The Scottish Parliament
Pàrlamaid na h-Alba

Official Report

HEALTH AND SPORT COMMITTEE

Tuesday 18 September 2012

Session 4

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HEALTH AND SPORT COMMITTEE
24th Meeting 2012, Session 4

CONVENER

*Duncan McNeil (Greenock and Inverclyde) (Lab)

DEPUTY CONVENER

*Bob Doris (Glasgow) (SNP)

COMMITTEE MEMBERS

*George Adam (Paisley) (SNP)
*Jim Eadie (Edinburgh Southern) (SNP)
*Richard Lyle (Central Scotland) (SNP)
*Nanette Milne (North East Scotland) (Con)
*Gil Paterson (Clydebank and Milngavie) (SNP)
*Dr Richard Simpson (Mid Scotland and Fife) (Lab)
*Drew Smith (Glasgow) (Lab)

*attended

THE FOLLOWING ALSO PARTICIPATED:

Sandra Auld (Association of the British Pharmaceutical Industry)
Melinda Cuthbert (NHS Lothian)
Dr Jonathan Fox (Scottish Medicines Consortium)
Dr Rachel Green (NHS Greater Glasgow and Clyde)
Dr Frances Macdonald (Association of the British Pharmaceutical Industry)
David Pfleger (NHS Grampian)
Andy Powrie-Smith (Association of the British Pharmaceutical Industry)
Professor Angela Timoney (Scottish Medicines Consortium)
Professor David Webb (Royal College of Physicians of Edinburgh)

CLERK TO THE COMMITTEE

Eugene Windsor

LOCATION

Committee Room 1

Scottish Parliament

Health and Sport Committee

Tuesday 18 September 2012

[The Convener *opened the meeting at 09:34*]

Decision on Taking Business in Private

The Convener (Duncan McNeil): Welcome to the 24th meeting in 2012 of the Health and Sport Committee. As usual, I remind everyone present to switch off mobile phones and BlackBerrys, as they often interfere with the sound system.

Agenda item 1 is a decision on taking business in private. I propose that, as discussed, we take in private item 3, which is consideration of the committee's approach to the scrutiny of the Scottish Government's draft budget 2013-14, and item 4, which is consideration of our work programme. Are members agreed?

Members *indicated agreement.*

New Medicines (Access)

09:35

The Convener: Agenda item 2 is a round-table session on access to new medicines. Members will recall that the committee agreed to hold the session to help our understanding of how new medicines are approved for use in the national health service in Scotland and the system of individual patient treatment requests.

Although the issue is clearly a matter of wider public interest, the committee's involvement in it was originally brought about through consideration of three petitions—PE1398, PE1399 and PE1401. Today's session is intended to allow the committee to gain a fuller understanding of the way in which the processes currently work. The committee will discuss its work programme later today and next week. During that discussion, members can and will consider whether the committee should carry out any further work on the topic.

I invite MSPs and witnesses to introduce themselves before Richard Simpson asks the first question.

Richard Lyle (Central Scotland) (SNP): I am an MSP for the Central Scotland region.

David Pfleger (NHS Grampian): I am the director of pharmacy at NHS Grampian.

Jim Eadie (Edinburgh Southern) (SNP): I am the MSP for Edinburgh Southern.

Dr Rachel Green (NHS Greater Glasgow and Clyde): I am from NHS Greater Glasgow and Clyde.

Drew Smith (Glasgow) (Lab): I am a member for Glasgow.

Professor David Webb (Royal College of Physicians of Edinburgh): I am professor of therapeutics at the University of Edinburgh.

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

Dr Jonathan Fox (Scottish Medicines Consortium): I am chair of the Scottish Medicines Consortium new drugs committee and one of the vice-chairs of the SMC.

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

Professor Angela Timoney (Scottish Medicines Consortium): I am chair of the Scottish Medicines Consortium.

The Convener: I am Duncan McNeil, the convener of the committee and the MSP for Greenock and Inverclyde.

Dr Frances Macdonald (Association of the British Pharmaceutical Industry): I am one of the industry representatives on the SMC and chair of the SMC user group forum.

George Adam (Paisley) (SNP): I am Paisley's MSP.

Sandra Auld (Association of the British Pharmaceutical Industry): I am operations director with the Association of the British Pharmaceutical Industry.

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

Andy Powrie-Smith (Association of the British Pharmaceutical Industry): I am a director of the ABPI.

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

Melinda Cuthbert (NHS Lothian): I am the lead pharmacist for Lothian medicines information service and yellow card centre Scotland, which is the centre for adverse reactions to drugs.

The Convener: Because there are so many of us, allowing people to join in the discussion will be a bit of a challenge, but it is the MSPs' clear intent that, if possible, we will have a broad discussion with our witnesses. To that end, we will try our very best to do more listening than talking, although an MSP—Richard Simpson—will have the first word.

Dr Simpson: You were looking directly at me when you said that, convener. Other than to ask the first question, I hope that I do not talk too much, although the topic under discussion interests me considerably.

Since the petitions were submitted to the Public Petitions Committee and action was taken, the Government—through the chief medical officer for Scotland—has introduced new guidelines on how medicines should be treated once they have been approved by the Scottish Medicines Consortium. In today's discussion, we may want to consider every aspect of the process, from a new drug being licensed through to its becoming available to patients, but I want to focus on the area of the process that goes from the SMC to the patient.

In England, as everyone will be aware, once the National Institute for Health and Clinical Excellence makes a specific recommendation primary care trusts are supposed to provide that drug, although I think that the reality is slightly different from what has been instructed. However, technically the drug is supposed to be available and the Government is making a lot of noise about wanting to know why a patient does not get those drugs.

However, in Scotland, the recommendation goes to 14 different area drug and therapeutics committees; not only that, but it might then be referred to a regional working group on the disease for which the drug is licensed. Indeed, I have also learned that Healthcare Improvement Scotland has established a new consensus meeting. All that means that recommendations have to go through multiple levels of bureaucracy before a patient can actually receive the medicine.

The guidance clearly states that a drug should be made available within 90 days, but there are escape clauses and loopholes—for example, if there is no protocol in place, availability can be delayed—and the tables that the ABPI has helpfully made available to us show that the responses from different area drug and therapeutics committees are widely different.

The drug ticagrelor, otherwise known as Brilique, was approved in April 2011 but, a year later, is still not available to our patients. Although the Scottish Medicines Consortium gave general approval to the drug, the regional group in the west has given some sort of approval only for its use in specific aspects of acute coronary syndrome and the group in the east only for its use in other aspects of the syndrome. I would be grateful if Professor Timoney could confirm this, but according to evidence submitted to the SMC the drug would save 200 lives a year. Therefore, one reaches the inevitable conclusion that these delays of more than a year, or indeed 15 months, since the drug was approved, which have resulted from the bureaucratic process, have cost 200 Scots their lives.

I would like the people round the table to comment on whether the process in Scotland, from the SMC recommendation through to delivery of the drug to the patient, is fit for purpose.

The Convener: Dr Simpson has asked a number of questions that we are all interested in about the complexity of the procedure, the layers of process and delays. Given that at least one of those questions was directed at Professor Timoney, I will let her respond first.

Professor Timoney: It is probably important to start at the beginning, which is when a drug gets licensed for use in this country. Most of the time, the licence comes through the European Medicines Agency, which examines the product's quality, safety and efficacy and basically performs a benefit-versus-risk assessment to determine whether the product is sufficiently safe and effective for use in the Scottish population.

However, the regulatory authorities are specifically precluded from considering cost in that

process and someone in the NHS has to determine the additional benefit that we would get from the medicine compared with the medicines in current use and what we would pay for that additional benefit—in other words, whether it is value for money. In effect, that is what the SMC does under health technology assessment. We look at the products to determine whether they are cost effective for NHS Scotland and then expect area drug and therapeutics committees to consider the range of cost-effective products and decide which should be used in their formulary.

Without going into the specific issue of ticagrelor, I point out that, under Scottish Government guidance, that product or its equivalent has to be made available; the fact is that NHS Scotland has other medicines that do the same thing and boards have certainly made those choices from a range of cost-effective products. That shows me that the process is working.

The Convener: Does anyone else wish to comment? Is the process efficient and seamless?

David Pfleger: From the ADTC perspective, I point out that the SMC's initial cost-effectiveness analysis that provides us with advice would, historically, have been carried out by us; the consortium itself was set up to remove such duplication and to bring together and use to best effect the expertise that we have in Scotland. As a result, the way in which boards use ADTCs has changed; now, it is more about considering with local clinicians where a new drug might fit in the pathway of local care, what other drugs are in use and how the new drug on the market fits with the rest of the available choices.

On timing, we moved in April to a 90-day timescale from pre-publication, so there is a 60-day period post-publication. We work hard to meet that and, in the vast majority of cases, we achieve it.

09:45

Sandra Auld: Brilique is one example, but I would like to speak more generally about issues of access to new medicines. As part of the written evidence that we submitted, we included a snapshot of the most up-to-date decisions that we could illustrate since the CMO's guidance came into being. Our evidence demonstrates that there is still marked variance among boards on implementation of the guidance.

The ABPI is not asking for anything new. We are just asking for the CMO's guidance to be implemented in the intended spirit, and for protocols to be put in place within the 90-day period. There will be instances when that does not happen, but we believe that they should be the exception rather than the rule—it might not happen 5 per cent of the time, for example. The system should not be used as a loophole.

Nanette Milne: The ABPI submission states that the "hurdles", as it describes them,

"generally lack transparency"

and

"clear processes".

Will you comment further on that?

Sandra Auld: That comment relates particularly to the consensus meetings. I hand over to my colleague Frances Macdonald, who can say more about that.

Dr Macdonald: We have a view on the consensus meetings that have occurred so far. Although we understand the logic of trying to come to a Scottish view on how a product should be used, we are concerned that it is unclear where the consensus meetings fit. They just seem to represent another hurdle on the pathway, because they do not give clinical guidance—they make clinical statements on use of products, but without a thorough clinical review. It appears that health boards still have to do their own protocol development, so the consensus meetings are more of a hurdle than a help. Our point on the lack of transparency is that the consensus meetings do not appear to undertake thorough clinical reviews, although they make clinical statements.

The Convener: Does anyone want to respond to that?

David Pfleger: I am aware of only one true consensus meeting having taken place—that was on dabigatran—so we should talk in the singular rather than depict consensus meetings as a common state of affairs. Hindsight is always good, but if we look back in 2020, I think that we will see that what we did was absolutely the right thing to do.

The introduction of dabigatran was clinically complex because it involved prioritising patients and working out who would most benefit, and managing the entry in terms of changing clinical practice. There were some concerns about ensuring that we targeted patients who would get the most benefit and would not be harmed by the drug. To some extent, that has been borne out. There are safety concerns around dabigatran, and its use in Scotland has not grown as much as we anticipated. It is one of a group of drugs that are coming. There is natural caution in the adoption of a new treatment—especially in that area—and the consensus statement on dabigatran was useful. I do not see the approach as a step that has been introduced permanently, across the board or for all medicines. A meeting was brought together for

that particular drug, and to that extent it was useful.

Dr Macdonald: That is quite right: there was only one such meeting.

Some of the lack of clarity is about why that specific disease area was chosen—most new products attract the same question about long-term safety and efficacy, because they are assessed in trials that are of limited duration. That is one of the questions that we have. The value of consensus meetings to the health boards is therefore unclear to us as well, because the meeting did not appear to make the decision making faster in terms of adopting or not adopting the product thereafter.

Jim Eadie: I want to clarify a point that Professor Timoney and Mr Pfleger touched on in relation to their respective roles—Professor Timoney's role with the regulatory body, the Scottish Medicines Consortium, which is a consortium of area drug and therapeutic committees, and Mr Pfleger's role with an area drug and therapeutic committee.

Professor Timoney talked about the role of the regulator in assessing safety and efficacy. One issue that I have as a constituency member, having received correspondence from constituents, is that there appears to be evidence that a medicine is not being made available. The area drug and therapeutic committee is not putting it on to their local formulary on the basis that there are safety concerns about that medicine. Do the experts round the table think that that is an appropriate rationale for refusing to place a medicine on the formulary and for not making that medicine available to patients? I understand the rationale that alternative treatments are available, which is often the explanation for not placing on the formulary a medicine that the SMC has approved. However, real clarity is needed around safety and efficacy. If that issue was dealt with by the regulatory body, why is it being used as a reason for not placing medicines on the formulary and for not making medicines available to patients?

David Pfleger: It would be useful to have some specific examples, but our committee would not take an assessment of the safety of the medicine. It would be interested in the safety of using that medicine and perhaps in some of the issues that go with its use, but there is no assessment of safety because it is accepted that that is part of the licensing process. I do not recognise what you describe as a response of the Grampian ADTC in terms of saying why we would not use a medicine.

Jim Eadie: This is meant to be an informal discussion and that is a helpful clarification, but are you saying that in your experience of how the process operates in Grampian, you would not fail to place a medicine on the formulary simply on the ground that there were safety concerns, and that there are no examples of when that has happened? I am thinking about my experience with NHS Lothian, as an MSP.

David Pfleger: In general, I agree that you have given a summary of what I just communicated. To be clear, there are discussions at formulary groups about the benefit versus safety profile. That is different to saying that we would not use a drug because of safety concerns. There might be a drug that gives more benefit and less risk; that drug would be better than a drug that gave less benefit and more risk. Those discussions are legitimate.

However, I would not recognise a situation in which we would say "No, we are not going to use a licensed drug on safety grounds." That is because assessment of safety is one of the prime purposes of the licensing process. If there has been such a situation, I would be interested to know about it.

The Convener: My colleague Jim Eadie referred to his constituents. As a layperson who is representing constituents, I find that they cannot understand the reasons for the various layers—European licensing, NICE, the SMC, then the boards—which become further hurdles. People get the support of their consultants for an appropriate medicine that they see being issued in England, Scotland and Wales. They feel that a massive bureaucracy is being applied to a medicine that they believe is vital to their quality of life, and that they are being prevented from using it. No other area in the health service is subjected to such scrutiny over outcome in terms of whether it is good value. My constituents say to me that that is not done for any other procedure in the health service but that they are subjected to that bureaucracy and when they are successful, the issue goes into the health board system for another six months or a year, sometimes. Is anybody at the table prepared to defend that system?

Professor Webb: Are you talking about the whole system?

The Convener: It is a whole system for my constituents. For people round the table, it is different parts of a system and we can all justify our part of it. However, the cumulative effect on the person who wants the medicine is pretty drastic.

Professor Webb: I have been involved in the SMC in the past and I think that the SMC system is timeous, robust and covers all medicines, so in some ways it has advantages over the NICE process in England. I believe that the SMC

provides good decisions that help health boards to do their work and that its doing that for all the health boards is a helpful process. What perhaps has not been captured in the discussion so far is that the SMC will approve all drugs that are cost effective, which does not mean that they are cheaper than what has come before; they may be more expensive and may offer no additional benefits. Another piece of work must therefore be done to decide whether the benefits that would be provided, or the additional cost, would make a drug worth while for use in Scotland. Knowing that a drug is cost effective is good, but knowing whether it is the best option for patients is a separate issue.

The Convener: Are you saying that all procedures that are carried out in the health service are cost effective? Why am I asked by constituents why there is a higher test for medicines than for any procedure in the health service?

Professor Webb: No I am not saying that. One could argue that that level of scrutiny should occur for surgical operations and for other medical practices, such as nursing care or homeopathy. A lot of things that we do could be brought under scrutiny. I think that such scrutiny should be widened, not narrowed.

The Convener: Andy Powrie-Smith wants to come in.

Dr Simpson: Can I just ask Professor Webb to clarify something?

The Convener: No, Richard. I will let you in later.

Andy Powrie-Smith: I echo some of the things that you said earlier, convener. It is easy to be drawn into examining individual sections of the system and the rationale behind them, but if we look at the overall picture, the UK is—I think—11th in Europe in terms of access to and uptake of new medicines. Within the UK, we see huge variability between the devolved nations and England, and within Scotland we see huge variability across health boards. From a patients' perspective, that is hard to justify. Whatever the individual rationale behind different parts of the system, ultimately the whole system is making access in Scotland low and slow for patients.

Professor Timoney: I tried to describe what we do in the context of the whole system; the convener is right that we should not just look at individual parts of the system. It is important to remember that we spend £1.3 billion a year on medicines in NHS Scotland. We would fail in our duty to the public and to patients if there was not appropriate scrutiny of that expenditure. What we do at SMC in looking at new medicines is just one part of that, but our hope is that early testing of cost effectiveness and being able to advise clinicians and prescribers about best use of medicines will help in terms of treatment access.

Andy Powrie-Smith's perspective of "low and slow" is an interesting soundbite, but the reality is that it is recognised that prescribers in Scotland are a cohesive clinical community who are, perhaps in some ways, conservative in their use of medicines. That is not necessarily a bad thing. When new medicines come to market there are still issues about safety, efficacy and risk. What the licensing authority does, in effect, is say that a medicine is safe enough to try in the population—not that it is generally safe. That measure of clinical conservatism and the advice that we can provide helps good practice.

The Convener: Do you really believe that all the stages need to be as rigorous and that they are all absolutely necessary in the process? Why is it necessary for a board to embark on another year's work on work that the SMC has already completed? As a lay person, I ask you why all that is necessary.

10:00

Professor Timoney: I am a member of the Tayside ADTC. We do not go over or even look at what the SMC does; we accept the SMC's assessment. However, if the SMC says that a drug for blood pressure is cost effective, there could be seven medicines in the same chemical entity and the same therapeutic class. Putting all seven medicines on the formulary would be inappropriate; that would not be the most appropriate way to guide practice.

What is important is not whether a drug is on the formulary but whether, if the SMC has said yes, our clinicians in Tayside can access it—and they can access a drug while we go through the formulary processes. That gives us cohesion in the systems and processes that we use. There is cohesion between primary care and secondary care and we are working as one community.

Bob Doris: I want to clarify a few things. The process seems to have two separate parts. I would like information on the time that the SMC takes to decide whether to approve new medicines in comparison with the time that NICE takes. We must get on the record how timeous or otherwise that part of the system is.

Richard Simpson's concern was not about that process but about how the ADTCs do their jobs after that. My concern was partly allayed by the fact that, in the case that he described, equivalent drugs that could have the same effect were being prescribed. There would be no evidence of loss of life, because equivalent drugs could be used.

Will you clarify how general practitioners and other clinicians can prescribe a drug before an ADTC puts it on the formulary? Will you reassure us about that process? Is how health boards give information to the public and how they account for it not at least unsatisfactory? A lot of the uncertainty could be relieved if health boards reported the information more consistently. I hope that those three points are helpful.

Professor Timoney: You have asked about timelines, about the situation for GPs and other prescribers once the SMC has given advice and about giving information to the public.

Bob Doris: Yes.

Professor Timoney: On timelines, the SMC uses a rapid health technology assessment system. Our entire process takes 18 weeks, which represents one of the most rapid systems in the world and is recognised as such. An article in the *BMJ* online in January compared the SMC's timelines with those of NICE and concluded that the SMC provides guidance within a median of 7.4 months of marketing authorisation whereas NICE takes 21.4 months, so evidence is available to Scotland at an early stage.

GP prescribers may prescribe whatever medicines they feel are effective and useful for their patients. We encourage them to look to the SMC guidance in making such decisions. They are not precluded in their prescribing; they do not have to wait for a drug to go on the health board formulary before they can prescribe it for their patients.

Bob Doris: Is that separate from individual patient treatment requests? Can GPs prescribe drugs directly?

Professor Timoney: Yes.

Your final point was about information to the public, on which I agree with you. The position is probably not very well communicated to the public. The most recent guidance from the Scottish Government, which was issued in February, said that health boards must make their information available on websites within 14 days and required that to be enacted by 1 April. The committee might wish to check whether that is happening, because such practice has in the past possibly not been as good as it could have been.

Bob Doris: That is helpful.

David Pfleger: I will offer a broad perspective. I agree with the previous comment about communication. We got the guidance at the end of February and it was to be enacted by April. The priority was providing access in that timescale. The communication about access is extremely important, but I would be the first to admit that we prioritised access, to ensure that patients can get access when they speak to their consultants and clinicians. I agree that we recognise—certainly on my board—that we have more to do to communicate decisions, to ensure that they are clear for patients.

It is six months since the guidance was issued, so we are at the point of reviewing locally how we are adhering to it. We have worked to implement the guidance and, six months down the line, we are at the natural point of working to see how we are adhering to it. We would welcome an extra push on that, but we must recognise the natural timeline for boards to ask how they are doing and whether they are doing as well as they should and could do.

Melinda Cuthbert: At NHS Lothian, we have always had a website where the information is freely available to healthcare professionals and patients. We publish on that website our decisions on whether medicines have been added to the list of medicines that are prescribable.

Even if we decide that a like-for-like medicine is already in use and that we do not wish to use another medicine, that does not preclude any prescriber from requesting that medicine for their patient if they truly think that there would be additional benefit from the patient receiving it. That is done via a non-formulary process, so if the SMC says yes to a medicine but locally we decide that it is not the one that is preferred, that does not prevent any prescriber from prescribing that medicine if they find that there is a niche for the patient to receive it.

With regard to the new guidance on implementation within 90 days, the situation is currently being tracked very closely by our formulary pharmacists and our medicines management team. The information that we have from 1 April to the present is that we are achieving implementation within the 90-day decision period. A couple of medicines have been delayed for protocol. One of those is one of the newer antibiotics that have just come out. The reason for the delay is that, as you are aware, antibiotics are developed less and less, so when we get them we are concerned about resistance. We therefore want to ensure before we initiate use of the medicine that protocols are in place within our antibiotic alert policies to allow it to be used appropriately.

Drew Smith: I am not entirely clear that improving the communication of the decision necessarily helps, because issues of principle are involved. If I, or a relative of mine, believe that a particular medication is likely to extend my life or improve the quality of my life and I know that someone in a different part of the country is able—as far as I am concerned, because of their postcode—to access that medicine but I cannot

and the only explanation that I am given is that my health board does not want to spend money on it, how does improving the communication process help me?

David Pfleger: Sorry, but I am struggling to see how we jumped from Melinda Cuthbert's comments to the situation that has just been described. If the SMC has said yes, I truly do not see that there is an access issue. It has been mentioned that there are occasions when a protocol, local guidance and getting clinicians to decide how they will use the drug mean that the process takes slightly longer than 90 days. The requirement for us to implement within 90 days is challenging at times, but it is correct and we work to achieve it. I do not recognise the situation in which we would, on the basis of cost or affordability, say no to a drug that has had a yes from the SMC.

Sandra Auld: It is clear from the evidence that we have gathered recently—I reiterate some of what I said previously—that there is still wide variance among the boards in the interpretation and implementation of the CMO guidance. It is a fact that, as of this month, in five boards in Scotland no information is available about the formulary decisions on new medicines.

On Mr Smith's comment about communication, I think that, from the patient's perspective, it is important for them to be able to find out whether the medicine that they need is available and how they might access it. Transparency in relation to the processes in health boards is also important, and—I am picking up on the convener's point—a system in which there are 14 different processes across Scotland is difficult for patients to understand and come to terms with.

Andy Powrie-Smith: It is great that we are hearing examples of good practice, but this is about the wider picture. An analysis of just one medicine that went through the SMC and the decisions that have been made in the 90-day period shows that in five boards the medicine has not been included on the formulary because boards are awaiting a protocol; in another five boards no information is available; in three boards no decision has been published; and in one board the medicine has not been included. To some extent, the examples that we are hearing around the table of what happens locally are less relevant than the wider picture, which shows the reality of what is going on.

There seems to be an assumption that prescribing something that is not on the formulary is an easy process for a GP, but that is not necessarily the case. This is anecdotal evidence, but clinicians who have shared their experiences with us say that there is an administrative burden attached to prescribing something that is not on the formulary. For example, the medicine does not appear in the drop-box on the electronic prescribing system, so the clinician needs to go and find it elsewhere.

We should be careful. SMC says, "We don't all sit round the table and assume it's fixed," but, from an industry perspective, although we absolutely support the pace and speed at which the SMC makes its decisions, which are timeous, it is about what happens afterwards. That is when the issue kicks in.

Jim Eadie: It is helpful to hear a range of views. Professor Timoney said that it is absolutely right that medicines spend in Scotland, which is more than £1 billion, is subject to the scrutiny to which it is subject, and I accept that.

Professor Timoney, Mr Pfleger and Ms Cuthbert all said that there are no barriers to a patient receiving a medicine that is not on the local ADTC's formulary, if the SMC has said yes to the medicine. My interest is in representing the people who sent me here and, when I have written to NHS Lothian on such matters, I have been told that a medicine is not available because it has not been placed on the formulary. Therefore, I am genuinely struggling to understand how patients can access medicines that the SMC has approved but which are not on the local formulary. If the witnesses can provide further reassurance on that, today or in writing, that would be helpful to MSPs who represent their constituents and write regularly to their local NHS boards to ask why a medicine that is SMC approved is not being made available.

David Pfleger: First, we must accept that the ABPI data give a snapshot. I talked about communication versus access, and what the ABPI presents shows that we are not achieving the aspirations—indeed, the requirements—of the CMO's letter in relation to communication. It would be interesting to see the access data behind that. It is right that we expect the chief executive's letter number 17 of 2010—the CMO guidance—to be adhered to.

Secondly, on SMC yeses that do not get on to the formulary, the formulary is largely about peer review and peer consensus on how medicines will be used locally. If a prescriber wants to use something that is not on the formulary, in essence, their view is not in line with the peer view. Jim Eadie said that we said that there are no barriers to getting a medicine in those circumstances; I think that—although we would never get to the point where we would say no—there has already been a view locally: clinicians collectively have decided that they want to use a particular treatment rather than another treatment, and they will have reasons for that view. We need to

discuss with clinicians why those reasons do not apply.

10:15

Bob Doris: I will ask a very brief question, because I know that other members want to come in.

The reply to Jim Eadie's question was that, although a drug might not be available because it is not on the formulary yet, GPs can still prescribe it. The important thing is the information that is given to clinicians post-SMC approval so that they know that if they judge that a drug is best for their patient, they can go ahead and prescribe it. I accept that the formulary might be a strategy for rolling out a specific medicine across a health board area, to replace other drugs and to explain for which particular conditions it is most appropriate. I understand that that protocol can take some time. However, what information goes directly to clinicians at an earlier stage after SMC approval so that they can make a judgment call irrespective of what the health board says? That would be useful to know.

Professor Timoney: I am happy to answer that from an NHS Tayside perspective. A general practitioner's terms and conditions require him to provide the medicines that a patient needs. If those medicines are licensed, he is entitled to provide them.

A formulary is not a list of the only drugs that can be accessed. Most boards would like to see 85 to 90 per cent formulary compliance and would accept that around 10 per cent of medicines will not be on the formulary. All clinicians know that.

The process in primary care is different from that in secondary care. In the secondary care that is provided by consultants and clinicians in hospital, the medicines must be stocked in the hospital pharmacy. There will be a non-formulary process and consultants and clinicians will all be familiar with the need to fill in a non-formulary request form so that the medicine can be ordered in. That is a standard process that has been in existence for some time.

The Convener: Can you help me? I do not know about all that. A constituent of mine had consultant support for a particular medicine but could not get access to it. You are suggesting that if a GP had prescribed that medicine, it would be okay. Is that the message?

Professor Timoney: Is it an SMC-approved medicine that your constituent wishes to access?

The Convener: It is an SMC-approved medicine. That is an example of the insider language that confuses people.

As I understand the situation now, if the SMC approves a medicine but it is not on the health board's formulary, the GPs can prescribe it. Is that right?

Professor Timoney: GPs are monitored on their formulary compliance.

The Convener: How regularly would GPs prescribe medicines that are not on the formulary? How common is that?

Professor Timoney: Our local compliance rates show that our GPs are incredibly compliant with their local formulary. The rate of compliance is probably 1 or 2 per cent higher than it is for our hospital prescribers, to be honest. GPs will mostly look to the local formulary to give them guidance on the medicines that they use.

The Convener: There may be an issue with communication. That insider jargon and knowledge is all completely familiar to you, but it is excluding people across the board from a simple understanding of how to access a medicine that might improve the quality of their lives. No one around the table is so far taking that into account.

Andy Powrie-Smith: To pick up on something that David Pfleger said, it would be interesting to see the data on the access to and uptake of those medicines. We are slightly missing that element. If we want to understand whether we are driving new medicines to patients who need them, it would be useful to have in the public domain the data on what health boards are using and at what levels, so that we can make decisions.

Richard Lyle: I have listened intently. I agree with the convener's point, and especially with Jim Eadie's point.

I want to ask about costs. First of all, I will read some facts into the record. The ABPI submission says:

"The discovery, research, development and clinical trials of a medicine takes on average over a decade and costs over £1 billion ... figures show that the price of medicines in the UK is amongst the lowest in Europe".

Of course, some people might disagree with that second point, and I would like to hear the panel's views on it.

However, in its most interesting comment, the ABPI says:

"Prescribing volumes have increased from 69.5 million items in 2002/03 to 94.6 million items"—

an increase of a third—

"in 2011/12. NHS ISD states that this growth 'reflects not only the availability of new or more effective medicines, but also increasing patient expectation and demographic changes and latterly the implementation of clinical guidelines and recommendations'."

Interestingly, it then points out:

"Meeting the increased demand of patients for medicines is costing NHSScotland progressively less in real terms".

Do the witnesses agree with that? Concentrating, again, on the question that the convener asked, I have to wonder why, if the SMC has approved a drug, other boards are then looking at it themselves. Why are they not passing it on to the people we represent who want it? We are told that the system is working, but why is it not working? If we are dispensing more medicines, why are people saying that they are not getting them? Like others, I got more than 100 e-mails about one particular case. Why are people not getting the medicines that they need? If, as we have been told, costs are going down, the money is there and the SMC has passed these drugs, why are clinicians not dispensing them?

Melinda Cuthbert: Your question raises a number of issues. First, a newer medicine is not always a better medicine; there might well be an equally effective drug in the system. Secondly, as Professor Webb and Professor Timoney have suggested, when a new medicine comes out we do not have the full safety profile for it. Giving any medicine to a patient involves a risk-versus-benefit process. Some medicines have greater risks than others, but patient factors such as the other medicines they are taking and other disease states they might have introduce further risk. Sometimes, the patients to whom we end up giving those medicines post-trial were not those who were exposed to them during the trial; they might have been excluded because of other medicines that were included or because of decreased kidney or liver function. We need to consider additional risk when we start to give out medicines in the actual environment.

If our formulary already contains known and trusted medicines in which we have confidence, we are not going to jump automatically to a newer medicine just because it is new. We have to find out whether prescribing the medicine would have additional benefits for patients. If we already have a medicine that does this or that and there is no additional benefit from switching to a new one, health boards might decide not to give that as the preferred medicine. However, as I have said, if a clinician makes the case that their patient might get additional benefit from the medicine without the risk, they will get it.

As for access, if a medicine is approved by the SMC, that is different from a medicine that is not approved by the SMC. I am not quite sure about the circumstances to which Mr Eadie alluded—

Jim Eadie: I was talking about an SMC-approved medicine.

Melinda Cuthbert: It is probably inappropriate for us to pick up a specific case right now, but I am more than happy to discuss it outwith the meeting. I do not know, for example, whether the clinicians supported the use of the drug or any other issues that might be involved; in any case, without the patient's permission, it would not be appropriate to discuss the matter here.

Jim Eadie: I make it clear that I gave the example on an anonymised basis.

The Convener: We are trying to resist getting into individual constituency cases. There will be an opportunity for that after the committee.

Dr Macdonald: It may be worth clarifying a few points. As was said, the SMC is different from NICE and the industry is supportive of the SMC role. It reviews every medicine, so the situation is different from that of NICE. However, given that the SMC reviews medicines and that the medicines will already have an EMA licence, it will already have been assessed that the benefit risk ratio is acceptable. In the SMC review, it is clear that the product is cost effective. It is therefore wrong to say that the products may not be valuable in themselves. If they are not any better, they have to be cheaper, or they will not get through, and that is rarely the case. In most cases, they are at least equivalent and bring some value.

I take your point that not every medicine can be put on the list. There might be a range of alternatives. However, every medicine that comes will have a limited safety profile—that is just a fact and cannot be a factor in not putting it in a protocol; otherwise, no medicines would be picked up. The need is to put them within a protocol that puts them in the context of the other options that are available. The big need for the patients is to get those decisions on the protocols made as rapidly as possible, in a rational manner, and then to put the medicine in place where it is going to bring most value. There is a frustration that the development of those protocols and the placement of medicines where they will bring most value are happening too slowly.

One also has to remember that the local regulatory body—in this case, the Medicines and Healthcare products Regulatory Agency—will monitor the safety of the medicines carefully. There will be a rapid turnaround in ensuring that the patients are not being exposed to unnecessary risk. That takes us back to the issue that safety should not be a reason not to put medicines on the list. We need quick decisions on protocols.

Professor Webb: I agree with the essence of what you say, but it is important to recognise that new drugs are generally more expensive. When they offer an absolutely equivalent benefit, we may also look at the relative safety of the two drugs. That is quite important. Dabigatran is a blood-thinning drug that is useful for treating heart

attacks, strokes and blood clots, just like warfarin. We hate to use warfarin, as it is a complicated drug and it is difficult to individualise treatment, but the effect can be terminated when someone has a bleed and with the newer drugs it is difficult to terminate bleeding. There are safety issues that must be borne in mind. The area is too detailed to go into it too far, but safety comes into play when everything else is equal and the price is much higher.

Dr Macdonald: In many cases, that is not the issue. You are quite right—if two products are exactly the same but one is more expensive, it probably would not be seen as cost effective to use the expensive one. However, there might be reasons why someone does not want to use the other medicine.

The frustration on the industry side and among some patients is the decision about where the product fits and what sub-group might get most value from it. There is often variation between health boards and a lack of clarity. For example, there are probably some high-risk patients who would benefit from dabigatran, but the consensus statement is a blanket, "It's the last therapy," which is almost too simplistic. There is a need for a broad and transparent discussion and a decision, so that everybody can see what the factors are and there can be some equality.

Professor Webb: I think that I agree with you on that point. There is room for more clarity, perhaps more speed, more consistency and more transparency—I do not disagree with that.

Dr Simpson: The word "consistency" is the one that really interests me. We have 14 different area drug and therapeutic committees, and we have evidence that they come to their decisions at different speeds and in different ways. Okay, if there is exact equivalence such as Dr Macdonald has described, I can fully understand the decision. I was the chair of a pharmaceutical liaison committee—probably the first in Scotland—back in 1980, and we had 35 different iron products. We were trying to get clinicians to agree that we needed only four or five, and it took two and a half years to achieve that just in relation to iron products, for heaven's sake. Clinicians are eccentric and need a degree of management—I accept that fully. However, my constituents in Tayside get one drug and my constituents in the Forth valley get another. Their doctor may tell them that he would like to prescribe the drug that the people in the other area get, but he cannot do so because the process is so bureaucratic and difficult and he is under such pressure that there is no point in pursuing it.

10:30

Brilique is the example that interests me. In the west of Scotland, it has been decided that it should be available for certain things, while NHS Lothian has decided that it should be available for other things. However, the SMC gave the drug general rather than restricted approval.

The consistency across Scotland is such that if you have a particular type of heart attack in the west, you will get the new drug, whereas if you are in the east, you will not, or vice versa. My constituent finds that confusing. The consistency to which Professor Webb referred seems to be lacking in the system, and there is no central monitoring. There is no monitoring of timing, and no recourse if ADTCs do not pick up a drug within 90 days, as they do not have to report to anyone centrally at present. We have a system that is deeply flawed at that level rather than at the SMC level.

The Convener: Do you want to respond, Mr Pfleger?

David Pfleger: I have no argument with the application of CEL 17/2010, which contains the CMO guidance. If we have not done that, we must hold up our hands and say so, and get it right. That relates to my earlier comments on communication.

I want to clarify two issues, first with regard to the formulary. We have heard this morning that the process runs from the SMC down to developing the formulary in the board ADTCs. However, we have not acknowledged that the formulary processes are generally open. There can be an application for any drug—the drugs are normally licensed, but some ADTCs will consider others. If a prescriber wants to prescribe a drug that is available in Tayside, there is a route for them, with peer support and peer review, to get that drug on the formulary—supported by the board, in general—so that it is clinically available.

The other issue is GP prescribing, on which there is a danger that the committee will go away with slightly the wrong impression. If the SMC says yes to a drug, one of the ADTC functions is to agree on which clinicians will be responsible for using it. Many of the SMC drugs are hospital focused, and in many cases it would be wrong for a GP to prescribe them. That would be outwith their expertise, and they would not have the support to do so, and therefore it would not be appropriate for them to use those drugs.

Angela Timoney is right to say that the general medical services contract arrangements mean that we cannot force or restrict what GPs can prescribe, but that is slightly different from the professional and clinical consensus that is gained in developing a formulary. The reason why

formulary compliance is high is that clinicians themselves—we should remember that the ADTCs are full of clinicians—have agreed on the use of those drugs.

Richard Lyle: On the point that Melinda Cuthbert made earlier, is the clinician frightened to take on board new drugs because they have the old drugs that they have used for 20 years in their cupboard and they do not want to use the new stuff?

It seems to me as a layperson that, if the SMC approves a drug, every board and clinician throughout Scotland should be able to use it if they wish, in consultation with the patient. It is the patient that counts—the person who possibly wants to extend their life for a number of years—and not the layers above.

I gave some facts earlier that show that we are giving out more drugs than ever before in this country at a lower cost, and that we are doing the best for the people of Scotland. However, we are not grasping what we should be. The new drugs are available, and clinicians should be giving them to their patients if they are required. The old drugs are good, but they are perhaps past their sell-by date. We should move on and consider how we can do better for the people of Scotland. Do the witnesses agree with that?

Dr Green: I will make a few points as a clinician. There is certainly a comfort in using drugs that you have used for many years, as you know the safety profile in a variety of different clinical situations. If a new drug is SMC approved and you believe that it is beneficial for the individual patient in front of you, you can prescribe it through a non-formulary application. That requires filling in a form, but it is not laborious or difficult, and it happens all the time in a hospital.

The difference is between drugs that are SMC approved and those that are not. The committee needs to recognise that, if the SMC approves a drug, it can be used if there is a clinical advantage to the patient. That is between the doctor and the patient.

Dr Fox: As well as being involved in the SMC, I am a clinical consultant in a kidney unit. I can say honestly from my clinical practice that an element of conservatism—with a small c, I hasten to add—in the use of medicines is entirely appropriate and in line with the best traditions of British medicine. I do not think that the idea that we automatically use new medicines is appropriate or correct. I include my own family in that, and if I was to treat myself I would prefer in the first instance to use medicines with a long track record of benefit and a low risk of harm.

I will make one other point, on the introduction of medicines and comparisons of Scotland with other countries in the world. We should bear it in mind that many other countries do not have effective systems for assessing medicines—in fact, Scotland is the envy of some of those countries, and some of them come to us for advice on how to introduce such systems. It is entirely appropriate to assess the cost effectiveness of new medicines. There is only one cake in healthcare, and it is appropriate to assess value for money and to consider what we might not be able to afford if we spent too much on new medicines.

Sandra Auld: On that point, page 3 of our written evidence shows a graph of data that was supplied by the Office of Health Economics. It demonstrates that the uptake of new medicines in Scotland in comparison with England, Wales and Northern Ireland is lower over a period of up to four years from launch.

Dr Fox: I disagree with that interpretation. I have the graph that we are talking about in front of me. If we were to apply a scientific method to the analysis of that graph, we would say that three countries—Scotland, Wales and Northern Ireland—are effectively identical in uptake. Whether or not they differ significantly from England is another matter.

We have already discussed what uptake means in this context, and I do not think that we should go through that again.

Professor Webb: First, I reassure Richard Lyle that clinicians want to give the best treatments to their patients and to have access to the best medicines. In my field of cardiovascular medicine, I feel that Scotland has allowed me to access medicines sooner than in other countries. Our system is very good: it is envied throughout the world, and we get early access to the medicines that bring important new benefits.

I will give a couple of examples from some time ago. Imatinib and Herceptin were launched extremely fast in Scotland after they were approved by the MHRA. Our patients with cancer got very early access to important and radical new treatments.

The Convener: Therefore, we have the best system in the world and all the people out there are complaining about nothing. Is that the message from those who are deeply involved in the system of reviewing and giving access to medicines? The system is perfect and cannot be improved, and those people—particularly with regard to orphan medicines and cancers—are being hysterical.

Dr Fox: We have already heard some suggestions for improvement—for example, monitoring the response to CEL 17, which states that decisions should be made in 90 days and

communicated. It is clear that we could move forward by ensuring that in an appropriate timescale—it is too soon to take a snapshot now—the response is appropriately monitored and the guidance enacted.

Nanette Milne: I have found the discussion extremely interesting. Perhaps those around the table can elaborate a bit more on the assessment of orphan drugs, which are prescribed or developed for very rare diseases such as certain cancers. Doubts seem to have been raised about the process, and it has been suggested that the health technology assessment should be modified for such drugs.

I am no expert in that sort of thing—although I have an ancient medical background—but I am interested in hearing a bit more from the experts in the field about what should be done. Should the SMC, as the ABPI suggests, set up a short-life working group with representation from boards, the ABPI, academia and patients to examine ways to approach the National Institute for Health and Clinical Excellence with regard to those drugs?

Professor Timoney: Perhaps Frances Macdonald can speak about that, as she used to be involved in a drug company that produced orphan medicines.

Orphan medicines are designated as such by the European Medicines Agency under a separate part of the regulatory process. That new system came into place around 2001, and there are currently a lot of orphan medicines in development.

The regulatory system was set up effectively to encourage drug companies to produce medicines for rare diseases when it may not be in their commercial interest to do so. The European Union rules state that fewer than five in 10,000 people should be affected by the disorder and that it should be of a serious nature.

In return for producing the medicines, the companies get back market exclusivity for 10 years, with an additional two years for paediatric medicine. They get additional time to recoup the costs of development and support to go through the regulatory system. They get a lot of meetings and the fees are reduced so that it is easier to some extent for them to get their drug to market.

From an SMC perspective, an orphan medicine is a medicine that has been designated as such by the European Medicines Agency. Because an orphan medicine will have been supported to get through the regulatory system, our clinical trials programme for that drug will be smaller than it would be for other medicines. The evidence base will not be as robust and the nature of the trials may be slightly different, and we will be dealing with a great deal more uncertainty around the medicine's benefits.

The SMC is explicit that we accept that uncertainty. The medicine will have a licence, and if it is an orphan medicine we accept uncertainty and some of the weaknesses that are associated with that. The SMC is used to dealing with orphan medicines: we have looked at more than 75 such medicines since the new designation came in, and we have said yes to more than 60 per cent of them.

Effectively, the SMC has to consider the reasons why we might want to say yes to a drug, despite the uncertainties, and whether we are comfortable in doing so. We may sometimes say that there are reasons for accepting a higher cost threshold in order to say yes to a drug. That is the process that we use.

Some of the submissions from rare diseases organisations have suggested that certain conditions or drugs are so different that we cannot do that. I do not see any evidence of that. In general, the submissions are very supportive of the SMC process, but people are saying that their drug, disease or treatment area is different and that we should allow something for them but not for everyone else.

The SMC uses a committee to decide whether there is a sufficiently strong reason or evidence that our processes cannot consider the drug in a comprehensive way. We at the SMC feel that the application of our modifiers allows us to say yes in the difficult cases where we do not have as much clinical evidence as we do for other medicines. We hope that, as a result, patients in Scotland get access to cost-effective medicines.

To an extent, the issue is not only that there is less of an evidence base around orphan medicines but that quite a price premium is sometimes attached to them, which means that the value-for-money case is more difficult to prove. The companies must decide what cost they think the market will bear.

10:45

Dr Macdonald: I fully agree with Angela Timoney's summary of the SMC's position on orphan medicines, but a discussion could be had on the topic. There are a lot of orphan medicines because the epidemiology says there should be such medicines for a group of 500 per 1 million patients, so a lot of medicines are now targeted at orphan diseases. It is therefore perhaps better to consider the particularly rare diseases that are sometimes called ultra-orphans. There are not many such patients in Scotland: perhaps 10 or 20. The term is used in England for a break-off of fewer than 500 patients.

From the drug development perspective, those medicines will still cost an appreciable amount of money to develop. The fact is that every drug that comes to market must accept the weight of the medicines that did not make it through development; it therefore pays not just for itself but for those that did not make it. The SMC accepts more variability, but some medicines for rare diseases will not meet the accepted criteria for being cost effective, which is often £20,000 or £30,000 per the quality-adjusted life years measurement—QALY. A company that is going to invest in orphan medicines cannot make the price the same as the statins, for example, because there will be a price premium simply because of the recovery costs.

There is perhaps a need for societal discussion of what society wants to pay for, because it is impossible for the orphan drugs to come in at a very low price. England considered the issue by putting it under the hat of the advisory group on national specialised services—AGNSS—which advises on specialised commissioning, and it has now given it over to NICE. The approach was to see whether the drugs could be evaluated in a slightly different manner by looking not only at cost effectiveness but the extent of unmet need, the severity of the disease, the societal impact of the disease and the societal benefit of treating the patients—if, for example, the medicine benefits a paediatric patient whose carer is looking after them.

NICE does not have the answer yet, but it has said that it thinks that there is a case to be opened up and discussed. If we do not do that, the ultra-orphans are highly unlikely to get through a conventional HTA assessment. That is probably the case in the Scottish context, because very few of those products will get through. That is not through any fault of the SMC but just because of the fact that they are high-priced products.

Bob Doris: I suspect that Richard Lyle was motivated to talk about restrictions on people getting certain drugs because of the 100 or so e-mails about an individual patient that we have had in the run-up to this meeting. The expression "postcode lottery" is sometimes used for such cases.

We got a helpful briefing ahead of the meeting about medicines for orphan conditions that are available in England but not in Scotland and vice versa. It is all about the clinical evidence that is used. For example, Imatinib, a medicine for the orphan condition of gastrointestinal tumour, is available in Scotland, but it was turned down for use in England. I am not criticising the English system, which undertakes due process for what it puts in place.

Rather than just scratch the surface and compare the position on different drugs, which is not helpful for progressing the situation, I am keen to look at the modifiers that are in place in the SMC—not just those for orphan conditions but the end-of-life and other factors that are taken into account. How would the witnesses modify or enhance the modifiers to make them more reflective? Have we got the balance right on the modifiers? Could we go further? I would be interested in the witnesses' comments in that regard.

Dr Macdonald spoke about societal benefits, but I am more interested in how we can build into the SMC process value-based pricing. For example, there can be thresholds of £20,000, £30,000 or £40,000 for the QALY, depending on the modifiers that are used, but I would like to know what the consequent benefits in savings would be to local authorities from a new medicine being prescribed. Given that we are looking at integrating health and social care, we should consider such aspects in detail.

How do we get the modifiers right, so that we map out the economic benefits of new medicines? Where does all that sit in the context of integration of health and social care?

Dr Macdonald: The questions are clearer than the answers. That is why we suggested that a short-life working group should be established to discuss the issue. You are right to say that, if we consider the societal costs in some disease areas, we can see that savings can be made, but not every new medicine will save money. The question is: what is the boundary within which we should look when we are considering the costs and benefits? Why should the societal benefits of care provision at home, for example, not be considered, when days in hospital are considered? There are costs to the system. There should be a discussion about what society wants and what the benefits and costs to society are. The discussion should be widened in certain circumstances.

Professor Webb: It is important to acknowledge that, if we are talking about health and social care, the discussion needs to be broader than one that is just about medicines. Sometimes, we are talking about rare diseases for which there are very expensive medicines that have marginal benefits, and in those cases the money might be better spent in other ways, such as on better carer support or on changes in the home so that the person can get into the bath more easily. It is not just about medicines; there are other things that we can do to improve patients' lives.

Professor Timoney: I agree with Professor Webb.

Bob Doris wanted to talk about modifiers. I think that, given that we do not really know what the public in Scotland want us to do, it would be worth while researching public attitudes—the committee could recommend that. I do not think that we can set up a short-life working group, because to some extent it would be a talking shop for people's views and opinions on what people in Scotland want. It would be helpful not just to the SMC but to the NHS as a whole if we understood what patients value and what the public would like us to spend our money on. Research into public attitudes would be a helpful contribution.

The evidence around rarity is confused, but it mostly suggests that the public do not value rarity. The view seems to be that, if someone has a severe disease that can be treated with a medicine and the person can get benefit from the medicine, they should have the capacity to benefit, whether the severe disease is common or rare. It is not clear that we want to value rarity on its own. That is an important point, and we need to get a perspective from people in Scotland on the issue.

The Convener: Do we not need to extend that to all health services in Scotland? What we spend at the end of life is a significant amount, which probably costs the health service a damn sight more than some of the medicines would cost. That is part of people's grievance.

The view of people who tell me, "I can't get that medicine, which would radically transform my life, because of the cost", is sometimes confirmed by the SMC, because when a drug company reduces the cost and offers a bargain, that triggers consideration of and access to the medicine. Access is triggered by a reduction in cost, not by the medicine being made safer or anything else. The SMC tells us that one of its major jobs is to reduce costs, and it is proud that it keeps the cost of medicines down. Is it any wonder that people say, "This is not about safety—nobody cares about that; it is about cost"?

Professor Webb: I want to open up an issue, which is opportunity cost. In the health service we have a limited pot. We are in a recession and there is not going to be much growth in the pot. We must give the best value for every pound that we spend. The reason why things change when drug costs come down is because when costs come down the value goes up, and we can spend the extra money. Money that is spent on the drug while the costs are high cannot be spent on things that are better value for money. That is what it comes down to in the end.

The Convener: Was the SMC consulted when the Parliament introduced free prescriptions? Is that the most cost-effective way of providing medicines?

Richard Lyle: Convener, I gave—

The Convener: I asked the witnesses a question. I will let you come back in, Richard.

Richard Lyle: Nobody wants to answer, so I will.

Professor Timoney: My recollection is that, when the then Health Committee considered free prescriptions, it had an evidence session at which the SMC was present.

The Convener: We were talking about costs. We are denying people what they believe to be life-saving and life-changing medicines. Those medicines are judged scientifically and on the basis of cost benefit. We cannot argue with that process, but people say, "Why only me?"

Andy Powrie-Smith: On what Professor Webb said, there is an opportunity cost around all spending in healthcare. The medicines bill accounts for 12 per cent of overall spending. The figure went up by 2 per cent last year, which is obviously less than inflation. When we are making decisions, the medicines bill comes under a level of scrutiny that is different from that for any other kind of healthcare intervention.

Perhaps we need to have a debate about all our healthcare spending, what we get the best patient outcomes from, and how we can deliver most for patients in Scotland from every pound that we spend. Currently, it feels very difficult to have that conversation, as the SMC has a rigorous process around cost efficacy that we do not apply in other areas. When patients or companies make the case for a new medicine, they encounter a different level of hurdle and investment compared with the levels in other areas of healthcare spending.

Jim Eadie: Professor Webb made a useful observation about opportunity costs, and he gave the example of dabigatran. Cost savings can be made from introducing what might be a high-cost medicine—I think that the example that was given involved saving NHS funding through reducing the use of warfarin clinics.

The substantive point is that, if people listened to only part of this discussion, they might think that no funding for orphan medicines or ultra-orphan medicines is available, but it is clear that, although the area is difficult, funding has been made available for those treatments. I am interested in high-cost medicines for orphan or ultra-orphan conditions that might have a low-budget impact because of the small number of patients across the country who are involved. I know that arrangements are in place north and south of the border. There was a reference to AGNSS south of the border, and there is a system in Scotland for considering how we can pay for orphan and ultra-

orphan medicines. I am interested in the panel's views on the distinction between the high cost of medicines and the budget impact that they will have on the health service in general, and on the systems that we use for assessing whether those medicines should be funded north and south of the border. Should the system in England be learning from the Scottish system, which is less formalised, although it could be argued that it is not as open and transparent, or should we look to adopt the system south of the border? Given the experience of the experts around the table, I am interested in their understanding of and views on that subject.

Professor Timoney: In Scotland, there is an NHS National Services division system. A process is in place for medicines that have gone through the SMC, particularly rare orphan medicines for patients whose illnesses perhaps have a genetic component. Three patients in one health board area might have such an illness, so provision may fall unequally across the health boards. Therefore, there is a risk-sharing scheme among the health boards that is administered by the NSD. The medicine has to go through the SMC and if the SMC says yes, it will go to the chief executives as a group, and they will decide whether to put it in the risk-sharing scheme. Money will be taken from the health boards for the risk-sharing scheme and effectively used to fund that medicine. That is quite a good and fair system across Scotland in which the chief executives agree to share the risk.

We have talked about AGNSS in England. That system, which was developed to look at orphan medicines, has struggled and has to date been unable to give any advice on orphan medicines. AGNSS will now be incorporated in NICE, so I will look at how NICE develops the system. We worked closely with AGNSS on our systems and processes and we helped AGNSS quite a lot in developing its systems and processes. We wish the people involved luck in developing a process that works.

11:00

Jim Eadie: Will you comment on the point about the budget impact as against the high cost of an orphan or ultra-orphan medicine?

Dr Fox: I will add to what Angela Timoney said. We ask companies for information about the estimated budget impact, but that has no role in the SMC's decision. Whether the impact would be low or high is not a matter for the SMC.

Jim Eadie: What is your view as a clinician, as well as an SMC representative?

Dr Fox: As you might imagine, I am very much in favour of the whole cost-effectiveness and value-for-money argument. I am also well aware of opportunity cost. I agree with Angela Timoney that we must find out how the public value various interventions in various conditions and their treatment. That information is lacking.

The convener asked why medicines should be subject to such scrutiny. I accept that many other interventions should be subject to the same scrutiny.

Dr Macdonald: I agree that AGNSS—which will ultimately be taken over by NICE—does not yet have the answers on how to assess ultra-rare diseases, but one advance is that it has recognised that we need to consider looking at them differently through having a wider definition of value that goes beyond the slightly traditional cost per quality-adjusted life year. We ask whether it is also relevant in the Scottish context to consider widening the picture of what is valuable.

I do not disagree that rarity per se is not the only issue; I agree with the point that severity must be considered. However, there are potential treatments for many rare and severe diseases, so they need to be considered differently.

We appreciate that the SMC does not take into account the budget impact, which makes sense, considering that it judges everything. I do not think that the industry would say that, when a treatment costs a lot, the SMC should approve it if it is for just one patient. Treatments still need to be evaluated in a systematic framework; budget impact is just one factor on the side.

Professor Webb: I endorse that point. We are trying to get at value for money. For instance, if we had said five years ago that everyone in Scotland should be treated with a statin, because that would reduce heart attacks and strokes, the Scottish Government probably could not have afforded that. That is a question of affordability for the Government. If we think that a treatment is good value for money, we recommend it.

Professor Timoney: I will offer reassurance. I took over as the SMC's chair in May last year. Since then, we have approved dabigatran, which the manufacturer estimated would cost NHS Scotland £20 million over five years. We have also approved treatments for hepatitis C—that was a massive step forward. Those decisions were easy for us; the treatments were value for money and we thought that patients should have access to them. The companies estimated that that would cost NHS Scotland £50 million.

Our decisions were based not on affordability for the NHS but on what is best for patients. I am proud that we made those decisions and that those medicines are now used in Scotland. Using our rapid process to allow access to good treatments is what this is about.

The Convener: Campaigners for the prostate cancer drug abiraterone complained bitterly about the delays and the time that the process took. That was contrasted with what happened down south, where the drug was available. People who were in the terminal stages of that illness were denied that treatment while the negotiations to reduce the price went on—but at what price for those who were left in that situation? How long did that process take?

Professor Timoney: NICE's decision on abiraterone came out a couple of months before ours did. As I said, in nine out of 10 cases, the SMC gives advice before NICE, so it could be argued that people in England generally wait in a way that people in Scotland do not.

We looked at abiraterone in April and said no to it. The drug company had to address the weaknesses and uncertainties in the case and consider some of the issues to make the product cost effective, and it was then able to market the drug. That is a right and proper process.

The Convener: What was the game changer? Can you give us an insight into that discussion? The game changer was reducing the cost of that drug to the health service—it was not to do with effectiveness, safety or anything else. The cost negotiation went on for months, with terminally ill people at the heart of the decision. That is the way that the campaigners saw it.

Professor Timoney: I think that that is right, which is unfortunate because that is not how it was. Since our meeting at the end of August with you, convener, I have looked at the resubmissions to the SMC to see which cases related purely to a difference in cost. That was the case with one cancer medicine, which was not abiraterone, and one set of eye drops.

Apart from those, we have never had a resubmission from the companies that was just about cutting the cost, because we do not engage in that. The companies have to identify the population and provide us with certainties that the evidence will show benefit for that patient group.

The Convener: Cost is at the heart of the QALY, is it not?

Professor Timoney: It is part of that—the QALY is about value for money and how well the drug works against the cost that we are expected to pay for it.

Bob Doris: We should state the obvious just for the public record. I have spoken to many pharmaceutical companies ahead of today's meeting, and I respect the evidence that they have given. They are clearly experts in their field, as are the clinicians from the SMC. However, it is true that pharmaceutical companies would wish to get as high a price for their medicines as possible. Given that fact, there will always be some form of negotiation.

Perhaps this information cannot be provided today, but for every medicine that the SMC has approved on resubmission when the price was lower, what would the differential be if the drug had been approved at the first time of asking? How much more money would it cost the Scottish taxpayer each year? That would effectively mean taking money away from front-line health services. We must talk about the costs for everyone who is an NHS patient, where that money comes from and where it is going.

Each time that a pharmaceutical company has resubmitted a drug at a lower cost with greater efficacy arguments, how much more money would the drugs bill have been for the NHS in Scotland if that drug had been accepted at the first time of asking? It is important that we get some perspective on the issue.

David Pfleger: I will expand on that point somewhat. It is about transparency around the submissions from industry. What are the drivers that would make a drug cost effective and acceptable to the NHS? I think that we lose sight of that. When there is a no from the SMC on the grounds of cost effectiveness, it will often give the value of the QALY or say that the cost effectiveness is not in the realms of acceptability.

I and the board would really like to know what the key drivers are that make a drug cost effective. To answer Bob Doris's question, is the cost element the key driver? Are the companies asking for too much money for the effect that people will get from the drug? In other words, is the cost effectiveness too low, and are companies asking us to pay too much for that level of effectiveness?

On the other hand, is the drug not effective enough? Do we need to demonstrate that it is more effective than the evidence currently shows? Perhaps the drug just needs more evidence over the longer term.

We would definitely value access to the drivers around how the QALY and the financial case are constructed. That would be particularly useful for IPTRs, which we have not touched on this morning. We need to understand how the general case for the population in which industry is looking for the drug to be used applies to the individual patient.

That information is somewhat hard to get at board level, because we are restricted in the information that is available to us. We need to ask how we can get better access to some of the submissions to the SMC. That builds on Bob Doris's point about the reasons behind the assessment of cost effectiveness.

Dr Macdonald: A range of comments have come up. On the price of medicines, I cannot defend every company's pricing policy, but I do not think that companies are trying to charge the absolute highest prices. However, Bob Doris is right in that companies have to get a return on their investment in products, including in the products that did not get through.

I have heard comments around the SMC table about a particular price being high or reasonable. We cannot paint all prices as being at the extreme top end. Of course, once a product goes off patent, we get the flip-side and get it at a reasonable price.

Sometimes, what stops the product getting a yes from the SMC is that the cost per QALY is high—I will come back to that in a second. At other times, it is because there is uncertainty about the clinical data and not the cost per QALY.

That can be seen in the DAD. It is reasonably easy to judge what the ground for refusal is because the DAD will say that the economic case has not been made or will say more about the clinical case. It is not a matter of the drug not being safe and effective—that is for the EMA to say; rather, it is the case that when we link the end point to the economic benefits, uncertainty is the issue.

We have not touched on one factor that sometimes makes the cost per QALY difficult to determine. Remember that the QALY concerns the patient's mortality—how much longer they live—and their quality of life. In some cases, it can be hard to measure the quality of life. It can be hard to put into the trial an instrument that is sensitive and can be measured between 0 and 1, which is the measure that ultimately goes into the QALY. We might find that a patient with a chronic disease walks a little bit better or that a patient with Alzheimer's functions a little bit better, but it is quite difficult to find an instrument that allows us to measure that sensitively and can be converted back into the 0 to 1 scale. Therefore, it is often difficult to show the quality-of-life benefit, although the cost is still shown.

For example, in Alzheimer's, the social costs cannot be counted. All the benefits have to go into a rating of between 0 and 1 and, with Alzheimer's, we are not increasing the lifespan, so the 0 to 1 factor is the only thing on to which the benefit can be hooked, but we still have all the costs.

As the industry submission says, we need to reconsider the QALY for some chronic diseases. Those things all add to the uncertainty and can add up to a number that might be too high or a degree of uncertainty that is simply not acceptable.

Does that answer some of David Pfleger's question? I think that the DAD explains the matter reasonably well. If it does not, that is perhaps something for the SMC to discuss. I think that it is reasonably clear, but perhaps that is because I read a lot of DADs.

David Pfleger: The DAD is useful. I am suggesting that there is room to draw additional information from the cases that the industry puts to the SMC. That additional information should be drawn through to the DAD or, preferably, made open so that patient groups can see it as well. Boards at least should have access to it, particularly for individual patient treatment requests. We would benefit from a little bit more data when dealing with those.

Jim Eadie: It would be useful to remind the committee what DAD stands for. I believe that it stands for detailed advice document.

Professor Timoney: DAD stands for detailed advice document. It is an eight to 10-page document from the SMC that provides advice not only about cost effectiveness but about a product's clinical advantages and disadvantages.

David Pfleger: My request comes not only from me; it comes out of the discussions that the group of ADTCs in the north-east of Scotland had about sharing best practice on IPTRs. It is not only one board's view; other boards also have the view that more information would be useful.

Bob Doris: Can I check what happens if the initial price at which a pharmaceutical company asks for a medicine to be approved is refused? I understand and fully accept that such refusals are based not only on cost but on cost effectiveness and the QALY. If the company comes back with a lower price within the formula, there will be a cost saving that would not have been made if the SMC had initially said, "Yes, that's fantastic. Let's go for that." Is anyone counting those numbers?

The committee has a political judgment to make. The convener mentioned abiraterone. The SMC could simply have approved that drug, but we cannot simply pick out abiraterone; we have to be consistent with every pharmaceutical intervention. I want to know the saving to the Scottish taxpayer of getting a lower price, separate from the wider social and medical benefits. We have to put that in context. Is anyone collecting those numbers?

Dr Macdonald: That situation is very rare. I cannot answer as to whether anyone is counting numbers. Those of us in the industry do not regard the SMC as a price negotiating forum. Such a situation is very rare among the 700-odd applications that the SMC has processed. In general, the company puts in a price that is influenced by its global pricing policy. More recently, some patient access schemes have

influenced the price, but there is very little bartering back and forth. That is not a way we would wish to use the SMC and it is certainly not a strategy that companies would adopt.

11:15

Bob Doris: Let me clarify. I am not suggesting that there is bartering. The SMC has been consistent in saying that it is not a price negotiation but rather a measurement of cost effectiveness based on the QALY with modifiers.

I am a politician looking at the numbers because our budget process is coming soon. There are no negotiations on the price and a company applies its global pricing strategy. If a drug at price X is not recommended and it comes back at a lower price of Y, surely someone should be counting up the figures? Perhaps that should be done by the SMC.

Dr Macdonald: It is also confidential information.

Professor Timoney: Frances Macdonald referred to patient access schemes, which are part of the reserved UK pricing system for medicines. The patient access schemes allow medicines that may not be considered cost effective to become cost effective because of a discount in the price.

I can provide the figures relating to the medicines that have gone through patient access schemes. Since the patient access scheme advisory group was introduced in May 2009, 44 schemes have been assessed by it, of which 27 are what we call simple schemes with just a simple discount and 17 are complex schemes that may have complex financial or clinical issues associated with them. Of those 44 medicines, 36 were accepted by the group as feasible for implementation in NHS Scotland. Of the 36 medicines that the group considered, all went before the SMC, which approved 20 of them, with 15 being simple schemes and five being complex schemes. However, those prices and discounts are considered to be confidential under reserved UK arrangements and therefore we have not looked at the cost. It can be assumed that there will have been additional cost benefits.

Bob Doris: I will not pry further. The convener made the point that had we just said yes to certain drugs at the outset, that would have had benefits for patients. I am trying to find out what the budget consequences would be of saying yes at the outset each time. That is important information for us when we are looking at the health budget.

The Convener: Richard Lyle has been very patient with me.

Richard Lyle: I want to return to two earlier comments. As far as I am concerned, the policy of free prescriptions has been excellent. The figures that I quoted earlier show that the policy has benefited many people in this country.

The Convener: I let you in to ask a question.

Richard Lyle: It is a brief comment.

Professor Timoney made a comment about what the public would like. The public would like the best health service in the world, with access to all the drugs that they require and better life expectancy.

The British pharmaceutical industry made an excellent submission. I am interested to hear the panel's views on its recommendation that the Scottish Parliament should

"examine the best use of the estimated £316 million of savings being made in the cost of medicines to NHS Scotland between 2012-2015, with a view to identifying what proportion can and should be reinvested in meeting patient expectations of access to the latest medicines".

In case I do not have an opportunity to comment again, from what I have heard this morning it seems that we are frightened to access these new medicines. When they are passed by the SMC, that should be the end of the story. The clinicians should then be able to access the medicines that they want for their individual patients wherever they are in Scotland. We should have the best health service in the world, and I think that we do. You all do a good job but sometimes we should cut through the red tape and get rid of the excess clutter.

Professor Timoney: I do not know whether this will be of help. You will have seen from the ABPI submission that in effect we are spending more money on medicines than we spent previously. The £316 million that has come from drugs going off patent has been used to treat other patients with the other medicines that are available, particularly generics. Branded medicines have a patent life of exclusivity and the drug companies make their money from that; when that stops, the medicine becomes a generic medicine.

As the population in this country gets older, they get more and more medicines. The increase in the drugs bill is mostly to do with the increase in volume, because more people are living longer and getting more medicines. That is what is happening to the money, and that is a good thing.

The Convener: We are running out of time, but there are a couple more issues to discuss, such as individual patient treatment requests and the stuff in the submissions about clinical research needing access to new medicines; the witnesses might want to respond to that.

Dr Simpson: We should move on to IPTRs, but before we do that I want to make two small points. First, it seems to me that if the horizon scanning that the SMC does for boards is effective,

protocols and preparation for the budget costs should be well in place before approval is given. It would be helpful to understand the timeline in that regard.

Secondly, it has been made clear to us that a reason why a drug does not go on to a formulary—apart from equivalence—is the innate conservatism of the system and the protection that that affords patients, because it is not always wise to rush into new medicines. Is the MHRA's system for monitoring medicines post-approval, when they are being used in the general population as opposed to the highly selected trial population, working adequately? It is a UK system, but from a Scottish point of view are the witnesses comfortable that SMC-approved drugs that are being used are being properly and effectively monitored, so that—before we get value-based pricing—we can see whether, in reality, the drugs are delivering the outcomes as safely as was envisaged in the licensing process?

Professor Timoney: I will talk about horizon scanning and perhaps Professor Webb will talk about pharmacovigilance. The SMC works closely with the pharmaceutical industry to identify what drugs are in the pipeline, and we send information to boards so that they can have advance notice and do comparisons.

At that stage, the drugs have not been through the licensing process and we cannot guarantee that that will happen. A drug might get through the licensing process but not the SMC process. Boards keep a watching brief on what is happening, but it would be inappropriate for them to start preparing all the protocols at that stage, because only about 35 to 40 per cent of medicines get through, so they would be doing a lot of work that is not necessary. I hope that that explains what we are trying to do. We try to ensure that plans are in place, but everything is refined as a drug goes through each step of the system.

Professor Webb: The MHRA acts on behalf of the UK to undertake pharmacovigilance and support safety. It works closely with the European Medicines Agency and I think that it does a good job. I do not think that there is a problem with the analysis of safety, which is robust.

Dr Simpson's question might have contained an implicit point about real-world effectiveness, as well as safety, which is not quite so well addressed. There is no body that takes on that issue in quite the same way as the refinement of safety is taken on. We often have to rely on clinicians and the NHS supporting research to look at real-world effectiveness when rather more complicated patients are getting the drugs than was the case in the original trials. That sort of information can take a number of years to come through.

Dr Simpson: We have a unique data linkage system—the CHI, or community health index. There would be capacity for Scotland to be a leader in that field if we were getting it right. We could provide a very useful service.

Professor Webb: The CHI could and should do that. If we are not careful, the English system—the CPRD, or clinical practice research datalink—will soon have the same capacity.

Dr Simpson: That is why I raised the point. It leads into the research issue.

Melinda Cuthbert: David Webb has covered the MHRA perspective. The yellow card scheme, which the MHRA runs on behalf of the whole of the UK, was one of the first spontaneous reporting systems established in the world for monitoring the adverse effects of medicines. The major problem with spontaneous reporting is on-day reporting, but that issue is not unique to the UK—it is international. One of the remits of the yellow card centre Scotland is to promote the yellow card scheme and facilitate education and training for healthcare professionals, to increase the reporting profile. Obviously, other things also inform the MHRA's approach to safety, with regard to clinical trials and post-marketing evaluation.

Jim Eadie: There is acceptance that Scotland has particular strengths in clinical research and clinical trials. There is expertise round the table—I refer in particular to Professor Webb and the work that he has been associated with at the University of Edinburgh.

However, some of our leading cancer clinicians and researchers have written an open letter to *Scotland on Sunday*—one of Scotland's national newspapers—in which they highlight a number of concerns that may be related to what they perceive to be the effectiveness, or lack of effectiveness, of the patient treatment request process for cancer patients. They highlight that the ambition of the Scottish life sciences strategy—I speak as the convener of the cross-party group in the Scottish Parliament on life sciences—has an "aspiration" to

"double the economic contribution by the life science industry by 2020".

They also suggest that the operation of the various systems in Scotland may impact on our ability to reach that objective. Because of the lack of availability of cancer medicines—I am paraphrasing—they say that

"due to Scotland in many situations no longer treating patients with the standard of care used in other parts of the world, Scotland may not be able to take the lead or take part in global clinical research studies, NCRN"—

national clinical research network—

"clinical trials or many other commercial trials which require the standard therapy in such trials to be what is approved by the regulators, but which is increasingly not the standard of care in Scotland."

They also refer to a number of adverse effects that would flow from that, which would undermine Scotland's strengths in

"basic science and translational medicine".

The letter was written by Professor David Cameron, Professor Jeff Evans, and Dr Marianne Nicholson—who I believe was once a member of the SMC—who will be known to members of the panel. It is a very serious challenge to the NHS and the Scottish Government and one that the committee needs to put on the record. Do the panel members share those concerns and what is their view on them?

Professor Timoney: The SMC would share your concerns if we thought that there was a challenge—

Jim Eadie: I am sorry, Professor Timoney—I was just putting on record the concerns that were expressed by the cancer clinicians. They may or may not be my own concerns.

Professor Timoney: Thank you for that clarification.

Basically, we would be concerned if we thought that Scotland was not able to participate in clinical trials. We think that that is really important for our patients, as well as for our clinical research and practice. At the SMC, we have had only one instance in which, after we had already said no to a medicine, it came back to us and the experts said that it was becoming the standard of care, and that it was really important from a clinical trial perspective. That was part of our judgment when we said yes to that medicine. For every medicine that we look at, we get clinical experts' views.

The issue to which Jim Eadie referred has not been reported to us in another environment. However, if it is a real issue, it would be worth investigating it. We need to see whether there are issues for Scotland, because I would not wish to see that happening. The SMC would wish to play its part in any necessary solutions.

11:30

Andy Powrie-Smith: From the perspective of the organisations that invest in those trials, that is an issue. We know that, and clinicians are stating it in open letters. If someone wants to run a trial, they must be able to compare against standard care or a comparator.

To give some context to the numbers, life sciences—as Mr Eadie will know—bring around £800 million to the Scottish economy and employ approximately 8,000 people. Scotland has not only a rich history of clinical trials, but future opportunities in informatics and other areas. It would be a great shame if we stifled our ability to grab those opportunities by not using some of the most new and innovative medicines.

Sandra Auld: We know that patients in areas where clinical research takes place do better, irrespective of whether they are involved in such trials. The benefits to the wider population are evident.

Bob Doris: One concern has been raised with me by representatives from the pharmaceutical industry. If a new drug is not approved by the SMC, that is one issue. However, if the company is looking for a successor drug five or six—or however many—years down the line, the clinical trials at that point will compare the drug that was not approved in Scotland with the new drug that is coming to market. The SMC's approval process may in that regard cause difficulties for the new drug.

I hope that I am clarifying the situation. Does that pose a challenge? If a new drug is not approved for cost-effectiveness reasons, and a pharmaceutical company then produces another drug with added benefits but the clinical trials are based on the drug that was initially not approved, would that undermine the company's ability to have the new drug approved at a later date?

That makes sense to me, Professor Timoney—I hope that you are following my explanation.

The Convener: Professor Webb?

Professor Webb: I was just nodding along—Bob Doris was making a good point.

Bob Doris: You were not nodding off.

Professor Webb: No—I got your point. To return to what Professor Timoney said, we have the industry view on that, but we perhaps need a wider piece of scoping work to understand how much of an issue it might be.

The Convener: That is wise counsel. I do not know how much progress we can make on that issue in committee, but we can certainly consider it for our future work programme.

Jim Eadie: I beg your indulgence, convener. Can we have on the record a response from Dr Macdonald and Professor Webb to the point that I raised about proper research?

The Convener: Yes, absolutely. On the question whether we can go further on the issue, it is important and needs to be addressed. We must decide as a committee whether we take it forward, as it is an area on which we could consider hearing evidence.

Another issue for consideration concerns individual patient treatment requests. We have had evidence about clinicians not participating in the process because it is not worth all the bureaucracy and hassle, and because outcomes are uncertain and can raise patient expectations when that would not necessarily be a good thing. There are issues to do with barriers, which we have heard about in evidence. As Jim Eadie clarified earlier, we are not necessarily speaking on our own behalf, but trying to reflect some of the evidence that we have received.

The transparency of the process has been raised in evidence. As someone who has a constituent who went through that process, I know that it is not a nice thing for any individual and their family to go through. Another point is that individuals who are dealing with rare conditions face the same problems that are faced collectively, along with the catch-22 around having some sort of clinical expertise in that condition.

Does anyone want to comment on the individual patient treatment request process?

Professor Timoney: Let us be clear. Medicines that go through the individual patient treatment request process have been determined by the SMC not to be cost effective so they should not be used generally. However, there must be a system. The SMC cannot necessarily make a recommendation for all 5 million people in Scotland—we have not seen those patients—so, if a clinician has identified that a particular patient has particular circumstances and they might benefit from a medicine in a way that we would not normally expect, or in a way that would not be expected under the SMC general advice, the board has a system in place so that they can access that medicine. That is the purpose of IPTRs.

In essence, there are two reasons why the SMC might not recommend a medicine. Either we have assessed the medicine and determined that it is not cost effective, or we have not assessed the medicine because the company has not submitted it to us and we say that the medicine is not recommended by reason of non-submission. I have been to all the ADTCs in Scotland during the past year and they have told me that one of their biggest challenges is getting an IPTR for a medicine that the SMC has not assessed because the company has not submitted it. Work should be done to encourage companies to submit new and active substances so that the boards have SMC guidance when they have to undertake an IPTR.

The Convener: Yes. Again, the situation can sometimes be affected by the cross-border or postcode issue, whereby although the SMC has not endorsed the medicine, a request is made on the basis that if the patient was living somewhere else in the United Kingdom, they would be able to access it. That further complicates the situation.

Sandra Auld: From the ABPI perspective, there are a number of issues around IPTRs. If the SMC framework was more broadly based, some IPTRs would not be made. We are not just talking about the rarer diseases; there are also issues around central nervous system medicines, for example. The cancer medicines have the highest profile, but the IPTRs do not come only from that therapy area. I reiterate that we support looking at a broader base.

The Convener: Can you help me out with a further explanation of a broader base and why that would improve the situation?

Sandra Auld: We need to look at the impact on society of some of the medicines, for example, and not just use the QALY-based assessment system that is in place. The SMC does a superb job within its framework, but some medicines will never get through the SMC, which is why some companies choose not to go to the expense of submitting them in the first place. That is one issue.

In some respects, I feel as if we are answering the wrong question with IPTRs. It is clear that the process does not work in the way that it was set up to work, or was expected to work; in some ways, it is answering the wrong question.

David Pfleger: I want to come back on a few of those points. The Scottish Parliament information centre briefing for the Health and Sport Committee shows that around 60 to 65 per cent of IPTRs get an acceptance. I find reassurance in that, because it shows that clinicians can identify the people for whom they can make a successful case and that they are not afraid to say that they think they have a case when peer reviews demonstrate that they might not. If the figure that I mentioned was 100 per cent, I would be concerned, as I would be if it was 20 per cent. There is no right or wrong level, but I have gained some reassurance from the figure. Interestingly, on oncology, we had a board review earlier in the year, which found a significantly higher level. That is important.

On the call for a broader base, I would absolutely welcome a discussion about drug versus non-drug interventions. We build a wall round our drug budgets and nothing can get out or in, which is not right. Drugs are the second most expensive item for the board, after staff. That is why there is lots of management round the issue and why we should ask questions about how we use that spend. Those questions include whether the people of Scotland want a fourth-line oncology treatment or a guarantee about palliative care. Obviously, we want both, but we might not be able to have both.

Some of the questions about the integration of health and social care will be about whether we should invest in a new drug for dementia that is on the horizon or put our money into social care to support patients. Those are real questions, so we need to engage in that discussion. All the discussions about the broader base for the QALY should be taken into a bigger discussion about how we spend our money in support of health and social care.

On the industry choosing not to submit drugs, I find it disturbing that the industry chooses not to play ball, because that causes us problems. I understand that there are reasons for the industry not submitting certain drugs to the SMC, but that is not a particularly helpful or mature response for healthcare in Scotland. We should encourage all drugs to go through the submission and assessment process, because that aids transparency.

Dr Simpson: I accept the figure of 65 per cent, which is the defence of the IPTR system. We must remember that the system is a modification of the previous exceptional needs system and that it was designed to have more consistency and read-across. However, I am not convinced that we have that read-across yet. There is a lot of anecdotal evidence that clinicians are not submitting IPTRs, either because they feel pressure from the board or for other reasons. That point is coming through to us strongly.

I am really quite concerned about the issue. In the past few weeks, I have been in discussions with a pharmaceutical company that arranged for two clinicians to speak to me, but they were then gagged by their health board. I would like the health board witnesses who are present to tell me that they deplore that action and to put it on the record that any clinician who has concerns about the system should be able to talk to members of this committee about those concerns. We cannot get to the bottom of the anecdotal issues unless we get hard cases that show that clinicians have had pressure put on them not to submit requests or that raise concerns about the bureaucracy or meeting the exceptionality conditions under the IPTR. If we cannot have discussions on those issues, there is a real problem.

David Pfleger: If that is the case, there is a mismatch between what we are trying to achieve and the reality on the ground, and I would be disturbed by that. I would welcome openness and discussion on the issue. I was a co-ordinator for the pre-IPTRs and I was involved in the development of some of the advice on the IPTR process. My view has always been that the board works with the clinician to overturn the advice that we do not use a certain drug. If we can do that legitimately and in a way that seems reasonable to the rest of the patients whom we look after and the rest of the population, that is great. If we cannot do that, we should not say yes to the request.

Such situations are often presented as though the board is trying to prevent a clinician from using a particular treatment, but I have always seen it the other way round, which is that we will push the issue as much as we can and try to get the drug for the individual patient, but the case has to stack up—it must meet the criteria in the IPTR process and get broadly towards an acceptance of cost effectiveness. That is not about calculating an individual QALY, but there are factors that send the process in that direction. It is about being able to say, accountably and reasonably, that this is an acceptable use of a treatment and that the individual case is different from the case of the group to whom we have said no.

I support what you are saying. What you have said surprises me and I hope to goodness that the board to which you refer is not my board.

11:45

Dr Simpson: They could not even tell me that.

David Pfleger: On the broader issue, I hope that the clinicians would have the confidence and the access to discuss the matter locally.

Dr Green: I agree that we want openness and transparency. I would be very disappointed if the clinicians that Dr Simpson mentioned were in my board, because they have not made themselves known to me so that I could help with the discussion.

Clinicians have now made quite a lot of use of the forms, so they have got used to what they need to do and what evidence they need to submit. The submission is made on an individual patient basis. I think that the clinicians understand what they are meant to be doing and I hope that the process does not put them off. The process is all based on the individual patient and their clinician's sense that the drug will benefit their patient with that particular clinical condition.

Dr Macdonald: The industry certainly knows that the IPTR is not just a way of turning a negative into a positive for all patients. I understand that there is a lack of clarity in general about how the processes work and that, as has been stated, many physicians find it difficult to work their way through the process and therefore view the hurdles as not worth crossing. Perhaps education is required.

There is a lack of clarity about the process and exactly what evidence has to be provided. There are also differences between health boards. The lack of consistency and lack of transparency

across the 14 health boards makes it difficult for all parties.

Coming back to the disappointment that was mentioned, the industry clearly wants to work in partnership with others, but when the process is not transparent, the hurdles are not clear and there is no consistency, it is difficult for the industry to work in partnership and say, "This is what we should be contributing." All those problems need to be fixed before the process works fully for any party.

Sandra Auld: I concur with a lot of what Frances McDonald said.

The ABPI surveyed oncologists about the IPTR process and also convened a short-life working group. Although we thought that there was an issue with the IPTR process, we wanted to establish whether others also thought that. The short-life working group included industry representatives, clinicians, patient representatives and pharmacists. The group concluded that there were issues, some of which were around the criteria for the submission of IPTRs.

The survey of oncologists produced comments similar to the anecdotal evidence that Richard Simpson referred to. Some clinicians said that they did not submit applications because they found the process very frustrating, difficult and time consuming. I will quote what some oncologists said:

"My experience was purely negative."

"I stopped putting them in a long time ago as they were always rejected."

"It is hugely frustrating for patients and clinicians."

Those were some of the comments that were fed back to us.

Jim Eadie: I have a question for the health boards.

David Pfleger said that 65 per cent of IPTRs were approved. I notice that the number of applications has declined, so that in 2012-13 the figure is down to 135 applications compared to 359 in 2011-12. Do you have any insight into why there has been such a decline? Do you recognise some of the comments that have been made about clinicians perhaps not having confidence in the system or not understanding how best to utilise the opportunity to make an application?

David Pfleger: We are only six months into 2012-13, so the data are incomplete for this year. That explains a large amount of the difference.

The data used to compare between the boards are difficult to interpret. For every one IPTR, we deal locally with four or five individual requests for unlicensed and off-label treatment. The slightly more complicated issue with that is that if the board is a teaching board, its clinicians are perhaps more likely to go down the route of using those unlicensed and off-label requests than they are to use an SMC no, because they are at the leading edge of what they are trying to do in their practice.

We can always do better in terms of communication. We have done local training and we have switched our IPTR process to the service level rather than the board level, where it used to be. We are always trying to improve communication.

My feeling is that we do not have people who are disengaged. People are engaged right from the top, so our clinical lead—certainly within the acute sector—chairs a lot of those panels. However, the ABPI has its views and those views have to be valid—we cannot put them to one side. We need to explore whether those views are representative of more than oncologists or haematologists—I am not quite sure which group you are talking about—and, if so, we need to ensure that we communicate the processes better.

The review of IPTRs for oncology showed a 90 per cent plus approval rate for oncology treatments. That does not quite stack up with the response that the ABPI had about a lack of confidence in the system. If there is a lack of confidence, we have to do something about it because those drugs are getting approved through that process. We need to look at it a bit further.

Melinda Cuthbert: I fully endorse what David Pfleger has just said. From an NHS Lothian perspective, we implemented the CEL 17/2010 and the good guidance that came after that. We took the time and we engaged with the clinicians in primary and secondary care to ensure that what we finally put in place was acceptable and met requirements.

As David Pfleger has already indicated, I am sure that trying to get that message down to each individual clinician is sometimes difficult. We went down the route of using the clinical management teams. We also have clinical pharmacists associated with all the specialties and primary care pharmacists out there in the community who are associated with the GP surgeries. Advice is available and clinicians are encouraged to ask for it. They know that on a day-to-day basis they can ask for advice on medicines. That network is there and clinicians will often call up the medicines management team or call me up asking questions about how to submit and how to do the forms.

I would like to think that we are open, encouraging and helpful so that, in those circumstances when clinicians think that it is appropriate to submit an IPTR because their patient meets the criteria as per CEL 17/2010 and

the good guidance, they are doing that. If they are not, I want to know why, so that we can try to address that locally within health boards.

Sandra Auld: I ask the committee whether Scotland needs 14 different IPTR processes. From a patient perspective, it is an absolute maze. There is a huge disparity between health boards as regards their decisions and their decision-making processes. When we did our research, in one of the boards in Scotland one of the criteria for consideration for an IPTR was that there was significant media and political pressure—that was on a health board website. That is an illustration of the differences and I am not sure that that is what we want decisions to be based on.

Professor Timoney: To move us forward, perhaps we could suggest that all health boards use a common IPTR form. We should not have a single system—a central committee is not a good thing because this is about timeliness and about local circumstances—but we could ask the boards to have one form so that everyone must submit the same form in a similar process, which should help address the issue, if there is a problem.

David Pfleger: I am looking slightly uncomfortable because we are just trying to make our form electronic and I am thinking of all the things we would have to unpick.

We cannot say that the decisions are different. We are talking about individual patient treatment requests and we cannot analyse it at that level. By all means have a national forum, but we would have to consider what we would lose in reactiveness or responsiveness if that was the case. Some decisions must be taken quickly.

I cannot speak for other boards, but our board has certainly tried to take the process down as close to the level of the responsible clinician as possible. That keeps the process responsive, keeps the clinicians engaged and allows them to be confident in the process—at least, I hope so, given the comments that Sandra Auld made earlier. We have the guidance, which should lead to consistent process. If it is not consistent, it is that bit that needs fixing. We do not need new guidance or new systems. We need to ensure that we have consistent compliance.

Returning to the issue of communication, confusion arises because access to drugs can be gained through trials, through unlicensed or off-label use, and through IPTR. The patient does not want to know that. They want to know whether they can get that drug and are not so much interested in the route. Some of the problem is a lack of understanding—that is completely acceptable—about the route to access a particular treatment and the comparisons that result from that.

Melinda Cuthbert: Further to Angela Timoney's comment and for the committee's information, NHS Lothian and NHS Greater Glasgow and Clyde already use the same application form. In addition, the west of Scotland and NHS Lothian use the same evidence-briefing templates. We are aware of the issue and we are speaking to other people in our networks about that.

Dr Green: Thank you, Melinda. You got in before me.

A regional association of health boards will often deal with cancer drugs, so any decision will be made by one IPTR panel for a variety of boards. That means that there is relative consistency, although it may not be Scotland-wide. For example, in the west of Scotland 50 per cent of all cancer drugs will go through one IPTR panel.

Dr Simpson: Are the cancer networks the basis for it, rather than individual boards? Is that what you are saying?

Dr Green: There is an agreement among individual boards that one board will lead.

David Pfleger: Just to clarify, that applies in the west, but not in the north of Scotland.

Andy Powrie-Smith: To be clear, we were not suggesting a single process for Scotland. We hoped that the same process would be applied in 14 places. Those two things are slightly different. My former role as a director of a patients organisation leads me to say that there would be no way to make patients understand why there were 14 ways of doing something. Simplicity at the time when a patient needs to go through an IPTR is crucial. As Angela Timoney said, it is about having consistent forms and so on. Applying the same process in the same way in 14 locations is the consistency that we are aiming for.

The Convener: I should say that the committee is not looking for solutions at this point. We just want to identify areas that may require further inquiry.

Nanette Milne: My question is not exactly related to the previous point. Do people have comments on the cancer drugs fund that is available for patients in England, which we do not have in Scotland?

12:00

The Convener: Does no one want to comment? Jim Eadie wants to speak, but I am going to wait out the witnesses.

Professor Webb: The cancer drugs fund represents a stopgap for the English Government before it moves to value-based pricing. We do not know what that will look like.

Andy Powrie-Smith: I agree with Professor Webb that we do not know what value-based pricing will look like. The cancer drugs fund demonstrates that dedicating funds to access to medicines can have implications and can push those medicines through the system, so funding can work. Rather than having support for a mechanism to drive access, what is important is having in place drivers to ensure not only that patients receive medicines, but that provision is well thought through. That need not relate to a particular disease area, but it is clear that there is an impact on the service.

Professor Timoney: The cancer drugs fund in England enables patients to access medicines even when NICE has said that they are not cost effective. The result might be that the industry is less inclined to make its products cost effective for not just England, but Scotland.

Bob Doris: I have a brief point on the cancer drugs fund. I would like the witnesses' thoughts on the overall spend on cancer treatment, which is wider than just medicines. For example, the Scottish Government has the £30 million detect cancer early fund and has invested £20 million in radiotherapy equipment. Money can be spent on that or on a cancer drugs fund.

What is the response to individuals who call for a special drugs fund for Alzheimer's or heart disease, for example? How would we create equity in the system?

It is right for Nanette Milne to suggest a cancer drugs fund, but a substantial majority in the Parliament was against that when we debated it. It is important to get on the record whether the witnesses support a cancer drugs fund or are—like the majority in the Parliament—against it, for the reasons that I have given.

Dr Simpson: I agree entirely with Bob Doris, but the corollary is that we must get the IPTR system right and we must get public confidence that it is right. Some of the suggestions about a read-across for IPTR applications are interesting.

What is going on must be monitored centrally. The confidence of clinicians, as well as patients, in the system must also be assessed. The committee will have to discuss a job of work to make that system the more equitable alternative to a cancer drugs fund.

Jim Eadie: Earlier, I put on record the concerns of cancer clinicians. For completeness, it would be helpful to have a response to that from Dr Macdonald and Professor Webb.

Dr Macdonald: Will you repeat the question?

The Convener: In a round-table format, you are not compelled to respond to any question.

Jim Eadie: Given the expertise that is available, it would be helpful to have a response, but whether to respond is of course entirely at panel members' discretion.

Leading cancer clinicians have put into the public domain concerns about the impact of what they perceive to be a lack of availability of innovative treatments and about the knock-on effect that that might have on Scotland's very good reputation for clinical research.

The Convener: Dr Macdonald, does your view differ from that of Mr Powrie-Smith?

Dr Macdonald: I fundamentally agree with the point that the situation needs to be monitored. If the leading products are not picked up in a country but they become the standard of care, a foreseeable conclusion is that a trial cannot take place there, as that country does not have the standard of care.

Monitoring is needed to see whether what has been suggested happens. The effect might not be noticeable at the point of SMC approval, when a drug is brand new, but it must be monitored thereafter, because that is when a drug will—or will not—become the standard of care. If the standard of care is not available, the trials will not be there.

The Convener: I am reluctant to open up that issue again.

Professor Webb: I will not open it up again. Obviously, we would be very concerned if we were not internationally competitive in our cancer research, as we are in many areas. I think that Professor Timoney suggested that we need a scoping exercise to define exactly what the problem is. First, we should see what the problem is. We have one view on that, but we do not have a broader view.

The Convener: I think that that is where we are. Jim Eadie's question was very helpful for the committee's purposes, and it is on the record. It will certainly be part of our work programme considerations.

Jim Eadie: I heard Professor Timoney's helpful suggestion earlier. I simply wanted to hear other views from around the table.

The Convener: You must let us get on, Jim. I know that this is your final Health and Sport Committee meeting and that you are getting in your last word, but we need to push on.

We greatly appreciate the witnesses giving us evidence in public. The committee has, of course, engaged with them over weeks and months individually and collectively in other sessions to try to get a better understanding of the confusing and complex world that they live in and the jobs that

they do. We have at least some understanding of how complex things are, but we have also spoken to patients groups, which have communicated with us and which we have heard, and we thought it important on their behalf to try to convey that charged situation.

We may not have covered everything that you wished us to cover, and you may now want to raise briefly issues that you think have not been raised. If you do not want to do that now, we would be happy to hear your views on this session and issues that may have been missed in good time before our final deliberations on the work programme, which will be in a week or so. Does anyone wish to raise any issues that they think we should have covered?

Dr Macdonald: I would like to reiterate briefly that, in the context of the UK, the pharmaceutical price regulation scheme and value-based pricing, we ask that the committee seriously consider how medicines and, potentially, other interventions are valued. The issue is a British one as well as a Scottish and English one, and I ask the committee to consider what value is and how it is measured, as the issue has a wide context.

The Convener: Again, I thank the witnesses very much for their attendance and participation, and for making the session interesting.

As previously agreed, we now go into private session to deal with the remainder of our business.

12:08

Meeting continued in private until 12:23.

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