The Scottish Parliament
Pàrlamaid na h-Alba

Official Report

HEALTH AND SPORT COMMITTEE

Tuesday 7 May 2013

Session 4

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HEALTH AND SPORT COMMITTEE
14th Meeting 2013, Session 4

CONVENER

*Duncan McNeil (Greenock and Inverclyde) (Lab)

DEPUTY CONVENER

*Bob Doris (Glasgow) (SNP)

COMMITTEE MEMBERS

*Mark McDonald (North East Scotland) (SNP)
*Aileen McLeod (South Scotland) (SNP)
*Nanette Milne (North East Scotland) (Con)
*Gil Paterson (Clydebank and Milngavie) (SNP)
*Dr Richard Simpson (Mid Scotland and Fife) (Lab)
*Drew Smith (Glasgow) (Lab)
*David Torrance (Kirkcaldy) (SNP)

*attended

THE FOLLOWING ALSO PARTICIPATED:

Dr Aileen Keel (Scottish Government)
Alex Neil (Cabinet Secretary for Health and Wellbeing)
Professor Philip Routledge (Cardiff University)
Professor Bill Scott (Scottish Government)
Professor Charles Swainson

CLERK TO THE COMMITTEE

Eugene Windsor

LOCATION

Committee Room 5

Scottish Parliament

Health and Sport Committee

Tuesday 7 May 2013

[The Convener *opened the meeting at 09:45*]

Decision on Taking Business in Private

The Convener (Duncan McNeil): Good morning and welcome to the 14th meeting in 2013 of the Health and Sport Committee. As usual, I remind those present to switch off mobile phones and BlackBerrys, as they can interfere with the sound system. To ensure that there is no confusion, I point out that some committee members are working from iPads. We can assure you that the iPads are being used to replace paper—we are not playing games with them, but are using them to aid our work on the committee.

Under agenda item 1, I seek the committee's agreement to take in private item 3, which is consideration of our draft report on teenage pregnancy. Are members agreed?

Members *indicated agreement.*

New Medicines (Access)

09:46

The Convener: Item 2 is evidence on the review of access to new medicines, which, as members know, the Scottish Government published last week. We have Professor Philip Routledge and Professor Charles Swainson with us this morning; I welcome them both. Both professors wish to make an opening statement. I invite Professor Routledge to speak first; he will be followed by Professor Swainson.

Professor Philip Routledge (Cardiff University): Thank you, convener. I have very much enjoyed looking carefully at the Scottish Medicines Consortium's processes. I highlight in my report what I regard as the characteristics of an ideal appraisal process: timeliness, which is very relevant to the speed at which Scotland assesses drugs; the relevance, in-depth nature and usability of the information; the efficiency of the process, which I looked at carefully and I believe the process in Scotland to be very efficient; and, in particular, the independence of the process—I am satisfied that the process in Scotland is very independent.

My conclusion is that the process that Scotland uses to appraise new drugs is very good and one of which it should be proud. My recommendations relate largely to trying to increase the transparency of the process, so that all those who are involved in it and who have an interest in the outcome can see the qualities of the process that Scotland uses.

Professor Charles Swainson: Overall, I found the systems of the area drug and therapeutics committees and individual patient treatment requests to be reasonably sound. However, I was struck by the evidence presented to the committee in written form and during its oral evidence sessions, and from the people to whom I spoke, that the quality and consistency of the arrangements left something to be desired. Many of my recommendations are therefore about tightening up and improving on the arrangements that are already in place. In essence, I am asking for more transparency, public reporting and, in some cases, involvement in these important systems.

I was also struck by the petitions to the Parliament that started the process and by the particular plight of patients who suffer from ultra-orphan—or very rare—diseases, for whom very few medicines have been recommended, even though medicines might be effective in many instances. That is why I made an interim

recommendation about the establishment of a rare diseases medicines fund.

Nanette Milne (North East Scotland) (Con): I found both reports extremely interesting. I was on the Public Petitions Committee when it received the first petition on the issue, which was about cetuximab, and I have been involved with the process right the way through. There is no doubt that a lot of concern has been expressed, some of which is valid, although perhaps some is not. However, I welcome what has been said about the SMC. No one here would disagree that, overall, the SMC has done an excellent job in the past decade in assessing new medicines. I welcome the suggestion about making the SMC meet in public session, which might clarify a number of the problems.

On the IPTR system, I welcome what you have said about orphan and ultra-orphan diseases and the setting up of an orphan drugs fund. However, beyond that, many patients and clinicians who are involved, as well as the industry, have raised concerns about access to cancer medicines. We now have a fund for rare diseases, which is excellent, but could a case be made for setting up an IPTR fund so that patients can access cancer medicines that are thought to be of significant benefit clinically, but which might not be cost effective, because some of them are fairly expensive?

Professor Routledge: Obviously, I am not part of the SMC process, but I have concerns about identifying one particular condition over another in providing access to medicines. Rarity of disease is another issue. Some of the indications for cancer are very rare and relate to small groups of individuals. Nevertheless, if a patient has severe heart failure, it could shorten their life considerably, so it is only fair that all serious conditions be given the same consideration as cancer. Clearly, it is a serious condition, but it is only one.

Nanette Milne: Would some of the rarer cancers come under the rarer diseases fund?

Professor Routledge: By definition, they would fall within the ultra-orphan or orphan category, so they would be part of that, but I would be loth to single out cancer from other conditions that shorten life or reduce the quality of life significantly.

Professor Swainson: I agree with Professor Routledge. No evidence was presented to me that demonstrated that drugs for cancer are treated any differently from other drugs in the decision making by the SMC, or in the decision making on IPTRs. From the single snapshot of work that has been done, roughly two thirds of IPTRs are successful and the same proportion applies to IPTRs for drugs for patients with cancer.

Singling out a particular condition would lead us down a very different road. I presume that many patients with different conditions would argue that the same should apply to their condition. One difficulty in the area is that, as soon as we agree that the health budget has to be limited, you begin drawing lines around different parts of it, of which medicines is simply one.

Nanette Milne: During our evidence taking, some clinicians raised the point that Scottish patients are perhaps losing out, principally because of the cancer drugs fund, which is available to patients in England—not just because they are not getting the drug now, but because new drugs coming on stream will be assessed against the current state-of-the-art drugs, and Scottish patients will not be able to take part in clinical trials of medicines in the future because we are not using the state-of-the-art ones, if you see what I mean. That was expressed to us as a significant concern.

Another issue is that clinicians with an academic interest, who will not be able to use what they regard as state-of-the-art drugs, are already showing some signs of reluctance to come and work in Scotland.

Professor Swainson: The only evidence that I have on that is from the research end of things. I inquired at the chief scientist office, and you will have seen the small section in my report about that.

The picture around clinical trials has changed all over Europe. We no longer do the same larger-scale trials in Scotland, or indeed in England and much of western Europe. The evidence is that the trials that are being done now in Scottish health boards and universities have been approximately the same over the past three years or so. The value of those studies has also been approximately the same. The emphasis of research has 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 is done elsewhere in the world.

The Convener: You refer to the limited evidence that was available from the chief scientist office, and you have spoken about the changing nature of what is happening across Europe and whether we can compete with other countries on price. I do not know whether it is your view that we need to consider that in relation to how we ensure the best prices, outcomes and qualities in that competitive market—and in relation to the Scottish Government's ambition for life sciences. That issue was raised in evidence, but was not fully

developed. Do you have a view on whether more work needs to be done to address those issues, or on whether more evidence needs to be brought to the table?

Professor Swainson: I have little direct evidence about those matters. My general impression has been that the life sciences industry in Scotland is doing very well, and I know that the universities punch considerably above their weight in terms of research funding in the United Kingdom. Despite representing only about 10 per cent of the population, we attract about 14 per cent of the biomedical research funding that is available in the UK. That part of the scene seems very healthy. However, I have no figures that break that down into research to do with new medicines.

I am sure that the issue of pricing is relevant to the whole question. You have already seen that the patient access schemes, which involve a negotiated price agreement for new medicines, can make the difference—very properly—between a submission being approved by the Scottish Medicines Consortium and its not being approved. Against a background in which there are very large companies whose annual results look pretty good to me, the question is really about pricing and how to set a price that makes a medicine affordable in the context of cost effectiveness.

The Convener: We have had lots of evidence that the issue is not simply one of pricing, but one of the people who are caught in the middle of the negotiation. That is why we are discussing the matter today.

10:00

Dr Richard Simpson (Mid Scotland and Fife) (Lab): You say that there have been 600 phase 2 trials and that we have moved mainly from phase 3 to phase 2 trials, but phases 3 and 4 are really important, too. The figure for them for the UK as a whole has gone down from 6 to 2 per cent, so we are not competitive. I will encourage the committee to consider recommending that we should have a review in that area that is separate from our report.

I do not have any problems with the 10 recommendations in Professor Routledge's report, which take the issues a little bit further, but I am slightly disappointed that we have not been a little more radical with regard to the structures. We have the Scottish Medicines Consortium, which is highly regarded—the committee's evidence and comments that have been made in the previous session and this session have shown that—but we also have 14 area drug and therapeutics committees, or 15, if we include the Golden Jubilee hospital, which sometimes meet as consortia and sometimes meet independently. Professor Swainson's report says that only 74 per cent of the medicines that are approved by the Scottish Medicines Consortium are then agreed for inclusion on formularies, either awaiting a protocol or not. In Lanarkshire's case, 23 out of 23 in the audit were introduced; in Lothian's case, only 13 were introduced. One more was okay with a pathway, but the rest were not going to be included.

When the Scottish Medicines Consortium recommends a drug for general or restricted use, the public cannot understand why that drug is not then used throughout Scotland. For me at least, as an ex-clinician, that is understandable if the drug is what we used to call a me-too drug—in other words, if it is a reformulation, a very minor advance on what was previously there, or a brand product that is being introduced when a generic one is available that is a lot less expensive. I can understand the ADTCs saying, in those circumstances, "We feel that this is not a sufficient advance for us to pursue it." However, if a novel medicine is not introduced and committees are second-guessing the SMC with different restrictions, that is completely not understandable, and the public do not understand that, either.

I have raised before the issue of ticagrelor, which the Scottish Medicines Consortium approved for unrestricted use. The west of Scotland got together and said, "We will restrict its use for certain conditions," and the east of Scotland said, "We will also restrict its use, but for a different set of conditions." As far as I know, neither had any evidence base whatsoever. That was merely a clinical judgment. Sitting as a general practitioner in Stirling in the middle of Scotland, I would be looking at my heart attack patients and saying, "Well, you had better go to the east because you've had one type of heart attack; you had better go to the west; and you might have to go to the north, which has not even made a decision yet, but it may come up with a less restricted use." That is not acceptable.

I wonder why we do not have a recommendation from the two reports that the Scottish Medicines Consortium should give very clear guidance on the different drugs that it approves for restricted or unrestricted use, and that such drugs should be divided into those that are a significant advance and will save Scottish lives, as ticagrelor did after the submission that was accepted by the SMC; those that might improve patient safety, have lower side effects, and are therefore a significant advance; and a third category of minor advances and me-too drugs that might be considered.

I do not approve of the structure of the ADTCs, of which we have far too many. We need only

three; that is what operated for ticagrelor. That number is sufficient. Those three, allowing for the locality, the pathways and the rest, should do.

I do not understand why you did not criticise one bit of the system. I think that ADTC decision category 4 or 5 is to do with the medicine not being introduced because clinicians were not keen to introduce it. As the chairman of the first pharmaceutical liaison committee in Scotland, I still have scars on my back from clinicians who adopted that attitude. It is unacceptable that a small group of clinicians in one of our 14 health boards should decide that drugs are not available for their patients if the drugs are a significant advance. Why did you not make much more radical recommendations in that area?

The Convener: There was a lot in that, gentlemen. I will allow you all the time that you need to respond to the questions that were asked.

Professor Swainson: I will do my best, thank you. I did not make the recommendations that you suggest, Dr Simpson, because that was not exactly what people told me. However, I understand your difficulty around specialist medicines where there is a deal of clinical uncertainty, as shown in the example of ticagrelor that you mention. The drug was approved and you are quite right—why should it not be used more uniformly and effectively for the people of Scotland as a whole?

I heard the same evidence that ticagrelor was used differently in the east and west of Scotland. That happened because of the enormous clinical uncertainty about who would benefit. It was not at all clear whether the drug would benefit all patients or just some patients and, because it was a completely new drug, my understanding from the clinicians involved was that they wished to try it with groups of patients in an incremental fashion. There was therefore one patient group in the east, as you say, while the specialists in the west started with different groups. We do not know where they ended up, of course.

I have a great deal of sympathy with Dr Simpson's view. When we have brand-new highly specialist medicines that will largely be driven by a small number of specialists in Glasgow, Aberdeen, Dundee and Edinburgh, we could certainly have a more robust and uniform way of dealing with those medicines.

I pointed out in a table in my report that we could use existing clinical networks to make some of those decisions rather better. A number of clinical networks—cardiac, diabetic and cancer—drive and maintain specialist standards across regions in Scotland. The cancer networks already work in that way, and I agree with Dr Simpson that the process could be made to work a lot better for specialist drugs.

The situation is not quite the same with all the roles that the ADTCs perform. I understand Dr Simpson's concern about the ADTCs—why do we need 14 or 15 of them when often one would do? However, the ADTCs do a lot more than simply agreeing that new medicines from the SMC recommendations can go on to a local formulary. They do a lot of work with prescribers, both in general practice and in hospitals, on ensuring the best use of medicines locally and on the detail of how medicines are used. For example, they look at the conversion of medicines that are given by vein and when they should be taken orally, and special medicines for the skin—they do a range of stuff locally that people need to agree to and understand. Much of that work is uniform across Scotland.

I have not looked at all the formularies in detail, but I have looked at some where the health boards work closely together. For example, smaller NHS boards such as Orkney and Shetland in effect adopt the NHS Grampian formulary. They adapt little bits of it for local use, but essentially they use the same one. The total number of formularies in that sense comes down a bit.

The other key aspect of formularies and the limited evidence that is published about them is around agreement about the use of those medicines in a community of prescribers. You can achieve that agreement when you have people who know each other, work with each other and get on locally—they can agree how to use the particular medicines.

Such agreement is difficult to achieve in any type of national forum. The key to that, as I am sure Dr Simpson will recall, is the general practitioners. They prescribe the largest amount of drugs by volume in Scotland. GPs are free to prescribe any of the medicines that are licensed in the UK or that are approved for use—that is around 12,000 of them. GPs can in fact prescribe any drug at all that is licensed. Legally, GPs can do that whether or not the drug is approved by the SMC. However, the fact is that GPs, because they have worked together so well locally in the groups that Dr Simpson mentioned and now with the ADTCs, have a broad understanding of the effectiveness of the drugs—locally and for patients in particular groups. They have a good understanding of how the budgets work and how costs are maintained in health boards. Having a single ADTC for Scotland would result in the loss of all that local collaboration and understanding, and most of that local budget control.

I did not recommend that we should have only one ADTC, but for the introduction of specialist medicines—Dr Simpson gave an example of that,

but there are several—I think that it would be a very good idea. It would introduce a small delay, but that would be relatively small. The gain would be that everybody would understand how a drug was to be introduced, and patients would benefit more uniformly.

The Convener: Do you want to comment, Professor Routledge?

Professor Routledge: Professor Swainson and I have mentioned the importance of transparency, which is especially important in this matter. If the SMC has deliberated about a medicine and made a recommendation, it should be taken seriously. The implementation of the medicine should be actively tracked so that we can be clear about the reasons why those decisions were made and what the evidence base was.

It is important to recognise that the SMC is—from my examination of it—very representative of the health boards and the ADTCs. It was drawn from them, and as a forum it has tried to represent views throughout Scotland. Its recommendations are therefore very important, and should be seriously addressed and whenever possible taken up.

Dr Simpson: That is helpful. I agree entirely with Professor Swainson's reply regarding the other elements of the ADTCs. They have an important local role in interacting with all the prescribers.

The whole system is drawn from the prescribing community in Scotland. However, it is still up to the individual prescriber whether they use a particular drug, so there is a caveat. If a prescriber feels that they wish to be more conservative, they can be, as some quite rightly will. It is one of medicine's strengths not to adopt novel things too rapidly, as that can cause problems.

The IPTR process seems to be the area in which we have the greatest problems. I will leave aside for the moment the separate issue of ultra-orphan drugs. Conditions that are subsets of significant conditions are a problem, as are conditions that only one clinician is dealing with. The example that was given to the committee—the convener will be aware of it, because his constituents were involved—concerned paroxysmal nocturnal haemoglobinuria. A clinic at Monklands deals with PNH in Scotland, and it had 21 patients who might have been eligible for the new drug. The drug was recommended for 14 of those patients, but not all of them were given it because boards took different approaches.

When there is one expert clinician in Scotland who is comfortable with prescribing a new drug—which, in the case of PNH, has not been approved but nevertheless appears to produce a significant benefit—and when, as with PNH, a decision must be made very quickly because it would be fatal for the patient to have to wait, a system in which an individual clinician must apply to 14 IPTR panels is totally dysfunctional.

The existence of 14 IPTR panels is a problem. The recommendation that the patient must be different from those in the trial group process that led to the licensing of the drug seems to produce a fundamental catch-22 for many conditions. The panel must decide what is different about the patient—not what is socially different or the length of life extension that might be worth while, nor what comorbidities may be present, because the clinician would not recommend the drug if the comorbidities were likely to be fatal in the time period. The individual clinician will look at those issues. If an individual clinician feels that a drug is appropriate, and yet they must apply to 14 IPTR committees, we have not got the system right.

The fundamental point is that it is just not practical to have an expert on each of those 14 committees for those conditions. There can be massive delays, or whatever. The point that I made about novel medicines applies to this group too, in my view. We need a different approach for this group—people with some of the conditions that Professor Routledge quite rightly pointed out in his report—which would take us forward in a much more transparent, fair and timely way.

10:15

Professor Swainson: I agree with much of what has been said. The limited snapshot that we have of IPTRs is that two thirds or so succeed. In that sense, the system works for a number of people. The evidence that I have had, particularly from NHS Greater Glasgow and Clyde, is that its IPTR process can be very quick. It is slickly organised and things can be done electronically, so a decision can be made quickly. That is probably true in the larger boards where the expertise is immediately available.

The question that you raise about rarer conditions is important. One of my recommendations tries to address part of that by asking the national services division to maintain a register of approved specialists so that where we are dealing with a condition on which there are a limited number of experts in Scotland, somebody is nominated who is able to provide the information quickly to the clinician in a board who is treating the patient.

Given that the interaction between the individual clinician and their patient and the people on the IPTR from whom they are seeking support is essentially local, I still believe that the process should be carried out locally. However, a number of things can be done to improve it. The issue

about having specialist support for the doctor who is making the application is important. That aspect could be improved very quickly.

I made a number of recommendations about the IPTR system and how the general process works. Having seen some of the best examples and heard from some patients and their representatives about examples of not so good practice, I think that the process can be very considerably improved.

Professor Routledge: For certain conditions, one might need to go outside Scotland. Certainly in Wales we look in England for specialists when the condition is so rare that perhaps no one in our own country has the relevant expertise. From that point of view, these are very much UK issues.

Bob Doris (Glasgow) (SNP): I will touch on some of the matters that Dr Simpson referred to. I seek some clarification on your recommendations. A lot of what I have heard so far is general assertions about your reports rather than specific questions about your recommendations, which are one of the reasons why we are here.

One recommendation is that, when the SMC approves a new medicine, the ADTC should make a statement within 30 days about what its policy is likely to be on making the medicine available across the health board area and that information should be rolled out with complete clarity within 90 days. I thought that that was supposed to be the situation already. Is there anything new in that recommendation or does it just ensure that health boards are doing the job that they are already supposed to be doing?

Professor Swainson: It is largely about ensuring that health boards do what the latest chief medical officer letter from the beginning of 2012 asks them to do. The publishing bit is important. The audit that I attached to my report, which was conducted by Healthcare Improvement Scotland, showed that the ability to find information easily on boards' websites was patchy. The majority of the boards that HIS looked at published the information, but individual patients whom I spoke to had had some difficulty in finding it, so there is quite a lot of work to do to make information a lot easier to find.

The SMC publishes its advice—it gives a clear recommendation and its advice is usually accompanied by quite a deal of text explaining why the recommendation has been made. However, if people try to find out how such a decision flows down through their health board in that health board deciding whether to include a medicine in the formulary, the central difficulty is in matching up the two aspects.

The recommendation was aimed at getting boards to make a decision quickly—the decision is made by a consortium of ADTCs, so that should not be difficult. I appreciate that the process for whether clinicians want something to be on the formulary and any particular issues that may exist with new drugs—which Dr Simpson mentioned—need to be sorted out. That would introduce a bit of a delay but, if a board could at least say, "We have looked at this quickly since the SMC published its decision and now we are going to do X, Y or Z," with regard to the categories that HIS has already agreed on for how to handle the recommendations, that would improve the speed and the transparency of the decision making.

Bob Doris: I find that helpful. Recommendation 1 in your report is in effect restating the Scottish Government's expectation about what should already be happening but, more important, it is saying that there should always be transparency and openness in how health boards come to such decisions.

I am more interested in recommendation 2 in relation to when such medicines find themselves on local formularies—or not, as the case may be. I am relaxed about whether the medicines are on local formularies, because that is a local decision—as long as the process is open and transparent and the health board explains how it has got to a decision. That is fine, so I am relaxed about recommendation 2—it is important.

However, we heard in evidence that, if individual clinicians wish to prescribe a non-formulary medicine, that can be a bureaucratic, time-consuming and difficult process. Somewhere in our inquiries, the fact got lost that, when health boards do not put a medicine on the formulary, individual clinicians can still prescribe it and use it in treatment. However, the ease of doing that could be an issue, whether that is to do with the information technology systems, particular medicines being in stock or the clinician not being used to going through the process that is needed.

Did you look at that issue at all? There is a huge safeguard—on one level, it almost becomes irrelevant what health boards decide about local formularies if individual clinicians feel confident that they can prescribe an SMC-approved medicine if they want to. Do the report recommendations take into account any other barriers to prescription that may exist in such cases?

Professor Swainson: I recognise what you are saying, but those particular issues were not raised with me. I remember that part of what you are saying came through in the *Official Reports* of the committee meetings but was not pursued.

I cannot give you a detailed answer. My only feedback is that applications for non-formulary medicines are handled internally by all health

boards—that is a separate process from the SMC process that we were particularly asked to address. I did not hear any particular criticisms of that health board process. It may be bureaucratic in some places but not in others—I have no further information on that.

I was trying to make the point that, if we get a much clearer line between SMC decisions and what appears on board formularies, that might avoid people having to ask for an additional step in getting a medicine on to a formulary. I want that line of decision making to become quicker and more transparent and to be published more easily than it is now.

Bob Doris: I completely agree with that point, but that feedback is important to tease out the fact that you did not identify particular problems with the prescription of non-formulary medications.

On the IPTR process, I have suspected for some time that there has been—for all the right reasons—an unfortunate misunderstanding by the public over what IPTRs were set up to do. The issue came up a lot in the committee round-table evidence sessions and it is mentioned in your report. Will you say a little bit about that?

Professor Swainson: I think that there is such a situation. One view that I have heard is that an IPTR is simply a back door to get access to medicines that are not approved by the Scottish Medicines Consortium, but that is not the reality. The IPTR process was a compassionate response to enable doctors to access medicines for patients whom they are looking after who are genuinely different in some respect from the generality of patients who are described in the evidence that the pharmaceutical company submitted to the SMC to gain approval or not.

Not all patients are the same. The patients who have been studied might be one particular group; trials often exclude particular kinds of patients. The IPTR's purpose is reasonably clear to me and to many people, but it perhaps has not been well enough explained or discussed publicly so that everybody has a good understanding of it.

Bob Doris: Much of the IPTR process still hangs on what was previously described as exceptionality—the fact that an intervention will give a response of a higher quality that is beyond what would be assumed from the peer group or the trial group. We can change the terminology however we see fit, but people understand clearly what is meant by the old terminology of exceptionality, even if we no longer particularly use that expression.

Recommendation 10 is that

"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."

Does that recommendation point to a situation where, if a patient is trying to get their clinician to prepare a case for an IPTR and they want to cite specialist evidence that there will be exceptionality—that the response would go above and beyond what would be expected from the peer group—they should go to that national register? If your recommendation was accepted, would that national list be a mandatory requirement under the IPTR process?

Professor Swainson: I do not know whether it would be mandatory; I guess that that depends on whether the recommendation is accepted. What I was trying to get at is that the specialist knowledge and skills of doctors vary, yet they have to look after the patients who come to them.

The cancer networks provide an example. Because cancer specialists generally work in other hospitals—outside the specialist board area, if you like—the knowledge and skills of those doctors are available to all the patients who are being treated for cancer in the regional network. I referred to that in an answer to Dr Simpson.

However, for many other conditions, the doctor who treats somebody outside one of the teaching centres might not have sufficient specialist knowledge or expertise and, in addition, they might not submit an IPTR very often. That is what I have heard. The situation is difficult; when a clinician has a patient who is extremely anxious about their illness and wants to get the best treatment that they can, the process is a daunting prospect. A clinician must consider how they can be sure that or test whether the patient whom they are looking after has clinical features that differ from those of the patients in the studies that were submitted to the SMC.

I am trying to get at the point that we could make specialists more readily available to doctors who do not have the specialist knowledge and skills, so that everybody gets treated on a level playing field and the quality of the IPTR application is as good as it can be.

10:30

Bob Doris: I suppose that that is what I am trying to tease out, because we will ask the cabinet secretary about this as well. Under recommendation 10, an individual clinician could submit an IPTR on behalf of a patient, although they were not on the national list of specialists for that condition. However, if a patient went through a clinician who was on that list, that could give the

submission added weighting for consideration. I am trying to tease out whether that would be standard practice. Is the recommendation intended to support individual clinicians? I have no opinion either way on the matter; we are trying to tease out the implications of the recommendations. However, could the practice undermine an individual clinician's opinion, simply because their name does not appear on the national list? What is the recommendation's intention?

Professor Swainson: The main intention is to support clinicians.

Bob Doris: Recommendation 12 states that the rare conditions medicines fund

"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 want to focus on the words

"after a successful IPTR ... has been agreed."

Is it suggested that, if an individual with an orphan or ultra-orphan condition gets their IPTR accepted, every patient with a similar condition will, as a matter of course and without having to go through the IPTR process, get access to the same drug? If not, would they have to put in a second, third or fourth IPTR? Will you explain what recommendation 12 means in that respect?

Professor Swainson: By definition, an IPTR is individual and for one patient and one doctor. In the report, I discussed whether we could use a system involving a group patient treatment request. A relatively small number of patients in Scotland have an ultra-orphan disease and a relatively small number of doctors look after them. There is perhaps a single specialist, in Scotland or outside, who can provide detailed specialist support to such patients. If the medicine for those patients is not recommended by the SMC, that opens the way for an IPTR-type approach, because the patients may be different.

I am saying that we should have 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. However, that rather depends on whether the patients have enough in common to enable that to happen.

Bob Doris: I am not sure whether I understand that yet. Are you saying that, if four patients in Scotland had a particular ultra-orphan condition and if the new version of the IPTR system was in place, all four patients—who could come from across Scotland—could be identified and put through via one specialist for the IPTR, and they could all access the medicines that they needed from only one decision?

Professor Swainson: Yes, but I prefaced my comment by saying that the patients would need to share the same clinical features of the condition that enable them all to be different, although not necessarily in the same way, from the patients whom the SMC had studied. We are talking about a not-recommended decision by the SMC, so if patients were to access a medicine through any kind of IPTR process, they would have to have some features that were different.

Bob Doris: Yes, but that is where the committee started with its evidence, because that becomes incredibly difficult to demonstrate with ultra-orphan conditions. We wondered whether your report changed the situation somewhat in changing where the bar sits for patients with ultra-orphan conditions in getting through the system to get approvals via the IPTR process. If the Government accepts recommendation 12, will it change the situation for patients with ultra-orphan conditions?

Professor Swainson: Yes, because funding has been put in place to enable boards to meet the cost of such conditions. It is helpful to have that fund. It does not change the overall system; we use the same process for those patients as for other patients. The process is not different from that for patients with any other condition.

Bob Doris: I am a bit clearer, but not exactly where I want to be. I might return to the subject later, convener.

Mark McDonald (North East Scotland) (SNP): I suspect that my questions will be predominantly for Professor Routledge. I note that you refer in recommendation 7 of your report to the need for the SMC to increase patient and public awareness of its role and the decision-making process. The committee has recognised that: the SMC perhaps does not have the most user-friendly processes, for those of us in the public eye who read reports about drugs in the media. Perhaps the SMC is not as effective as it could be in getting its point across.

Beyond a publicly available annual report—which recommendation 7 mentions—do you envisage the SMC making available some sort of regular update briefing about the decisions that it is making and the reasons that lie behind them?

Professor Routledge: I agree whole-heartedly about that. It is not sufficient to do a good job; one has to make sure that everyone knows about that, including the public. Regular briefings about work that is occurring and regular updating of health boards and area drug and therapeutics

committees are part of that. The annual report is only one thing, although it is important because it can be written to inform the public and health professionals about the SMC's work.

The second part of recommendation 7 relates to the fact that the SMC has shown, by its work on antibiotics stewardship, that it has the resources and skills to give broader central encouragement to safe and effective prescribing, other than just through appraisals. It is important to link the appraisal of medicine and access to medicine—that is only one aspect of safe and effective prescribing. There is also the range of ways by which drugs are used safely and effectively. With the appropriate resource, the SMC has the skills to broaden its remit nationally.

Mark McDonald: The committee's work and our debates have touched on the way in which certain drugs can be promoted through the media, often by companies, and that is not always balanced against the clinical information that the SMC or the National Institute for Health and Care Excellence has available. One of the more prominent examples of that comes from the issues around Herceptin when it was first recommended for approval. In advance of that recommendation for approval, there was an aggressive media campaign in support of Herceptin, when there had been no submissions to NICE at the time. Are there issues in the current process regarding the pressures that can be applied through just the weight of publicity? How can that be balanced against the clinical approach that needs to be taken?

Professor Routledge: That has always been a problem and it has not gone away. One way in which Scotland has tried to address the issue is by having good collaboration with the pharmaceutical industry from the outset. I see access to medicines in any health community as being a partnership between the industry and the health service. Therefore, the closer we work together and collaborate to avoid such problems, the better.

That means that medicines should be submitted to the Scottish Medicines Consortium at a very early stage, because problems can occur in the gap between the licensing and the marketing of medicines. A timely process with good horizon scanning—I believe that that is good in Scotland—will reduce the period of uncertainty.

It is a matter of mutual trust between the industry and the health service. The main purpose of both is to get clinically effective and cost-effective drugs to patients as soon as possible. We share the same role; we simply have to work more closely together to avoid such problems.

Mark McDonald: The point about cost effectiveness leads me neatly to the pricing issue. That is another of the issues that have dominated much of the discussion.

I note from your report your examination of the quality-adjusted life year system and the fact that health economists believe—unanimously, I think—that the QALY approach is the best one to take. That approach has been criticised, as there are certain medicines that, by their nature, will never achieve a QALY that would allow them to be considered, because of the size of the cohort or the stage of the illness at which the medicine would be applied. That has led to some people looking at value-based pricing as a different approach.

The discussions that I have had indicate that we do not know enough about value-based pricing to know whether it would make a significant difference to the number of drugs that are made available in comparison with the QALY approach. What have your investigations uncovered?

Professor Routledge: Even if value-based pricing comes into place in 2014, there will continue to be many medicines for which the current appraisal process is important. From the feedback that I received from health economists and my reading, I believe that the QALY is still the gold standard, in that it can be clearly measured, taking into account the quantity and quality of life, and the system can allow the comparison of different conditions and different medicines.

However, if we consider the QALY to be the most important or the only criterion on which to base a decision, that can be misleading, and one can get into difficulties. It is a necessary part of health technology appraisal but, if it were the only part, we would not need the Scottish Medicines Consortium or NICE—decisions could be made on a strictly numerical basis.

All the issues around social value judgments need to be developed in order to put the QALY into context. You mentioned some of the situations in which that might occur. It might occur in end-of-life situations and situations involving children or rare diseases, for example. The important point is that there should not be sole reliance on a QALY or any particular QALY threshold; other social value judgments should be used that may impact on the decision, particularly with ultra-orphan medicines.

Mark McDonald: To link that back to my initial question, would more open and transparent relationships between the public, politicians, the press and the SMC help to improve understanding? I get the feeling that the wider perception out there is that the price is the be-all and end-all in the process and that other factors do not play a role. Would the process that you

suggested in recommendation 7 in your report help to break down some of those perceptions?

Professor Routledge: I believe so. That is why I made recommendations 1 and 2, as well. If there is a transparent process, any misconceptions about the reasons for a decision can be held open to direct scrutiny, so any process issues will disappear. The decisions will then be made on scientific grounds. The SMC and the manufacturer might disagree on the decision, but nevertheless it will be made on scientific grounds.

The important thing about having the industry at the table is that that can ensure that the process takes into account all the issues that the industry feels to be important, so that the industry does not feel that the decision was made on inadequate grounds or in the absence of some crucial evidence. I firmly believe that that will help in arriving at what we all wish to achieve, which is to have a better understanding of how the decisions are made and ensure that they are made transparently.

10:45

Mark McDonald: One of the frustrations that the committee has had is that we do not feel that there is enough information at present about the difference that value-based pricing would make and how it would operate. Do you share that frustration? In the work that you have done, did you find that it was difficult to get a hold on that? Do you have any idea when that information might become more widely available so that we could, perhaps, hold up the QALY and value-based pricing systems and compare and contrast the approaches that will be taken?

Professor Routledge: I certainly want to know more about value-based pricing and I feel that I am in the same position as you. It is important that we liaise with the bodies that will be involved, to get clarity on the matter. I support your contention that we need more information.

Mark McDonald: Obviously, pricing is not a one-way street. Companies that have invested heavily in the development of a drug will want to see a return on it but, if the drug is not approved, they will not see a return on it. What can the industry do to examine the pricing set-up that it adopts, so that drugs can be made more affordable and therefore more available?

Professor Routledge: The industry should have, and already has, a major role. One strength of the system in Scotland and the liaison with industry has been the development of patient access schemes, which are a form of value-based pricing, in that the price is set at a level that makes the drug cost effective—or not, as the case may be. An attempt is made to make it cost effective. We should encourage that dialogue with the industry to ensure that the price is set at a level that ensures that the drug is not only clinically effective, which it may well be, but cost effective.

The Convener: I would like some clarity on the quality-adjusted life year. The evidence that we have received showed significant concerns about the limits of that equation, as if we were talking only about health budgets and prescribing costs. I thought that, in our discussions with it, the SMC took some of that on board, but it has not been reflected in the recommendations. The cap that has been in place for 10 years was set arbitrarily and has not changed over those 10 years. There is also an absence of recognition of the impact and wider benefits of medicines and drugs on the wider family, the community and the care services that are in at an earlier stage, for instance.

There is no recommendation about that in the review, although I certainly expected one. Some of the evidence that the committee received should have been reflected on.

Professor Routledge: I hope that I pointed out in my report that, although the QALY is still the gold standard and I was unable to find any alternative, it must be considered in the context of all the other issues, particularly the social value judgments. That is where there needs to be further exploration. The SMC modifiers allow some of those social value judgments to be addressed, but perhaps that could be considered further.

Having a process in public also allows those issues to be examined much more closely. It allows not only the industry but patients and patient representatives to be present. It is important to involve them more closely in the process that leads up to the appraisal. Their submissions can be extremely valuable in helping the SMC to come to a judgment that is based not solely on the QALY but on all of the representations of the stakeholders in the process.

The Convener: Those remarks are helpful—we should be considering a wider assessment. However, your explanation does not bring us clarity, transparency and a clear understanding about what has been evaluated in the process. I do not want to misrepresent you, but that is almost like an add-on—something that could be considered, rather than something that should be considered.

I am clear about the people who would be on the Scottish Medicines Consortium and their expertise, but would we need other people on the SMC to help evaluate the impact on the community budget? We should be considering the extended public purse, and there should be clarity about the whole cost.

Professor Routledge: I do not think that there are any major shortcomings in the structures of the consortium, but the patient and public involvement group representatives have an important role, which could be strengthened further. That strength could be utilised in making judgments.

The ultra-orphan issue is an example of where the cost per QALY would not meet the threshold that is set by NICE. Nevertheless, such drugs can be approved because of the societal issues, which mean that a representative committee, drawn from the health service and including representatives of lay groups and clinicians, can make a judgment that, although the cost per QALY might not reach the normal thresholds, the drug should be made available. That illustrates to me that the SMC does not just work according to the cost per QALY; it takes into account much broader issues and tries to represent the patients of Scotland as best it can.

The Convener: You can understand the perception, given the negotiations about the to-ing and fro-ing that are often, unfortunately, covered in the press. The point of agreement is then reached for the authorisation of the medication, but it is predicated on a reduction in its cost to the health service. You can understand why people think that the issue is one of cost.

Professor Routledge: Yes, absolutely.

The Convener: You can understand that, given the cap that has been set on the QALY for the past 10 years.

Professor Routledge: Yes. I certainly did not make any comment about whether the QALY was at the right level. Although I used the word "threshold", there is no absolute threshold. To the best of my knowledge, there is no cap either. A drug might be approved at a level significantly higher than £30,000 per quality-adjusted life year. However, if a drug is priced at a level that makes it less than £20,000 per quality-adjusted life year, one would expect it to be approved unless there were very good reasons not to approve it. That is more of a spectrum against which judgments are made, but judgments are made at a cost per QALY significantly above £30,000 in some cases.

The Convener: That goes back to your fundamental recommendation, which is that people have to understand the process.

Professor Routledge: Yes, I think so.

The Convener: You have told us something that has not been particularly clear at previous evidence sessions. Do you wish to add anything, Professor Swainson?

Professor Swainson: I support Professor Routledge's view that the SMC is the right place to take into account the wider societal and other issues around the use of a drug beyond its clinical effectiveness and cost. It can have ramifications down the line for other services—both in health and elsewhere—if a drug works in a different way from whatever has gone before, and the wider societal gains might be considerable. I welcome that approach, which certainly helps with the general process of understanding and getting drugs approved for use.

Drew Smith (Glasgow) (Lab): In the context of what has been said about social value, Professor Routledge's recommendation 6 is that the SMC

"should be encouraged to set up ... a 'Citizen's Council' or 'Citizen's Jury'".

Will you say a little about the parameters of such a body and which part of the process it would try to influence?

Professor Routledge: I think that the comment was made that social value judgments are really owned not by health professionals but by society. Therefore, it is crucial that society be involved in deciding how it will use its limited budget to support access to medicines, recognising that if we spend our money on one group of medicines there might be less money for other medicines and treatments—there is an opportunity cost with every decision.

NICE set up a citizens council, which tried to represent society as a whole—obviously with a limited number of participants. The citizens council looked specifically at ultra-orphan medicines and made recommendations, which were not unanimous but helped with decisions about the approach that would be taken to such medicines. The strength of the council's input was that it came from individuals who were not suffering from a disease, were independent in their views and were from a variety of walks of life. They therefore represented what society feels about a particular issue.

A slightly different approach has been taken in Wales. Groups of 12 people—called citizens juries—have been asked to listen to expert advice and then come to a judgment about specific issues to do with health and medicines.

Those are two ways to involve the general public in informing the decisions that we make, given that it is their health and their money—taxpayers' money—that we are dealing with. A citizens body is one way of ensuring that there is ownership of the decision-making process.

Drew Smith: How would such a body consider the issues? Would the process be driven by the SMC saying, "This is an issue of concern and interest to the SMC and the wider community, so we will present you, as an external group, with the

parameters for your thinking"? Would there be a public engagement element, too? I am sure that members of the citizens council or jury would be impartial, in the sense that they would not be directly affected by the issues. Would they respond to groups that are directly affected, or would that need to be done separately?

Professor Routledge: It would be ideal if the body considered issues that were directly relevant to medicines access in Scotland. The SMC should be very much part of the process, but it could be widened to include much broader representation in Scotland. The crucial point would be to ensure that a representative sample of the general public was involved and was giving advice independently, as you said.

Drew Smith: Do you envisage such a body being a one-off or something that meets fairly infrequently, when an issue comes up? There is currently great public concern about the issue, so this is a good time to take the temperature—if you like—of the views of the wider public. Alternatively, do you envisage input from such a body being more integral to decision making? Should there be a standing body, which is engaged with a programme of work?

Professor Routledge: I certainly think that one would need to see how it worked, first. As I said, the citizens council did a valuable job, and the citizens juries came up with helpful advice. It might well be that if such an approach was successful in Scotland, it could be continued. There are cost implications of such a process, of course. I think that initially one would want to address a particular area, such as end-of-life or ultra-orphan medicines.

11:00

Bob Doris: In recommendation 4, you say:

"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".

Can I infer from that that, currently, once a manufacturer submits evidence and a price, the process is fairly rigid from that point onwards and that the recommendation would change that? Is the current process quite inflexible from that point of view?

Professor Routledge: The process is not inflexible, but because it is swift, it tends to continue once the clock has started. That may mean, for instance, that there is more than one submission for a particular drug for a particular indication. That can cause some confusion if you have several decisions. Therefore, if an issue is, as you say, integral to the decision-making process, the SMC should feel empowered and it should have that opportunity to reflect on that rather than have to go through the whole process because it has started. I say the SMC because I think that it should decide how the process develops and what timelines there are.

That recommendation is based partly on my own experience. Sometimes issues can be dealt with that might mean a pause in the process but nevertheless make the outcome much clearer. That is why I made the recommendation.

Bob Doris: NICE currently has a pause—a review period—in the process. Can the price that pharmaceutical companies initially submit for the medicines be altered during that pause in the process? In other words, do companies sometimes aim high, get a refusal and then pitch the price a bit lower so that they get their drugs approved at the resubmission? Can drugs companies use the process as a bargaining chip by aiming high, getting refused, then pitching the price lower? Would that pause that you recommend allow those discussions to take place within one submission process?

Professor Routledge: I did not see the issue as being about agreeing a price. What is important is that the SMC's process is so timely that anything that systematically impacted on it would be unhelpful. I am trying to get at the issue around any possible concern about the model that is being used—about the comparators. In those circumstances, if it is clear that there can be no clear outcome, it is important to clarify such issues. However, the SMC's role is not to negotiate the price of the medicines.

Bob Doris: I completely agree with that, and the SMC has made that point perfectly clear to us. However, if a resubmission comes in with a lower price, that is a longer process than a process that could pause to allow the drugs companies to take stock of a submission and amend it if necessary. I guess that the pause that you recommend could allow that to happen. Are there any examples of pharmaceutical companies putting in a submission to the SMC and then fairly quickly afterwards putting in the same submission to NICE but with a price that is lower than in their SMC submission?

Professor Routledge: I am not aware of any such situation. I have no knowledge of that.

Bob Doris: I have no further questions.

Dr Simpson: I have two or three quick questions. One is on procurement. Professor Swainson's report referred to some of the bigger health boards having an advantage in procurement and to that being a factor in their ADTC approach. I was slightly surprised by that, because I thought that we purchased drugs on a Scotland-wide basis.

Professor Swainson: I did not go into that in detail. Those were just some of the remarks made to me by the directors of pharmacies.

Dr Simpson: Should we be asking the Government to pursue that a little bit and examine what is actually happening?

Professor Swainson: Yes, I am sure that improved knowledge about all that would be helpful.

Dr Simpson: I noted that the patient and public involvement group—PAPIG—stopped being part of the annual report in 2008, which surprised me, particularly in light of Professor Routledge's welcome recommendations about greater involvement of patient groups. Do you know why that has occurred?

Professor Routledge: I did not get any comments on that.

Dr Simpson: Perhaps we will ask the SMC about that.

You were positive about horizon planning, but did either of you feel that the boards' use of horizon planning was appropriate? For example, various boards took a little while to look at the new drug that partially replaces warfarin, and they did it in different ways. Horizon planning was clearly part of that issue. Did you do any work to look at how horizon planning was playing into the ADTCs and local use?

Professor Routledge: I did not look at that. Horizon scanning work in Scotland is a beacon and is highly valued by other health technology organisations elsewhere. The process is excellent, but I cannot comment on the ADTCs apart from to say that horizon scanning is available and it is extremely helpful.

Dr Simpson: There are two bits to my point. One is the bit that you have praised, which you have rightly said is a world leader, but the second question is about what the boards actually do with it and what is the practical part of the implementation of horizon scanning. Are there delays because the boards are not getting ready for the budgetary requirements and refinements of use to which you referred, Professor Swainson?

Professor Swainson: Horizon scanning is very valuable. It gives almost a year's warning about what is going to happen. I did not look specifically at how long it takes, but I guess that in a country of our size, with the relationships that we have, we could get all that prepared and accelerated. That is partly what I was saying about the ADTCs' general response.

Dr Simpson: We have talked about all the new drugs that are coming in, but does the SMC have a role in saying that certain drugs, although they are still licensed, are no longer regarded as being of clinical benefit? In other words, what can we stop using? Such drugs might not be hugely expensive, but the added cost of the prescription to the cost of the drug is significant. Should the SMC be recommending that a medicine should no longer be used in the Scottish context?

We all recognise that we have a global package of costs and we have decided that we should not have prescription charges in Scotland, which reduces income to the health services by something between £56 million and £70 million. The previous prescription charging system was antiquated and bankrupt and it needed radical alteration, but we have got rid of that money. Should we consider taking the approach that is used in Oregon and other countries of saying that some medicines should not be on prescription? That might go slightly beyond the witnesses' remit, but I am interested to know that because of our budgetary considerations. Some drugs incur the added cost of issuing a prescription that means that the medicine can cost two, three, four or even eight times the cost to the patient buying it over the counter. I am referring mainly to OTC medicines.

Professor Routledge: That certainly was not part of the review of the SMC process. However, you make an important point about the need to look at acceptable disinvestment in some of the things that we do in the health service. I hinted at that in my recommendation that the SMC could help in the broader context of a consideration of general medicines management. Clearly, that is part of the work of the area drug and therapeutics committees, but there is also a need for central co-ordination of that and resources that help to move the use of particular medicines that are being overprescribed in certain circumstances, particularly if there is variability across Scotland. The skills of the people in the SMC are appropriate to helping with disinvestment in treatments that are of limited value or are potentially harmful. It is important to try to link together the assessment of medicines with all the other issues around the use of medicines, which are not necessarily all the province of the SMC alone. Given the appropriate resources, the SMC could take a much broader role.

Professor Swainson: I support that general view. There are some good examples in Scotland of how decisions to stop using medicines are made. For example, some drugs that are used for treating rheumatoid arthritis are monitored very closely at the ADTC level to ensure that only patients who require those drugs get them. The safety monitoring that must be done is done to a high standard. A great example from recent years involves the change of use of antimicrobial drugs. For various safety reasons, we agreed that,

although certain drugs might be effective, the side effects, in terms of the promotion of Clostridium difficile in the environment, were no longer acceptable, so we changed the way in which antimicrobial prescribing is done and we effectively no longer use certain drugs in Scotland, or use them only in very much reduced amounts.

Dr Simpson: Should it be a requirement to publish all trial data when submissions are being made? That is a very topical issue. The BMA—membership of which I should declare—has a campaign on that matter at the moment, mainly in relation to Tamiflu but also in relation to other drugs. If we are going to recommend that a drug be used, should we ensure that the pharmaceutical companies all sign up to making available all trial data as part of their submissions?

Professor Routledge: I should declare an interest as well, as I am the president of the British Pharmacological Society, which has signed up to the all-trials campaign. I firmly believe that all trial data should be made available. That is important. Again, it comes down to the issue of transparency. I am delighted that many pharmaceutical companies are signing up to that.

Professor Swainson: Yes, I absolutely agree.

Dr Simpson: Convener, I think that I have taken up enough of the committee's time.

The Convener: I see that you have prompted a couple of questions from members.

Bob Doris: This will be a brief question, I hope. Mr McDonald asked about value-based pricing, the cost effectiveness and social benefit of drugs and the lack of information at a United Kingdom level. Of course, value-based pricing has a Scottish context, which is recognised in these reports as well. Soon, this committee will be considering the integration of health and social care. I have had many discussions with pharmaceutical companies, which, I assure you, have not been backward in coming forward to tell committee members what they think the SMC process should look like. In those discussions, I have asked whether they have considered whether a medicine will benefit not only the national health service but also social care providers, such as local authorities, in two years, five years, seven years and so on. There seems to be a dearth of economic modelling in that regard, whether on the part of pharmaceutical companies or anyone else, so we are unable to come up with an evidence base on that issue.

I raise that point in the context of value-based pricing because, if those economic models could be built, we could release money from the system to provide drugs and medication that would otherwise seem not to be affordable. Do you think that, if Scotland thought imaginatively within the process, that would be achievable? Do you have any comments to make on that? The committee will be looking at the integration of health and social care as part of our other work.

11:15

Professor Swainson: That is a fascinating question. In a sense, this represents an opportunity for us to integrate health and social care and to think of them as whole systems, which is how people think of them and have to deal with them. The ability to take into account the effects downstream, on subsequent health and social care costs, of using a medicine at a particular time in a person's life must be important.

My only experience of that is with some of the ultra-orphan diseases, particularly those that affect children and that can result in profound disability later. If we are able to prevent all or part of that with a course of treatment early in a child's life, I would have thought that any economic model should be able to take that into consideration.

Bob Doris: When looking at cost effectiveness during their current approval process, neither NICE nor SMC will look at the savings for local authorities or other public sector bodies should a drug be approved. Such savings are not considered or quantified just now—is that correct?

Professor Routledge: That is my understanding.

Bob Doris: The problem is that we do not know what value-based pricing is going to involve. In your opinion, would it be deficient if it did not address the question of what the savings could be for local authorities and other public bodies? If value-based pricing followed a merely health economic model, would that be a weakness?

Professor Routledge: If the models of health and social care have been integrated, that issue must be addressed in order to see the true value of the benefits. That supposes that that has happened, but that is not the case at present.

Nanette Milne: I have a brief question. I confess that it is not on your current remit, but it is prompted by the references to disinvestment. Do you think that there is a case to be made for processes and procedures in the NHS being scrutinised and evaluated in the same way as medicines? That does not appear to happen at the moment.

Professor Routledge: Medicines have led the way in terms of both an evidence-based approach and an economic approach, as the price of a medicine is clear and benefits can often be measured much more straightforwardly. However, the same principles can—and should much more—apply to other forms of care in the health

service. Medicines have led the way in showing how valuable that more objective approach to assessing all treatments can be. We should be assessing the value of treatments, not the cost of the treatments in isolation, and investing in those that give patients the best outcomes.

Nanette Milne: With the increasing demands on the NHS, it will become increasingly important to know what value we are getting out of the entire service.

The Convener: There are no further questions. Thank you, gentlemen, for sharing your work and recommendations with us this morning—and sometimes going a wee bit beyond those, as well.

11:18

Meeting suspended.

11:24

On resuming—

The Convener: We continue with item 2 and welcome our second panel, who are Alex Neil, the Cabinet Secretary for Health and Wellbeing; Professor Bill Scott, the chief pharmaceutical officer; and Dr Aileen Keel, the deputy chief medical officer for the Scottish Government. I welcome you all, and invite the cabinet secretary to make an opening statement before we move to questions.

The Cabinet Secretary for Health and Wellbeing (Alex Neil): Thank you very much for the opportunity to appear this morning.

As the committee knows, I listened to the concerns that were raised through the petitions and acknowledged the need for an independent review to identify improvements in licensing and availability of new medicines. Medicines are an essential part of clinical care for patients, and NHS boards are responsible for making sure that clinical care is optimised through the most effective and efficient use of the most appropriate and cost-effective medicines. However, there are in existence about 12,930 UK marketing authorisations, or licences, for prescription-only medicines.

Clinicians are key to optimising care for their patients, which involves critical decisions around use of medicines and other therapeutic interventions. At its heart, that is about a therapeutic relationship between the clinician and their patient, and the care that the NHS provides for families in difficult circumstances.

The Scottish Medicines Consortium has the challenging job of appraising newly licensed medicines and providing advice to NHS boards in Scotland about their clinical effectiveness and cost effectiveness. Its process uses SMC expert clinicians, who provide guidance on the place of the medicine that is under consideration in the best therapeutic care for patients, which may involve comments on best-practice clinical guidelines. The SMC considers such views alongside other available evidence.

The SMC has published advice on 854 medicines indications to date, of which 595, or 70 per cent, have been accepted, or accepted for restricted use, within the NHS in Scotland. People who have given evidence to the committee have acknowledged the value of the SMC's work. I believe that we have a national system to be proud of. At this point, I will quote some speakers who presented at the recently held 10th anniversary conference of the Scottish Medicines Consortium.

Professor Lloyd Sansom, who is a special adviser to the Department of Health and Ageing in Australia, said:

> "I extend my congratulations to the SMC on its 10th Anniversary. In that 10 years the SMC has developed into one of the most respected Health Technology Agencies in the world. In Australia, which has a much longer history of HTA, the evaluations from the SMC are widely read and are considered to be one of the bench marks for assessments."

Similarly, Dr Mary Baker MBE, who is president of the European Brain Council, said:

> "The SMC punches well above its weight and has become a powerful influence in Europe."

I could quote many other experienced experts in the field making similar comments.

Obviously, difficult decisions have to be made around the value that different groups place on relative cures versus very short life extension—possibly two to three months—where quality of life is uncertain. SMC decisions to publish not-recommended advice about medicines reflect that the evidence that the manufacturers submitted to it about the medicine's benefits does not justify its cost. Although many pharmaceutical companies have engaged in the national process to offer discounts on their medicines, others have chosen not to do so. That has to be viewed within the context that prices of medicines have escalated significantly in the past 20 years.

The SMC has been able to publish advice to confirm its acceptance of some 25 medicines on the basis of a patient access scheme, since the scheme was established in 2009. I want to see us build on the scheme's success. A group of more than 100 world experts in chronic myeloid leukaemia recently published an article in the journal *Blood* arguing for the need to lower prices of cancer medicines to allow more patients to be offered them and to maintain long-term healthcare

policies. The committee may wish to consider that aspect in its deliberations—I certainly agree with the clinicians' view.

Ideally, I would like the political parties to work together to find a pragmatic approach to facilitating shared understanding of and support for the need to ensure the best possible outcomes for patients and their clinicians in this challenging environment, while meeting our responsibilities to patients and the public to achieve that within the resources that are allocated to the NHS.

On how we will move forward, we will not take any decisions on the Swainson or Routledge reports until we have seen and considered this committee's report and recommendations. We will then engage in a period of public consultation, with the objective of achieving cross-party consensual agreement on the way forward on this difficult issue: I think that it would be to everybody's benefit if we were to take it out of party politics.

11:30

The Convener: Thank you, cabinet secretary. The first question is from Mark McDonald.

Mark McDonald: Thank you, convener, and thank you, cabinet secretary, for coming along today. In my questions to Professor Routledge, I said that the committee had concerns about the lack of information on value-based pricing—what it entails, what difference it will make and how it will be distinct from the QALY system. I do not wish to put words in Professor Routledge's mouth, but I think it was clear that he has similar concerns about that lack of information. Do you share those concerns? What discussions have you had with the UK Government on it? Has there been a lack of information coming forward?

Alex Neil: I emphasise that the pricing of pharmaceuticals is still a reserved matter; it is not one for which we have legislative responsibility. Therefore, decisions and leadership on value-based pricing, or indeed any other aspects of the pricing of medicines, have to come from the Department of Health in London. The department has said that it is happy to consider the issues and to discuss them with the devolved Administrations in Edinburgh, Cardiff and Belfast.

I have to say that we were expecting to be much further down the road in terms of preparations for value-based pricing, which was originally due to be introduced in January 2014. From the very limited information that we have on the status of the value-based pricing proposals, it looks as though January 2014 will be an extremely ambitious—if not already unachievable—date for their introduction.

We are very much in favour of the principle of value-based pricing, particularly in terms of taking a range of additional factors into consideration in pricing medicines. However, we also need the proactive co-operation of the pharmaceutical companies, which have to be much more open about how they price their products. I heard Professors Routledge and Swainson being asked earlier whether there is any evidence that the pharmaceutical companies use the SMC as a dry run for their presentation to NICE and, if so, whether there is a difference in the price. The honest answer is that we do not know, because the companies are very secretive about their pricing. I can up to a point understand the commercial interest in being secretive; nevertheless, much more openness all round would be helpful for everybody.

We absolutely support the principle of value-based pricing, but pricing of pharmaceuticals is a reserved matter, which is led by the Department of Health. We are very disappointed at the lack of progress.

Mark McDonald: I explored with Professor Routledge the point about the industry. The issue of pricing cuts both ways. The industry will have spent large amounts of money developing pharmaceuticals and will obviously want them to be purchased. Do you detect from discussions a willingness for a different approach to be taken not just to pricing but to submissions? Might we be able to have more flexibility in the future?

Alex Neil: Flexibility is very important and I am very supportive of having as much openness as possible. Having worked in the commercial world for many years before coming to Parliament, I recognise that each company is operating in a commercial and very competitive environment. There are, however, areas where companies could be more forthcoming, particularly with regard to the cost structure and the development costs of medicines. The core argument from the pharmaceutical companies is that it often takes 10, 15 years or more to develop a medicine before it comes to market and that they have, therefore, to make up for the costs that they incurred during that period.

Also, some medicines—particularly new ones—are not on the market for long before they are superseded by another drug.

I recognise the challenges that pharmaceutical companies face in that they are, ultimately, in business to make profits, but I also think that it would be in their interests, our interests and—most important—the interests of patients if they were a bit more forthcoming and open with us, particularly on pricing and the cost structure. Although we accept that they have to turn a pound or two on pharmaceuticals, many companies are

making extremely substantial profits; one wonders whether we are getting the biggest bang for our buck.

Mark McDonald: Okay. I note that Professor Routledge made recommendations on the transparency of the SMC approvals process and on improving public awareness and perception of what it entails. Do you believe that a more open process, by which I mean one that is publicly accessible and in which information on the decisions that are taken is more publicly available and more user friendly for those of us who are not well versed in the terminologies, would be of benefit? I get the feeling that a shroud of mystery still hangs over some of the decision taking, which often leads to misconceptions about what has led to a decision on a particular drug. Removal of some of that shroud of mystery might help to improve public perception.

Alex Neil: I think that there have probably been a couple of misconceptions in the public mind. The first misconception is that the SMC turns down more drugs than it approves. As I have said, the SMC approves for use or for restricted use 70 per cent of the drugs for which it receives applications, so the percentage of approvals is high.

Secondly, I think that there is also a misconception on the part of the public about IPTRs, which is sometimes reinforced by consultants wrongly advising patients that it is a waste of their time to apply for an IPTR—I have certainly heard anecdotal evidence of that. Nearly two thirds of IPTRs, including IPTRs for cancer drugs, are approved. In other words, there are far more approvals than there are rejections. It is very important to get that message across.

One of the big advantages of far greater openness and transparency in the SMC process and right across the board—including on the part of the pharmaceutical companies—would be much-improved understanding of the system, which is much better than some people think it is. That is not to say that the process does not require further refinement and improvement—it absolutely does. That is why we will listen carefully to the committee's recommendations and to its view of the Routledge and Swainson recommendations before we make a final decision.

My principle, not just in relation to drugs but across Government, has always been to maximise transparency and openness, because that gives everyone a much better understanding of the complexity of the issues that are faced. Such openness and transparency would allow people to see just how much information the SMC is or is not getting from the applicant for a new drug. Applications are sometimes turned down initially not because the SMC has reached the conclusion that the drug in question is not the right drug, but because it has not received the evidence to show that it is the right drug, because other drugs are available or whatever.

The more openness there is, the better the understanding will be. We are talking about extremely difficult decisions, which are not unique to Scotland or the UK. The availability of drugs at the right price in the right place for the right patients is a global issue. We should be as open as we can be, while always maintaining patient confidentiality.

The Convener: I know that pricing is part of the process, but do you agree that the UK should be leading on establishing a value-based pricing model?

Alex Neil: That question can be interpreted in two ways. Under the current constitutional arrangements that is absolutely the right thing to do. However, if we had different constitutional arrangements, although we would still very much co-operate with the rest of the UK, that would perhaps be on a more equal basis and we would be driving the matter far faster than it appears to be being driven at the moment.

The Convener: I look forward to the cabinet secretary explaining how Scotland—by itself—would be more able to take on big pharma. I am amazed that you introduced the constitution into the discussion. I will now ask the question that I intended to ask before you did so. Although you suggest that under different constitutional arrangements you would be leading the process, you say that there is a delay in the matter. Have you suggested a different approach to the UK Government to address the issue?

Alex Neil: We are very much in the dark about what is happening. I do not know whether the Department of Health has more information than it is giving us, but we certainly do not have a great deal of information on what is happening other than the clear indications that the chance of value-based pricing being introduced on the original timescale is getting less and less likely.

Related to that is the fact that the cancer drugs fund down south was established on the understanding that value-based pricing would come in in early 2014. We have seen in recent weeks a substantial reduction in the number of medicines that are being made available under the cancer drugs fund in England. I would have thought that it would be in the interests of the Department of Health to be further down the road on value-based pricing so that it could—if nothing else—decide on the future of the cancer drugs fund. I am not blaming the Department of Health; I am just saying that the position is very unclear. That is disappointing because right across the UK

we are agreed that value-based pricing is the right thing to do.

Aileen McLeod (South Scotland) (SNP): I welcome the cabinet secretary's awaiting the Health and Sport Committee's recommendations on the review before making a final decision on what recommendations to make, and the fact that there will be a brief consultation over the summer.

I seek clarification on the timing of your recommendations and when they will come into play. There are concerns that it is less likely that value-based pricing will be introduced by the 1 January 2014 deadline. We also have the new cancer drugs fund coming in to replace the current system.

Alex Neil: On where we go from here, I presume that the committee's report will be published with recommendations before the summer recess. Once we get that report, we will have about one month during which we will consult key stakeholders on the committee's report recommendations and the Swainson and Routledge reports. We will then form a view on how to move forward.

I am keen that at that stage we will sit down with Opposition spokespeople to see whether we can agree on the way forward in a detailed way, because it is not in anybody's interests to revisit the issue in another year or two. It would make much more sense if we could get a robust system up and running, that we are all signed up to. The system needs to be robust enough to cater for the introduction of value-based pricing. The processes that we are talking about are precisely that—they are processes rather than particular pricing strategies. However, the process must be robust enough to deal with any radical changes in the pricing strategy.

Aileen McLeod: We need to ensure that we have as much confidence and trust as possible in the new system to make sure that we can progress it.

Alex Neil: Absolutely.

11:45

Dr Simpson: I join my colleague Aileen McLeod in welcoming the approach that the cabinet secretary is taking on the matter. It is in all our interests to get it right this time. It is the second time that we have had a go at the issue. The first one resulted in the IPTR process instead of the exceptional needs approach. That has clearly worked to some extent, but has not worked always.

However, there are areas in which we should not wait for reports and consultation, such as the need for the area drug and therapeutics committees to publish their decisions in a timely manner, which has been made clear by the chief executive and CMO letters.

In the last iteration of the issue, the 90-day rule came in. We have found from the audit that the rule is not always being obeyed and the decisions are not being published adequately. Will you, rather than wait for the committee's full report, take action on that now? I know that the instruction has been reissued to the area drug and therapeutics committees, but what are you doing to ensure that the rule is implemented?

Alex Neil: As Richard Simpson rightly says, we have reminded the local area drug and therapeutics committees that there is a 90-day period to adhere to. It is in place for good reasons and we are talking to individual boards, including chief executives, to ensure that publication happens much more timeously than it has in the past. If any board does not adhere to that, we will consider what we need to do to ensure that it does. However, I am very much of the view that the rule should be adhered to as far as possible.

I ask Bill Scott, who is the man in charge, to add anything he wishes to say.

Professor Bill Scott (Scottish Government): HIS has now set up an audit process to examine how boards perform so that we can give feedback and achieve the target of publication within 90 days.

Dr Simpson: That is good. The other issue is the variation that occurs when the Scottish Medicines Consortium recommends a drug for full or restricted use. The audit, which was helpful, showed a variation between NHS Lanarkshire, where 23 out of 23 drugs were put on to the formulary, and NHS Lothian, where only 13 were put on to the formulary.

The review suggested that one of the main reasons for clinicians not wanting a drug on their formulary is that it is not applicable in the area—they do not have the specialist element. That is not borne out by the fact that NHS Lothian has one of the lowest inputs of newly approved drugs and used the excuse of clinicians not wanting a drug. I assume from Professor Scott's answer that the audit will apply to that as well as to timely publication.

Aside from continuing the audit, we need much clearer reasoning for why a medicine is not being accepted quickly, appropriately and in a timely manner. Only 74 per cent of the medicines that the SMC recommends, which is itself 70 per cent of the applications, get through to the patient. We are talking about a fraction of a fraction—it is still a majority but it is also still a fraction of a fraction.

Is the cabinet secretary prepared to take some steps now, without waiting for the committee's report, to ensure that, first, the reasons for a medicine's not being accepted on to a formulary are made clear and, secondly, if a medicine is not applicable on a given formulary—which was Professor Swainson's suggestion—that is laid out as a seventh reason, separately. In other words, the board would say that it was not needed on its formulary because it would be dealt with at tertiary level.

Alex Neil: If I may, I will correct your maths, because 70 per cent of 70 per cent is 49 per cent, which is not a majority.

Dr Simpson: I think that it was 74 per cent. We can quibble.

Alex Neil: We are at one on the general point that you make. The last thing that we want is any kind of postcode lottery on the availability of important drugs.

When we analyse the 26 per cent of drugs that, in NHS Lothian's case, were not made available within the 90 days, we find a range of reasons why they were not made available. Some clinicians say, for example, that they are not keen to offer a drug because there is something else available that they think is superior. I am not going to overrule clinicians, and I do not want them to feel that they will be overruled because of a rule that does not allow them the degree of freedom that they need to make proper clinical decisions.

With regard to the brand-new drugs that are coming in, there is no excuse for delay. That issue will be part of the audit that HIS is undertaking, because we want a much more consistent performance in that area.

Dr Simpson: The conservatism of the profession is one of the patient's safety guarantees, and I am not advocating that everyone adopt every medicine overnight, but the prescriber does not have to prescribe a medicine. The issue is that some medicines are banned according to the formulary for an area that covers the whole of the Lothians as well as the Borders, which works on the same formulary. A tranche of people are therefore excluded, whereas just over the border in Lanarkshire, people can get all 23 drugs.

Alex Neil: Absolutely. We need a consistent approach throughout the country, as Professor Swainson's report makes clear; there are issues around how we will achieve that. There is a case for saying that, once the SMC takes a decision, we should implement it nationally rather than have 14 boards implement it at different paces.

I certainly do not think that there is a case for abolishing the area drug and therapeutics committees, because—as the committee heard in evidence this morning—they do a fantastic job in a range of areas, and it would be a huge mistake to lose that local connection. We are not going to throw the baby out with the bath water, but we need to raise the temperature of the bath water to ensure that the process is consistent and timeous, and that we do what is required for everyone everywhere, which is to make those drugs available consistently throughout the country.

Dr Simpson: That is particularly the case for the novel drugs.

Alex Neil: Absolutely.

The Convener: Are there any other questions for the cabinet secretary?

Gil Paterson (Clydebank and Milngavie) (SNP): Good morning, cabinet secretary. There is a lot of discussion around the IPTR process. Recommendation 9 in Professor Swainson's report suggests that consideration should be given to putting a member of the public on each panel. Are you attracted to that idea?

Alex Neil: Professor Swainson's recommendation on the IPTR process and Professor Routledge's recommendation for a citizens council or jury are both very welcome. I do not want to pre-empt our discussion of the committee's recommendations, but we would be happy to support those.

The public need to be, to be seen to be and to feel as though they are more involved in those decisions. That will lead to far greater understanding among the public of the complexities of some of the decisions, and it will also—I hope—lead clinicians to better understand the public perception of such decisions. We need to close that gap, because misconception is an element of the problem.

Gil Paterson: The public's expectations and knowledge about how the process works are an issue. One member of the public on an IPTR panel might not provide the transparency that is required nor act as a way to inform the public.

Have you any ideas over and above that recommendation for how you would inform the public about how the process operates?

Alex Neil: There is a big difference between the public having access to the SMC's decision-making process and having access to an IPTR panel. By definition, an IPTR process concerns an individual patient, and as such there are issues with patient confidentiality. We have to be much more careful about any arrangements that we put in place for involving non-clinicians in an IPTR process. I am talking about the principle rather than whether there should be one or two—or however many—members of the public involved.

We have successfully run a system of public partners in Healthcare Improvement Scotland's inspections, for example. That system, in which there are certain obligations on how the public partners go about their business, might be the kind of model on which to build this kind of participation. I would like to see the committee's recommendations on exactly that kind of thing, and then discuss it with the other parties.

I draw a clear distinction. A discussion about a decision on the availability of a drug in which no particular patient interest is involved can be much more open than a discussion about an individual patient's requirements, which is where I am slightly more cautious. The principle is a good one, but we need to be absolutely sure that such a discussion in no way compromises patient confidentiality.

Gil Paterson: I understand that fully. I was talking not about individual cases but about the overall work of the IPTR panel. We were informed earlier about the impact on a person's wider life and on their family. Giving a member of the public the opportunity to engage in the process might be beneficial.

The element of decisions being taken—not on individual cases but in the panel's overall work—could be publicised to inform people. There is a lot of misconception about exactly how the process works and there is a bit of noise that it does not work in some cases, when in fact the evidence would say otherwise.

Alex Neil: I have had anecdotal evidence of some consultants telling patients that it is not worth their while making an IPTR. That is a misconception; it is worth while making an IPTR and it is worth while for the clinician and the patient to take some time to make sure that they submit a good-quality application. The purpose of the IPTR is to assist patients who feel as though they need access to a drug that is not generally prescribed.

On wider involvement in the IPTR process, the more people understand about it, the better. How we go about that is wide open for discussion. We will consult widely on that recommendation, as there are a lot of implications.

Nanette Milne: Recommendations 9 and 10 in Professor Routledge's report concern keeping a register of IPTR decisions—centrally, I presume—so that we know what is going on in different parts of the country, and regular sharing of experiences between area drug and therapeutic committees. Can I have your comments on those recommendations, please?

Alex Neil: They seem very sensible and certainly fit with other recommendations made by Professor Swainson. Again, we will wait to see what the committee says and we will discuss it with Opposition parties. They seem to be the type of recommendation that would be broadly welcomed, because they are sensible things to do.

They would help us to gather intelligence and identify any part of the country in which the process does not appear to be working as well as it could and should. In some senses, the more information that we gather about what is happening out there, the easier it is for us to monitor the situation and make sure that the system is working as robustly as it could and should be.

Nanette Milne: Thank you. That is what I hoped that you would say.

Bob Doris: Cabinet secretary, you said that some clinicians might say to patients that it is not worth their while to make an IPTR. I hope that those would be examples of clinicians who are trying to inform patients and give clarity but are expressing poorly what IPTRs are for. Some patients should not go through the IPTR process and have their expectations raised falsely if clinicians know at face value that their cases evidently would not meet IPTR criteria. It is important to say that. Professor Routledge's report teases out a quite understandable lack of understanding on the part of some members of the public about precisely what IPTRs are for.

On area drug and therapeutic committees and the time that it takes for drugs to go on the formulary, another misunderstanding has been about drugs that do not go on the formulary. Even so, they can still be prescribed by clinicians locally, and that needs to be made clear. I asked this question in the previous evidence session: do you have any views on the ease of access for clinicians wishing to prescribe and use drugs that are not on the formulary, and on whether there are any barriers to doing that locally? Is there a way to make that easier? I would rather that we had the formulary right to begin with, but individual clinicians should be allowed to make independent decisions based on their own knowledge, expertise and views.

12:00

Alex Neil: I will bring in Aileen Keel and Bill Scott to answer parts of that question and to give their views, particularly on the latter point.

Underlining your question is the need for all of us—the SMC, health boards and the Government—to be much clearer to both clinicians and patients about exactly what can and cannot be done at each stage of the process. I would like there to be what I would call a taxi driver's guide for patients about how they can use the IPTR process if they feel that they are not getting a drug

that they think is essential. This is a complex area, and it would be good to have an easy guide that ensures that patients know what their rights and their chances are—without unduly building up expectations that are not going to be realised. There is a wide degree of discretion around the final IPTR decision and a whole range of factors are taken into account, and the patient might not totally appreciate that unless we explain it properly. There is more work to be done to get the message across to clinicians and patients and to ensure that everybody has a full, proper and comprehensive understanding of the IPTR process.

I invite Aileen Keel and Bill Scott to respond, especially on your latter point about how the process is working on the ground.

Dr Aileen Keel (Scottish Government): The response to the question that Bob Doris posed to Professor Swainson was that he had no evidence of the barriers that you were discussing being in place. You are absolutely right, Mr Doris, that any doctor can prescribe any medicine that is licensed.

The main thread running through the two reports is the need for greater transparency and more openness around the processes, so that we can demystify them. If there is evidence—we need to look for it—that clinicians who want to prescribe licensed products that are not on a local formulary are coming up against undue barriers in the process, we will address that as part of our information gathering.

Professor Scott: As Dr Simpson pointed out, new medicines are not all necessarily the best medicines available. It was interesting to consider Professor Swainson's report, in which he wrote that specialists should be brought in to help to get uniformity in formularies.

It depends which literature we look at, but there are a number of medicines—perhaps 80 per cent—that are me-too. They might have a different side-effect profile but are not on the formulary. In cases where a clinician wants to use such a medicine and it has gone through the SMC, there should be a simple process, I believe, whereby the clinician can get that medicine for their patient. As Dr Keel said, we will consider that point.

Alex Neil: I stress the importance of the local formulary. It is clear that since local formularies were introduced, they have allowed us to manage prescriptions. This year, we will spend £1.4 billion on prescription drugs. Around £1 billion of that will be spent in the primary care sector and the rest will be spent elsewhere, primarily in the acute sector. That is around 15 per cent of our entire budget. The local formulary is part of the process of ensuring that we manage that money as effectively as possible. We want maximum flexibility and we want to remove any unnecessary and artificial barriers, but I do not want to throw the baby out with the bath water. The local formulary is a key tool in managing the prescription budget.

Bob Doris: It has been stated that a number of health boards pool their approach to local formularies. I think that Dr Simpson gave examples of that. There are 14 ADTCs, which have important functions other than local formulary functions. Could the Government take an approach that standardises some of that joint working? Rather than allowing health boards to feel their way when they co-operate or otherwise with other health boards, could some of that work be standardised and formalised?

Alex Neil: That is definitely an area for us to look at. Obviously, we need to consult clinicians and the boards in particular, but we also need to consult other stakeholders. I will be interested in seeing what the committee recommends on that.

Bob Doris: Another recommendation in the reports is that there should be a national register of experts for local clinicians who feel that they might not have the detailed expertise to demonstrate an evidence base for their individual patients' use of the IPTR process. Are you initially supportive of that recommendation? My gut instinct is that the idea is excellent, as long as it is seen as being supportive of local clinicians. We would not want a local clinician to think, "I am an expert in this area, but my name is not on the national list and I feel that I have to go to someone who is on it." I am not suggesting that that would happen, but there are potential dangers with the approach, although by and large, it is an attempt to support local clinicians.

Alex Neil: Absolutely. We want to stress that point: it is a support mechanism, not a substitution mechanism. The relationship between the clinician and his or her patient is extremely important, particularly where the patient has a rarer disease or condition—I mean not necessarily a rare one, in the sense that very few people have it, but a disease or condition with which perhaps only a handful of specialists in Scotland deal so that it makes absolute sense to put their advice at the disposal of the clinician who is dealing with the patient. However, the person is still the clinician's patient, and I do not think that the specialist would want to usurp the clinician's role.

Bob Doris: That is helpful.

Finally, at the end of the previous evidence session I asked about value-based pricing. My question is nothing to do with the speed of value-based pricing being rolled out or the lack of clarity; rather, it is about the Scottish context. We are moving to health and social care integration and are looking at pooled budgets, single budgets,

early intervention and keeping people at home and healthy for longer. Where a pharmaceutical intervention or medicine can make that happen and people are more mobile and healthy and need less support at home, there will be financial savings for local authorities. My understanding is that none of that is taken into account in the modelling work anywhere in the UK on the cost effectiveness of medicines. Whether as Scotland or as the UK—let us put the constitutional arguments to one side and speak about what is best for our constituents—when we look to approve a drug for a certain use, should we look at not only the costs of prescribing it, but the downstream savings to local authorities and social care? It is my understanding that we are not doing that now. Is that where you see things going in future years?

Alex Neil: The more robustly we can model, the better. I will give an example. On average, it costs £4,500 a week to keep someone in an acute hospital in Scotland; £1,800 a week to keep someone in a community hospital setting; between £500 and £600 a week to keep someone in a nursing home; and around £300 a week to keep someone at home. If by taking a new medicine somebody is kept out of acute hospital—even for a week—that saves £4,500. If the patient is at home, the net saving is £4,200. If they are in a community hospital instead, there is still a net saving of £700 per week. By any standards, those are very substantial savings, especially if they are applied across a large cohort of patients. It makes absolute sense for us to try to have robust modelling and evaluation systems that can take those factors into account.

It may be very difficult to do that at patient level; it might have to be done at a more generic level. Either way, we need to develop more robust models as part of the process. I do not think that anybody would disagree with that.

Drew Smith: Clearly, if all the recommendations were to be pursued—although I understand that we are not agreeing that today—there would be implications for the amount of drugs that are prescribed in Scotland and, therefore, implications for the budget, which might go up or down. We can come back to that; I know that we are not at that stage yet. I appreciate the cabinet secretary's offer to listen to what the committee has to say before coming to a final judgment. I presume that an initial assessment has been made and that nothing in the recommendations presents a challenge to the Government in terms of the resources available for more reporting, holding meetings in public, or setting up citizens juries.

Alex Neil: The recommendations are not costly recommendations to implement, to be frank. What is at issue here is not cost, but whether we are doing the right thing. The key measurement must always be, "Does it improve patient outcomes—are we improving the satisfaction of the patient journey?" We must never lose sight of the fact that we are trying to create a person-centred health and social care system in Scotland. I think that transparency, improving the process, making it simpler, informing people, citizens juries and so on, do help us to improve the patient journey, especially understanding, and that in doing that we improve the patient outcome.

Drew Smith: Thank you. It is helpful for the committee to know that. My final question is on a broader point. When we have taken evidence on this before, a number of people have made the point that medicine spend is probably the most scrutinised part of the process. Do you have a view on whether other parts of the picture around treatment for patients should be subject to a much more rigorous examination of what value really means?

Alex Neil: We have to look at the totality of health provision. Prescriptions and drugs are a very important part of that, taking 15 per cent of the entire £12 billion per year budget. However, there are many other parts of the health service. We are having a debate tomorrow, for example, on bed numbers—and not just the crude issue of bed numbers, but the balance between, say, surgical beds and medical beds. That is part and parcel of how well people are treated at accident and emergency.

Last week I spoke at the conference for dementia champions and the graduation award for the first dementia champions. The work of the dementia nurse consultants, who are paid for by the Alzheimer's Society in Scotland, has resulted in first-class improvements to the care of dementia patients—particularly in the acute sector but also in the primary sector. We must always look at the totality—every aspect—of the system and scrutinise it constantly to ensure that we are getting answers.

I have been in this job for just over seven months, but the goalposts move all the time, especially as new medicines come forward. I remember the days when a cataract operation in one eye required hospitalisation for weeks; now it can be done in a morning and the patient is back out of hospital. So clearly we do not need all those beds that we needed 20 or 30 years ago when a cataract operation meant being in hospital for weeks at a time. Things are changing all the time and we have to ensure that we all change with them fast enough to stay ahead of the game to ensure that the best treatment that is available anywhere is available to people in Scotland. That is our objective.

12:15

The Convener: Some aspects of the discussion about access to new medicines are very complex. People have petitioned the Parliament and we have had, as I am sure you have had cabinet secretary, emails from the relatives of Janice Glasswell about her quest to get cetuximab, the cancer drug that is freely available on prescription in England but is not available here in Scotland. How does this review change the situation? I think it was yesterday that a consultant here in Edinburgh claimed that 40 such drugs are available in England but are not available here, and that people were going through their relatives in England to get access to cancer drugs. How does the review help people who are in that situation?

Alex Neil: I met Janice Glasswell and her husband and I am sure that any of us who were in that situation would feel exactly as they did. As a politician, I am not in a position to overrule clinical decisions, and I do not think that anyone would want me to. If we got into that situation, it would be a regressive state of affairs.

One of the misconceptions around the issue is that it is all one-way traffic, in that some drugs are available in England that are not available in Scotland. Many drugs are available in Scotland that are not available in England. It works both ways. We have to make sure that the SMC and IPTR processes and all the infrastructure around them are as robust as possible so that, when it makes clinical sense for someone to get access to a particular drug, they get access to it.

The key issue is defining what makes sense. For example, earlier this year we had the issue of children who have cystic fibrosis. It is estimated that just over 50 children in Scotland have a particular Celtic mutation of cystic fibrosis that means that they would benefit substantially from Kalydeco. It is claimed that that drug would extend their lifespan by up to 16 years. That is a very expensive drug, but the condition is very rare; in Scotland, it would be for just 50 people. Therefore, in my view, it makes sense to make that drug available. Hopefully we will find a cure for cystic fibrosis within 16 years.

Obviously each case has to be decided on its merits. By making the system more transparent, consistent, and robust, I hope that we will end up by making better informed, better understood and more robust decisions, so that people who would benefit from a particular drug receive it, within the parameters of what we can do.

The Convener: Do you accept that the IPTRs, which were introduced not so long ago, have caused some confusion? They were presented as a solution to the problem of access to drugs but they have not proved to be so.

Alex Neil: It was right to bring in the system itself but, by definition, the committee would not be conducting this investigation and I would not have had Professors Routledge and Swainson conduct their reviews if the system had been working as well as we hoped it would. Their recommendations and those of the committee are designed to improve the system and how it works.

The fact that more than two thirds of IPTRs are approved is an indication that the process is working far better than it is sometimes perceived to be. That said, there are improvements to be made and I hope that we can reach agreement on what those improvements should be and implement them as quickly as possible.

Every country in the world is dealing with the issue of the availability of medicines at different levels. As people are living longer; as demands on the national health service are growing; as resources are becoming scarcer because of the budget squeezes in recent years; as the complexity and incidence of co-morbidities are increasing; and as both the cost and the potential benefits of new drugs are increasing, the allocation of resources and the setting of priorities become the issue. Where do we set the parameters? That is not an easy question—anyone who pretends that it is is not being honest—and it is not easy because we are dealing with matters of, literally, life and death.

The Convener: Do you agree with the SMC's contention that there should be a wider debate and opinion polling to agree the priorities, or should the priorities be agreed simply by politicians or clinicians?

Alex Neil: It has to be driven by the evidence—primarily the evidence of the impact of a drug but also the evidence of the cost of the drug and whether it is the best use of resources. Every time that we spend money on one drug, less money is available for other drugs and it may be that more people would benefit from another drug than would benefit from that drug. I do not think that we can make decisions on these things on the basis of opinion polls; our decisions must be based on hard evidence. That is why, ultimately, the process has to be driven primarily by clinicians rather than by politicians or opinion polls.

The Convener: To go back to Drew Smith's point, why should that sort of scrutiny of outcomes, value and quality not be applied to all health services? Bill Scott is nodding at that. Do you agree that all services that are provided in the NHS in Scotland should be assessed in that way?

Alex Neil: There are many different processes in the national health service but they are all

fundamentally about trying to allocate resources on the basis of clinical priority. My answer to Drew Smith was that that is already happening. This is about resource allocation, which is a difficult thing to do.

Let me give you an example. Last year, we spent about £45 million on research directly in the national health service. One of the benefits of the results of that research, over the past couple of years, has been a dramatic reduction in the incidence of C diff. Another benefit of the informatics science of that research has been a substantial reduction in the number of amputations, particularly among patients with diabetes. We invested money for that research and it has paid off—although it might not have done; it was a risk. Such are the decisions that have to be made, and they should be scrutinised regularly. This is about the most fundamental of decision making: the allocation of scarce resources within the national health service.

The Convener: Yes, at that strategic level, but are you claiming that the rigorous assessment that is applied to drugs and new medicines is applied to every service that is delivered in the health service?

Alex Neil: We try to apply rigorous assessment. From time to time, a number of boards undertake a review of clinical services in their areas and the configuration of those services. As you know, some years ago I opposed the proposals arising from the review of accident and emergency services at the Monklands and Ayr hospitals. The result of that was a very rigorous review led by Dr Andrew Walker of the University of Glasgow. Day in, day out in the health service we rigorously review all such things. I am happy to ask Aileen Keel to give you more detail on that.

Dr Keel: That is absolutely right. That work is the bread and butter of what NHS boards do day in, day out. We are around this table, I suppose, because what we are talking about is the discrete, high-profile—in terms of media coverage—high-cost area of new drug developments, which have really come on board over the past few years, particularly in relation to cancer drugs. Many of the drugs that we are talking about are monoclonal antibodies, which began to be developed about 10 years ago. We now have a swathe of those drugs coming online and being licensed and made available to the NHS, but only at very high cost. I guess that explains why this committee has focused on the area and why there have been two reports on it, because it is distinct from the more routine areas of NHS delivery, which the boards scrutinise daily.

Dr Simpson: I have a brief comment on the Kalydeco issue, because I do not quite understand why the SMC did not make the decision on that drug. As we go forward, we are going to be faced with drugs that have been produced for specific sub-groups within a disease area. We are at the beginning of an era of personalised medicine. Unless we face up to that and recognise it in the structures that we put in place, we are going to have a real problem, are we not? We will have to replicate the one-off decision that the Government made on Kalydeco. The question is what the threshold level for that will be.

The SMC turned down a bowel cancer drug, but it was subsequently discovered that, because of a genetic marker, it would be beneficial to 70 per cent of those with bowel cancer, which is a very big number. The drug was still very expensive, but its application was narrowed down to a special sub-group. I just wonder what your views are on the politics of the issue. If we start making decisions on individual drugs, will we not be in a very difficult situation? We could of course hand off such decisions to the SMC.

Alex Neil: My view is that we need a system that does not involve politicians in making decisions about whether drugs are available. I think that it would be a huge mistake for politicians to be involved in that. We would end up in a very bad place if decisions on drugs were political decisions. That is why I am very keen to try to get cross-party agreement on what system we go forward with. If we all sign up to it, we can agree to take day-to-day politics out of the whole question of the availability of particular drugs. So, I think that Richard Simpson poses a very valid question.

One of the more exciting developments in the life sciences in Scotland at the moment will be the new stratified medicine innovation centre on the campus of Glasgow southern general hospital. I think that that will help Scotland become a world leader in stratified medicine. However, by definition, the area of stratified medicine raises a host of issues of the kind that Richard Simpson touched on in his question. Therefore, there are bigger issues that we will need to tackle in respect of getting more personalised medicines. Within the next five years or so, there will be diagnosis of the genetic mutation—not just type—of the particular cancer or tumour that an individual has. Presumably, that will require very individualised treatment. There will be huge questions around how we resource that, ensure that the costs can be met and all the rest of it. Therefore, this is not the end of the debate but the beginning of a new debate about how we tackle bigger and broader issues.

The work that we are presently discussing is about trying to get a robust process in place that we can move forward with. However, I absolutely agree that big issues still need to be addressed at a strategic level.

Dr Simpson: One of the differences that I think we very much agree is fundamental is that, unlike in England, where NICE looks only at medicines that are referred by a minister, in Scotland the SMC looks at everything. That leads me on to a final point on which the cabinet secretary might like to comment. I understand that a number of countries around the world follow the SMC's lead and adopt its approval of particular medicines. I wonder whether we have done any research to find out which countries do that. Given that we cannot prevent them from using our very good, world-leading decisions, in future we could invite them to contribute to the costs of a system that might have to be more robust and go into more detail.

12:30

Alex Neil: We should perhaps franchise it and raise some money.

Our understanding of the SMC's influence is the same as Richard Simpson's, but it rests on pretty anecdotal information. However, I am happy to ask officials to see whether they can do some research that would inform the committee on that. Earlier, I quoted comments from Australia about the SMC. I would not say that Australia copies the SMC's decisions, but Australian clinicians have stated clearly that SMC decisions inform their decisions.

Of course, I should make the general point that, for the past six years, anything that this Government does has been widely copied around the world.

Dr Simpson: I think that I would extend that period to 13 years, cabinet secretary, just to end on a more consensual note.

The Convener: If there are no other questions for the cabinet secretary, I thank him and his colleagues for being with us today and for their evidence. We look forward to working with him on this issue over the coming weeks and months.

We previously agreed that we would go into private session at this point to discuss teenage pregnancies, but I suggest that we defer that session until next week's meeting. Do members agree?

Members *indicated agreement.*

Meeting closed at 12:31.

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e-format first available
ISBN 978-1-78307-986-5

Revised e-format available
ISBN 978-1-78307-999-5

Printed in Scotland by APS Group Scotland