Tuesday 29 January 2013**

1. **Access to new medicines evidence session:** The Committee took evidence from—

   Professor David Newby, British Heart Foundation Chair of Cardiology and Consultant Cardiologist, NHS Lothian and University of Edinburgh;

   Keith AA Fox, Duke of Edinburgh British Heart Foundation Professor of Cardiology, University of Edinburgh;

   Ian Mackersie, Secretary, and Len Woodward, Treasurer, aHUSUK;

   Professor Tim Goodship, Professor of Renal Medicine, Newcastle University;

   Nancy Greig, Network Development Officer, Health and Social Care Alliance Scotland, (the ALLIANCE);

   George Grindlay, Angus Long Term Conditions Support Groups, Lead Facilitator of ALTCSG and Non-Executive Director, Health and Social Care Alliance Scotland (the ALLIANCE);

   Alastair Kent, Chair, Rare Disease UK;

   Professor Peter Hillmen, Consultant Haematologist, St James University Hospital, Leeds, and Lesley Loeliger, Founder and Chairman, PNH Scotland, PNH Alliance;

   Dr Mark Eldon Roberts, Consultant Neurologist, Salford Royal NHS Foundation Trust;

   Joan Fletcher, Family Support Officer, Clinical Nurse Specialist, Association for Glycogen Storage Disease (UK);

   Marion Ferguson, Chairperson, Ivacaftor Patient Interest Group.

**14th Meeting, 2013 (Session 4)**

**Tuesday 7 May 2013**

2. **Access to newly licensed medicines** The Committee took evidence from—

   Professor Philip Routledge, Professor of Clinical Pharmacology at Cardiff University;
Professor Charles Swainson, former Medical Director, NHS Lothian;

Alex Neil, Cabinet Secretary for Health and Well-being, Professor Bill Scott, Chief Pharmaceutical Officer, and Dr Aileen Keel, Deputy Chief Medical Officer, Scottish Government.

15th Meeting, 2013 (Session 4)

Tuesday 14 May 2013

2. Value Based Pricing: The Committee took evidence from—

Katy Peters, Head of Pricing, Prescriptions and Supply, Medicines, Pharmacy and Industry Group, Department of Health, UK Government.

16th Meeting, 2013 (Session 4)

Tuesday 21 May 2013

2. Access to newly licensed medicines: The Committee took evidence from—

Professor Angela Timoney, Chairman, Scottish Medicines Consortium;

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

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

David Pfleger, Director of Pharmacy, NHS Grampian;

Dr Frances Macdonald, ABPI member of SMC, Association of the British Pharmaceutical Industry;

Eric Low, Chief Executive, Myeloma UK;

Leigh Smith, Chair, Melanoma Action and Support Scotland;

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

Professor Charlie Gourley, Clinical Lead, Scottish Cancer Research Network (South East Scotland);

Dr Richard Casasola, Consultant Clinical Oncologist, Clinical Lead for Oncology, Tayside Cancer Centre, NHS Tayside and University of Dundee, Scottish Cancer Research Network (East of Scotland);
Dr Stephen Harrow, Consultant Oncologist, Beatson West of Scotland Cancer Centre;

Ian Mackersie, Secretary, aHUSUK;

George Grindlay, Lead Facilitator, Angus Long Term Conditions Support Groups;

Natalie Frankish, Development Officer, Rare Disease UK;

Lesley Loeliger, Chair, PNH Scotland;

Joan Fletcher, Clinical Nurse Specialist/ Family Support Practitioner, Pompe Disease, Association for Glycogen Storage Disease (UK) (Pompe Group);

Vicky Crichton, Senior Public Affairs Manager, Cancer Research UK.

19th Meeting, 2013 (Session 4)

Tuesday 11 June 2013

1. **Decision on taking business in private**: The Committee agreed to take item 4 in private. The Committee also agreed to take consideration of a draft report in private at future meetings.

4. **Access to newly licensed medicines**: The Committee considered a draft report. Various changes were agreed to, and the Committee agreed to consider a revised draft, in private, at its next meeting.

20th Meeting, 2013 (Session 4)

Tuesday 18 June 2013

6. **Access to newly licensed medicines (in private)**: The Committee considered a draft report. Various changes were agreed to, and the Committee agreed to consider a revised draft, in private, at its meeting on 25 June.

22nd Meeting, 2013 (Session 4)

Tuesday 25 June 2013

5. **Access to newly licensed medicines (in private)**: The Committee considered a draft report. Various changes were agreed to, and the Committee agreed to consider a revised draft, in private, at its meeting on 26 June.
1. **Access to newly licensed medicines (in private):** The Committee considered and agreed a draft report on its inquiry into Access to newly licensed medicines.
ANNEXE D: ORAL EVIDENCE AND ASSOCIATED WRITTEN EVIDENCE

24th Meeting, 2012 (Session 4) Tuesday 18 September 2012

Written Evidence
Patient and Public Involvement Group of the Scottish Medicines Consortium
Scottish Medicines Consortium
The Royal College of Physicians
Association of the British Pharmaceutical Industry
Association of the British Pharmaceutical Industry

Oral Evidence
Scottish Medicines Consortium;
Royal College of Physicians of Edinburgh;
NHS Greater Glasgow and Clyde;
NHS Grampian;
NHS Lothian;

Supplementary Written Evidence
Association of the British Pharmaceutical Industry

33rd Meeting, 2012 (Session 4) Tuesday 4 December 2012

Written Evidence
Scottish Melanoma Group
Cancer Research UK
James Whale Fund for Kidney Cancer
Macmillan Cancer Support
Myeloma UK
Melanoma Action and Support Scotland
Prostate Cancer UK
Beatson West of Scotland Cancer Centre

Oral Evidence
Scottish Cancer Research Network;

Supplementary Written Evidence
ABPI Scotland

3rd Meeting, 2013 (Session 4) Tuesday 29 January 2013

Written Evidence
aHUSUK
Rare Disease UK
PNH Alliance and PNH Scotland

**Oral Evidence**

NHS Lothian and University of Edinburgh;
University of Edinburgh;
Newcastle University;
Alliance Scotland, (the ALLIANCE);
Angus Long Term Conditions Support Groups;
Salford Royal NHS Foundation Trust;
Association for Glycogen Storage Disease (UK);
Ivacaftor Patient Interest Group.

Supplementary Written Evidence

**Alliance Scotland**

**14th Meeting, 2013 (Session 4) Tuesday 7 May 2013**

Written Evidence

**Scottish Government**

**Scottish Government**

**Oral Evidence**

**15th Meeting, 2013 (Session 4) Tuesday 14 May 2013**

Written Evidence

**Oral Evidence**

Department of Health, UK Government.

**16th Meeting, 2013 (Session 4) Tuesday 21 May 2013**

Written Evidence

**Association of the British Pharmaceutical Industry**

**Pompe Disease, Association for Glycogen Storage Disease (UK) (Pompe Group)**

**Association for Glycogen Storage Disease**

**Oral Evidence**

Scottish Medicines Consortium;
University of Edinburgh, Royal College of Physicians of Edinburgh;
NHS Greater Glasgow and Clyde;
NHS Grampian;
Myeloma UK;
Melanoma Action and Support Scotland;
NHS Lothian;
Scottish Cancer Research Network
Beatson West of Scotland Cancer Centre;
aHUSUK;
Angus Long Term Conditions Support Groups;
Rare Disease UK;
PNH Scotland;
Cancer Research UK.
ANNEXE E: LIST OF OTHER WRITTEN EVIDENCE

Pfizer UK
Novartis Pharmaceuticals
David Cameron, Professor Jeff Evans and Dr Marianne Nicolson
Merck Serono Ltd
Roche Products Ltd
Bristol Myers Squibb
Professor Charlie Gourley
On behalf of Oncologists specialising in the treatment of colectoral cancel within Scotland
Shire
Requests questionnaire
Pfizer UK
Caroline McManus individual
Anonymous Submission
Personal statement by Paul Sharp
Andy Carver, Prevention and Care Adviser, British Heart Foundation Scotland
Karen Facey individual
Beating Bowel Cancer
Beating Bowel Cancer
ANNEXE F: INDIVIDUAL PATIENT TREATMENT REQUESTS QUESTIONNAIRE

The Committee sent the following questionnaire on Individual Patient Treatment Requests to the 14 area NHS boards.

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

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

3. How many of the 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?

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?

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?

NHS Ayrshire and Arran;
NHS Borders;
NHS Dumfries and Galloway;
NHS Fife;
NHS Forth Valley;
NHS Grampian;
NHS Greater Glasgow and Clyde;
NHS Highland;
NHS Lanarkshire;
NHS Lothian;
NHS Orkney;
NHS Shetland;
NHS Tayside, and
NHS Western Isles.

Responses to the questionnaire.
Members who would like a printed copy of this Numbered Report to be forwarded to them should give notice at the Document Supply Centre.