The Scottish Parliament
Pàrlamaid na h-Alba

Official Report

HEALTH AND SPORT COMMITTEE

Tuesday 25 February 2014

Session 4

Tuesday 25 February 2014

CONTENTS

HEALTH AND SPORT COMMITTEE
6th Meeting 2014, Session 4

CONVENER

*Duncan McNeil (Greenock and Inverclyde) (Lab)

DEPUTY CONVENER

*Bob Doris (Glasgow) (SNP)

COMMITTEE MEMBERS

*Rhoda Grant (Highlands and Islands) (Lab)
Colin Keir (Edinburgh Western) (SNP)
*Richard Lyle (Central 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)

*attended

THE FOLLOWING ALSO PARTICIPATED:

Myles Fitt (Breakthrough Breast Cancer)
Joan Fletcher (Association for Glycogen Storage Disease UK)
Professor Jonathan Fox (Scottish Medicines Consortium)
Alastair Kent (Rare Disease UK)
Anne Lee (Scottish Medicines Consortium)
Lesley Loeliger (PNH Scotland and PNH Alliance)
Eric Low (Myeloma UK)
Dr Frances Macdonald (Association of the British Pharmaceutical Industry)
Ian Mackersie (aHUSUK—A Patients and Families Support Group)
Karen McNee (Kidney Cancer Scotland)
Dennis Robertson (Aberdeenshire West) (SNP) (Committee Substitute)
Emlyn Samuel (Cancer Research UK)
Leigh Smith (Melanoma Action and Support Scotland)
Professor Angela Timoney (Scottish Medicines Consortium)
Professor Matthew Walters (Royal College of Physicians and Surgeons of Glasgow)
Professor David Webb (Scottish Medicines Consortium)

CLERK TO THE COMMITTEE

Eugene Windsor

LOCATION

Committee Room 2

Scottish Parliament

Health and Sport Committee

Tuesday 25 February 2014

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

Access to New Medicines

The Convener (Duncan McNeil): Good morning and welcome to the sixth meeting in 2014 of the Health and Sport Committee. As usual, I ask everyone in the room to switch off any mobile phones and BlackBerrys that do not need to be used. People should take note that members and officials are using tablet devices instead of hard copies of their papers. We have received apologies from Colin Keir, who is unable to attend, and we welcome back Dennis Robertson as the Scottish National Party substitute.

For the first agenda item, we return to our work on access to new medicines. As everyone will be aware, following the Routledge and Swainson reviews and the committee's report, the cabinet secretary asked the Scottish Medicines Consortium to review its processes, which it has now done. We will hear from the SMC later, but first we will have a round-table session with patients' organisations and a few clinicians to hear their views and verdicts on the work that has been done and to discuss what still needs to be done.

It would be useful if, as is usual with round-table sessions, those present could introduce themselves. That would also save me having to make all the introductions.

Ian Mackersie (aHUSUK—A Patients and Families Support Group): I am a trustee of and secretary to aHUSUK, which is a charity and support group for people with the ultra-orphan disease, atypical haemolytic uraemic syndrome.

Bob Doris (Glasgow) (SNP): I am an MSP for Glasgow and the committee's deputy convener.

Joan Fletcher (Association for Glycogen Storage Disease UK): I am Pompe family support practitioner with the Association for Glycogen Storage Disease UK, which is a charity that supports patients with glycogen storage disease.

Myles Fitt (Breakthrough Breast Cancer): I am the policy and public affairs manager for Breakthrough Breast Cancer in Scotland.

Gil Paterson (Clydebank and Milngavie) (SNP): I am the MSP for Clydebank and Milngavie.

Dr Frances Macdonald (Association of the British Pharmaceutical Industry): I represent the pharmaceutical industry and sit on the SMC in that capacity.

Emlyn Samuel (Cancer Research UK): I am policy manager for Cancer Research UK.

Dennis Robertson (Aberdeenshire West) (SNP): Good morning. I am the MSP for Aberdeenshire West, and I am substituting for Colin Keir.

Karen McNee (Kidney Cancer Scotland): I work in communities development at Kidney Cancer Scotland.

Dr Richard Simpson (Mid Scotland and Fife) (Lab): I am a Mid Scotland and Fife MSP.

Eric Low (Myeloma UK): I am chief executive of Myeloma UK.

Leigh Smith (Melanoma Action and Support Scotland): I am from Melanoma Action and Support Scotland.

Rhoda Grant (Highlands and Islands) (Lab): I am a Highlands and Islands MSP.

Professor Matthew Walters (Royal College of Physicians and Surgeons of Glasgow): Good morning. I am a consultant physician at the Western infirmary in Glasgow, and this morning I am representing the Royal College of Physicians and Surgeons of that city.

Aileen McLeod (South Scotland) (SNP): I am a South Scotland MSP.

Alastair Kent (Rare Disease UK): I am the chair of Rare Disease UK.

Nanette Milne (North East Scotland) (Con): I am a North East Scotland MSP.

Lesley Loeliger (PNH Scotland and PNH Alliance): I am founder and chair of the charity PNH Scotland and an executive member of the PNH Alliance.

Richard Lyle (Central Scotland) (SNP): I am a Central Scotland MSP.

The Convener: Good morning, all. I am the MSP for Greenock and Inverclyde and the committee's convener.

I believe that Gil Paterson is going to kick us off.

Gil Paterson: I will start with a general question. Will those who participated in the task and finish group tell us about how well it functioned, their ability to participate and whether any problems or difficulties arose?

Alastair Kent: As a member of the SMC's task and finish group—I should say, however, that I was unable to attend the final meeting—I found it a very careful and thorough exercise. I certainly felt able to contribute to the workings of the group

and the various discussions, and I suspect that all stakeholders felt the same way. I had no impression that the agenda and the outcome had been predetermined. The process itself was very fair, open and transparent, and it reached some very sensible conclusions.

Myles Fitt: I second those comments. I should say that, on the charitable side, I sit on the Scottish cancer coalition and that I linked in with it to provide updates on what was going on in the group. I also checked on the patient and clinician engagement—or PACE—element. There was broad consensus in the coalition that things were going in the right direction, and I fed that information back to the task and finish group. We have since had further correspondence on how the new group will be set up and what input the coalition can make in that respect.

In short, we are broadly happy with how things went from a patient and a charity perspective, and we felt that the process was satisfactory.

Dr Macdonald: From the pharmaceutical side, I back what has already been said. The group was run very well, very fairly and in a very open manner, and everyone had the chance to say what they wanted. Our side had three participants—me and two economists from member companies—and, without unduly disclosing the nature of the discussion, I had enough time to go back and get input from a wider range of companies to ensure that I could put forward the views of a wider group.

Gil Paterson: I wonder whether those present who were not participants in the group were consulted in any way. Did any of you have a way into the deliberation that was taking place?

Myles Fitt: As far as the charity side is concerned, I repeat what I said a minute ago about my acting as a link to the Scottish cancer coalition. The coalition itself did not have a direct input, but I apprised it of what was going on and it was able to feed in views through me. As I have said, the response was broadly positive.

Gil Paterson: It sounds as if the group operated in a satisfactory way and that it went wider than the actual group members who participated.

The Convener: So there are no complaints about the process, but did the group reach the right conclusions?

Lesley Loeliger: From a petitioner's and a patient's point of view, I think that the report has been compiled with remarkable speed. I also find it remarkable that our thoughts and concerns have been listened to and included. Obviously, some of the fine detail needs to be gone over and some extra information needs to be looked at, but the experience for us as petitioners has been remarkable. If we can clarify some of the points in the report, Scotland will have a world-leading system for drug appraisal and, for that, we as petitioners and patients are very grateful.

Professor Walters: The views of clinicians in Scotland are enormously well aligned with those that Lesley Loeliger has just articulated. From the practitioner's perspective, it is important to have a robust and equitable system that, crucially, delivers treatment to patients in a timely fashion. All clinicians feel the pressure of time, particularly with regard to some of the conditions that the review deals with, and my personal view is that the entire group who prepared the report should be commended on the speed with which they have conducted the review and the quality that they have achieved.

Alastair Kent: I do not want to rain on everyone's parade, but although on paper the framework is great and I endorse everything that has been said about it, how it works in practice will make all the difference to whether patients can access innovative medicines. I am very confident that the framework will deliver, but we need to keep a watching brief on the process to ensure that it lives up to the expectations that have been built up around it.

Eric Low: I must emphasise the importance of the point that Alastair Kent has just made. The devil is always in the detail. Although the report is commendable and although the SMC and the group have done a fantastic job, it will all be for nothing unless we discuss, implement and properly monitor the detail. It is critical that the SMC gets the resources to do that, but a major issue for the consortium for many years now is that it has not had the resources to do right by patients.

I just hope that the Scottish Government can resource the SMC to the level that we need so that we can deliver this world-class way to approve new drugs in Scotland.

Dr Simpson: I wonder whether the previous two speakers and, indeed, any of our other guests have specific concerns about the three different areas—end of life, orphan and ultra-orphan. We are treating them all as one. Have you any particular concerns about implementation in relation to one or other of those three groups of medicines?

Alastair Kent: No. Our principal concern is the rare drugs or the ultra-orphans. As I say, the framework that has been created looks excellent, but it is only by seeing innovative therapies go through the process that we can see in process how the framework has worked, the sort of evidence that is given weight and how those submissions can be supported. That is particularly true when we are looking at interventions involving

ultra-orphan drugs, which are required by a tiny number of patients in Scotland and, indeed, the whole United Kingdom. The ability of patients and the patient groups that support the submissions to the process will need to be helped in some cases—not many people have experience of making such a submission—particularly if they are also struggling to cope with the impact of a rare condition.

Ian Mackersie: I agree that the report was an outstanding piece of work. What impressed us particularly was the political will to make it work and to implement the recommendations. I am sure that it will have the desired result.

To take up Alastair Kent's point, the increase in opportunities under the new arrangements for patient interest groups to put forward their views is welcome. However, in our experience, PIGs vary in their capabilities and some will need significant help—possibly even financial help—to get them to perform, as it were, at the required standard.

The Convener: Let me see whether I can cut to the chase and stir up some debate here. We all agree that the process was inclusive and that people got their say, and I presume from what is being said now that that input has been reflected in the language of the initial report. Everyone is happy with that.

From the committee's point of view, the objective was to see whether we would get more yeses out of the system, and everyone agrees that we will and on when we will. There is an expectation that, in the first year, 1,500 more people will get access to drugs. Everyone believes that that will happen.

Lesley Loeliger: As we said before, and as Alastair Kent mentioned, it is all about the detail and the wording of certain parts of the report. I have always talked about the idea of including the correct specialist, and the patient and clinician engagement meeting is listed in the report as one that will include a specialist clinician.

I still want to know who would be classed as a specialist. As paroxysmal nocturnal haemoglobinuria patients, we were entirely reliant on our local specialists while believing that they were not actually the experts on our condition. If the wording can ensure that the correct expert is asked and consulted every single time, it will be a remarkable change to the system that we have and will completely eliminate any chance of a postcode lottery. It would be great to get that clarification.

10:00

Myles Fitt: Breakthrough Breast Cancer thinks that, on paper, the new system works well. The difference between the current system and the new one is the PACE step. We clearly have to get that right, get the new group established and get the process worked out and set up. We know that work is being done on that. We have to wait and see what happens, but we think that the new system should work quite well in practice.

From a breast cancer perspective, the SMC last year rejected on grounds of cost two medicines that were clinically effective. We would like to think that if the medicines were resubmitted under the new system, they would be approved. We will watch the situation with interest to see whether that happens.

We have an interest in what happens when a very high-cost medicine that is hugely clinically effective and which is supported by patient groups and clinicians comes through the new process. Under the current system, it would probably stand next to no chance of being approved. We would like to think that, under the new system, it has an increased chance of being approved. We accept that the national health service is not a bottomless pit of money, but if there is a strong will for a medicine to go through, it should at least stand a bit more of a chance under the new system. We would like to see how that works out in practice.

Eric Low: It is also important that we empower the SMC to continue to do a very robust appraisal of clinical effectiveness and cost-effectiveness. It is not a case of just saying yes to everything, because in the long term that is not in the best interests of patients or the industry. If our goal is to say yes to everything, there is no point in having the SMC. We need to balance out the desire for more yeses with the need for those yeses to be to the right drugs—in other words, drugs that demonstrate clinical effectiveness and cost-effectiveness and which represent a use of NHS resources that, in the long term, is best for patients in Scotland.

We should not dumb down the work of the SMC. We need to empower it to carry out a very robust and fair appraisal of the new drugs, with additional clinical and patient output to refine some of the uncertainty about some of the drugs that the SMC has said no to in the past. We should not be saying yes to everything.

The Convener: I do not think that the report says that. It says that 1,500 patients would benefit under the proposed new system, which would be robust, not careless. That is not insignificant and I want to be excited about that on behalf of people but I do not want to get overexcited about it to the extent that we create an expectation that is not met.

There are patients who are not getting access to medicines under the old system. From what has

been said in the debate and the announcement that was made in the chamber, it appears that patients are being denied medicines that they would be receiving if the new system were in place. That is an important point, because people are being denied access to medicines now and will be denied such access for however long it takes to arrive at the system that we want to create. It is therefore important that the new system is able to deliver. Everybody seems to accept that the new system can do so, but are there no doubts?

Emlyn Samuel: Cancer Research UK welcomes the review and thinks that the findings are very promising. However, as has been said a number of times, the devil is in the detail. As well as being robust, the system also needs to be consistent. When the details of the changes emerge, the structure and framework will have to be consistent to ensure that everything is reviewed in the same way and the process must be transparent.

Dr Macdonald: I do not want to repeat what everybody has said but, from the industry's point of view, we are also very pleased with the direction of travel and we believe that there will be more yeses.

As everybody has said, the key is how the PACE group will work. There are a lot of detailed questions on the table about how the experts will be involved, how industry will contribute and how the timing will work. I have every confidence that the SMC will continue to work in a collaborative manner to develop the processes. Industry will obviously have to understand the new system in enough time to make submissions in accordance with the new guidance rather than the previous guidance. I see no sign that the situation will not change.

Bob Doris: I want to ask a brief question, just so that we can get a couple of things on the record. I will maybe come back in later with a more substantive question.

From my notes, I see that the definition of an end-of-life condition is a condition for which, under normal existing treatments, there would be a life expectancy of less than three years on average. Is that balance about right? How does that contrast with what the National Institute for Health and Care Excellence would deem an end-of-life condition in England?

The report before the committee sets out a prevalence level to try to provide an official definition of orphan and ultra-orphan drugs. The committee used to use the terms "orphan", "ultra-orphan", "rare" or whatever, but there was no statutory guidance as to what they meant.

Those are quite technical questions, but do you think that we have the balance right in terms of what we deem end-of-life conditions? How does that compare with how others would view end-of-life conditions? Are you content with the definition of orphan and ultra-orphan drugs?

Eric Low: Yes. I think the definition is broad and helpful. It is broader than NICE's definition of end-of-life conditions. It is important to note that, going forward, NICE is not going to use the end-of-life criterion, which will be subsumed within the "burden of illness" criterion as part of its value-based assessment strategy. From a Scottish perspective, the definition is appropriate.

Bob Doris: Could those who are involved with orphan and ultra-orphan drugs put on the record their approval or otherwise of what is proposed? The committee wants to go through this in a structured fashion.

Joan Fletcher: We very much welcome the recognition of ultra-orphan drugs, which is what the original public petition was questioning. We feel as though the ultra-orphan drugs were very much at a disadvantage in the past, because of the difficulty of getting evidence from a small amount of population. We very much welcome the guidelines.

Alastair Kent: Orphan drugs are defined in European legislation as being for the treatment of a condition with a prevalence of fewer than five in 10,000 or one in 2,000 in the European Union. However, it is important to acknowledge the ultra-orphan category. I agree with Joan Fletcher that drugs for incredibly rare conditions have fallen through the net on too many occasions, and the specific recommendation is to be welcomed.

Bob Doris: I might come back in later, but I just wanted to get that on the record.

The Convener: Certainly.

Nanette Milne: I want to ask ABPI about the PACE process, at which stage you will be able to put forward revisions to patient access schemes. What are your views on that? Do you think that the pause that will be allowed will encourage you to propose a patient access scheme more often?

Dr Macdonald: Until we see how everything works, it is difficult to answer your question definitively. Companies have the option to do that at the beginning; when they first make a submission, they take a view on the value that their medicine provides and the extent of the need for it. You are right that what is proposed gives them a second option to do that. It will just be a rethinking of what they decided at the beginning.

We have to keep the PACE and patient access scheme processes quite separate, because they are two different things; they might just happen to occur in the same time period. However, you are

right. Companies can reconsider the position that they took at the beginning of the submission.

Nanette Milne: If there are issues other than price, such as data or modelling, will you have the opportunity to be involved more in the process?

Dr Macdonald: That is an open question. The Scottish Government response to the original committee report said that companies will have an opportunity to have a discussion with the SMC at the beginning. We have not started that discussion yet, because there have been other priorities to address before working that out. There is no view at the moment about having that discussion in the middle of the process—all the detail has still to be worked out.

Gil Paterson: As I understand it, if the SMC puts a new application on pause, it would go to the clinicians and patient interest groups for a recommendation. It would then go back to the SMC and then perhaps to the industry to see whether a discount would be available. What are people's thoughts on that? Coming from a commercial background, I wondered whether the process might be better if the opportunity for the industry to engage came before the application went to the patient and clinician engagement process; whatever the outcome of the industry engagement, the application would then go to the patients and clinicians and then back to the SMC.

Would there not be a benefit in that, rather than the recommendation coming from patients and clinicians? Everyone would know where they stood, instead of there still being some dubiety. It might be more wholesome and better for everyone if the intervention was earlier. Have I understood the process correctly?

Dr Macdonald: The opportunity for the company to put forward a revised or new patient access scheme runs in parallel with the PACE meeting. Your question is whether it should be one after the other: the industry first, then the PACE. The industry has that opportunity when it first makes the submission, so it knows what its cost effectiveness numbers are and it should have a good idea of the medicine's value from a clinical perspective. The industry makes its submission and, in many cases, it offers a PAS at the beginning. In many cases, the industry could probably judge reasonably accurately whether it is likely to see a minded no from the SMC new drugs committee when it first submits, so I think that it has already taken that decision at the beginning.

The industry can then revise the submission if it wishes, but I do not think that there is any rationale for saying that that discussion should happen first and then the patient should have the PACE; in some ways, that would be no different from the thought process that the industry went through at the very beginning. For strategic reasons, the industry can then decide either to put in a PAS or to change it. However, I do not think that there is any rationale for making those processes happen one after the other. The PACE becomes very valuable because it adds a lot more depth to the extent of clinical need than we get at the moment.

Lesley Loeliger: There is an excellent flow chart at the end of the report, which I found very helpful. From a patient perspective, I thought that the PACE was more to do with helping the SMC and the whole appraisal system to understand the real cost offsets and the difference that the drug could make, for example, from a PNH patient point of view. Other patients who are on the same drug as me no longer need monthly blood transfusions and can go back to work. Most of the patients have gone back to work and are therefore off benefits. From my point of view, the PACE was all about education and help for the SMC, from the appraisal-making process—I could be wrong on that—and not so much for the drug company, which already knows the drug's benefits. That was my take on it.

Bob Doris: This is one of the points that I had hoped to raise later. My colleague Gil Paterson makes a reasonable point. I apologise for speaking in layman's terms. The ABPI and the pharmaceutical companies do an incredibly important job and their submissions are based, hopefully, on robust evidence. However, they also go in with a maximum price for their business model to get that through an SMC process; I have no doubt that they consider making their original submission at a range of prices. In partnership working, it is reasonable, if there is a pause in the process, to talk to patients and clinicians about what added value the drugs have that is not being reflected by the traditional quality-adjusted life year process—in other words, to get more yeses. That is also an appropriate time for pharmaceutical companies to have a similar discussion with the relevant individuals within the sector to see whether they can reconsider their reimbursement rate—apparently we are not allowed to say "price".

I think that that is a reasonable position to set out, as a parliamentary representative who is proud of the work that this committee and the Government have done. Do others around the table, not just from the ABPI, feel that getting more yeses is not just about talking more to clinicians and patients but is also about members of the ABPI, in partnership, seeing what more they can do to get more yeses? If so, do you think that a pause in the process would be the ideal time to make that happen?

10:15

Dr Macdonald: I think that the process, as described, gives people that opportunity. The PACE will run and the company can reconsider whether to put in place a patient access scheme, or to revise it, if there is a scheme already.

All that I was saying earlier is that I do not think that it makes sense to have one and then the other; I think that they should be seen as two separate events that run in parallel. We do not want either party to play one against the other. The opportunity is there, but they should run in parallel.

Leigh Smith: I agree completely with the points that Gil Paterson and Bob Doris have made. I think that there would be an advantage in the drug companies negotiating at that stage before the full PACE is commissioned, because we are talking about the time of clinicians, and we cannot have everything. We cannot have patients being seen and treated in clinics if we also have clinicians serving on committees elsewhere. As far as possible, we should try to come to an agreement with the pharmaceutical industry about how much can be afforded for reimbursement in relation to the new product. If it is possible to bring that in at a point when it would be beneficial to the clinicians in terms of their time—their time is precious, in relation to patients and other matters—it would be important to try to do that.

Professor Walters: The process that has been set out recognises the value of enriching the dialogue and allowing a proper holistic appraisal of a new medicine by drawing on the views of the clinicians and patients, and of allowing a dialogue with industry, which, previously, has not happened quite so readily. The temporal sequencing of that dialogue is slightly more difficult. As a clinician, I agree with the point that was expressed earlier about wanting to have all the information on the table in order that we can make a timely decision. However, from my reading of the report as it stands, I think that we will have the opportunity to do that, with two processes running in parallel. I am not sure that we need to mandate a temporal sequence of meetings as the process plays out.

Eric Low: This is a very good discussion that is drilling down to the detail. My view is that the current SMC systems should be exhausted prior to a PACE meeting. I think that there should be a discussion between the SMC and the company around cost effectiveness prior to the PACE meeting; that would give clinicians and patients the opportunity to discuss the broader benefits and some of the issues and challenges around patient access schemes.

It is important to distinguish between the type of patient and clinician input that will take place at the PACE stage and that which will take place at the appraisal stage. If we are going to make this work in a world-class way, we want the appraisal system to work better, and we want to ensure that there is an appropriate amount of clinical and patient expert input into the process.

We should think about the PACE process as being useful only as and when things are not successful under the current system, otherwise there will be a doubling-up of processes. We would need to be clear about what additional input patients and clinicians would give in a PACE environment, compared with what would be the case in the normal appraisal system. That detail needs to be worked out.

It would make sense to me that the actual appraisal system should allow for the appropriate level of clinical and patient expert input. If there is a minded no, that is the opportunity for the SMC to sit down with the industry and discuss a solution to address uncertainty and cost effectiveness. Should that fail, and there will otherwise be a no, the process should default to a deeper, more solution-oriented discussion with PACE. That strikes me as the most sensible thing to do.

Emlyn Samuel: I agree. Given the potential extension of time for the process, there should be a clear need for the additional evidence. The patient and clinical involvement should happen throughout the process, not just at that stage.

Myles Fitt: The issue of where we position the additional PAS was debated at the task and finish group. There are pros and cons for both options, and I take on board the points that others have made on the matter. The key is that there is a very welcome second opportunity; before, there was just the one opportunity for the PAS. The second opportunity will provide an added chance for medicines to get approved, and indeed for medicines to come down in cost.

I agree with Mr Doris in that it should not just be the patients and clinicians making the decisions; we need to find a way to get the pharma industry to come down in price. The key for me, as I said, is the fact that there is an additional step. I am relaxed about where it is in the system.

The Convener: Does that take us to the health board level? Most of us agree that there should be a principle of having a second go. Perhaps there is a concern, however, that that could become a default position. I will address that point later in relation to the existing situation and the individual patient treatment request system—and that is despite the correspondence from the chief medical officer. I will come back to you to get some views regarding the letter to the committee from the Beatson clinicians.

Is there a concern about the second opportunity becoming a default position? How do the boards play into the process in that regard? We have established that there is political will and that the Government is on board. Given the SMC's review, things are changing, and there is a potential for further change. Are we sure that that will follow right through the system, and that we will get the changes that we need in the health boards? Should we pick up on that, rather than just worry about the budget?

Dr Macdonald: You raise a key point regarding the health boards. Nobody wants a situation in which, although there are more yeses from the SMC, the health boards have difficulty putting them into play. Clearly, the funding has to come on to the table.

It is worth mentioning the pharmaceutical price regulation scheme. At the moment, if the health boards do not have extra funds, presumably they have problems either in accepting the medicines or in finding the funds from elsewhere to pay for them. The money has to come from somewhere.

Opportunely, the PPRS has just been re-signed and started from January this year. The pharmaceutical pricing system is relevant for the whole of the UK. Within that, the Government and the industry have agreed that they will fix the branded medicines drug budget for the next five years. It is fixed at a 0 per cent increase for the first two years, and at just under 2 per cent for the next three years. That implies that if the branded medicines budget goes above that baseline, which was set at the end of last year, industry will pay back those funds.

There is an opportunity to use those funds. First, it has to be agreed with the Government that the funds come back to Scotland on the basis of usage. Then, the funds have to find their way back down to the health boards, so that the boards can fund the medicines that they have accepted through the system. Otherwise, there is no balance. That is a key part of it—otherwise, there might be all these yeses, but the health boards will not be able to fund the medicines.

It is important to note that the system is based on a quarterly assessment of spend—every quarter, the money will come back. That should help with the obvious cash flow aspect.

Alastair Kent: I return to the point about patient access schemes. It is essential that we are clear about the role that patients could and should play in such schemes. We must not make patients feel that they are being made to plead with industry for a lower price in order to get a drug that will work for them. We need to be careful to ensure that, while patients can contribute with regard to the value of a drug to their lives, they do not have an input on the price. It is not appropriate for patients to be involved in those commercial decisions.

The Convener: I want to put on the table the individual patient treatment request system, as it is now. Members have expressed concerns in committee and in the chamber debate that people who are already in the system should be considered while the transition is taking place, bearing in mind that it will take some time to get the new system right. That is important.

The committee received a submission yesterday from the Beatson west of Scotland cancer centre consultant committee that addresses the issue. The clinicians say that, despite the fact that the CMO wrote to NHS Greater Glasgow and Clyde to say that

"the concept of exceptionality should not be a factor in any"

current

"IPTR under consideration",

the system is still very "problematic." They contend that, for NHS Greater Glasgow and Clyde, it is "business as usual". Is that view reflected among the patient groups that are represented here today?

Joan Fletcher: We have had difficulties with the same health board. We welcomed the transparency and the introduction of the same processes in all the boards, but we have had difficulties with NHS Greater Glasgow and Clyde with regard to the process that is in place at present before the changes take place.

One issue concerns the question of which physician can submit an IPTR. That system has changed from when we first submitted our petitions to the situation that the board says is in place now. At present, our group has a patient who is stuck in the middle needing someone to apply for an IPTR until the system changes in 2014.

We would like to know whether there is any clarity on who can submit an IPTR, and whether the system will apply throughout all the 14 health boards or whether each board will have its own criteria on who can submit a request.

The Convener: I assume that there is a bit of a communication breakdown. The minister made it clear in the debate and at committee that the issue should be addressed, so it is concerning to receive a letter from the Beatson clinicians that suggests that the process is still problematic. Perhaps the committee can make representations to get some clarity on that. Has anyone else from a patient group experienced that issue?

If no one wants to come in, we will move on.

Bob Doris: I declare a slight interest in the topic. Without going into details, I think that I am involved in the particular case to which Ms Fletcher referred, and representations are on-going. I am sure that we would both be happy to share the information with the committee as appropriate, but, given the confidentiality of individual constituents, we cannot give it in a public forum. However, I did not want to sit quietly during this particular discussion and not put that on the record.

Joan Fletcher: We understand that the funding is available for the patient to receive treatment.

The Convener: That issue is pretty important to us, given that we are struggling to go on working with the Government to try to bring about a better system, with wider support for that objective, and given that people are stuck in the system for quite a long time and missing out on treatment.

Joan Fletcher: An important point concerns the timing of medication, because of the progression of the disease. Many of our patients cannot wait for a long time for these medicines and the treatment that is required, and I am sure that that is the same for patients who are represented by the groups at the table today.

10:30

Dennis Robertson: How did the group come to the calculation that 1,500 people would benefit from the decisions? Perhaps an explanation of cost effectiveness is required, because I am not terribly sure whether we are looking at best value or added value, or whether we are looking at decoupling added value from best value. Could we have some clarity on those points?

The Convener: We may have a greater opportunity with the next panel to address specific points about the numbers involved, the associated costs and how those numbers were projected. I do not think that there is anyone on this panel who can address that point at this stage.

We note from the written submissions that there has been general support for the proposals, but we have an opportunity to discuss that this morning.

Bob Doris: I have a procedural point, convener. We have come together as a committee to address the situation. I did not see anyone else raising their hands, but if there is any advice that the witnesses can give us, we will decide how best to scrutinise it and follow through how the proposals are implemented. How long do the witnesses think it would take to start to see a significant amount—let us not set a specific target—of new yeses coming through the system? What timescale is appropriate and how quickly should we move to scrutinise the success or otherwise of the new system?

Eric Low: From a patient perspective, the sooner the better, but it is important that we allow time to get the details right from the outset. Our ability to do that will be proportionate to the SMC getting the resources that it needs, because people need to be recruited to do that stuff. It is a question of getting the resources in place to make it possible, ensuring that we discuss the detail and getting the system as robust and as tested as it can be so that patients genuinely benefit. We need to do that as soon as possible, because there are patients out there who may die in the next six months because we do not have the system in place.

Professor Walters: That is an excellent point. Time is crucial. As we have discussed today, there are some broad exceptions, but it is a welcome initiative. There is a lot of detail still to be worked through and that will take time and effort, mainly on the part of the SMC secretariat. The crucial question, to my mind, is about the largest threat that underpins the whole endeavour, which concerns the provision of adequate funding to ensure that the system runs smoothly.

Emlyn Samuel: As well as the timescale, it is also extremely important that the changes are communicated effectively to patients and that they understand the changes and how the new process will work, so as well as the changes to make it speedy, it is also vital to have public understanding about how it is going to work.

Myles Fitt: There was a degree of intent to prioritise consideration of any resubmissions of medicines that were rejected in the past year or two, and we would like to think that the system would focus on inviting resubmissions as quickly as possible, so that they can be assessed.

Aileen McLeod: Some of the points that I wanted to raise have already been addressed, but I wanted to pick up on a comment that was made by Lesley Loeliger about the need for patient and clinician groups to have financial support to help them get to the required standing. I would like to ask the witnesses what kind of training and support they think would be necessary. Obviously, we have increased opportunities for the patient and clinician groups to engage with the process. Given that some of the groups are quite small, what other training and resources will those groups need to ensure that they are engaging and participating effectively with the process?

Ian Mackersie: To deal with that last point, one of the things that were not mentioned in the SMC report was expert centres for ultra-orphan diseases. In our experience, those are essential for effective treatment, as they concentrate

expertise and make it available to clinicians throughout the country. Examples that we have seen use encrypted communications so that orphan disease experts can offer advice, consultations and even diagnoses anywhere in the country. Where there might be a lack of clinical expertise in Scotland or it is uneconomic to set up a centre for a handful of dispersed patients, and a centre already exists in England, we understand that reciprocal funding arrangements exist in those circumstances. We think that the creation of expert centres for ultra-orphan diseases is essential.

On training, when we went through the process in England, we got terrific support from the NHS, which gave us access to a couple of consultants, who helped us to put together the patient voice. We found that particularly useful in making the case that we had to make in the three evaluations that we have been through. If there is one way that patient interest groups can be helped, it is along those lines. In some cases, those groups will certainly need a significant amount of help.

Alastair Kent: In the English system, before the advisory group for national specialised services—AGNSS—was abolished, the national specialised commissioning team made arrangements for patient groups that wanted support to be supported by an independent advocate who was familiar with the system, who understood the criteria on which decisions were made and who could give advice on how to put together a submission for patient groups that lacked that capacity. That independent expert help is incredibly valuable for patient groups, which might otherwise struggle to understand how the committee mind works, as it were.

Lesley Loeliger: That covers the patient aspect of the question. To return to the concept of an expert clinician being involved, that comes back to having the correct expert clinician. I know that I always bring the discussion back to my condition, but that is the one that I have experience of, and we have a fantastic centre of excellence. It is important to have the right expert who knows all the cost offsets, all the things that patients have to go through and the costs that would be involved if they did not get on a drug, and who has a real understanding of exactly who the drug would work for. That person has all the information already, so the training would come down to having an understanding of the documentation, but if there is the right expert person, they will have the detail that is needed to make the difference.

Dr Simpson: I am still slightly concerned about the relationship between the national approval system and the health boards. I wonder whether the witnesses feel that we have sorted that. Previously, there were delays in individual health boards giving local approval to what had been approved nationally, but have we changed that sufficiently? There are different circumstances for different drugs. Obviously, for the generality of conditions, if a national expert centre is not required, local approval depends on local clinicians who are engaged in the clinical process. However, if there is a national centre, as with PNH, why do we need the individual health boards to approve the drug? We could end up going back to a situation in which one board approves a medicine immediately while another board takes a few months, which could be significant for the individual patient. We have one expert centre, so the same clinician will recommend that a patient is suitable for treatment, but whether they get that treatment depends on which board they go to. Have we got that right?

The Convener: Does anyone want to take that question?

Dr Simpson: Does silence mean, "Yes, we have," or, "No, we haven't"?

The Convener: Mr Low will take the question.

Eric Low: I am not sure that it is fixed. The issue is partly that SMC guidance is not mandatory in Scotland, so nobody is obliged to implement it. The best-case scenario is that, as soon as SMC guidance is available, a drug should go on to the formulary of every health board in Scotland, and we should leave it up to clinicians to decide when and how they want to prescribe it in clinical practice. We cannot mandate that doctors must do what SMC guidance says; we need to get the medicines on to the formulary as quickly as possible and leave it up to clinical judgment—with the funding in place—to make them available.

The Convener: Harking back to the letter from the Beatson clinicians, how do we create a situation in which there is not a stand-off? Despite the intervention of the CMO, and the expectation that access should be easier, the clinicians say that the system is still "problematic" and that they cannot get access. How do we avoid that? That is a question for the next panel, too. It is essentially the whole point of the inquiry.

Lesley Loeliger: There is one single Scottish expert on my condition, so my hope for the system has always been exactly what the convener has described, which is that one decision would be made for the whole country. That would be the case for ultra-orphan conditions; I know that for other conditions there will potentially be experts dotted around. I have sat in my patient group with two patients: one who was on her knees and another who was racing back to work. The second patient asked the first patient, "Why are you well and I'm not?" and he said, "I'm not in your health board." That was it. My hope was that we would get a single decision for the whole country.

Eric Low: It is important to understand that the IPTR process is used typically when the SMC has said no. As we have discussed at the committee before, the system is not fit for purpose and is not the right way to adjudicate in such situations. The idea that we should demonstrate exceptionality in such clinical situations is prehistoric—it is not good. The system needs to change in the future, even when we get an answer of no from the SMC.

Dr Simpson asked whether the funding will be available to health boards. It should be, because the treatments are coming to market anyway, independently of whether the SMC guidance is in place or not. The SMC carries out a robust appraisal to ensure that those drugs are clinically effective and cost effective, and that they represent a good use of NHS resources. If we take that element away, the drugs are still coming, and health boards need to budget for them anyway, so we need to ensure that the funding is in place. It should be in place anyway so that we get fair and equal access to the best possible treatments for patients, as driven by a discussion between the clinician and the patient about the best option.

We need to join the dots. The funding needs to be in place; we need a robust system to approve the medicines; they need to be put on the formulary as quickly as possible; and we then need to leave it to the clinicians and the patients to ensure that the drugs are made available to the right patients at the right time in the right way.

Professor Walters: I do not think that you would find a clinician in this country who would disagree with what Eric Low has just said. That brings us back to the original point: the timeframe is crucial, particularly for some of these conditions, and we need to streamline the process from the yes from the SMC through to the pharmacist handing the tablets to the patient. The sooner we can minimise—in whatever way—the time that it takes to do that, the better.

10:45

Joan Fletcher: I want to go back to the issue that Lesley Loeliger raised about who the clinical experts will be. We recognise that there are not many clinical experts in Scotland with particular knowledge of the disease that I deal with. You asked about funding. Although we would welcome having the experts up in Scotland, because it would save patients who find it difficult to travel from travelling down to England, we feel that there needs to be some funding and some kind of bridge in-between to get the experts in Scotland, perhaps with help from clinicians from other countries in the UK. That is what happened with the centre of excellence that has now been achieved in Scotland for the condition that Lesley Loeliger has. Clinicians from outside came in to educate willing clinicians in Scotland up to a standard whereby they have become the experts.

We welcome the fact that the SMC task and finish group said that the IPTR system is not fit for purpose and that the process will change, but we question who the experts involved in the new process will be.

Dr Macdonald: I have a related point, which builds on the previous comments, about how the system is working in health boards. A comment that we hear from a lot of companies—I think that it was made in some of the submissions for today's meeting—is that they do not know what the process for the rare medicines fund is or how to access that money and that they are unclear about what the guidance is. I do not think that guidance about who to submit what to exists, so the process is inefficient when agencies, physicians or patient groups try to access that fund.

Alastair Kent: It seems to me that the nub of the problem is who is making decisions. We are talking about the national health service, whose founding purpose was to respond to patient needs. In this situation, there is potentially an effective drug that, in the opinion of an expert clinician, will benefit the patient and has been approved by the SMC, but the accountants are saying no. That is the wrong profession to be making the decision.

Dr Simpson: It seems to me that it is a case of different horses for different courses. If the condition is rare or ultra rare—or if there is only one centre or maybe two centres—it is inappropriate to have 14 health boards make the decision. As Eric Low said, in those circumstances the SMC's decision should be immediately followed by the drug going on the formulary. For conditions that are treated in, for example, the three cancer centres or by individual health boards, it may be that the individual health board has to work out the clinical pathway. It seems to me that the process needs to be refined.

I remain concerned about two things. First, I think that the IPTR system is not fit for purpose and that it should be a national system rather than a local one, particularly for conditions that are rarer and where there is not the expertise.

My other concern is a more global one. Although, as we have heard this morning, the review of access to medicines has been an excellent process and one which takes us to a much better place, the committee and the Government need to recognise that, when it comes to medicines, the pace of change will become even more rapid. We are entering an era of personalised medicine and genetic medicine, which will create far greater strains than we have seen to date—heaven knows, we have had

difficulty coping with those. I put on record the caveat that the process has not ended. The committee will have to continue to follow the matter up and we must recognise that the pace of change will increase, so it will be very difficult to match the increasing demands for expenditure in this area.

Richard Lyle: Most of the questions have been asked and I thought that I would not need to come in, but the point that Dr Richard Simpson has made is quite relevant and it leads on to the question of resubmissions. The T and F report noted that, over the past three years, there have been around 60 "not recommended" medicines or indications that may fit the definitions of end-of-life, orphan or ultra-orphan medicines and therefore be appropriate for review under the new process. Does the panel believe, both from a pharmaceutical and from a patient point of view, that the industry is likely to engage with the new process by presenting resubmissions for end-of-life, orphan or ultra-orphan medicines previously not recommended for use by the SMC?

Myles Fitt: Absolutely. I think that the pharma industry would re-engage and resubmit. If a medicine has been rejected in the past year or two and the parameters have changed, making it more likely that the medicine would be approved, I would expect and encourage companies to resubmit. I have in mind a couple of breast cancer drugs from last year, so the answer to that question is yes.

Lesley Loeliger: I am not quite so sure. Drug companies have been hesitant about submitting or resubmitting in the past because of the costs and the financial model and because they expected to get a "not recommended" decision. Things have changed. My request would be that the SMC communicates clearly to the drug companies, with details, how the new system will operate and how they can look at the overall impact of the drug, rather than concentrating so heavily on the costs. If that is well communicated, I have high hopes.

Dr Macdonald: I cannot comment for every company, but I would expect there to be some resubmissions. Many companies are looking at the devil in the detail once again, to see exactly how the system is going to work, and it will be an easier decision for some than for others. However, so long as we continue to work together with the SMC to get the processes right and to communicate them, the companies will have a little bit more certainty about what they are stepping into. A huge amount of effort has clearly gone into revising the system so that there can be more yeses, so I am sure that there will be some resubmissions, but every company has to make the decision based on what it is offering to the NHS and how it sees the detail working.

The Convener: If there are areas that have not been covered, now is the opportunity for witnesses to place on record any final points that they wish to make.

Dr Macdonald: I know that I have said it before, but I would like to reiterate that the availability of funding at health boards, so that they can pay for the drugs, and the chance to use something like the PPRS payback can provide an opportunity that was not originally in the design but which happens to be available at the same time as the SMC changes. That is why getting that money to the health boards is important.

The Convener: I thank all the witnesses for their attendance, participation and evidence throughout the process, and for all the help that they have given in the inquiry. We look forward to working with you in future to evaluate, at an appropriate time, the outcomes of the new proposals.

10:53

Meeting suspended.

11:00

On resuming—

The Convener: We continue with agenda item 1, which is on access to new medicines. Our second evidence session this morning is with the Scottish Medicines Consortium. I welcome to the committee: Professor David Webb, chair of the task and finish group; Professor Angela Timoney, chair; Professor Jonathan Fox, chair-elect; and Anne Lee, chief pharmaceutical adviser.

Do we have an introductory statement from Professor Fox? I am sorry—it is Professor Webb. That is what I get for reading from my brief today.

Professor David Webb (Scottish Medicines Consortium): I thought that, as chair of the task and finish group, the opening statement might fall to me.

I know that the committee has heard a little from the previous group of witnesses, but I thought that it might be worth making a few background remarks and running through our process and the thinking behind the report. I am grateful to the committee for inviting us to speak today.

First, it is important to remember that, through SMC, Scotland has one of the fastest health technology assessment systems in the world, and that SMC gets cost-effective drugs for common conditions to patients sooner than almost anywhere else in the world. There is a lot of interest overseas in what SMC does, and many people come to Scotland to try to understand the

system so as to implement it elsewhere. We have a world-class system.

Unlike NICE, SMC deals with all new drugs, all new indications for existing drugs and all new formulations, so the process is comprehensive. That is possible only because of the submissions that are made by the pharmaceutical industry; the process works in association with pharma, and it is the industry's efforts that help to make SMC cost effective.

Our proposals are based on an evolving process that would add value to the existing system and improve access while not interfering with the majority of the business of reviewing drugs for common—or more common—conditions.

The task and finish group included all relevant stakeholders such as clinicians, patient representatives and three ABPI representatives. The group was diverse, and I knew that everyone would come with their own opinions, which were not all consistent. We had a tight deadline of 20 December from the cabinet secretary to deliver our report, and I was delighted that so many people were willing to commit to three meetings in October, November and December. We had about 35 people at each meeting, and almost everyone came to two meetings—nearly all of the group members came to all three, in fact.

All the parties in the meetings fully recognised the Scottish Government and cross-party will to improve access to medicines for the end of life and for rare diseases. It was interesting that, although I was rather worried about the risk of entrenched views and people walking out of the meetings, the process was—as the committee heard earlier—a constructive and open discussion.

We started with a relatively blank sheet and a range of options. We explored, eliminated and added in new options, and we had a very open discussion that led to the report that was sent to the cabinet secretary on 20 December.

We dealt with the recommendations in two parts. I will start with the major part, which was on end-of-life and rare conditions—which, as the committee has heard, we are calling orphan conditions. Those conditions affect one in 2,000 people, and approximately 2,500 in Scotland.

When we considered what we had seen at SMC in the past two years, it was clear that most of the rare conditions were also end-of-life conditions; many of them were cancers. We felt that we could deal with end-of-life and rare conditions together under one review process. We used as our definition of "end-of-life condition" the expectation that death would result from that condition within three years. That is perhaps slightly broader than one of the definitions that was used by NICE in a previous incarnation, but we thought that our definition was fair and inclusive.

We started by looking at a QALY—quality-adjusted life years—weighting. The upper limit that NICE and SMC accept is usually around £20,000 per QALY. We looked at multiples: we tried a twofold weighting first, taking the amount up to £40,000, and then to £60,000 and £80,000. We had to get to about £80,000 for most drugs before we could include them as a yes. By the time we had played around with those figures, it felt terribly arbitrary and not an appropriate way to proceed, so we eliminated that element and decided instead to use the existing modifiers alongside an entirely new process called patient and clinician engagement, or PACE.

We have always used experts to tell us about the conditions, but we have not asked their views on the new drugs. The PACE process will get them in the room talking with patients and SMC members about the value that the new drug will bring to that group of patients, which is very different from the current process.

In establishing a PACE meeting, which would be done at the wish of the submitting company, we would seek greater clarity on the potential role of a medicine with regard to making a case for acceptance. We would look at treatment criteria that are relevant to current therapies; at which patients might be expected to benefit most from the medicine; and at continuation rules and for how long the medicine will be tried. We would also try to get further outcomes—we would like there to be a process for looking at long-term outcomes for those new drugs.

A key output from the PACE meeting would be that there would be more clarity on the views of patients and clinicians, as the current QALY measurement does not capture that value. We would be interested in disease severity, the level of unmet need and the impact on carers. The assessment would be much broader than the process that has previously been undertaken. Although it will not be clear what will happen until the system is in place, implicitly we very much expect that the system will substantially increase access to the new medicines.

We think that the use of the PACE process will substantially reduce the need for what were IPTRs and will be the PACS—peer-approved clinical system—because the IPTRs are there for clinicians and patients who feel that they are unable to use a drug that both think is of value. The patients and the physicians will be involved in the process under the PACE system, so we would hope that there would be less need for an IPTR-type process to follow on from that.

We would be able to use the existing IPTR system to see which drugs that the SMC has not recommended in the past are most heavily in demand among the Scottish population so that we can prioritise consideration of certain resubmissions if a very large number come in early on.

The second part of our report relates to very rare conditions. Those are the ultra-orphan conditions that affect less than one in 50,000 people, which equates to 100 patients or fewer in Scotland—so they really are very rare conditions. In such cases, smaller studies with simpler data are usually used.

We examined a range of options, and we would still like to see a cost per QALY, but we did not think that that should be the focus of our discussion. We wanted a framework of explicit criteria that would help us to come to a decision. We would look at the nature of the condition; the impact of the medicine and the technology beyond the direct health benefits; the strength of the case; and the value for money. We would also plan, where required, that we would invoke a PACE if the company would like us to do so. We would still have the cost per QALY, but it would not be a key driver.

I will finally mention implementation. I know from having been involved in the SMC's work in the past that it is an extremely busy organisation. It has done a lot of out-of-hours work in the past three to four months to build up the process with no new funding. It is crucial that it is able to employ the new staff that are necessary to do the much greater body of work that will come through.

The SMC has promised that, if funding is available, it will start the process within two months, which means that drugs would come through the process over the following four or five months. Decisions would therefore come out in the second half of 2014. However, that is contingent on the new budget.

There will be a cost to the NHS in Scotland. There was an estimate of the cost in the first year that was based on drugs that we can see coming forward that would fit the bill and to which we would say yes. The figure was around £70 million, which would allow an estimated 1,500 additional patients to receive treatment. If the resubmissions that failed were brought into the mix and were successful, I think that there would be another 1,500 or so patients, with a pretty substantial additional cost that might be of the same order as the £70 million, but I am not sure.

In summary, the SMC and I and my colleagues on the task and finish group all believe that the changes will deliver substantially improved access to medicines at the end of life and for rare conditions. The changes are intended to be a bridge eventually—I hope—to a value-based approach, which is the Scottish model of value that has been discussed in the past.

I have no additional comments as preamble. I do not know whether Angela Timoney might want to add anything.

Professor Angela Timoney (Scottish Medicines Consortium): I will speak briefly, because I think that this is an opportunity for the Health and Sport Committee to speak to the SMC.

I was heartened by some of the positive comments made in the earlier evidence session by the patient groups, who have sat around our table and read our report, and by the clinicians and the pharmaceutical industry—all the stakeholders who we have worked hard to engage. I take this opportunity to thank Professor Webb for the work that he has done on behalf of the SMC.

The Convener: Thank you for that. We will move quickly to questions. Rhoda Grant is first.

Rhoda Grant: Professor Webb talked about costs to the SMC and NHS boards. The evidence that we received in the earlier session was that there were also costs to patient interest groups, who will be very involved in the process. Some felt that they are equipped at the moment to deal with that, but others were concerned that they would need support, training and, indeed, help with the costs involved. Has that been factored into the costs that you mentioned in your opening statement?

Professor Webb: I am not sure that that is in the costs. Angela Timoney might want to speak to what support the SMC currently gives to patients, but there is no new money in that area.

Professor Timoney: At the moment, the SMC has a patient and public involvement group. We have recruited some new public involvement officers to support patients and patient interest groups in making submissions. We have worked very hard with them to understand what the issues are for them and to ensure that they can contribute fully to our process.

To some extent, the PACE mechanism does not really require the patient interest groups to understand the SMC decision making—that is not what we need from them. We need to understand what the issues are for them and their condition. We want to hear about that from them, and I think that the mechanism gives them an opportunity to do that without having to understand the system in some detail. We have to reach out to them. However, the resource that has been made available does not have anything for any additional educational requirements that they might have. It

might be something separate for the NHS to think about as a whole.

Rhoda Grant: So you feel that it should fall to the NHS rather than the SMC to support patient interest groups. They will need some help through the process. Obviously, a large interest group can buy in expertise, but a small interest group that is involved in one of the sessions might need an awful lot more support to enable it to contribute and to put across the patient interest.

Professor Timoney: We have been very fortunate because between 50 and 70 per cent of our submissions have come from patient interest groups, so they have been able to contribute to the process. One of the speakers in the earlier evidence session talked about the importance of having a specialist clinician perspective. The PACE mechanism allows the clinician who understands the disease to contribute to the process as well. That gives us a richer sense of the situation, which I think will contribute to our decision making.

11:15

Anne Lee (Scottish Medicines Consortium): Our patient and public involvement group has already been planning for how it is going to communicate the changes to the patient interest groups. Members of our group will be working with the new public involvement staff that Angela Timoney has already mentioned. They will be doing what they can to raise awareness and to encourage submissions. An effort has always been made to help smaller charities and patient interest groups to understand the process. That will certainly continue.

Nanette Milne: I have a couple of questions from the pharmaceutical industry point of view. I have your briefing note, which we received this morning, headed "Progress towards improving access to new medicines" and outlining a timetable. The briefing says:

"Submissions received from companies in May onwards will be able to use the new process".

What is the position with medicines that have already been submitted but that have not, as yet, received a decision from the SMC? Under the old system, some medicines would be likely to receive a no; under the new system, they may well be approved. What is the position there? I am thinking in particular about the disadvantage to patients if decisions are put off for too long.

Professor Webb: There are lots of questions in there.

First, we all need to come away from this meeting clear that we have the support of the Scottish Government and that we are able to get the funding to employ the staff, appoint the people and train them up. That will be an additional burden on the SMC before it is relieved by the additional staff helping with the process.

Although I am sure that the SMC would like to start tomorrow, it is not realistically possible. That is the reason for the May start. We must have consistent processes that will not be challenged; they need to be right before we start. I think that May is a pretty ambitious start date.

It is a difficult situation for applications that are already in the process—we have the old process, and we will have the new process. There might be companies that are waiting to find out when the new process starts and are holding up a submission in order to put it through the new process. I am not aware that that is happening, but it is quite possible.

Companies might go through the process under the old system and feel that they would have been better advantaged by the new system. The SMC has never been a group that is not willing to consider resubmissions, unlike NICE, which offers a one-off shot. The SMC encourages new submissions when new evidence becomes available—and I guess that that applies when the new system is put in place.

Nanette Milne: I understand that the minister has said that he expects resubmissions to be dealt with expeditiously. He indicated £1 million of extra funding to allow that to happen and to cope with the additional requirements that the new process will bring. The report does not refer to that. How do you plan to cope with the demands of the extra workload, plus the likely resubmissions for the 60-odd applications—or however many it is—that were previously declined? Will the promised funding cover that?

Professor Webb: I do not think that that funding has been approved—I do not think that the SMC has seen the funding yet. Angela Timoney should probably answer the question.

Professor Timoney: Nanette Milne is absolutely right. The SMC has been asked to continue with its current work programme. We are still full speed ahead with all the other medicines that we are looking at. I will be at a meeting next week, and we have a lot of medicines to consider.

In addition, we have been asked to develop a new methodology for the particular groups concerned, and we have done that. As you heard earlier, we have a lot of detail to work through in that regard. That methodology will apply to the new medicines coming through.

We have also been asked to consider resubmissions. There is quite a lot of work for the SMC to do, and we need to have the staff and

resources in place in order to do it. However, we are keen to do that work: we want to produce medicines and advice to help the NHS in Scotland.

On resubmissions, our thoughts are that we will work with clinical groups and networks so that they can identify for us what they see as being priorities and what they need guidance on. We will work with the pharmaceutical industry to encourage resubmissions, but if we get lots of resubmissions we will be working with the cancer networks and the area drug and therapeutics committees to establish which applications have been causing them the most problems. It is through that process that we hope to act in a way that meets the needs of patients.

The Convener: We will pick up on that. When you produced your report and made clear that you wanted to do that work, which would take a couple of months to set up and five months to have an impact, did you outline the resources that you believed that you needed to tool up for it? What was your bid to Government?

Professor Timoney: The cabinet secretary made an announcement in October in which he asked us to undertake that work and to complete the rapid review by 20 December. At the same time, he announced the £1 million per annum that would be available for the SMC. We were asked to prepare a business case to describe the work, which we submitted on 20 December, the same day on which we submitted the report.

The Convener: What response have you had from the Government?

Professor Timoney: We are still in discussion with the Government on the receipt of resources. We are making progress on that, and we are meeting the Government tomorrow. However, so far we have not received any money, and we need to get that money to do the work. It takes time to employ staff, as the processes are highly specialised and the positions are highly challenging. In order for us to do the work, we need to be able to employ staff.

Healthcare Improvement Scotland has worked with us, and we have employed people in a number of temporary positions and dealt with some of the additional resources around those, but we need to have the budget in place to recruit staff to permanent positions to do the work.

The Convener: What have the barriers been? What is the problem with regard to how the health department or the Government views your bid?

Professor Timoney: I am not sure that I can answer that in any detail, to be perfectly honest. We put in our bid for a budget, and we set out work covering a period of time, saying, “This is what we think we could do”. We need to recruit staff and to grow the team appropriately. I think that the Government would like everything to be done yesterday, but the resources are not in place. I also think that it is challenging for the Scottish Government, given the resources that it has available.

I am hopeful that we will have a good discussion tomorrow, and I am happy to write to the committee after the meeting and confirm that we have received those resources and that we can proceed.

The Convener: Does the funding of £1 million that was announced reflect accurately the amount of resource that you believe you need to do that job? Did you bid for more than £1 million?

Professor Timoney: It will cost far more than £1 million to do the work, but we are a cost-efficient organisation. We put in a bid for something like £1.1 million. In a sense, we tried to cut our cloth to fit the resources that were available, and we have tried to ensure that the things that we have developed will fit within that envelope.

That has been a challenge for all of us, but we have a responsibility, as an organisation that assesses the cost effectiveness of medicines, to be cost effective in our work as well. Nonetheless, the work will be challenging, and we should not underestimate it. The SMC has been asked to undertake an enormous piece of work.

The Convener: What is your estimated price tag for being able to do the job proficiently, quickly and properly?

Professor Timoney: We have not produced an official estimate for that. My personal estimate was that the price would be substantially more than £1 million, but we can live with the resources that we have been promised, if the £1 million comes through.

The Convener: Would the estimated cost be above £1.5 million? Would it be £2 million or £3 million? Which figure is closest?

Professor Timoney: It is more like £2 million.

The Convener: Bearing in mind the impact of the proposed work and that the fact that we are dealing with end-of-life conditions and rare conditions that are progressive, is your timeframe for delivery slipping back because you have not been able to confirm the funds that you need to recruit and properly resource the organisation?

Professor Timoney: We do not wish that timeframe to be delayed. If we get the resource as agreed, we will proceed as we have described this morning.

The Convener: But has the timeframe slipped from your initial estimate?

Professor Timoney: It has not slipped yet.

The Convener: It has not slipped yet.

Professor Timoney: Yet.

The Convener: So when is the drop-dead date when the resource issue will start to impact on the work?

Professor Timoney: We said that we needed to know by the end of February.

The Convener: By the end of February. Thank you very much for that.

Dr Simpson: I just want to put on record what Professor Timoney has already suggested. The SMC system is incredibly cost effective in comparison with other systems. Frankly, we have had a bargain for years—at least I believe that we have. I invite Professor Timoney to give us some international comparisons—I want just to get an idea rather than a comparison of the absolute costs.

A lot of other countries follow what we are doing here, including New Zealand and Australia. As we gear up with our new system, is there some potential for us to say that, if they want to follow it, we would like them to make some contribution, instead of simply picking up our results? In other words, although this will be difficult because we want to be transparent, we will not make available to them our background papers and our workings. Is there some way to encourage those other Governments that are piggy-backing on our excellent work to make some contribution to the additional costs?

Professor Timoney: Thank you for recognising the SMC's position in the past, Dr Simpson—I appreciate that. We are a remarkably cost-effective organisation. We make decisions on about 80 medicines per year, and we have been told that the cost that we represent to the NHS comes to about £12,500 per piece of guidance that we issue. For comparison—I add the caveat that these are not official figures—it has been suggested to us that it costs £160,000 per piece of advice for NICE to produce a single technology appraisal and £250,000 per piece of advice for a multiple technology appraisal. That is more than 10 times as much as what the SMC costs the NHS—that is the order of magnitude. I am not suggesting in any way that the SMC should get the same. We believe that we should work effectively.

The proposal on charging outwith the NHS is interesting. I would encourage the Scottish Government to consider that—it could do it if you wished it to. However, we have tended to work with our colleagues across international boundaries to develop things. We have learned from them, and they will learn from us. That all helps the NHS in Scotland to take things forward.

Bob Doris: My colleague Nanette Milne has responded to some of the issues raised by people in the pharmaceutical sector. We heard from Dr Frances Macdonald, on behalf of the ABPI, about the new PPRS that is in place. If my notes are correct, the overall branded medicines bill for the next two years should have increased costs of 0 per cent. That reflects branded medicines coming off patent, although there are still a lot of cost increases within the system. It is not as simple as saying that cost changes will be 0 per cent over the next two years; there will be cost increases in the system. Over the following three years, there will be a 2 per cent increase per annum.

Dr Macdonald asked about what would happen if the ceiling were to be breached because of more yeses in the system—I suppose that is the best way of putting it—and about how the money would find its way back from the UK level to the Scottish level and, ultimately, into health board budgets.

I know from evidence that the SMC previously gave the committee that the discussions to which the SMC was privy were very limited in relation to the whole renegotiation of UK pricing systems. Have there been any discussions at a UK level with you or with the Scottish Government—if you are privy to that information—in relation to what would happen should the 0 per cent ceiling be breached over the next two years? Would money come back to Scotland?

Professor Timoney: That is really not for the SMC to say. I understand that there will be on-going discussions between the Scottish Government and the Department of Health around how that is to happen. I am aware that a Scottish Government working group is considering what happens with the PPRS and what goes down to health board level, but that is really a Scottish Government issue.

Bob Doris: I have two reasons for asking the question. One is that the matter was raised with us during the previous evidence session, and it is therefore appropriate for us to raise it with you now.

The second reason relates to additional costs that will be put on the Scottish NHS. There is the political will to ensure that those are met, but has the SMC done any financial modelling work? Perhaps you could put on record what you think the additional costs might look like. How will they compare with overall drugs budget costs, given that a significant volume of medicines will come off patent and therefore become dramatically cheaper? There is a to-ing and fro-ing as far as costs are concerned. Has the SMC done

modelling work on that, or would that be the Government's responsibility?

Professor Timoney: Someone from the SMC sits on the short-life working group and has been discussing the figures that we have been discussing here as far as the additional costs for the new methodologies are concerned. That would be our contribution.

On the subject of benefits to the NHS and medicines going off patent, you should be aware that the increase in costs in medicines is due to an increase in volume. We have changing demographics and lots of older people, who get more medicines. Therefore, most of the cost is to do with the increase in volume, rather than price changes. I think that the short-life working group will consider some of those issues.

11:30

The Convener: Dennis Robertson asked the first panel about the workings behind the overall figures. Do you want to ask your question again, Dennis?

Dennis Robertson: Thank you, convener. I am trying to understand the formula that was used to calculate the benefit of the new system to patients. The SMC estimated that 1,500 patients would benefit. How did you arrive at that number?

I am also interested in cost efficiency, which is a bit of a generic term. Are we looking at best value or added value? Have you decoupled added value from best value?

Those are the areas on which I would like a bit more detail.

Professor Webb: I can certainly respond to your second question, on cost efficiency. It is a normal arrangement to use the cost per QALY gained as a way of judging a range of technologies. It is easy to do that with drugs; we have a lot of information to help us to generate the cost per QALY.

The economists take a utilitarian approach, which is about trying to get the greatest value for the greatest number. Whenever we spend on a high-cost-per-QALY drug, if the budget does not change we lose out on better-value medicines. The risk is that if we spend on lower-value, high-cost medicines—it is the value that matters here; we have to pay more to get an extra year—we lose out.

Added budget needs to go to the health boards to cover the new drugs. It is the will of the Scottish Government that the group of disadvantaged patients that we have been talking about should gain greater access to medicines. There is no problem with that, but it is not done with the idea of improving overall cost efficiency—it slightly reduces overall cost efficiency.

The estimate of 1,500 patients was based on the SMC's forecasting work on new drugs that are coming through. The SMC has done such work for a number of years and there is a formal report each year. If we look at the forecasted drugs that fit the bill in relation to end-of-life and rare conditions, we can consider how many of them will get a yes, compared with how many would have got a yes in the old system. That is where the 1,500 and £70 million figures came from.

Dennis Robertson: Will the figures go up? The estimate of 1,500 relates to patients who will benefit in the first year of the new system. Will the number increase in the years to come?

Professor Webb: As new drugs keep coming through, the costs will amplify until the drugs come off patent and become cheaper. The long-term advantage of approving and using drugs is that they become cheaper, eventually. However, you are right: the costs will go up, and they will go up further if there are resubmissions to the SMC. As we heard from the ABPI representative, that is likely. However, those costs will probably be spread over a longer period.

Dr Simpson: You said that 1,500 is the forecast in relation to new medicines coming through in the new system. However, did I hear you say that it is possible that resubmissions would lead to a further 1,500 patients benefiting? That would mean a total cost to health boards of £140 million—£70 million for each group. That is a very big sum.

Professor Webb: Angela Timoney is reminding me that we thought that the cost associated with resubmissions could be £50 million.

Dr Simpson: So we are talking about £120 million, which is roughly 10 per cent of the current drugs budget. That means that boards need additional funding of 10 per cent, which will be a real challenge.

The Convener: Since we met the SMC and the Government in private, have there been detailed discussions with the Scottish Government about the thinking behind the proposals and the funding that the NHS will need?

Anne Lee: A member of our team is contributing to the work of the Government's short-life working group, which Angela Timoney mentioned. We have been asked to look in a bit more detail at the figures in the report that Professor Webb mentioned.

We know that there is uncertainty around the estimates—they are estimates. Some medicines might not come to market. We are making predictions about the list price to the service, so our estimates do not take account of patient

access schemes or discounts that might be agreed. The usual caveats around the estimates, which are based on a very uncertain pipeline, need to be factored in. However, we try to help where we can and give further advice on how the figures were derived.

Professor Webb: Because of all those uncertainties, it will be important to come back in a year or 18 months' time and look at how the system has operated. We will need to consider what drugs have come through and at what cost, and explore the utility of what we have done.

The Convener: As far as I know, the Scottish Government and the cabinet secretary welcomed your report. I have not heard about any caveats from the Government. I do not think that it has said, "Well, we welcome the report but we do not welcome this bit and we question that bit." What detailed discussion on the report has taken place between you and your team and the cabinet secretary?

Professor Webb: The only meeting was the one that you and I had with the cabinet secretary.

The Convener: Which was all of 10 minutes.

Professor Webb: Yes.

Bob Doris: It was 10 minutes involving us—

The Convener: Well, yes, but I think that the SMC left shortly after we left.

Professor Webb: Yes. I am not aware of any detailed discussion.

The Convener: There has been no detailed discussion with the Scottish Government on the matter.

This is important, given all the expectations about the Government's commitment and political direction, which were evident in our discussions with our previous panel. The more I hear, the more concerned I become. If there is difficulty in securing £1 million or £2 million to get the work done, what problems are we going to have in getting £120 million out of the system—if there is £120 million to get?

Professor Webb: That is why we were a little anxious about adding new patient support, when the question was asked. It is not that we would not do it if there were money to do so, but it is important to have the money. The anxieties are around that area.

Dr Simpson: The Government is going to have to get a handle on that. The new system might cost £120 million, which is 10 per cent of the drugs budget. We can perhaps take away the rare conditions medicines fund, because that issue should be okay under the new system—maybe—although it might be politically difficult to take away the fund. I presume that the IPTR system will be dropped, so some of the current IPTR funding could be used. The net amount might therefore be less than £120 million. The committee needs to understand where we are going and to support the Government, because the new system will be a real challenge. We need to get some figures clarified.

The Convener: Does the SMC have figures? Professor Webb said that in addition to getting resources for the SMC to deliver the new scheme—those resources are nothing in the scheme of things, it seems—the health service will need to be funded. Have you put a figure on what will need to go into the health service to fund the system for a year or 18 months?

Professor Webb: To put a figure on that would be speculation. The figures in our report are probably as good as we can get without doing some pretty complex work, which would still have lots of caveats around it.

The Convener: As Richard Simpson said, the benefits will reduce some costs—there is a balance. However, there has been no detailed work on that, has there?

Professor Timoney: I think that that is what the Scottish Government short-life working group is trying to do. We will need to see the output from the group. We have talked about the SMC part, but you can see that that is just one little part of it.

The Convener: When is the group due to report?

Professor Timoney: I do not know.

The Convener: We do not know that, either.

Bob Doris: Richard Simpson has made some reasonable points, but I want to split the issue into two parts. We are talking about funding of £1 million to £2 million—we will see what happens in your meeting with the cabinet secretary—for the process aspect, which will involve ensuring that the mechanisms are in place, and you are planning on that basis.

However, we have to separate that element from the national Scottish Government budget for health boards and how that resource flows, because they are two separate things. The political commitment exists, and in-year budget amendments are made in Parliament as a matter of course. We will need to see at a later date how the short-life working group's conclusions will impact on budgets and subsequent budget amendments, but the political will exists.

None of that concerns me. What is important is the need to ensure that the process that will drive the funding liabilities—or opportunities, depending on how we look at them—is not compromised.

That is the key aspect of the committee's scrutiny, at present.

We have to assume that the money is there, and the committee will scrutinise the matter because the political commitment has been made. There has been a budget and there are always in-year budget amendments, and there is a short-life working group looking specifically at the matter, so we have to assume that its conclusions will be identified in NHS budgets.

To return to the funding of £1 million to £2 million, you said that your meeting with the cabinet secretary is taking place tomorrow. Are we on course to deliver more approvals—I am not talking about funding for individual drugs—as has been outlined in your report and accepted by the Scottish Government? Has that aim been compromised or are we on target to achieve it?

Professor Timoney: I said earlier, and I will say again, that if we get the resources by the end of this month we will proceed according to the time limits that we have described.

Bob Doris: Thank you. That is helpful.

Dr Simpson: That is great.

Professor Jonathan Fox (Scottish Medicines Consortium): For accuracy, I just want to say—although I will not be at the meeting tomorrow because I am doing a clinic—that we are meeting the Scottish Government rather than the cabinet secretary tomorrow.

Rhoda Grant: I have a supplementary on Bob Doris's point. I understand what he is saying about your process, but—to go back to the £140 million—if the process works according to the timescale and you start giving more approvals in two months' time, when will the cost start to impact on NHS board budgets?

Professor Timoney: According to the timeline that we have described, it will probably be September or October—probably October—before some new medicines come through the system. The impact will start to hit boards in quarter 3.

Professor Webb: The impact will undoubtedly be incremental, however, because a few drugs would be coming through each month. The amount will build up over a year.

Rhoda Grant: You say that the amount will build up, and that the cost will be £70 million or £140 million, depending on how many drugs come through. Will that be the future annual cost, once the backlog has been cleared, or is that the amount at which you think the cost will peak?

Professor Webb: That is the amount that we predict the cost will build up to at the end of the first year—or maybe the second year—of full operation.

Rhoda Grant: Will that cost continue year on year, thereafter?

Professor Timoney: Other medicines will be licensed and will come through the process. The cost that we are discussing relates to the medicines that we expect to come through in the first year of the process. Following that, there will be more medicines and different decisions, so the first medicines will be there as a background and there will be new ones on top.

Professor Webb: The matter is complicated, though. Some medicines that we might think we will see on the market in six months will have safety issues and will not get to market, or companies may decide not to submit some medicines to SMC. Some medicines will come through with patient access schemes, and the cost that we had thought was going to be very high may be substantially lower.

Those are all uncertainties, so we cannot give an absolutely straight answer. It is not that we do not want to; we just do not know.

The Convener: Has there been any interaction with, or feedback from, the NHS boards? We have had positive feedback from the people round the table who have engaged in the process. Boards will eventually have to face this problem whether there is funding or not, although we hope that there will be. What has been their reaction to your report?

Professor Webb: The chief executives of the boards were involved in the task and finish group, and they recommended clearly that we put the estimated financials in the report. We were not sure whether we should do that, but they were clear that we should.

The Convener: So, the chief executives had their say: they were part of the recommendations and they insisted that the funding issue be included in the report.

Professor Webb: I believe that the boards would be happy with the outcome if they felt that funding would flow from decisions.

11:45

Nanette Milne: I have a question on predictability and transparency under the new system. There seems to be an acceptance that the old process was lacking in transparency and that there was no clear understanding of how the SMC modifiers worked. The QALY has been ruled out under the new system, and there does not appear to be an indicator like it. How do we ensure transparency for the public and some predictability for the industry? What levels of cost effectiveness would you accept or decline in a submission? Has that been worked out?

Professor Fox: A lot of detail is still to be worked out. We were given the go-ahead for the system only on 31 January, which was three weeks ago. With that caveat, we will try to make the criteria for the inputs into and the outputs from the system as orderly and evidence-based as possible.

We will, once we have the resource, create pro formas that give the type of information and background that Nanette Milne is talking about. The cost per QALY will not be entirely irrelevant, and we will ask companies to submit that cost for the groups concerned. However, we will also look at all sorts of other factors, such as what NICE used to call—until it changed its terminology—the wider societal benefit. That includes benefits to carers, employment issues and all sorts of other things.

You are quite right to say that we need to make the process as explicit, transparent and understandable as possible; we will try to do so. That is probably the best answer that I can give, at present.

Nanette Milne: It is work in progress.

Professor Fox: Yes—absolutely.

Anne Lee: Just to add to that, the new processes around the PACE flow from just one of the recommendations from this committee that we are implementing. In addition, we will be meeting in public from May 2014, which will support the demand for transparency.

The Convener: We have met the SMC at least a couple of times since the report came out, and we appreciate the benefit of those meetings. However, I want to put something on the record that seems to be a bit contradictory: it appears that the QALY is still part of the process but does not really matter. Is that a statement that holds water?

With regard to openness and everything else, we felt from our discussions on the previous process that people want at least to understand the rules that have been applied and how a judgment has been made on access to a medicine. What you say about the QALY seems to be a bit of a contradiction, and I cannot understand it.

Professor Timoney: The QALY does matter.

The Convener: There we go.

Professor Timoney: I will be sitting in the SMC—well, I will not; Jonathan Fox will be sitting there because he is currently the chair-elect—and we will receive the draft recommendation from the NDC, which will be based on a company's submission and will include an assessment of the cost effectiveness of the product. If the SMC is minded to say no at that stage, the company will have the opportunity to submit another pass and give us an improved discount, and the submission will come back to the SMC. We will have a number of pieces of information: the NDC draft recommendation; the feedback from the company, which we currently get and which may correct some of its assumptions or any factual errors or sensitivities that we have asked it to address; a new patient access scheme, potentially; a patient interest group submission; and a view from the PACE group on whether patients and clinicians would really value having that medicine. All those factors, especially the PACE mechanism, will be important deciding factors in the SMC's coming to a judgment at that time about that medicine.

However, we have a responsibility to ensure cost effectiveness and that we listen to patients and stakeholders. That balance and judgment are important. At the moment, the cost per QALY is not the deciding factor for the SMC, but it is a major factor. Now, there are other factors that will help to inform our judgment in a significant way that responds to the needs of patients and clinicians.

The Convener: So, the weighting has changed and it is broader?

Professor Timoney: Yes—there is a broader perspective.

Professor Webb: In the past, we have often said that if it was just about cost per QALY we would leave the economists in the room and they could make the decision for us. It is always about clinical judgment, and there will be more inputs to the clinical judgment in the future.

Dr Simpson: That leads nicely on to my question. For some conditions, there is limited expertise in Scotland, and sometimes even in the United Kingdom. My first question is this: are you comfortable that you will be able to get that expertise, from whatever source?

Secondly, a real conundrum is presented by the growing body of criticism that says that clinicians who have been involved in the research that led to a drug's being approved should not then approve the drug for use. I do not agree with that, particularly in respect of areas in which limited expertise is available, because the people who are experts will be the ones who are involved and their expertise is being confounded by that. I do not know how clearly I am making my point. The matter came up originally in relation to vaccines, when there was an attack on the Joint Committee on Vaccination and Immunisation, almost all of whose members have been involved in research and approvals. Can you find an expert who has not been involved in such work? I do not think that you can, so I would welcome your comments on that.

Professor Fox: On the first point, we are prepared to ask as wide a range of experts as is necessary, and we intend to do that, certainly within the UK. Even for most of the ultra-orphan medicines, we can find experts in the UK, and for some of them we can find experts right here in Scotland. The answer is that we will be casting our net as wide as is necessary.

On the second point, declaration of interests is the general issue. The SMC and the NDC have strict rules so that people with specific personal interests have to leave the room. We declare other levels of interest, too. However, in the task and finish group we will have to relax some of those tight rules, bearing it in mind that the group will not be making the final decision, which will still be made by the SMC under its tight declaration and disclosure policies.

People who are involved in the PACE mechanism will necessarily have conflicts of interests; the patient groups will and many of the experts will too, but that will not bar them from taking part, although their interests will be disclosed publicly.

Dr Simpson: That is helpful.

Bob Doris: I apologise if I am repeating something that was said before, but I had to leave the room. Concerns were raised by the previous panel about the transfer from the IPTR system to the peer-approved clinical system, and the chief medical officer and the cabinet secretary have made it clear that, although there is a hiatus while IPTR continues until the new system is brought in, there should be flexibility.

In my local health board there are various views on whether or not the spirit of that flexibility is being used with IPTRs, and I do not have enough evidence on that, although I hope to learn more about it in relation to the case of an individual constituent. I have already put my interests clearly on the record. How quickly can we get the new system online? A bit of certainty about the process would help my constituent, who falls between two stools at the moment, so any information would be most welcome.

Professor Timoney: The peer-approved clinical system is a Scottish Government system to replace IPTRs. The Government has said that it will issue guidance on the system in May; we look forward to that.

Bob Doris: Will you have any input at all into that Scottish Government guidance?

Professor Timoney: We have not been involved at all—the system is separate.

Bob Doris: There is a gap in my knowledge, which I should not use the evidence session to fill. Who, that you know of, is the Government speaking to about bringing online the new criteria?

Professor Timoney: I honestly cannot answer that question, but I can repeat what Professor Webb said. We expect that, if SMC systems and processes are up and running, some of the medicines that are currently not recommended and which go into the IPTR system will not go into that system, because we will have the PACE mechanism. We hope that we have helped with that part. However, the separate issue that you raise is outwith the SMC's responsibility.

Bob Doris: One thing that is within the SMC's scope is prioritising resubmissions, to which you have referred. If not giving medication to a patient with an ultra-orphan condition could have a degenerative impact on the person—although they are not in an end-of-life situation—would you prioritise that medicine for speedy reconsideration?

Professor Timoney: You have put a strong case to us, which involves the clinical factors that we would consider. We would listen closely to the service about the particular challenges in the system for it that mean that it would like us to put through a medicine as quickly as possible. We would respond to that as well as we could.

The position also depends on the company's making a resubmission; we cannot consider a medicine if the company does not resubmit it. We must encourage companies to make resubmissions and we must listen to the NHS about where there are problems for it in the system. We will try to do that.

Bob Doris: I am just trying to represent my constituent without breaching confidentiality.

Dr Simpson: There are lots of mostly positive differences between NICE and the SMC. A fundamental difference is that once NICE approves the limited drugs that it considers—its range is far more limited than the SMC's—foundation trusts or whoever in England must deliver those drugs and have no choice in the matter, whereas the SMC is an advisory group to the area drug and therapeutics committees.

Do Professor Timoney, or Professor Fox as the incoming chair, feel that there are circumstances in which the SMC should be able to say that a medicine should be introduced immediately and should not be subject to prolonged consideration? I raised the issue with the first panel in relation to drugs for ultra-orphan conditions and end-of-life drugs, for which rapid decisions, following the SMC's decisions, are imperative. Prolonged delay could seriously affect a patient's end-of-life circumstances. Would you like the power to say that drugs in some categories must be introduced, although the clinical pathway might have to be

carefully considered for a period for other drugs, because many clinicians are involved?

Professor Fox: I would like a bit more power, which would be great. As was set out in a letter that was issued last year, health boards are already expected to have the medicine or an equivalent treatment—as we all know, not all the medicines are immediately life saving and many of them have good alternatives. However, the SMC does not have the power to mandate that availability, and we would not immediately seek that.

We see ourselves as advising and facilitating. We allow medicines to get on to formularies—we are permissive—but health boards still have the power that you described. Important medicines are expected to be made available within 90 days. That is where we are.

12:00

Professor Webb: It is probably worth mentioning for those who are interested—the health boards, the area drug and therapeutic committees, and clinicians—that the narrative of an SMC report gives a very clear idea of how important a new medicine is.

Dr Simpson: You feel that that is probably sufficient. The decision by the area drug and therapeutic committees sometimes depends, however, on their definition of equivalence, and there is evidence out there that that is another postcode lottery. For the new anticoagulant drugs, for example, the interpretation of equivalence is such that some patients are being deprived of a new medicine that has a significant advantage. I just wonder whether we have got that right. I am trying to anticipate the next set of problems that the committee will have to face.

Professor Fox: To be frank, I feel that we have enough on our hands at the moment. I do not mean to be evasive. As you might imagine, there are differences of opinion about how strong the advice or instruction from the SMC should be. However, that is a different debate, which we do not have the energy to engage in at the moment, I feel.

Dr Simpson: Okay. Thank you.

Bob Doris: I apologise to our witnesses because I am going to ask a similar question. It is a bit like groundhog day for Dr Simpson and me; we had very similar discussions a few months back.

I would like clarification. Is the expectation that any drug that is approved under the current process, or a future process, and which is about to come online, should be made available on the formulary within three months, but individual clinicians in every health board area have the power to prescribe the drug after an SMC approval, with the issue being whether it is to be prescribed routinely or not? That was the situation that we experienced during our reporting. We have to be careful to ensure that clinical decision making for individual patients is paramount in a drug's prescription. Is the situation as I described it, or have I got it wrong?

Professor Timoney: This is an important matter. As I said, we have 80 pieces of guidance a year, many of which are around medicines for common medical conditions for which there are lots of therapeutic options. Our job is to say which of those products that have come to the market from the pharmaceutical companies are cost effective. It might be that for a particular therapeutic area there are six or seven medicines that are all cost effective. We do that work for the boards in advance and tell them that they may choose some of those medicines to put on their formularies. They will be able to arrange buying policies to secure the medicines even more cost effectively for patients. That is a formulary decision. The ADTCs must do that for their boards in order to secure best value.

Nevertheless, if the SMC has said that a product is cost effective and a clinician really thinks that it has particular benefits for their patient, even if it is non-formulary, there will within boards be a non-formulary request process that the clinician will be able to access. You are therefore right in your understanding, Mr Doris.

However, we need to make it clear that not everything that the SMC approves goes on to a formulary—I would not expect that. If they did, that would crowd our formularies out and inhibit clinicians in some of their decision making. Part of our job is to give them guidance on a limited range of products in order to improve the systematic use of medicines in a health board.

Bob Doris: That is helpful.

The Convener: As there are no other questions, I thank all the witnesses for their attendance and for the work that they have done. I wish you all the best in the future, Professor Timoney. I have no doubt that we will be seeing Professor Fox again.

Agenda item 2 was to be on public petitions. I seek the committee's agreement to defer consideration of the petitions until next week's meeting. Is the committee agreed?

Members *indicated agreement.*

Meeting closed at 12:05.

Members who would like a printed copy of the *Official Report* to be forwarded to them should give notice to SPICe.

Available in e-format only. Printed Scottish Parliament documentation is published in Edinburgh by APS Group Scotland.

All documents are available on
the Scottish Parliament website at:

www.scottish.parliament.uk

For details of documents available to
order in hard copy format, please contact:
APS Scottish Parliament Publications on 0131 629 9941.

For information on the Scottish Parliament contact
Public Information on:

Telephone: 0131 348 5000
Textphone: 0800 092 7100
Email: sp.info@scottish.parliament.uk

e-format first available
ISBN 978-1-78392-822-4

Revised e-format available
ISBN 978-1-78392-836-1

Printed in Scotland by APS Group Scotland