The Scottish Parliament
Pàrlamaid na h-Alba

Official Report

HEALTH AND SPORT COMMITTEE

Tuesday 21 May 2013

Session 4

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HEALTH AND SPORT COMMITTEE
16th Meeting 2013, Session 4

CONVENER

*Duncan McNeil (Greenock and Inverclyde) (Lab)

DEPUTY CONVENER

*Bob Doris (Glasgow) (SNP)

COMMITTEE MEMBERS

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

*attended

THE FOLLOWING ALSO PARTICIPATED:

Jackie Baillie (Dumbarton) (Lab)
Dr Richard Casasola (NHS Tayside, University of Dundee and Scottish Cancer Research Network (East of Scotland))
Vicky Crichton (Cancer Research UK)
Melinda Cuthbert (NHS Lothian)
Joan Fletcher (Pompe Group of the Association for Glycogen Storage Disease (UK))
Natalie Frankish (Rare Disease UK)
Professor Charlie Gourley (Scottish Cancer Research Network (South East Scotland))
Dr Rachel Green (NHS Greater Glasgow and Clyde)
George Grindlay (Angus Long-term Conditions Support Groups)
Dr Stephen Harrow (Beatson West of Scotland Cancer Centre)
Lesley Loeliger (PNH Scotland)
Eric Low (Myeloma UK)
Dr Frances Macdonald (Association of the British Pharmaceutical Industry)
Ian Mackersie (aHUSUK—A Patients and Families Support Group)
David Pfleger (NHS Grampian)
Leigh Smith (Melanoma Action and Support Scotland)
Professor Angela Timoney (Scottish Medicines Consortium)
Professor David Webb (Royal College of Physicians of Edinburgh)

CLERK TO THE COMMITTEE

Eugene Windsor

LOCATION

Committee Room 2

Scottish Parliament

Health and Sport Committee

Tuesday 21 May 2013

[The Convener *opened the meeting at 09:46*]

Decision on Taking Business in Private

The Convener (Duncan McNeil): Good morning and welcome to the 16th meeting in 2013 of the Health and Sport Committee. As usual, I remind those present to switch off all mobile phones and BlackBerrys, as they can often interfere with the sound system. People around the table and in the public gallery may also notice that some members are using iPads instead of hard copies of committee papers.

Agenda item 1 is a decision to take item 5, which is a work programme discussion, in private. Such discussions are normally taken in private. Does the committee agree to take item 5 in private?

Members *indicated agreement.*

New Medicines (Access)

09:47

The Convener: Under item 2 we return once again to access to newly licensed medicines. As everyone is aware, the Scottish Government reviews of the Scottish Medicines Consortium and the individual patient treatment request system have been carried out.

We normally ask people around the table to introduce themselves but, in the interests of time, we will make some progress. We all know that we are here to look at the reviews and the responses to them. I will go directly to the first question. I ask those who come in with a comment or question to introduce themselves at that point, to help us to make progress. Bob Doris will ask the committee's first question.

Bob Doris (Glasgow) (SNP): I want to go straight into the body of some of the recommendations in the review reports that are before us, because I know that witnesses will want to give some opinions on them.

An issue with IPTRs that has been raised is how individual clinicians can get expert clinical opinion and medical advice that will allow them to demonstrate a response to the medication in question for their patient that goes beyond what would be expected in the peer group for whom it was initially not accepted by the SMC. Recommendation 10 of the review of IPTRs is that

"a register of approved specialists to support IPTR"

should be established and maintained. I see some advantages of that in supporting the consistent application of IPTRs across Scotland, but rather than just giving my views on that, I am keen to hear witnesses' views.

David Pfleger (NHS Grampian): We broadly support the availability of or easy access to that advice. I am sure that having that national list would be a useful addition to the IPTR process.

Bob Doris: If an individual clinician chose not to consult a nationally recognised specialist before submitting an IPTR, could that compromise the clinician's ability to make an effective request? Have the witnesses considered that scenario? I am hoping to get some comments on the recommendations that are in the reports.

Professor Charlie Gourley (Scottish Cancer Research Network (South East Scotland)): I am also from NHS Lothian and the University of Edinburgh.

The answer to your question depends on the context and the disease that is being treated. Some diseases are treated only by national

specialists anyway, so it is difficult to see how those submissions would benefit further from a list of specialists, because the clinicians making the request would probably be on a specialist group anyway. However, I imagine that there are other conditions for which a generalist might decide that an IPTR would be appropriate. Under those circumstances, if the clinician is not familiar with the process, such a group of experts would be beneficial.

David Pfleger: If we were to say that it would be necessary to access the group of experts every time, it would probably just slow the process down and be inappropriate for many requests. However, expert advice would be a useful resource in cases where the clinician making the request felt that they needed extra support.

I guess that the danger is that we might set ourselves up for the situation in which, if the clinician does not access that support, questions are asked about whether they have done their best for the patient through the IPTR process. The question is when the support and resource would require to be used. To my mind, it is really about the clinician's need for it rather than some strict protocol that says that they must access it.

Dr Rachel Green (NHS Greater Glasgow and Clyde): I will speak from NHS Greater Glasgow and Clyde's perspective. We support the ability to access specialist advice. We are probably fortunate that, as a larger board, we have quite a lot of specialists who can help one another through the process, which is good.

There might be some difficulty if the expert advice came from south of the border, because clinicians there are not aware of the IPTR process and decision making. That might be worthy of some further discussion, but the general principle of having experts is good because clinicians' submissions are much improved if they are supported by their expert peers.

Leigh Smith (Melanoma Action and Support Scotland): Dr Green's comment more or less mirrors what I was thinking. If there were a requirement to have advice from another clinician in Scotland, no one might be available. In the case of melanoma, there is probably only one clinician in Scotland who would have any experience of using the drug that was being requested. Therefore, they would not have a colleague to go to and would have to go to London for help. However, the clinicians there do not know the process.

Bob Doris: I know that the question seems process driven, but the Cabinet Secretary for Health and Wellbeing has asked us to consider the recommendations of the reports and report back to him before the Government considers the steps that it will take.

It has been suggested that the time that the 14 health boards' area drug and therapeutics committees take to get medicines that have been approved by the Scottish Medicines Consortium on to their local formularies has been unduly slow. The target is, of course, three months, and that has been achieved—or, as the case may be, not achieved—inconsistently throughout the country.

One of the questions that this committee considered is why on earth we have 14 ADTCs in the first place. We had some strong evidence that they do much more than simply putting drugs and medicines on to formularies so that, when the SMC approves a drug, patients get it speedily at a local level—which everyone should do, although that is simply not the case at the moment.

I accept that we have to stick with 14 ADTCs for the moment, but should we in future—say, one year, two years or three years from now—look at establishing a national formulary to ensure that the time that Glasgow, Edinburgh, Aberdeen, Dundee and so on take to get SMC approvals on to the local list of available drugs does not differ? We have regional formularies at the moment and I think that managed clinical networks, too, do some work on this issue, but how might we address the system of getting SMC approval for drugs in the first place and then ensuring that they are speedily and consistently available at a local level?

Melinda Cuthbert (NHS Lothian): I support what Professor Swainson says in his report about ADTCs. I certainly do not think that they should be dissolved because, in doing that, you could disengage many of the local clinicians who contribute to and feel that they have ownership of the formularies. There is regionality in certain models such as those for cancer and there might be some room for manoeuvre in that respect; however, with regard to the timelines for bringing medicines into formularies, I do not think that this is a race. You have to give clinicians the opportunity to consider the medicines and where they fit into their treatment guidelines to ensure that they can be introduced safely. If you try to introduce them faster than clinicians need to make those decisions, you could introduce more risk into the system. Moreover, there might be an increase in cost if the local prescribers disengage.

David Pfleger: In his report, Charles Swainson was quite clear about the advantages of the 14 ADTCs over the other regional and national approaches that Mr Doris has mentioned.

However, the crux of the question was about the timescales for considering SMC advice and formulating the local response. Healthcare Improvement Scotland's scrutiny of those

processes since the introduction of the 90-day guidance has certainly made us focus on ensuring that our systems try to comply. We have a tool that we can use; the clear indication is that we need to make these decisions by 90 days, and Charles Swainson has usefully recommended that, during those 90 days, we be more open and transparent about the steps that we take, where we are in the process and what we are trying to achieve to ensure that patients and other stakeholders understand what is going on.

As for the regional work, it grows naturally and—to use an overused word—organically. As a result, regionalisation is more advanced in some areas than it is in others; indeed, the cancer networks are a key example of that. As Charles Swainson has made clear, however, there is not a massive disparity in the formularies and the choices that we make across Scotland. Of course there are differences, but we need to accept that ADTCs are not just about new drugs; they play a much wider role in medicines governance. If we ensure that we hit the timelines that we need to hit, we can have the advantages of local clinician engagement and local medicines governance as well as meet the timelines and aspirations for the implementation of SMC decisions.

Professor David Webb (Royal College of Physicians of Edinburgh): I do not want to say much more than endorse the point that ADTCs do a lot more than simply consider new drugs. They provide local ownership and engagement in the process and a forcing ground for training clinicians who might subsequently join the SMC and take on a national role. Moreover, certain local factors affect the introduction of drugs. For those reasons and others, I think that maintaining that local structure is valuable—indeed, I think that that was Charles Swainson's intent.

Bob Doris: That was certainly his intent but—and I do not want to get bogged down in this—he also said that, although ADTCs do a variety of things that we should support, they could be working far closer and in a more integrated way to get drugs to the local formulary. My constituents would be confused about why the process is speedy and safe for some ADTCs but far more laborious for others. Frankly, I would rather that access to a new medicine was delayed in order that it could be introduced safely. However, I am looking for a way whereby we can change the structure to ensure that there is consistent best practice in making new medicines available quickly at the local level. Is there room for improvement? Is there room not for the merger of ADTCs but for the integration of some of their functions? I would want to ensure that they were keen to work closely together rather than to defend their own institutional viewpoint about what they do locally.

10:00

David Pfleger: Absolutely. I am not sure about the term "integration", but ADTCs certainly work together. For example, north of Scotland boards met around the IPTR process to share best practice and some learning. It is also about how we share our resources. We combine across ADTCs in the Scottish Medicines Consortium because there are certain things on which we need to come together, particularly at the regional level. That becomes more important for the MCN work, because it seems sensible for all the ADTCs to work together to engage with a regional MCN.

We are therefore less wedded to the integration model, but we are absolutely wedded to working together to reduce duplication and maximise our use of our expertise and resources.

Bob Doris: I have another question for Mr Pfleger, and then I will let my colleagues in.

I do not want to get hung up on the process, but you said that the process could grow organically—you did not talk about integration, but you talked about working more closely together and being more speedy. If we were to find out in 18 months' time that there were still considerable differences between, say, Aberdeen, Dundee, Glasgow and Edinburgh, do you think that structural steps would have to be taken to deal with the issue? If the 14 ADTCs are working more closely together, making greater use of managed clinical networks, being more open and transparent and sharing best practice, and given the focus that the Swainson report provides on speeding up the process consistently, should we expect to see minimal differences in 18 months' time in the approval processes at local level?

David Pfleger: Just to clarify, are the differences that you are focusing on in the approval of SMC medicines?

Bob Doris: No. I am talking about the placing of SMC-approved medicines on local formularies so that they are available routinely at the local level in all 14 health boards.

David Pfleger: We need to accept that medicines that are approved by the SMC will have alternative treatments locally. Part of that decision-making process is about local clinicians coming together in a peer-review process to decide which medicine is their first choice, so there will be differences. When Charles Swainson interviewed stakeholders for his report, he did not find any issues around accessing SMC-approved medicines that were not on local formularies. We need to remember that formularies do not cover 100 per cent of usage. He did not find that to be an issue, because there were alternatives in place. If people were not going to use the alternative and

wanted to use the SMC-approved medicine, there were routes for them to be able to do that.

I am therefore less convinced that the outcome would be one that would trigger a full structural change. It is about having the appropriate medicine on the formulary, being transparent and open, and engaging with members of the public and patients in those processes. I am just less convinced that the outcome that we are looking for is necessarily having absolute uniformity of SMC medicines on our formularies.

Bob Doris: That is a good clarification. I should be careful what I say. We are more concerned about the time that it takes for each health board to come to a decision about whether it is appropriate to use a new medicine. It is those differences that we want to iron out. You make very good points about me-too medicines in different parts of the country, traditional usage and risk-averse local clinicians, but the committee has been through all of that. We are more concerned about the time that it takes all 14 ADTCs to decide—inconsistently—how to use the medicines on the local formulary. Nevertheless, I thank you for that clarification.

David Pfleger: If you come back in the timescale that you are talking about and we have not achieved those timelines but do not have reasons for that, you should hold us to account for that—absolutely.

The Convener: Why did it take the review and the inquiry before you addressed that issue? It was clear that different health boards took different lengths of time to give people access to licensed medicines that had been agreed by the National Institute for Health and Care Excellence and the SMC. We have another role, as our inquiry started by looking at why there was delay and denial of access to new medicines. The people who gave us evidence on the process told us that it could take up to eight months, in some cases, for health boards finally to prescribe those medicines and give people access to them. We were talking about end-of-life drugs and medicines then, and there would not be much point in the drugs after that time. If we have known about the problems and are dismissing them so lightly today, why have we not acted?

Eric Low (Myeloma UK): There are different things at play here. We have heard from the national health service perspective. There is a difference between something going on to a formulary and clinicians having the ability to provide what they think is best for their patients. Those are two different things. A medicine can go on to a formulary, but a doctor is not required to prescribe it if they do not think that it is safe or the best medicine for their patient.

It is important to differentiate between clinical practice and a formulary. We can put things on a formulary as quickly or as slowly as we want, but nobody holds a gun to a clinician's head, saying that they must prescribe the drug to their patient. Clinicians must have the ability to choose from the available options what they deem to be the best treatment for their patient. The decision should not be influenced by the cost of drugs. That should not be the big factor in not getting a drug to patients.

From the patients' perspective, some of the concerns may have come from the fact that, on the ground, they had experienced delays in getting access to treatments that had been approved by the SMC. In some circumstances, that is not appropriate. We cannot deny patients access to treatments that have been approved, so we need to address whatever local factors are preventing the timely adoption of effective treatments on the formulary. I am not sure that it is clinicians not wanting to prescribe the drugs—they want to get good drugs to their patients.

We need to be clear that getting stuff on the formulary is one part of the problem. We need to do that quickly and efficiently to enable clinicians to prescribe the drugs that they think are best for their patients as soon as possible.

Dr Green: NHS Greater Glasgow and Clyde strives to achieve the 90-day target. When we do not, it is usually because getting clinicians to agree where a treatment fits within a patient's pathway and protocol sometimes takes us over the 90 days. Given the Swainson report, we will look for ways in which we can get something on to the formulary in that interim phase. I hope that many boards will read the report and look at how we might get things on to the formulary earlier.

Eric Low: The job for clinicians is to get ahead of the curve and anticipate innovation and developments for early adoption. However, one of the issues is that, if a clinician has decided that the best place in a patient's pathway for a drug is different from what is stated in the SMC guidance, how do we marry up the fairly strict SMC guidance around using drug A at point B with the clinician's desire to use drug A at point C? That could well be the situation. How do we overcome that issue?

Dr Green: A standard protocol is used for individual patients. If a clinician wishes to use a drug, they will have to go through some minor administration to get it prescribed.

Eric Low: You have the ability to effectively ignore SMC guidance and use that drug at a different point in the pathway.

Dr Green: If it has been SMC approved.

Eric Low: You can use the drug at a different stage of the disease. I think that what you said

was that you would deliberate to work out where in the pathway to put it, but it is clear from NICE guidance where the appraisal is.

Dr Green: SMC guidance goes not generally tell you where the drug is in the pathway. The clinicians wish to decide where they would put it in their priority order for prescribing. If a clinician had evidence that it would be better for one patient to receive a drug at a particular time, there is an ability to do that.

The Convener: What will you take out of the recommendations? What actions will you take as health boards to address the delay? Do you take from the review and its recommendations that everything is okay and that you are all doing fine?

Melinda Cuthbert: NHS Lothian has looked through the reports. We are meeting a lot of the stuff and ticking the boxes in them. Some things that need to be achieved relate to resource issues. If we are going to deliver training to all doctors on IPTR processes or provide greater support to patients so that they can engage in the IPTR process, we need to look at resources.

On the recommendation for making our formulary decisions more visible, we already have an external website. The issue identified for us was that that fact was not put in an important or noticeable place on the NHS Lothian internet page so that patients could access the information. However, once someone visits the external website, they see that the information is there.

The Convener: Are there any other recommendations that you have looked at that will require increased resources from the board?

Melinda Cuthbert: I cannot speak for the clinicians; they would have to make the decisions on where to put treatment in their protocols. However, I would think that most clinicians' main remit is seeing the patient in front of them and delivering care. Finding the time to have meetings and make decisions can be another resource issue. I will let the clinicians around the table speak to that element.

David Pfleger: I will speak in general terms before making specific points.

The broad themes that have come out of the two reports are increased transparency; an increase in the ease with which patients and the public can access understandable information; an assurance about public involvement—while not increasing it—in ADTC processes; and the introduction or improvement of scrutiny, with a role for Healthcare Improvement Scotland.

All that is to be welcomed but, as Melinda Cuthbert says, scrutiny comes with resource implications and that resource comes from elsewhere, so there is an opportunity cost. I accept that we need to rebuild public and patient trust in our processes and systems. That resource may therefore be well used, but we must remember that it comes from somewhere else in the system.

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

The question is how we can implement some of the recommendations without impeding the responsiveness of the system that we have. The SMC is very quick to generate advice to Scotland and we do not want to impede that. The question is how some of the recommendations can go into play at SMC level, ADTC level and IPTR level without the system being slowed down, because we do not want that to happen. We need to be wary of that. In general, however, I found the recommendations to be most useful.

10:15

As far as the recommendations for ADTCs are concerned, I did not see any that I would not want to go back and either begin implementing or provide assurance on that we are achieving the aspirations in them. There are some bits of detail that need to be clarified because, if they are not, we will be back discussing and arguing about some of the detail. There are some bits that we will need to agree on, but in the main the recommendations are welcome.

The Convener: Have you calculated an increase in the prescribing bill as a result of any of the recommendations?

David Pfleger: That brings us to the crux of the matter. The Association of the British Pharmaceutical Industry submission clearly states that the start of the process was related to access to new medicines. For once, I agree with the ABPI on something, which is that the discussion in itself does not necessarily improve access to new medicines in Scotland.

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

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

Professor Webb: Speaking as a clinician, I have been pleased to hear at this meeting and previous meetings the support that generally exists for the work of the SMC. It has never been about cost; it has always been about trying to get the best value for money from NHS funds in an equitable way for patients.

I, too, broadly support the recommendations and more openness. The recommendations from Professor Routledge, who runs the All Wales Medicines Strategy Group, look very much like they provide for the inclusion for the SMC of many of the things that his group does. If we take up his recommendations, we will move towards the activity that happens in Wales. The SMC started before that group and has a substantially greater business than it has. We look at all medicines, whereas it looks at a relatively selective group of medicines and not always the difficult ones.

Although the Wales group has open meetings, much of the work happens—and the decisions are made—in camera. There is always a private final discussion and decision. My concern as a clinician is that, if we go to a much more open process, with the public then leaving so that we can have decisions in camera, the meetings might be substantially longer. That is another resource issue—and not just in relation to time—for the clinicians, pharmacists and other people who are involved in the process. I worry that clinicians, who already give of their time to come to the meetings, will find that the workload will double or more, and that that will make them disengage from the SMC process. That would be a tragedy.

Eric Low: I think that David Pfleger got to the crux of the matter eloquently. The issue, which was not part of the review, is why we get noes from the SMC. We need to focus on that, because ultimately what we want is to get more yeses and reduce the reliance on individual patient treatment requests, which are not the way in which to make decisions on access to medicines.

We need to work with the SMC, which has a willingness to engage, and focus on its mechanisms and modifiers, to ensure that we can get more yeses. We need to strike a balance between magnitude of benefit and high prices. As Professor Webb said, we need to strike that balance; that is the crux of the matter. However, that is not the question that has been asked, nor the debate that we have had. In fairness to the SMC, to some extent it has held back from addressing the issue because we have been working on the question of value-based pricing and waiting to understand what role it will have in Scotland.

As far as I can see, VBP may not be practical. That fact gives us a wonderful opportunity to sit down with the SMC and stakeholders and address the issue of how we can strike the balance between demand-orientated healthcare from clinicians and patients and the ability and willingness of payers to pay a price that is fair and represents value. That is key; if we can fix that in Scotland, the issues that we are talking about—formularies and individual patient treatment requests—will become the exception rather than the norm. We are focusing on the wrong issue.

If there is not an appetite in Scotland for a cancer drugs fund—which there is not; that is fine—the rare disease approach may work in some situations. Although VBP is not going to be practical in Scotland, if we know that current mechanisms still need improvement we must focus on that. We have to find a solution to help the SMC make better decisions and get more yeses; a solution that empowers the Scottish Government to have a grown-up discussion with pharma about a price that represents good value for NHS resources will mean that no patient is denied access to a drug that their doctor thinks they will benefit from, and which gives pharma some sustainability, security and certainty about how the market is going to respond.

That is the solution, and it is not that complex. We have not had the right debate; we have been looking under the wrong stone for the solution.

Dr Frances Macdonald (Association of the British Pharmaceutical Industry): I want to build on the comments that we have just heard—namely, that the bottom line has to be that patients will get access to valuable medicines that the SMC has accepted. We support most of the recommendations, but we were disappointed that they did not say very much about some of the SMC-related methodological issues

We appreciate that the SMC is excellent and does a very valuable job, but some of the evidence put forward related to some of the methods that it uses. There were three aspects. The first was the societal aspect: there is the suggestion of a Scottish citizens council parallel to that in England, in which we could have a discussion on whether society wants to pay more in certain areas. That is step 1, which is very valuable.

Step 2 relates to the methods per se. As Professor Routledge said, the QALYs are less than perfect in some disease states. It is true that they are probably the most validated tool at the

moment, but that does not mean that we cannot further enhance the methods that we use. In some of the long-term central nervous system conditions, for example, the patient can get used to their condition and the utility does not show up—Professor Routledge refers to that in his report. If somebody has motor neurone disease, for example, they may say that their utility is fine. We can develop an effective medicine, but we will probably not get the sensitivity in a QALY. Yes, the QALY is still the best tool, but that does not mean that we cannot add something on top. There is a methodology discussion, therefore, that has escaped us; it has been touched on but has escaped any recommendation.

The third aspect is the wider one of value—namely, how to deal with diseases where there is a wider social burden, be it on a provider-supporter for a child or somebody with Alzheimer's, or a burden on other aspects of social care that are being paid for by the social care bill. That point has also been missed out of the recommendations, although it was certainly mentioned by many who gave evidence.

Those are the three key points. The social value aspect has been picked up, but neither the methodology nor the question of wider value has been. That is a disappointment from our point of view, given that they are some of the hub issues that need to be addressed. Otherwise, we are back to discussing IPTRs, which is not the best route to giving patients access to medicines.

Professor Gourley: I am glad that we have come round to the crux of the issue. Many of the recommendations in the reports are very welcome. The issue is that, if IPTR rules are applied properly, they are not a mechanism to access SMC not-approved drugs. It is not fair of us to give patients the message that they might access SMC not-approved drugs in that way. For the vast majority of patients, GPTRs and the rare conditions fund will not change the situation. If we are going to improve access to drugs, we need to find another way.

SMC does a good job but it can do only what it is told to do, which is to make a decision regarding cost effectiveness. The fact that there is a rare conditions fund in Scotland and a cancer drugs fund in England shows that there is a social and political will to provide access to drugs beyond that which is mandated by the SMC and NICE.

We have an opportunity in Scotland with value-based pricing. We have some strengths, and we should use the SMC as a route to get drugs rather than how it is perceived now, which, for clinicians, is as a bit of a barrier. We are a nation of 5 million people and we have fantastic data acquisition through the Information Services Division. On the cancer side, we are moving towards a single electronic chemotherapy prescribing system. Most of the drugs that are coming out have just been licensed. We have the potential to say to the pharma companies, "We could provide you with post-licensing data on safety and tolerability. On the basis of that, we want a good price for the drug."

Some of those negotiations would have to go on behind closed doors because, if we are negotiating a price that is specific for Scotland on the basis of what we are giving back, it may not be good for pharma for that to be broadcast. However, we should look at that sort of model in order to ensure that Scottish people get all the best new drugs. We would have to expand the SMC slightly, but as I understand it there will be a drug saving in the Scottish drug budget of about £316 million between 2012 and 2015. There is scope in that.

Professor Angela Timoney (Scottish Medicines Consortium): I have not spoken yet because I think that it is important to hear everyone else's views. I particularly liked what Professor Gourley was saying. We have not had a discussion about that issue.

You will see from the SMC submission that we have no desire to prevent people from having access to medicines; what we want is for people to access good medicines at a price that is fair for all. We really have to think constructively about how we are going to achieve that.

The recommendations in the report are good but they are about process. At the SMC we are concerned that all we are doing is putting in more processes. They might slow us down a bit, and we might get more resource so that we do not slow down, but they will probably not change our decisions—possibly a little around the ultra-orphans but not necessarily. Will that address the concerns that this committee has raised? I would like to look at a different way of working; we have to think about a different way.

This inquiry arose because of concerns from cancer patients and patients with rare diseases. There is a challenge in whether the Scottish Parliament wishes to treat those groups differently. You heard clearly from Professor Swainson and Professor Routledge that those groups should not be treated differently. I do not think that they should be treated differently, but we should find a way that is fair for all in relation to all medicines. We need to look at that.

Leigh Smith: I agree with Professor Timoney. Within its remit, the SMC does a superb job, and I was delighted to hear her come up with what I thought was one of my kooky notions that we should look at the real value that we get from

products, for example via post-marketing surveillance.

Scotland is not a huge country and we have four centres for cancer treatment, so it should be possible to collect the data on the real outcomes from these very expensive drugs. If the outcomes are not as good for our population, there must be a way for us to go back to the companies and renegotiate a price. We could perhaps say, "Can we have a probationary period? We will pay you that price for a year until we have a bit more knowledge of your product, and then we will sit down and renegotiate." I am delighted that Professor Timoney has come up with the same kooky notion.

10:30

Dr Macdonald: It is a difficult issue to comment on. The principle of looking at the long-term outcomes for medicines is welcomed by all, including the pharmaceutical industry, but it is remarkably difficult to get the information. In a prior life, I worked in the industry on some rare diseases, and we were desperate to get the long-term outcome data. There were a few specialist centres, but they could not agree on end points, the data systems did not work and so on. The industry would welcome the ability to get some long-term robust data.

The most recent pharmaceutical price regulation scheme—which is going to run out soon—contains some text that allows for flexible pricing in both directions, and the industry has signed up to that. The principle that we are talking about is not that contentious, but it has so far been a challenge to get it to work practically. The outcomes can go in either direction once the real data is collected, so we would agree with the principle.

Nanette Milne (North East Scotland) (Con): Much of what I was going to ask has been very ably discussed in the past while, but I will touch on value-based pricing. As you know, we had a briefing last week from the Department of Health on the plans for value-based pricing. Does anyone think that it will make any significant difference to the situation in Scotland that we are discussing?

Eric Low: The principles of value-based pricing are sound, and we have just discussed the need for a broader assessment of value in order to better understand the value of a drug to patients, to the NHS and to the taxpayer. However, there are some practical issues with the introduction of value-based pricing in Scotland. One could argue that, by default, what the SMC does anyway is a form of value-based pricing.

We need to focus—and this is consistent with the discussion that we have just had—on how we can work with the SMC on potentially producing a broader assessment of value that is based on the principles of value-based pricing. That would allow more informed decisions to be made about the value of a drug to Scotland, provided that we can agree on an appropriate price, because that is the issue.

Scotland now has an opportunity either to embrace VBP as part of the SMC's process, or to take the principles of VBP and modify some of its methods to undertake a broader assessment of value and have an additional discussion with industry—perhaps involving the Government—on price if the outcome remains a no.

Professor Timoney: I was at the committee's meeting last week for the presentation on value-based pricing. The situation is still confusing, and it is unclear what value-based pricing will mean for Scotland. It seemed that Katy Peters was saying that there would be free pricing at the point of launch to comply with the European transparency directive and that, thereafter, NICE would undertake a value-based assessment on behalf of England.

There seems to be an expectation that the launch price—the list price—will somehow be close to whatever comes out as the value-based price, and that that will be fine. However, as you know, NICE does not look at all medicines, whereas SMC looks at all medicines, so one could argue that we already assess the value-based price that has come through.

We perhaps need to undertake a wider assessment of values, and we may make some changes on that. However, if the outcome from SMC's perspective is that 30 per cent of medicines still do not meet their value-based price, what happens next? Katy Peters did not make that clear.

With regard to the medicines for which we say, "That is not a value-based price and it is not cost effective", is it proposed that we will go back and have a pricing discussion, or would it be a reimbursement discussion? If it is a pricing discussion it is reserved, but if it is a reimbursement discussion it is devolved. It is still not clear to me what Scotland's role is in that regard. It is important that we answer those questions, as that will determine whether we can get medicines at a price that is fair for all.

The Convener: Something about the issue dawned on me last week—apart from the confusion. Many people have been saying for some time—it has almost been convenient—that value-based pricing will be the single bullet, which will help us to resolve the issue. However, it was not obvious to me that whatever comes out of the discussions with the United Kingdom Department of Health and other bodies will be of any

assistance to us here. Why are we waiting for that? Why are we not just getting on with it and expanding the reputation of the SMC? Is there anything to prevent us from doing that?

Professor Timoney: The issue is that pricing is a reserved matter, and it is for the UK Government to determine.

When the idea of value-based pricing was brought up for the first time, it seemed to be different from PPRS. An element of health technology assessment—the assessment that we do—was suddenly being introduced into the pricing mechanism. If the choice is made to go for VBP, it is a reserved matter, and we will face a challenge in addressing that. We therefore have to see what VBP is going to look like, and I am still not clear about that. Clearly, some work has been done regarding the wider assessment of value, which might be of benefit for our decision makers in the SMC, but we have not seen that work yet.

Bob Doris: We are now moving to what I want to speak about; the earlier discussion was process driven because I was trying to elicit opinion on the two reports before us.

Last week, I was struck by what the witness from the Department of Health said. She seemed to say in one breath that value-based pricing is reserved but in the next breath that it is not. My understanding is that the consultation that started in late 2010 included discussions about value-based pricing. It was the committee's understanding that that was a major reserved issue that would have huge implications for the SMC. The issue seemed to be one of how we might develop modifiers in the future.

Last week, for the first time, I had the niggling feeling that there has been a hiatus for up to three years, during which we could have been getting on and developing modifiers, examining the appropriateness of cost per QALY, considering social care costs and pricing mechanisms, and having discussions with pharmaceutical companies about the time-release benefits for financial years 5, 6 or 7, about whether there could be a discount for years 1, 2 and 3, and so on. We could have been having discussions about those issues in relation to real, sensitive price modelling, but we have just been on hold for the past three years. Last week, a UK civil servant seemed to tell us that we could have been getting on with all that over the past three years.

I know that you have to be diplomatic in what you say, Professor Timoney, but I am interested to know whether you think that the lack of clarity from the UK Government means that we have wasted three years of our time waiting for something to emerge that is simply not going to emerge, and that patients and health boards in Scotland will suffer because of that?

Professor Timoney: I honestly cannot answer that question, because I do not know at what stage value-based pricing is currently at. Last week, Katy Peters suggested that there had been a lot of interaction with the SMC, but I did not recognise some of the statements that she made.

The SMC was involved, and we had a meeting. As you have said, VBP came out for consultation in late 2010. We were invited to a meeting at St Andrew's house on 2 March 2011, which involved the people at St Andrew's house, the Department of Health and the SMC. We discussed the consultation, and we then had a teleconference about two weeks after that, but we have not had any other discussions with the Department of Health.

We were invited to the technical workshops to which Katy Peters referred, which covered burden of illness, wider societal values and therapeutic innovation. Those were technical workshops at which people talked about the evidence, which in some instances was very conflicting. The workshops involved some of the technical challenges in trying to consider the wider societal benefits, but there was no outcome that allowed us to ascertain what those wider societal benefits look like. I am still waiting to see that.

Dr Macdonald: The negotiations between the relevant parties and the Department of Health are still continuing, and I am not party to them, but I have seen the consultation that came out at the beginning of the process.

A key point that is causing a lack of clarity lies in the question, "Value-based price, but which price?" Angela Timoney has already spoken about this to some extent. If it is the list price, a lot will have to change; if it is the reimbursement price, the methods will be developed, to an extent, by NICE and the SMC as they see fit. That is the key question about price.

Until the negotiations finish, we will not know the outcome. If it is the list price, that is a big change; if it is the reimbursement price, it is much a more devolved matter, and you can decide what is most appropriate for Scotland.

Eric Low: It is difficult for anyone to speak with authority on value-based pricing because no one really knows what is happening with it, but it is important to get a few things straight. First, it appears that VBP is not necessarily a pricing mechanism connected to the PPRS but part of a health technology value assessment. There appears to have been a change in thinking about that from the Department of Health in England. When the consultation first came out, VBP seemed to be very much a pricing mechanism

attached to the PPRS, but it has subsequently evolved to be something that NICE will do that will form part of a broader assessment of value. Those are two very different things.

As we know, pricing is the responsibility of the UK Government, whereas the assessment of value is the responsibility of devolved nations. Therefore, we need clarity on whether VBP is a mechanism for pricing attached to the PPRS or a mechanism for assessing value. If VBP is an assessment of value mechanism, it should be a devolved responsibility, which should give the SMC and the Scottish Government a bit of freedom to come up with a value-based price, by whichever means are deemed sensible and responsible, for the best use of our NHS resources.

If it is clarified that VBP is indeed an assessment of value mechanism rather than a pricing mechanism and is therefore devolved to Scotland, that brings us back to our earlier discussion about the need to sit down with the SMC to work out what we can do to improve methods to ensure that we have a broader assessment of value. We also need to have that all important discussion about what price point is acceptable to taxpayers, the NHS, patients and industry. That is really what the issue comes down to.

The Convener: One issue that has been put to us in evidence is that, whereas the difficult issue of access has partly been resolved in England through the cancer drugs fund, people here in Scotland are losing out. It might be right that we debate how to get the best value and best outcome for patients, but in the meanwhile we have a group of people who are being excluded from those drugs. That is one issue.

Another issue is the delay. It has taken our committee having an evidence session for us to discover that there might have been a change in direction in the UK Department of Health on whether value-based pricing is about wider societal issues or a pricing mechanism. It does not augur well that we are disjointed from the process, as seems to be the case now. That debate could go on for some considerable time with no discernible outcome for patients in Scotland. What do we do in response to that?

For example, we heard in evidence last week that the cancer drugs fund is expected to finish in 2014 but a final decision has not yet been taken. We also heard that those who have gained access to medicines through that fund will continue to receive those medicines beyond 2014. People in Scotland are being disadvantaged, but there seems to be an expectation that we will see no real value or difference. Indeed, we could have been getting on with things ourselves.

Eric Low: We cannot change what has happened over the past two or three years, but on the back of the good discussion that we have had—not about IPTRs and all that stuff but about the crux of the matter—and the meeting with Katy Peters, it is very clear what the two or three issues are.

Whether through the SMC or through the Scottish Government, we need to take the issue by the scruff of the neck and go back to the DOH in England to seek clarification on those points. We want to be in charge of our own destiny, as it were, in terms of the approval and assessment of, and access to, medicines. We do not want the divergence between access to medicines in England and in Scotland to widen in the short term because we have not understood how best to move forward. That would be worse than where we are at the moment.

The path is quite clear. If we can get answers to these questions, we need to come round the table again and work out with clinicians and the NHS the best way for the SMC's methods to be adapted to allow a broader assessment of value, consistent with our discussions, and have the all-important discussion about the price that is valuable. We must then have a mechanism whereby doctors have the freedom to prescribe according to the outcome of that process in the way that they believe to be best for the patient.

10:45

Professor Webb: I agree about the need for clarity from the Department of Health. Time is running out for it; the Office of Fair Trading reported some five or six years ago. The idea was to make a value judgment through NICE, the SMC and the All Wales Medicines Strategy Group acting together to determine which drugs created sufficient value for their market price. For those that did not satisfy the market price, a pricing unit would negotiate with industry at arm's length from those organisations to set a price that would give value to the NHS. That was the original purpose.

The cancer drugs fund was a stop-gap to take us through to 2014, when all that was to happen. If it is going to happen, it will have to happen very soon. If it is not going to happen, we have to go our own way. We are at just the wrong moment to make our own decisions about this, because we need to know what the DOH is going to do.

Drew Smith (Glasgow) (Lab): People who followed the evidence from last week's meeting will be aware that we questioned the Department of Health quite closely on what its engagement with Scotland had been, so it is confusing to the committee that there seems to be so much confusion about it.

Can we have some more information, principally from the SMC, about the structure of the engagement and who—in your eyes, Professor Timoney—was responsible for it? Was it SMC's role to keep a watching brief? I understand that you said that you did not recognise some of the statements that the DOH made about its engagement with Scotland. Whose responsibility in Scotland was it to understand what was happening at the Department of Health level?

Professor Timoney: SMC is based in Scotland and we report to the Scottish Government. That is my route of communication. It would not be appropriate for me to contact DOH directly; I would not do that. I would always go through the Scottish Government, which is right.

I presume that Katy Peters did not understand that, because she stated at the committee meeting last week that SMC and NICE regularly meet about VBP. We have never had any formal or informal meetings with NICE around value-based pricing.

Drew Smith: Do you mean that you would request such meetings through the Scottish Government or that you understood that the Scottish Government would make the points that the SMC would wish to make in such discussions?

Professor Timoney: The Scottish Government has to liaise with DOH on what is happening. In effect, DOH has responsibility for PPRS. That is where it has been left at present. I presume that the Scottish Government is involved in those discussions.

Drew Smith: The SMC responded to the initial DOH consultation. What sort of things did you ask for then? I presume that it would have become clear that what you had asked for did not appear to be coming out.

Professor Timoney: There was a public consultation, to which we responded to raise some of the issues. Part of our concern was that a lot of the stuff on value-based pricing was motherhood and apple pie, and we wanted to know how it would work in reality. We were trying to test some of the systems around how they would value burden of illness and so forth. That is why we have been involved in the technical workshops, and I am pleased about that.

I understand that there will now be a meeting between the Scottish Government, DOH, SMC and NICE, which I welcome.

Drew Smith: Do you know when that will be?

Professor Timoney: I do not have a date for the meeting.

Drew Smith: I have one other question on the SMC, which goes back to the recommendations in the Routledge report. It is on the issue of medicines of which assessment has not been made because the company has not made a submission. The recommendation is that SMC could look at such medicines independently, based on public information. How practical do you envisage that being?

Professor Timoney: It would be pretty challenging, actually. You should not underestimate the work that pharmaceutical companies do when they submit to us. To do a clinical and cost-effectiveness assessment would require us to build models, which we do not do at the moment; we critically appraise what the company submits. We would need significant additional resources to do that piece of work.

I understand why it is being suggested that we do it, because the feedback that we are getting from the service is that it would like that perspective from the SMC. One of the reasons why it has been suggested is possibly that it might encourage companies to think that they should make their own submissions, rather than having the SMC do them independently. We certainly could do it, but we would require significant additional resources.

Gil Paterson (Clydebank and Milngavie) (SNP): My question is on a similar line to the point that my colleague Drew Smith has raised. If my memory serves me right, in the very first evidence-taking session on the issue, value-based pricing was raised. The nearest thing to the response that I could describe is a shrug of the shoulders. No one knew anything about it. They did not know what impact it would have and they did not understand the reasoning, given that it was based on price.

We have quite a few knowledgeable people around the table. Last week, we fairly pushed Katy Peters about consultation in Scotland. It is important that we put on the public record exactly who was consulted. The minister also said that he had very little knowledge about value-based pricing and that he had had very little feedback from the Department of Health.

Would any of the witnesses like to comment on that? Do they know of any other agencies in Scotland that were consulted?

Eric Low: To put the communication and lack of clarity on VBP into context, NICE attended the workshops that Professor Timoney attended on behalf of the SMC and was equally in the dark not about the principles of VBP—everybody understands what it is and what it is trying to do—but about how it would work in practice. Even though NICE was subsequently asked to do VBP, at the time of the announcement it still did not know what was going on.

Despite there having been a consultation and workshops having been held, there has been an underlying lack of clarity about some of the practical and implementable aspects of VBP. It is not something to which only the SMC has been subject.

We and other patient charities have been urging the Department of Health to speak to the SMC and the AWMSG more proactively. It is safe to say that the department has been relatively dismissive of that and has said that it is a matter for the SMC and for the Scottish Government, which is consistent with the evidence that Katy Peters gave last week.

However, we should not unpick what has happened because we are all in the same boat. It is important to establish a platform based on current evidence for how Scotland moves forward. That is down to getting clarity about whether VBP is a pricing mechanism or an assessment of value, about what scope the Scottish Government has to embody its principles in SMC assessments and about the ability to have a discussion about price as part of an assessment of value. The launch price is something completely different. It is a matter for the Westminster Government.

We do not need to tie ourselves in knots about the complexity of, and the issues in, what has happened in the past, because that would drive us completely mad. We must take the matter forward from this point with clarity about what we want to achieve in Scotland. We must get from the Department of Health in England the answers to some simple questions and, on the basis of those answers, come back and put in place a process and system that work for Scotland.

Dr Macdonald: This may be seen as a slightly simplistic comment—at one level, it is—but do we really need to wait? The value question will arise in some form or other, so could some of that work be initiated before value-based pricing is totally on the table? Be it on the list price or the reimbursement price, we still need to know what Scots consider to be of value. There is a societal aspect to that, for example—the social cost aspect of what is feasible.

Perhaps at least some of the initial thinking can start, even if we do not know the final answer.

David Pfleger: I want to go back to the question of non-submission that Angela Timoney commented on and give a health board perspective. NHS Grampian would, I think, absolutely welcome the SMC taking a proactive HTA approach to non-submission, but it would require resourcing. Like other boards that commit clinicians and pharmacists to the SMC to undertake some of that work, we are acutely aware of the workloads involved. However, I think that the approach fits with the transparency and consistency that we are all trying to achieve and with the SMC's ethos of reducing duplication of effort, if not process, at local level. If we have a non-submission, we still have to deal with that through IPTRs, et cetera. It is important that we do not undermine the driver for pharma to submit, because we do not want anything that disengages the industry from submitting. We must remember that 30 per cent of medicines are not submitted to the SMC. I have commented on that here before.

If a company thinks that it will not achieve an acceptable cost-effectiveness level, then our earlier discussions about joint working are a really powerful base from which to start. It is about how we can work with the industry either to demonstrate effectiveness that it has not been able to demonstrate—we must remember that we are considering drugs that are very early in their lifespan—or on targeting, because if we can target a more appropriate group of patients and are clearer that they will respond, effectiveness goes up and issues with cost-effectiveness drop. There are only several points in the process that we can manipulate: we can bring the cost down; we can improve effectiveness or targeting; and we can change our willingness to pay. Those are the three things that are in the mix; we just need to remember that.

Professor Gourley: If the SMC was expanded and given more power to negotiate for us and to help us to access medicines, that would also potentially allow it to reassess medicines when they come off patent. If a drug company submits a medicine to the SMC and it is not approved, the medicine will eventually come off patent but, according to the rules as they are just now, even though the medicine might become a lot cheaper, it would still not be available in Scotland. Unless there is a process through which such medicines are assessed when the price—and therefore their cost-effectiveness—changes, the position will remain the same. However, that would all fit well if the SMC was given more scope.

The Convener: I think that Drew Smith wants to come back on a point.

Drew Smith: I wonder where the driver would come from for particular assessments to be taken forward. The suggestion is that it would be when NHS Scotland requested it. Is that based on the fact that there have been a number of IPTR requests and on clinicians in particular fields saying that they have an awareness of an issue and are concerned that it is coming from the boards? What would you see as being the drivers? How does the voice of patients fit into that?

Professor Timoney: David Pfleger said that 30 per cent of drugs are not submitted to the SMC. However, those are usually just additional

indications and not brand-new medicines; most new medicines are submitted. Even for those that are not submitted, what we have done in the past—just to give a sense of that—is to go back to the ADTCs, which are our constituency, and ask: "Is this causing you a problem locally?" They are usually able to tell us and will link in with their local clinicians and indicate medicines that they have not had a submission for. We have been challenged by how we can address that, but it would not be difficult to identify the medicines that have caused problems because the service did not have an SMC view.

Dr Macdonald: I am sure that the industry would be happy to work to try to find solutions, particularly for the brand-new medicines. However, I welcome what David Pfleger said, because the issue for many is that it costs a lot of money to make a submission. We have surveyed our members, who have told us that the cost can be from about £50,000 to over £200,000, depending on the complexity of the model. That does not mean to say that the NHS does not need the information, but it provides a bit of a disincentive for a company if they know that they are going to get a no. Some of the prior discussions about value and so on are relevant in this context. Clearly, however, if the NHS needs something, the industry would be willing to work to try to find a solution. However, that is slightly easier to say than do and the context is wider than just writing a document.

Professor Webb: This is just an observation. I am not sure what the annual cost of the SMC is at the moment, but I suspect that it is under £1 million. If it is to take on the role that you suggest, it needs the health technologists to do that—I am not sure that Scotland has them—and the money that goes to support the activities of the SMC needs to be increased by an order of magnitude.

11:00

Professor Timoney: That is a really important point. The SMC costs £1.2 million a year for 80 assessments a year, so each assessment costs approximately £12,500. The figures that Frances Macdonald was talking about, which are to prepare the model, can be anywhere between £50,000 and £200,000. That is a huge amount of money, and the NHS must decide whether it wants to spend its money in that way. We could do it, but it would be challenging. It costs NICE £160,000 to do a single technology assessment, so it is not as though anyone else can do it more cheaply than the SMC.

Aileen McLeod (South Scotland) (SNP): I have a small point on the issue of transparency. As you know, we talk a lot about the need for greater transparency to build trust and confidence in the process among patients. What are your views on the idea of publishing all the clinical trials data? That would be one way in which to get greater patient involvement, trust and confidence in the system.

Eric Low: There is a lot of work going on around the transparency of clinical trials data. I mean no disrespect, but the average patient is not going to be able to interpret clinical trials data. It is hard enough for doctors to extrapolate stuff from that. I am all for greater transparency, but it should be around process, as we have discussed. It is not going to increase confidence in SMC decisions. It may make them more visible, but I do not think that it will change those decisions significantly.

Transparency is not about the short pass or the long pass; it is about being pragmatic and sensible. The issue is more about focusing on the crux of the matter, and is the issue that David Pfleger talked about right at the beginning. Nevertheless, patients should have access to clinical trials data.

Dr Macdonald: The industry association would agree that clinical trials data should be published. Guidance has been put in place to ensure that the data is put on www.clinicaltrials.gov, but that has not always been done on time. Similar to some of what we have discussed this morning, the industry has now put in place agreements to audit and encourage that so that it happens. There is no disagreement with the principle that the results should be published.

Jackie Baillie (Dumbarton) (Lab): I want to take a step back. Notwithstanding all the technical detail that we have heard, the message has come across loud and clear that, although we have two interesting reports, they are essentially recommendations about transparency in the process and do not deal with the central issue of how we can improve access to medicines. I have heard nothing to suggest that the IPTR process is going to improve to let more people in.

Notwithstanding the SMC saying yes more often, which will take time and a different set of recommendations, I keep coming back to the fundamental issue of fairness. Some patients are considering moving to England to gain access to medicines that they cannot get here. Where in the recommendations is that issue resolved? Somebody mentioned the potential to save £300 million on our drugs budget. Could some of that money be pressed into play? If somebody were to offer a solution to the unfairness in the system, what would that be?

Eric Low: I think that that is right. We have been discussing how we can make the SMC methods give more yeses and have less dependency on IPTRs but at a price that

represents value, which is key. Some of the divergence between Scotland and England arises because England has slightly different mechanisms for getting to that point because of the CDF and so on. What we are saying is that, from this point on, we need to come up with our own solution that is not the CDF but is another mechanism. I think that we are all saying the same thing. We just need to get to the point. Let us get round the table and have the discussion, because we cannot have a widening of the disparity in access between Scotland and England. That would be terrible. Let us get on and have the discussion.

Professor Gourley: I back that up. Clearly, there is a big disparity, particularly with regard to cancer medicines, but I do not think that the creation of a cancer drugs fund is necessarily the best solution. The idea is to find a Scotland-specific solution that is fair for all conditions, but flexibility will be needed by the SMC or any organisation that sits outside it that is going to do the negotiation and try to get the best possible value.

We need more flexibility. To a certain extent, the SMC is hamstrung by rules. There was a good example recently in relation to a drug for ovarian cancer. Clinicians in Scotland had done a big clinical trial using half the licensed dose. It might have made the drug cost-effective, but the SMC could not assess that dose because it is only allowed to assess licensed medications. I would like it to be given a remit to speak to clinicians in Scotland and to be allowed that flexibility.

Rather than having a fund that sits outside everything, such as the cancer drugs fund, which patients with non-cancer conditions could consider to be unfair, we should have a more global solution. We need to start working for that now. At present, there is a big disparity and patients who have diseases that they want to get drugs for are talking about moving south of the border.

The Convener: I will take some comments from other witnesses before I allow Jackie Baillie back in.

Professor Timoney: On Jackie Baillie's point about the £300 million efficiency savings, you need to be really clear that the NHS is spending more money on drugs now than it did last year or the year before. That £300 million has tended to go towards paying for increased volumes of medicines for mostly older people. It is not as if there is a pot of money to use for cancer medicines, because we are actually spending more on medicines this year than we did last year and the year before, and that is right—it is an appropriate way to treat the patients.

Melinda Cuthbert: If we get value-based pricing and we do not have the disparity between the north and the south, we will still have medicines that will not be funded. I suppose the question then is what the Scottish Government is going to do to manage the expectations of the Scottish population. You might not have answers to that today, but we certainly do not want to be back here in about three years' time discussing the same issues. We have hung our hopes on the peg of value-based pricing, but there are still going to be expectations. There will be medicines out there that patients will want but which will also be found not to be cost effective under that system.

Richard Lyle (Central Scotland) (SNP): As a member who has recently rejoined the committee, I am coming slightly late to the debate. I would like to know Professor Angela Timoney's view on the comment that

"the SMC is hamstrung by rules."

Should the SMC meet in public? Should you invite manufacturers of new medicines to give evidence? Should you explore other innovative approaches? While I have sat here listening the SMC has been continually criticised. You rightly pointed out that the £300 million that was saved has to be spent everywhere else. To me, as a layman, value-based pricing means that a drug would come to Scotland at a decent price that we could afford, and we could then give it to our constituents or patients. That is what it comes down to. I mean no disrespect to any drug company. The companies spend billions on developing drugs, some of which work and some of which do not, and they may want to recoup the price. I note that drugs are cheaper in Scotland than they are abroad.

Value-based pricing should mean that my constituents can get the drug that they require at a decent price, and that we are not being overcharged by drug companies. I am interested to hear Professor Timoney's reply to my comments, because SMC is continually getting battered unduly.

Professor Timoney: Actually, I do not share Richard Lyle's views. I was really pleased by the Routledge report, and by the comments that I have heard in this committee and from consultants and others about the high quality and international reputation of our work. Speakers from abroad have said that they consider what the SMC says.

However, I understand that the situation is difficult. It is very difficult if we say no; people do not want to do that. I mostly hear people talking about how often the SMC says yes, but at some point someone must say, "It's okay to say no, because that's not a fair price for the NHS." The industry is entitled to charge the prices that it wants to charge to make the profits that it requires,

but somebody needs to say when a price is more than we are prepared to pay. That is a tough job, and we take it seriously. Our committee makes very difficult judgments based on evidence and every single one of our committee members is, when they make decisions, thinking about the patients whom they see day in, day out.

Frances Macdonald, as a member of that committee, will acknowledge that, every time we say no, we tease out the evidence first to see whether we can find a way to say yes. At the end of the day, however, we may have to say, "I'm sorry, but this is not value for money", and we stand by the decisions that we make. We are not "hamstrung by rules", but it is important that we have rules, because we must treat every drug in the same way and give every patient the same chance, so that there is equity.

I am happy to meet in public, but my concern is that the prices in patient access schemes are confidential. Our most controversial decisions tend to be associated with drugs that are part of patient access schemes, so I am concerned that when we come to discuss the drugs that are really controversial we would have to ask people to leave the room; we would be criticised, although the reason is that it is a commercial issue.

Richard Lyle: Just a small comeback—

The Convener: I will indulge you, Richard, but Jackie Baillie is next.

Richard Lyle: Who decides the price of a medicine? Is it the drug company, or does the SMC have a say? Does the company just say, "Right—it's going to cost £X", and you have to say that you are not prepared to pay that?

Professor Timoney: No. What happens is that there is free pricing at the point of launch. The SMC has no role in negotiating a price; we are not able to do that. We simply assess the price and the submission that is made to us.

Richard Lyle: Should you have a role?

Professor Timoney: We cannot have a role, because that is part of the PPRS. Pricing is a reserved matter.

Richard Lyle: I knew the answer before I asked the question.

The Convener: If we had time, it would be nice to have the conversation, but we need to press on. Jackie Baillie has a supplementary.

Jackie Baillie: It is just a tiny point. I accept that the SMC operates by a set of rules that it is given, but it strikes me that we have had two reports on the wrong issues. The discussion that we want to have is being had round this table. I hope that the cabinet secretary will reflect on it, because I know that he pays close attention to what the committee says. However, given the time that it will take to get there, what do we do now?

The Convener: Does anyone want to come in on that?

Jackie Baillie: What do we do now for the patients who are making life-changing decisions?

Melinda Cuthbert: From an NHS perspective, we have been given the rules and regulations to follow. If the SMC decides that a drug is not recommended, the only avenue that we have, unfortunately, is the IPTR route. Unless something was to change, there is no other way for us to treat that issue.

The rare conditions medicines fund will be issuing guidance on the matter, but the request still needs to go through the IPTR or group process, and the onus is on us to show how the patient or individual in question is different from the population that was assessed. One of the things that has led us to this point—and one of the reasons why we are discussing the issue today—is the misconception that the IPTR route is a means of accessing medicine regardless. That is simply not the case.

11:15

Professor Webb: I certainly do not have an answer to the question—I am not sure that anyone in the room does—but I will say that the SMC has functioned very effectively for the past 12 or 13 years, and that it would be very harmful if any short-term measures were to destabilise its activity. To put in a stop-gap would be very difficult; the cancer drugs fund has not worked tremendously effectively and I do not think that it will be renewed. Given that, I would guard against making short-term changes and suggest instead that we deal with the matter head on.

Professor Gourley: I agree, but the fact is that inequity remains and that the period of time that we are talking about is a source of extreme distress to patients. The rare conditions medicines fund has been created, but the problem is that it is good only for rare conditions and many of the patients whom we want to treat have common conditions. I cannot think of anything that would be more palatable than a stop-gap fund, but obviously such a move would be very controversial.

Leigh Smith: My concern is that, if anything, the cancer drugs fund has stymied things and muddied the waters because the companies got the price they wanted from England, which made us look bad and mean. It might have been better if that had not happened, but we cannot change the situation.

I have to say that we would not have the patient access scheme, where drug companies can go

with a better price, if the companies were not using the fund. I have been cross with companies that have developed products for melanoma patients but which have refused to go with a better price. People say, "There's nothing we can do", and the SMC says, "We've got no more information", but I say that that is rubbish. The companies can drop their price to one that is agreeable. We need to say to them that although their drugs might be effective and that they might, on balance and under the circumstances, be reasonably safe, their prices are off the wall and so they are not going to sell the drugs in Scotland until their prices drop. There needs to be a place for such negotiations and for people to say, "We want your drug, but as patients we are not prepared to be held to ransom."

Richard Lyle: Hear, hear.

Dr Macdonald: Oh, dear.

I, too, think that the SMC does an excellent job and believe that there must be balances in the system, but we should bear in mind the context, and the fact that it costs a lot to develop medicines. Moreover, the PPRS controls profit within the UK and there have been a lot of redundancies in the pharmaceutical industry. As a result, we should temper our comments about the industry. I hope that we are developing a lot of medicines that meet a lot of needs—which is, to some extent, why we are having this discussion. A balance should also be struck on the side of the pharmaceutical industry.

Professor Timoney: A lot of this discussion has been about what the SMC has said no to, so it is really important that we remember some of the things that it has said yes to. For example, since I have been chair of the consortium, we have said yes to drugs for hepatitis C, which is a major public health issue. Those drugs were incredibly expensive—their estimated cost to NHS Scotland is £50 million at year 5—but they were also incredibly beneficial. We also said yes to all the new oral anticoagulant medicines that will prevent stroke in patients; we said yes first time to three new drugs, the costs of which are estimated to be about £20 million at year 5. That is £70 million for two sets of drugs. We are prepared to pay a high price for medicines that have great benefits. We should not lose sight of that.

To some extent, it seems to be that people think that all medicines have benefits and that when they are not provided it is because of the cost, but that is not the case. When the cost and benefit are clear, even though the SMC might be causing an affordability problem for the NHS—it is an awful lot of money for the NHS to find—we will still say yes because it is the right thing for patients.

David Pfleger: It would be very easy to say yes to a cancer drugs fund or to any fund that would fund all the drugs that we have deemed not to be cost effective because that would take a lot of issues out of the system. However, affordability and value for money must be questioned. We must remember that boards carry the onus of demonstrating value for money to the silent majority of the population in the board's area who are against using medicines for an individual patient through the IPTR. Boards have been doing the best that they can with that task, but the cancer drugs fund down south has taken all that away because it basically means that non-cost-effective medicines will be paid for.

We also need to remember equity. The clue is in the title—it is a cancer drugs fund. What about all the other conditions for which there are non-cost-effective medicines? Before any response is made, I want to see something that addresses that equity issue. Cancer is emotive and media friendly, but there are lots of other conditions out there for which there are non-cost-effective treatments that patients feel that they need access to. There is a need to address that across the piece.

The Convener: The cancer drugs fund is routinely funding non-cost-effective drugs on the QALY model.

Professor Timoney: Yes.

David Pfleger: That is what it is designed to do.

The Convener: Does anyone know how much we have spent on the cancer drugs fund? Has it been fully utilised?

Professor Webb: There is £500 million, I think, and it has not been fully utilised.

The Convener: The fund has solved a problem.

Professor Timoney: It has taken up the front pages of newspapers. Is that solving the problem?

The Convener: Is that not what the recent announcement on the rare conditions fund did, too?

Jackie Baillie: You could say that, convener, but I do not think that the witnesses can answer that question.

The Convener: Bob Doris has a question.

Bob Doris: Thank you, convener, for bringing me in at this point.

What I have to say is part statement and part question. I say to Ms Baillie that the two reports from Professors Swainson and Routledge flow into a lot of the committee's work, particularly on rare and orphan conditions and the transparency of the SMC process. Whether or not they reach the parts

of the overall view of access to new medicines is a reasonable discussion to have, but we should not diminish the fact that the reports are in part a direct response to the diligent work of the committee and the petitioners who came to the committee through the Public Petitions Committee. It is important to put that on the record.

We have also heard about the cancer drugs fund. Sometimes that is viewed in isolation from how our society or the NHS deals with money that is spent on cancer or any other life-limiting or terminal condition. When the SMC says no and an IPTR is refused, no explanation from a politician will cut it with an individual who has cancer and the family who love that individual. We must accept that, no matter what rules we have in place. There will always be situations in which people and their families are told no. Irrespective of how we change the system, those who experience directly the heartache and pain will not be appeased.

Sometimes, when we mention the cancer drugs fund, we forget about cancer prevention, early detection, and curative work. We go to the end-of-life aspect and how distressing that is for families, but in so doing we miss the bigger picture. Unfortunately, the bigger picture sometimes has to take emotion out of the equation and rely on evidence. As a politician, I hear about such issues weekly at my surgery, so that is a horrible thing to have to say.

How are patients who receive access to the cancer drugs fund treated compared to patients who have other terminal conditions? There are myriad patients who have other life-limiting conditions and whose quality of life will be seriously damaged if they do not receive a drug, whether or not it is deemed to be cost effective. Where is the balance between the cancer drugs fund and treatment of all patients who have heart-rending sensitive stories to tell—not to mention the stress that the situation puts on those individuals and their families? How does the cancer drugs fund create an equitable basis to deal with all patients?

The Convener: I need brief responses, please.

Eric Low: It seems to me that we are taking a backwards step in our discussion about the cancer drugs fund. I think that we have all decided that it is not the way forward; England has realised that it is not the way forward. The fund is not the solution, so I question whether we should use our time to backtrack and discuss the cancer drugs fund.

The previous discussions have set out the issues clearly. We are all united in that—that is what we need to focus on. There is a willingness round the table from industry, MSPs, the SMC, clinicians and patient groups to find solutions to our problems. The solutions have to be fair for all—for industry, the NHS, taxpayers, patients and clinicians. That should be our focus and I see no point in talking about cancer drugs funds and IPTRs. Such a discussion is not the solution, but will just remove the clarity that I think we reached half an hour ago. All our efforts need to be focused on finding our solution to our problem in such a way that everybody gets out of it what they need.

The Convener: I see a lot of nodding around the table. Is there general agreement about that?

Witnesses: Yes.

The Convener: Drew Smith has a final question.

Drew Smith: I may not get an answer to my question and it may lead us into the next evidence session. It goes back to Jackie Baillie's point about the questions that are answered by the two reviews and their reports. We are trying to focus not only on the wishes of the petitioners—which is where we started—but on the evidence that we have heard.

We were very concerned by evidence about clinicians and their experience of clinical practice, and about the issue of clinical research as a result of where we are on drugs in Scotland. There are not many recommendations on that in the reports, although we may hear more in the next evidence session. I want the health boards to say whether there are recommendations that would begin to answer some of the questions or whether, on the other hand, their position remains that the concerns are overblown and that we therefore do not need recommendations on them. If it is suggested that we need further work to be done on value in the process, do we also need to do further work on clinical practice and clinical research?

Professor Gourley: The Swainson report suggests that access to the latest clinical trials is not being restricted by inability to access drugs that are now regarded as being an international standard of care. The rationale that is given in the report for that is that the number of commercial studies in Scotland has not decreased since 2010. However, the report states that the number of big phase 3 studies has gone down. There is absolutely no doubt that a number of examples of clinical studies cannot be done in Scotland because we cannot provide what is regarded internationally as the standard of care—we cannot provide the standard drugs. If you introduce a trial to test a new drug, you are testing it against the standard. We cannot access the standard.

By the time that shows up in clinical trial activity data, we will have lost our position at the forefront of clinical research. That is really important for

Scotland. The clinical research and trials issue is a massive one; it is also a massive one for patients because patients who get on to clinical trials benefit from those clinical trials.

11:30

The Convener: Are there any other comments on or responses to that?

Leigh Smith: I, too, am concerned about the fact that phase 1 and 2 trials are continuing but we are losing out on phase 3 trials. I find that to be almost objectionable. It means that our patients are being used as guinea pigs for fairly untried stuff, but are not getting the benefit of the phase 3 trials, which is when we know the dose and so much more about the drug. That really is very worrying and I do not have an answer to the issue.

David Pfleger: We also need to tease out issues about the cost of running phase 3 trials, which is where we become less cost effective—less cheap, in a sense—for the industry. Some of our earlier discussions about post-marketing studies might resolve issues that have been described and might make up for the fact that it would be more expensive to do phase 3 trials here. Solutions other than just having access to the drug at the point of licensing might help, if that makes sense.

Professor Gourley: It is also worth pointing out that the relationship with pharma and big international phase 3 studies can bring a lot of income into Scotland; that could be another point of negotiation. Some clinical trials actually save the NHS money because they provide mechanisms that clinicians can use to access drugs that they cannot access routinely. That must also be thrown into the equation.

David Pfleger: I will back up that comment. It is absolutely clear that we make savings through trial activity—specifically on medicines, let alone anything else.

The Convener: I must bring the session to a close. I thank you all very much for the time that you have given us this morning and throughout the inquiry. We have got the message that a number of issues about the review and its conclusions still need to be resolved, but there is a lot of good will to create a better situation. Thank you all very much for your attendance.

11:32

Meeting suspended.

11:40

On resuming—

The Convener: I welcome everyone on our second panel. I propose to do as we did for the first panel. I will not ask everybody to introduce themselves at this point—many of us know one another, although I did not emphasise that during the first session—but the witnesses may wish to introduce themselves when they make their first contribution. That should cut down on the time. We therefore move directly to questions.

Richard Lyle: I am very impressed to see on our second panel doctors and others who are at the sharp end of this disease—the big C—that is unfortunately with us, which can come in many forms. Many of the witnesses around the table sat in the public gallery during the first evidence session, and I am interested to know what they learned. Do they have anything to tell us? Perhaps they wanted to respond during the first session. How do they feel about the SMC?

The Convener: That is a wide-ranging question, which should prompt someone to respond. In the first evidence session, there was a lot of agreement on many issues, but we do not know whether that agreement will continue or how the witnesses' thoughts will contribute to the process.

What was the witnesses' response to the review? Do they believe, as many of those on the first panel did, that the review was well meaning and made some good proposals about openness and transparency, but that it did not get to the heart of the matter?

Lesley Loeliger (PNH Scotland): I am a patient with the ultra-orphan bone marrow disease paroxysmal nocturnal haemoglobinuria, or PNH for short. I represent the charity PNH Scotland and the UK-based organisation, the PNH Alliance.

We absolutely welcome the Routledge report, and we completely concur that the SMC is internationally recognised as being excellent. However, we have an issue, as did the report, with how ultra-orphan diseases are dealt with. We were particularly grateful that the report acknowledged the term "ultra-orphan". As you will probably remember, we have been asking for the term to be recognised for quite some time.

We are interested in the IPTR system and in the rare conditions medicine fund and the issues that still surround it, which were not necessarily brought out in the report.

Ian Mackersie (aHUSUK—A Patients and Families Support Group): I represent aHUSUK, a charity and support group for patients in the UK with atypical haemolytic-uraemic syndrome and their families. It is an ultra-rare disease—we think

that there are about 20 to 25 patients with aHUS in Scotland. My interest is in ultra-orphan drugs and orphan drugs in the UK. I have a particular interest in access to a monoclonal antibody called eculizumab, which can be used in the treatment of aHUS.

11:45

Our general view of the report is that it was well researched, well argued and well written. The recommendations that relate to the areas that are of concern to us are useful, particularly Mr Routledge's recommendation 5, on page 25, that the

"SMC should develop a policy … to guide the process of consideration of all available evidence relevant to its advice on"

ultra-orphan medicines. No such thing has existed before, and ultra-orphan medicines have struggled to get past the SMC, so we regard that as a positive move.

We also strongly support Professor Swainson's recommendation 12, which is that the rare conditions medicines fund should concentrate on funding

"access to medicines for ultra-orphan diseases."

If it is administered independently in the way that he suggests, it will give fair consideration to ultra-orphan drugs that the SMC does not recommend.

Although it is somewhat vaguely expressed in the report, it seems that a group PTR could contribute to the funding of drugs that have not got past the SMC.

Our general position is that, where the reports relate to our interests, they are most welcome.

Dr Stephen Harrow (Beatson West of Scotland Cancer Centre): I am a consultant at the Beatson west of Scotland cancer centre.

The SMC report was fair. All the consultants with whom I work understand the complexity of the situation and are sensitive to the fact that all the drugs are expensive and that we must consider cost effectiveness. Transparency is always a good thing.

The IPTR report was really disappointing. It does not change my practice at all. We are still in the situation that we were in before the report was published, in that we have a difficult system to navigate and the recommendations will not provide any greater access to medicines that have not been through the SMC or which it has turned down.

Natalie Frankish (Rare Disease UK): Rare Disease UK would whole-heartedly agree with everything that has just been said. Although the reports are completely welcome and make some good recommendations, particularly in relation to the SMC policy on ultra-orphan medicines, there are some glaring omissions.

We want to ensure that the IPTR issue is addressed because the review will not change anything. Rare disease patients will still not be able to access medicines even though a fund is now in place. Our committee will need to take that forward.

Vicky Crichton (Cancer Research UK): I definitely agree with what was said in the discussion that the committee had with the first panel. In the areas on which the reports focus—ensuring that the system is equitable, evidence based and transparent—most of the recommendations are welcome, and some will be incredibly helpful for improving public awareness of, understanding of and trust in the system. However, I also agree with what has been said about the fact that there is not much in the reports that will increase access, which is almost a separate discussion.

Cancer Research UK wants to flag up some particular points about some of the recommendations, but I can come back to those.

Dr Richard Casasola (NHS Tayside, University of Dundee and Scottish Cancer Research Network (East of Scotland)): To reiterate the point, the review addressed the ultra-orphan situation extremely well but does not take us desperately far forward in how we manage cancer patients for whom there are drugs that are currently not recommended.

George Grindlay (Angus Long-term Conditions Support Groups): On the whole, the reports contain good recommendations but the IPTR system needs to be examined.

According to information that has been given to me, the IPTR process will still take a long time, and it will still take a long time for the SMC to get information to health boards. It is difficult for a person with a long-term condition to go through that process and to have to wait a long time to get a yes or no answer on the IPTR.

Joan Fletcher (Pompe Group of the Association for Glycogen Storage Disease (UK)): I am here to represent the Association for Glycogen Storage Disease, which is a patient support group that also covers Pompe disease. We were one of the original petitioners, along with PNH Scotland and Rare Disease UK. We very much welcome the work that the committee has done. I agree with and echo what has been said about the SMC. We are quite happy that it will take the issue further, and we very much welcome the transparency that it has spoken about.

However, although there have been discussions around IPTRs, we still believe that the requirement to prove exceptionality is a stumbling block. We believe that the drug that is used for Pompe disease—the enzyme replacement therapy—is effective. We are aware that it is very expensive, but it has been proven to be effective. How will proving exceptionality—saying that patients are different from those who were on the trial—make any difference? Why should we prove exceptionality when we say that the drug works? Why should the patients whom we want to use it for be any different from the patients who were used for the trial?

The Convener: Is that apprehension born of real-life cases that have tested the system? Can you test the system at this point? Is your apprehension based on your expectation that nothing will change?

Lesley Loeliger: The IPTR system as it stands—and as it is described in the report—is based on exceptionality, as Joan Fletcher said. Twelve patients in Scotland with my condition have been recommended to be on my drug, but three of them still require funding. It is almost impossible to prove exceptionality in such a tiny patient cohort. As I understand it, only one person was on the drug trial. It is very difficult to be different in that regard.

We cannot get through the IPTR system. Alex Neil, the Cabinet Secretary for Health and Wellbeing, said on the radio yesterday that IPTRs are based entirely on clinical effectiveness, not cost. However, it is very well documented that my drug, eculizumab, is 100 per cent effective for the patients for whom it has been recommended. It gives back a normal life expectancy to patients such as me, and therefore gives immense benefit. However, patients are turned down both at the initial IPTR hearing and at appeal.

A patient contacted me yesterday to say that she had been turned down at her initial hearing and has been through an appeal, which she is waiting to hear about. At both the initial hearing and the appeal, the drug was deemed to be clinically effective, but she was still turned down at the first hearing. I do not understand what the disconnect is.

Dr Casasola: We discussed that very drug recently at IPTR level, at which middle management and senior clinicians are represented. We agreed that the drug is effective—there is absolutely no doubt about that—but were reluctant to give the agreement to go ahead with its prescription because of the sheer cost. We agreed that, because we have an established appeals process, it should go to appeal. We also agreed that, because of the cost, the decision should be made by the medical director—in Tayside, as it happens. It was an issue of cost: the group was nervous about letting it through.

Lesley Loeliger: My slight issue is that there seems to be a disconnect. The Government portrays the reasons as being about clinical effectiveness, not cost, but the health boards think, "My goodness—the cost." I understand that. The problem is the disconnect, which is where patients find it difficult to understand and cope.

Dr Casasola: It is the magnitude of the costs. I do not know whether people are aware that the cost of a year's treatment of eculizumab is £250,000. That is why a local group is nervous about passing that drug.

Joan Fletcher: We appreciate and welcome the transparency that we have talked about, but we also welcome transparency for the patients, particularly around why they have been turned down. I fully understand and accept that there is a limited amount of money and that, because there must be cost effectiveness, not all drugs can be accepted. However, patients have been told that they are not getting a drug because of the cost. The letter that was sent to one of our patients when her appeal was turned down stated that that was because of the

"impact on the QALY cost, and subsequently on the opportunity cost implications for NHS Ayrshire and Arran."

So she had been turned down because of the cost implications for the local health board. Alex Neil has said that decisions about drugs are made on the basis of clinical effectiveness and not cost. The information is not transparent for patients.

The Convener: If we accept that cost is an issue and that there must be good value for the patient and the national health service, how can we make the process better? Should the transparency just be around telling people that there is a budget for prescribing and that there is simply no money to provide a particular drug?

Lesley Loeliger: That would be fine if there were no inequalities between health boards. I have reservations about saying this, but I would rather that none of us got a drug than that some of us could cherry pick a drug that had proved to be clinically effective.

Dr Harrow: The issue would not have arisen if there was not a cancer drugs fund in England. When I became a consultant, none of the drugs that I now deal with was offered across the UK, so they did not come up in clinics. The position would probably have evolved so that they would have gone through the SMC.

The way to access the drugs is to go through an IPTR. People have said that there is exceptionality, but exceptionality has now come

out of the IPTR. Basically, we are now being asked to ensure that the patient for whom we request the drug is different from the trial population. We must also demonstrate that the patient will do better than the trial population. So, clinicians are being asked to come out of the existing evidence base and try to present scraps of evidence that might show that the patient is different, but on a clinical ground only—that is all that is acceptable now.

The IPTR has a section where patients are supposed to make a statement, but I think that that is just cruel. Given that, as Alex Neil said, the decision is made on clinical grounds only, what would a statement add? For a drug for only 12 patients in Scotland, it will be impossible to try to tease out somebody who is different from the trial population and more likely to benefit. Even when we give that information, my experience has been that the drug is still turned down. I do not know where we go from here, but the IPTR process is not functioning as it should.

12:00

I agree that the decision making comes down to cost. However, the Swainson report commented that a lot of doctors could not understand the process—I agree that that is probably true—and that somehow we should be able to seek advice from specialists. With all due respect, we are the specialists, yet although we know the patient, the condition and the literature and are able to put forward the data, we are not involved in the decision-making process, which is taken out of our hands and taken over by management. That is wrong. We should definitely be sitting at the table, helping to make the decision.

Vicky Crichton: I want to go back to the discussion with the first panel. Although some aspects of the operation of IPTRs can be improved, this is not about putting in place an ideal IPTR system, because that is never going to provide population-based access for patients. The system is, by definition, designed to provide access to a small number of unusual patients. Although we need to improve that system for those patients, we also need to find out whether we can improve the SMC approvals system and ensure that people do not have to use IPTRs and do not see them as the answer. The fact is that they are never going to be the answer, and we should instead focus our efforts on the wider debate about value at the SMC level and on examining those processes to see whether we can improve the system for the vast majority of people.

George Grindlay: I agree with Dr Harrow that he should be present to support a patient's IPTR application. As we have heard, the decision comes down to cost, but if no one is physically present to speak for the patient it will go against the patient every time. For example, a request for an ultra-orphan drug for which specific exceptions cannot be provided does not go anywhere and is refused funding. I find that really sad for those who have ultra-orphan conditions.

Drew Smith: How would you ensure more clinical involvement in the decision-making process, particularly in cases involving ultra-orphan drugs? Is the issue simply that clinicians need to be more involved in the existing process, or is there a need for a new process? Should we entirely separate the issue of cost from such judgments, with, say, a solely clinical judgment being made by a clinician from another health board after they have examined the case? Is that a step too far?

Lesley Loeliger: Although I absolutely accept that in many regions there are amazing experts who deal with cancer, for example, I note that there is one expert for Scotland who deals with my ultra-orphan condition. I feel that, as was recommended in the Swainson report, the IPTR process needs a body of recognised experts, either from Scotland or from further afield if necessary, from whose exceptional knowledge of these rare conditions patients can benefit.

I know how expensive my drug is and I am incredibly grateful to have it, to have a life and not to have to claim benefits and so on. However, I would like to think that an extreme specialist in an ultra-orphan condition would be able to bring to the IPTR process or even an SMC hearing an understanding of the cost offsets. For example, I do not have to go through blood transfusions every six weeks, kidney dialysis and so on—all such elements could be considered as cost offsets. The drug is exceptionally expensive but there are many ways in which I am now not going to be a burden on society.

Ian Mackersie: With regard to the assessment of ultra-orphan medicines, there was until very recently a perfectly good system called the advisory group for national specialised services. However, it was disbanded in March and the HTA role with regard to orphan drugs was given back to NICE, which is presently undertaking a full review to develop a policy and process for making decisions about how those drugs are dealt with. If anybody were looking for a model on which to base such assessments, AGNSS would be the place to look. However, as I said, it has been disbanded.

On co-operation among the home nations, given that access to ultra-orphan drugs is under detailed review in three of the four home nations, is there not a case for real co-operation between the health departments in Scotland, England and Wales to find a common definition, a common

assessment policy and a common HTA process for orphan and ultra-orphan drugs? If that were to take place, surely negotiations with pharma would be easier; certainly, the smaller nations' negotiating position would improve. Until that happens, the divergence and disparity in how ultra-orphan conditions are dealt with in England, Wales and Scotland is likely to continue.

The Convener: That process was an earlier theme. It was suggested that, although we require resource, we have in our own hands in many areas—although maybe not in rare orphan diseases—the capability to move on with this work, recognising that the SMC's remit would need to be broadened and recognising that it has been difficult to keep apace with what, if anything, is happening with value-based pricing down south. Would that view be echoed among the panel in terms of how we could improve access and get more yeses, as someone said earlier?

Dr Harrow: The drug that I am keen to use has not been submitted to the SMC because it was part of a multiple technology assessment by NICE and NICE decided that the drug, along with two other drugs that it took it upon itself to review, was not cost effective. The drug company will not submit the drug to the SMC because it has already been turned down by NICE, which is the English equivalent of the SMC. I understand that, when NICE does a multiple technology assessment, that applies to Scotland.

In England, NICE turned down the drug but English patients can get the drug through the cancer drugs fund. In Scotland, the drug will not be submitted to the SMC but I am bound by the NICE recommendation and I cannot navigate around that. I have tried to navigate around it through the IPTR process. We, as a team of colorectal cancer consultants, all tried to sign the IPTR request to show that we were united behind the drug. I also got the surgeons to agree to the proposal for the patient. However, the request was turned down at the IPTR level. In the appeals process, we sought advice from 10 specialists across the UK who were using the drug and I presented that advice at the appeal. However, that did not carry any weight although they all agreed that we had met the criteria in the IPTR process. In addition, I had supporting documentation from the professor of medical oncology and the professor of translational research saying that I had met the criteria in the IPTR process. When that was turned down, I appealed the appeal decision with another letter from the professor of medical oncology, but again that was not deemed to be sufficient.

I am at a loss as to how I can access these medications for patients. I feel that I have done everything that I can do within the system and have sought as much advice and support from eminent colleagues around the UK as I can to support the application.

The Convener: Does the SMC need more flexibility to allow it to look at that, or was it bureaucracy that prevented it from looking at that?

Dr Harrow: I do not know. All I know is that I used to be able to get the drug, and then NICE carried out a multiple technology assessment of it, which stopped me being able to access it. Such an assessment applies in Scotland—in England, patients can circumvent the NICE guidance through the cancer drugs fund, whereas I am still bound by it here.

Vicky Crichton: There is a disparity in comparison with access in England because of the cancer drugs fund. We have always argued that we need improvements to the process in both England and Scotland so that we do not have additional processes tacked on the end. We need some of the drugs to be approved where they are deemed to be cost effective or where we decide that there are benefits from those particular treatments, and they should be valued at a higher level, which goes back to the discussion about value.

The Convener: We have just heard a story about the clinical expertise of front-line professors and the SMC's inability to respond. Is it the SMC's role to do so, or do we need to create the flexibility to allow it to look at those things and respond to that sort of recommendation, as was suggested earlier?

Does Jackie Baillie have a supplementary?

Jackie Baillie: Yes. I want to understand and appreciate the story. We are told—quite rightly—that clinicians and not politicians should make the decision. Lesley Loeliger was right to say that there is a disconnect between what is said—that it is not about cost—and people's real-life understanding.

I want to tease out something with Dr Harrow. Did you say that you sought agreement first from your own cancer team?

Dr Harrow: The Swainson report suggests that we should try to get some expertise, but in the cancer centre we work in multidisciplinary teams anyway. We collectively agree on protocols and management plans, and meet weekly to discuss complex cases, so we have a consensus.

Jackie Baillie: So your colleagues agreed with the IPTR that you were going to put in.

Dr Harrow: Absolutely.

Jackie Baillie: You then got 10 consultants with expertise in the field to agree.

Dr Harrow: Yes. We proved that the patient was different from the trial population, and that they were likely to gain more benefit than the people in other studies that had been published. We sought advice from consultants around the UK with expertise in that drug and that disease, and they all concurred with our finding.

Jackie Baillie: And that was backed up by an independent expert, in the shape of a professor.

Dr Harrow: It was backed up by Professor Evans at the Beatson.

Jackie Baillie: Okay. If a decision is based on clinical opinion, and the overwhelming weight of clinical opinion was in favour of the patient receiving the treatment, who is on the IPTR panel making the decision?

Dr Harrow: An IPTR panel was convened, which included the medical oncology clinical director, a general manager and a pharmacist. The appeals panel included the medical director and a layperson; I cannot recall who the other person was. The third appeal—because I appealed the appeal—was a virtual process, and I cannot remember the constituents of the panel.

Jackie Baillie: Would it be fair to say that, if clinical opinion is to decide the matter, the overwhelming weight of clinical opinion was in favour of treatment, and yet the IPTR process resulted in a rejection?

Dr Harrow: Yes.

Jackie Baillie: Okay.

Dr Harrow: I submitted evidence in written format and I went along to the initial appeal to give a verbal account of the situation, but I was not involved in the final decision-making process. That is where we need some inclusion of clinicians.

Jackie Baillie: Given the way in which you have approached the situation, the issue is not whether you, individually, should necessarily be included, but that you have tapped into a network of specialists and they have all agreed. Nobody has disagreed.

Dr Harrow: Nobody has disagreed.

When you get rejected, you think, "I have got something wrong," so you seek advice and support. We have done that consistently. It is not just me who has been doing that. It got to the point where we knew that we were not making progress with IPTRs, so we decided, as a group, to co-sign all applications.

Jackie Baillie: So if the issue is about clinical effectiveness, that demonstrates where the system is not working.

12:15

Joan Fletcher: I echo what Jackie Baillie has said. We had the same situation. Unfortunately, we are not as fortunate as Dr Harrow is in having the support of a number of full multidisciplinary experts.

However, for our appeals, two world-renowned experts gave advice. On two occasions, different experts advised that, on clinical grounds, the patient would benefit from the treatment, but we were still rejected.

Jackie Baillie: Was it about money?

Joan Fletcher: It would appear so. The patient and the others who were involved felt that way.

Bob Doris: I want to tease out some of this.

Dr Harrow, it is clear that you have tried incredibly hard on behalf of your patients to give them access to the medication in question. You said that you proved that your patient was different from the trial population, but that a final decision was taken, to which you were not party, that that was not the case. I assume that others made the same judgment call about whether the patient was different from the trial population and that, for whatever reason, they looked at the evidence and decided that that was not the case.

I fully appreciate your frustration about that, but I am trying to be clear—

Dr Harrow: I think that the phrase that was used was that the patient was not "different enough". At no point in the IPTR process are people asked to quantify the difference. They are just told that they need to prove that there is a difference.

Bob Doris: That is extremely helpful. That allows us to get more information about how that process has been dealt with.

Do you think that your presence at the final decision making, whether in an active or a passive role, would have been an informative addition to the process? From what you have said, my understanding is that you submitted the application and that a decision was made behind closed doors, of which you were informed. Is that the process that took place?

Dr Harrow: That was certainly the case for the appeal of the appeal. I was present for the initial appeal but was asked to step outside while a final conclusion was reached. On that occasion, the appeals panel found it extremely difficult to come to a decision and asked for extra time.

Such matters are highly complex. They involve a lot of basic translational science. What we are asked to do is to come out of the evidence base. We have to go and find very complex

supplementary evidence to back up the assertion that the patient will do better. I find that extremely difficult to understand, and I think that a layperson and a non-specialist would do so, too. I do not know how such a bold conclusion could have been reached, given that all the specialists and the professor of translational research backed up our claim.

Bob Doris: That is all very helpful.

Is there any possibility that the process by which the drug was not approved—it was never approved by the SMC; that process was bypassed because of the multiple technology assessment by NICE—could have been an additional consideration, whether rightly or wrongly, of the group in finally deciding that your patient was not different enough from the trial population? Do you think that the fact that the SMC has never considered the drug had anything to do with the process?

Dr Harrow: It is quite difficult for me to answer that, because I do not know what discussion took place in the room, but I think that the decision that was made was based on the evidence that I had provided, which was different from the evidence that was submitted as part of the NICE application. In fact, in relation to the drug that I sought, NICE stated that if evidence were to come to light regarding BRAF status, it would certainly welcome that in forming its opinion. Subsequently, we have started to get BRAF assessments done, and that formed part of the submission. We presented what NICE suggested might influence its further decision in our submission to the appeal, but it was still unsuccessful.

Bob Doris: I will not ask about BRAF genes, because that subject is way beyond my comprehension. I was trying to tease out whether there needs to be an improvement in IPTRs more generally—it is obvious that there needs to be; that is why we are sitting round this table—and whether there is an issue with the multiple technology assessments that you outlined. I do not have other questions on that just now, although I might ask questions about other themes later.

Drew Smith: I do not want to dwell on the issue for too long, but there seems to be a disconnect between the recommendation that we need to train other specialists to understand the process better and Dr Harrow's evidence, which suggests that a number of people understand the process very well; it is just that the process does not necessarily work.

A lot of work seems to be involved in clinicians and their colleagues having to make the case about whether a patient is different, or sufficiently different, and that is alongside all the other pressures on clinicians' time. It is clear from the review that we need to think about the resource implications at the other side of the appeals process, as well as the resource implications for the profession. Are we focusing on the wrong bit? Is more resource going into an adversarial process between clinicians and a group that is apparently making a clinical decision but is not made up entirely of clinicians?

Dr Harrow: My colleagues and I have spent absolutely hours on this work. The amount of email traffic regarding the issue is phenomenal. There is no doubt that that completely distracts us from seeing patients and doing clinical work.

Bob Doris: I think that the committee should consider some of the evidence that we have heard today. I would like to mop up some of the points that have been made, especially those that were made by Lesley Loeliger.

One of the issues is access to specialists. Dr Harrow made the point well that, although the pre-eminent specialists put in the IPTRs for many of the prevalent cancers, in the case of some of the ultra-orphan conditions the process can be more challenging. I consider that the recommendations in the report about identifying, and having a register of, national or international specialists may not be necessary. Indeed, the report gives a nod to the fact that that may not be necessary in terms of, for instance, managed clinical networks for cancer and the like. For clarity, were the report's recommendations on how to manage ultra-orphan conditions welcome? The committee considered a petition on how those conditions are managed.

Lesley Loeliger: Yes; I absolutely think so. Having a central list of specialists would be incredibly fair for patients in Scotland. One way of considering who is a specialist is to go through a centre of excellence. We happen to have such a centre for our condition, and I think that others—or perhaps someone who has published work in the specific field—are being considered for other ultra-orphan drugs. However, we would never want to take away from the expertise and very hard work that would be done, say, by a local clinician.

One suggestion is that the experts on the list would perhaps just need to be a signatory on the submission, so that a local clinician would still be responsible for the IPTR, but there would be a specialist as countersignatory to say, "Yes, I completely agree with this submission." Specialists could be accessed by the IPTR panel for things such as cost offsets, as I said, or just for an absolute understanding of the drug's clinical benefits. I have always wanted there to be a recognised expert for the ultra-orphan conditions.

Bob Doris: I will ask you about a situation that may never materialise, although I have been considering it.

We heard from Dr Harrow about a situation in which he feels that pre-eminent experts have given advice and made recommendations but, for whatever reason, the panel has not found them powerful enough. We have to look at that process. However, how would you see it if there was a patient with an ultra-orphan condition and a consultant sought out the pre-eminent expert, who said that, for whatever reason, they did not find the case compelling enough to support the IPTR? Should that have to be declared in any future IPTR submission? If I was the patient, I would say, "Go and find me another expert."

An expert's signature has weight and adds gravitas in the decision-making process. A consultant might identify an expert, who might say, "I would quite like the patient to get this medicine but, based on the criteria, I do not think that this is a goer." How would you respond to that? Should that be the end of the process? Should it be declared if the process continues?

Lesley Loeliger: I completely understand what you say about the concept of a gatekeeper and the idea that it is their signature or nothing. I can speak only for my condition and my situation. On the recognised experts, as I said, we have one Scottish expert who is based at the Monklands centre of excellence and we have several down south. They understand the condition so well that we have 30 PNH patients in Scotland and only 12 are recommended for the drug because the experts know whom it will work 100 per cent effectively for. I would sooner that that happens than that somebody goes through the entire IPTR process and perhaps is given funding for the drug for whatever reason and it does not work for them, because that would be a complete waste.

I cannot speak for other conditions. The position with my condition is pretty clear, in a sense, and I therefore have no issue with that. I would sooner that every patient had the chance to hear the expert say, "I understand this condition so well, and this drug will work for you."

Bob Doris: Okay. Are there any other comments on that issue before I move on?

Ian Mackersie: I entirely endorse what Lesley Loeliger says. The only way in which ultra-orphan drugs and patients with ultra-orphan diseases can be managed is by means of a centre of excellence or expertise. Generally, that is fronted by a distinguished senior clinician. The approach works extremely well in practice.

Bob Doris: I want to move away from the IPTR process in a moment, but from looking at some of the evidence that we have received, it seems to me that there is a lack of clarity about what happens during the final IPTR decision. Are the people involved judging on the clinical evidence? I am not convinced that cost comes into it, but I wonder whether they are trying to second-guess cost effectiveness, when that is actually the SMC's job. I am curious about that. The cost effectiveness is, of course, different from the raw cost.

My understanding—I stand to be corrected—is that there is a risk-share process between the health boards. Is the burden, if you like, of the additional cost for some of the more expensive medications borne by all health boards equally? How does that work?

Joan Fletcher: There is a risk share. The burden goes on all the health authorities. The drug that Pompe patients need is mentioned on the risk share, but we cannot access it because we cannot get through the IPTR process. Somebody has recognised that Myozyme is effective and should be on the risk share, but we cannot access it.

Bob Doris: You have highlighted something that I did not know. Is there a specified list of conditions in the risk share, by which—

Joan Fletcher: Yes.

Bob Doris: The committee might want to consider and reflect on how conditions get on to that list. That might be of particular interest to the witnesses who focus on orphan and ultra-orphan conditions.

I will move on to the issue that dominated the first discussion, which is whether the SMC gets this right in the first place. I think that it was Ms Loeliger who mentioned offset costs, but I will try not to personalise the issue and focus on her.

Across the board, there will be savings elsewhere. If someone is kept fitter, healthier and happier for longer, if less strain is put on carers and the family support network and if there is less breakdown of that network, there are economic benefits, as well as the social benefits that we all want there to be.

12:30

Part of the discussion with the first panel suggested that everyone had been holding their breath awaiting the outcome regarding that mystical thing called value-based pricing before considering the issues in greater detail. We found out last week for the first time—it came as a surprise to most people—that the value-based part of value-based pricing might not be reserved at all.

I will stop looking just at Lesley Loeliger; everyone can comment—my apologies. Do you think that the focus should now be on doing significant work to get the economic and social modelling right, so that the SMC's discussion with pharmaceutical companies is about the social

price of medications? That might put more pressure on drug companies, but it might also give them a stronger arm with which to argue their case, and we might we get more yeses. Is that where the momentum should now go?

Vicky Crichton: Yes—we completely agree with that. To echo what you have said, there was a lot of surprise at some of the comments and discussion at last week's committee meeting. Cancer Research has been quite involved at UK level in speaking to the Department of Health about value-based pricing. Our understanding has always been that it would be reserved because of the pricing element and that it would therefore apply in Scotland.

We have continued to call for further information on how the system would generally work and specifically on how it would relate to Scotland. We have also called for the Scottish Government to engage with the UK Government to determine how the system would work. Following last week's evidence session, the situation is not entirely clear.

As was said during the earlier evidence session this morning, the first thing that needs to be done is to get final clarification from the UK Department of Health on that issue. Is the approach just a development of the HTA process? If so, we should absolutely be getting straight into discussions about how we want to make progress on the matter in Scotland. Do we need to take into account a pricing element? The comments that have been made about the idea of a gap in the reviews regarding improving access are all about the fact that we have been waiting for value-based pricing. If it turns out that we have been incorrect in doing that, we will need to move fairly swiftly.

An idea is emerging from today's discussion that everyone is really on the same page as regards the principle of what we want to do, and there is agreement about the idea of value-based pricing. The question is how to apply it in practice.

Lesley Loeliger: To pick up on what Bob Doris said, we have an issue in that ultra-orphan conditions cannot get through the SMC because of the modifiers and the fact that our drug is too expensive to get through the SMC. If we can bring in the concept of cost offsets at that level, we can take away the need to push things through the IPTR system.

George Grindlay: The social elements will bring about a bigger difference for Lesley Loeliger, for instance. There is scope for change there. When the SMC meets and a decision is made about putting drugs for orphan conditions or any new drugs on to the formulary, that is an opportune point to bring in a citizens council or jury. Carers and members of the public with an interest in medications will then be able to make a valid input into the SMC's decisions. At present, that is not happening. That is a valuable point.

Doing that would also bring in the socioeconomic aspect. A carer's time is unpaid, but their value to the patient's life is immense. If a drug gives a patient a better quality of life, it will also give their carer a better quality of life, but that is not taken into consideration at all.

The Convener: The need for wider involvement was reflected in the review recommendations and, at a previous meeting, we talked about the need to test public opinion on what we want to pay for, in relation to rare and orphan diseases, end-of-life treatment for cancer and so on. Do the witnesses support such an approach, or does it offend them? We do not ask a citizens jury to decide whether to send a helicopter to help a mountain rescue team. How do you feel about using a citizens jury or opinion polling to decide whether the cost of treating you—or the people whom you represent or look after—is legitimate?

Vicky Crichton: We welcome the general point about having a wider public discussion of some of the issues. As I understand what was proposed in the review, such an approach would be applied not to individual decisions about particular treatments but to categories. For example, we might ask a citizens jury to think about end-of-life treatments or orphan diseases in general, and the approach might then be applied, through modifiers, to subsequent decisions.

Such an approach would be helpful, but we suggest caution. We would want to ensure that we spoke not just to the public but to patients. We know that people who are living with a condition have a view on what is valuable that is quite different from the view of members of the public, who perhaps have no concept of what it means to have the condition, although they have a stake in the debate, as taxpayers.

It is important that the debate should encompass patients. For example, in the context of end-of-life treatments, we are not talking about gains such as people being able to return to work or savings further down the line, because the treatments are often just life extending. However, there is a strong suggestion that the public put additional value on such treatments. We need to test and ratify that, so that we can say to the SMC, "The public think this has additional value, and your modifiers should reflect that."

Ian Mackersie: There is no difficulty with patients being involved in all such decisions. That is critical. However, I have reservations about asking members of the public to decide on what are extraordinarily complicated issues. The issues are medically, economically and politically

complicated. It strikes me that a very high standard of education would have to be provided for people if the citizens jury were to have the appropriate decision-making ability. I am slightly nervous about simplifying a horrendously complicated issue in such a way.

Natalie Frankish: I agree with Ian Mackersie. The principle of a citizens jury is wonderful, and it is about time that we asked what people want to pay for, but there is a danger. The rare and orphan diseases side of things is extremely complex, and people might not be able to take the complexity on board and make truly informed decisions. The principle behind the idea is very good, but we would have to do an awful lot of work on how a citizens jury would work in practice, to ensure that it operated fairly.

Dr Harrow: Engagement of the public is a good thing. However, the pathways are extremely complex. We have to understand how the drug is working and its side effects. The issue is not going to go away. The First Minister said that these are exceptional times, but they are no longer exceptional. This is how medicine is going—it is stratified, it has targeted agents and we are trying to tease out populations.

This is how it is now, and medicine is not going to return to a situation in which the same drug is given to the whole population. We have to get our heads round the fact that this is how it is going to be and that it is going to get even more complicated, with the drugs becoming even more targeted and probably even more expensive as time goes on.

Gil Paterson: I have a question on that point. I took a member's bill on palliative care through the Parliament, and I found a discrepancy between the palliative care that is available to cancer sufferers and that for people with other life-threatening illnesses. Someone with cancer had an 80 per cent chance of receiving quality palliative care, whereas someone with another life-threatening illness had an 80 per cent chance of not receiving quality palliative care. What does the panel feel would be equitable? Should we set up a fund specifically for cancer sufferers or should we have a fund for everyone across the board, with no exceptions based on what someone is suffering from, whether it is an end-of-life condition or otherwise?

The Convener: Are there no takers?

Lesley Loeliger: A £21 million rare conditions medicines fund was set up at the beginning of the year. It is meant to be for any ultra-orphan condition—that is, a condition that is suffered by fewer than one in 50,000 of the population, which would cover fewer than 100 people in Scotland. However, access to the fund has been pretty useless. People can get drugs through the rare conditions medicines fund only if they get through a successful IPTR, and we cannot do that. Only one group has been able to access the fund, and that was because its drug was deemed to be 100 per cent effective and because patients should not fight with each other. I take the opportunity to say that my drug is 100 per cent effective, too, and we would happily access the fund if it were possible. The fund exists; we just cannot get to it.

Dr Casasola: I have always been against the idea of a cancer drugs fund purely on the principle that it seems illogical to have one Government body deeming drugs not cost effective and another Government body asking which of those apparently not cost-effective drugs we want to use.

The Convener: A wider point is that the assessment is applied to new and developing drugs and to medicines for cancer and rare diseases, but a lot goes on in the health service that is not evaluated in a similar way. The comparison has been made with free prescriptions and some procedures and operations, which are not evaluated in the same robust way as new drugs and medicines are when they come on the scene.

Dr Harrow: I return to the point that we are not in extraordinary times. Such are the times that we are in, and we need to address the decisions that were made in the past that will perhaps not help us to move forward with drugs for ultra-orphan conditions and cancer drugs, if that is what society decides that we should spend our money on. This is not just a blip in the process and in how we clinically treat patients; this is how medicine will evolve and continue to be.

Jackie Baillie: I am blown away by the idea of all your clinicians being assembled on one side and an IPTR panel on the other side saying no consistently. If you had been in the room at the end, would that have made a difference to the panel's decision, or was it not about you presenting but about there being an expert in the same field on the panel? I understand that there was not an expert in the same field on the panel.

12:45

Dr Harrow: No, there was no expert from the same field on the panel.

Jackie Baillie: Is the issue perhaps not so much about the applying clinician presenting as it is about allowing a different expert to have a role in the decision making?

Dr Harrow: Yes.

Jackie Baillie: Given that we are told that clinicians make a decision based on clinical

effectiveness, but all the clinicians and professors may have lined up to say that a drug would be clinically effective, am I correct in saying that the only thing left, therefore, is money?

That might be difficult for you to answer.

Dr Harrow: The issue comes back to the point, which was said explicitly in the report that I got back—although I cannot remember the exact wording—that the effect was not different enough. That was a goalpost shifter, as that was not what we were asked to prove. We were asked to prove the two points, which we proved with supporting documentation, but it was then said that we had not proved that the effect was different enough. Because advances have been made since the multiple technologies assessment was done and because we can access different genetic code within the tumour—we presented all that—it is evident that some in the group who it was said would not benefit would benefit really well. Given that a proportion of that group who had been denied the drug might need to be given it, the issue must come down to cost.

Jackie Baillie: Let me put the matter another way by asking about the number of panels, which we discussed earlier. Should the decisions be left for individual health boards, which we know make different decisions on the same drugs even if there are the same kinds of indications from patients? Is there a view that we would benefit from having fewer panels? Might medical directors who have one eye on their budgets be slightly more relaxed then, because the decision would be made at a regional or national level?

Lesley Loeliger: That is where having an ultra-orphan section would help. For certain conditions, there are many experts who would be able to handle the decision but, from my point of view and from the point of view of those with other ultra-orphan diseases, that would be the ideal.

Dr Harrow: I have never thought of that before, but perhaps the SMC could take that on centrally. We could submit the request centrally to the SMC and all the experts who had appraised the cost effectiveness and the data up to that point could be involved. Any additional data could then be brought to those who are dealing with that system day in and day out. As I said, it took me hours of reading SMC multiple technologies assessments to try to understand the process, which is not clear.

Joan Fletcher: I agree with both the previous speakers. The review has shown that different health boards have different forms to fill in and different ways of filling in the application, so it would make sense to bring everything together centrally. Also, the £21 million budget is already in place. The process is halfway there, and it just needs to be taken a step further.

Dr Casasola: Let me make a couple of points. I can talk only about the Tayside IPTR system—I have no knowledge of the system in Glasgow—but there is a little bit of nervousness when there is a big group of patients. There is a desire to target treatment by defining which patients in the group will benefit the most. The concern is that a precedent may be set and that giving a drug to one patient will open the doors for everybody. I do not know whether that is the case in Glasgow, but that is certainly a nervousness locally.

The advantage of having a local group is that, whereas there might be a bit more time to make decisions about treatment for patients who have PNH, for patients who have late-stage cancer time is precious. I am not sure that having a national group to discuss all those difficult patients would be time efficient. At least with our local group, the majority of one-off requests—I cannot put a figure on it—get approved and receive a decision often within a week of the request being made. I do not think that that could be guaranteed with a bigger group.

Lesley Loeliger: I entirely mean it for a disease such as PNH, which is ultra-orphan and for which we have three patients who need the funding. I completely agree that it would be mayhem if there were too many patients.

Jackie Baillie: The issue is worth teasing out slightly. It can be the case that a drug is available in, say, NHS Borders but would not be available for the same patient in NHS Greater Glasgow and Clyde. Surely there is a way of balancing the local flexibility and speed with ensuring consistency and fairness.

Vicky Crichton: I do not have enough direct experience of the IPTR process to comment on that. However, to broaden it out to IPTRs more generally and the area drug and therapeutics committees, we have some concerns about ensuring that we reduce variation in the decisions as far as possible. Obviously, IPTR decisions are—or should be—about individual patients and, therefore, there will be differences in the decision. However, there should be parity in the decision-making processes.

We absolutely took on board the comments that Professor Swainson made about the wider role of area drug and therapeutics committees and the good reasons why those should be retained. However, attempts thus far to ensure that the process is consistent across ADTCs and IPTR panels do not seem to have worked, so, if we are going to continue with separate health board processes, there must be parity of process in the decision making to ensure that the issues that are

being considered and taken into account are the same. That must also continue to be audited because, if variation continues, we might need to consider a different approach.

The Convener: Do any of the witnesses wish to place on record something that we have not covered?

Vicky Crichton: I will go back to the research point that was raised in the previous evidence-taking session. When we spoke to clinicians in advance of the evidence-taking session before Christmas, a number of them raised concerns about trials not being able to run in Scotland, where the comparators are not in use. Although Professor Swainson said that he did not receive any evidence on that, it has been raised with the committee a number of times and, if it is true, it is of great concern. Therefore, we are keen that it be looked into further.

George Grindlay: As an ordinary person, I want person-centred healthcare. If I live in the Borders and have a problem, I want the drug to be available not only in that area but on the formulary throughout Scotland, because I would find it quite difficult if I moved from my health authority, which had agreed the drug and made it available, to another health authority in the north of Scotland where it was not available and, therefore, I had to put in an IPTR. In a person-centred healthcare system, anything that goes on to the formulary should go on it pan Scotland. At the moment, each health board has the authority to accept or not accept SMC recommendations for new medicines.

The Convener: This is an on-going process so if, on the bus going home, any of the witnesses thinks of something that they wish that they had said, they should not hold it in but should email us or get in contact with the clerks. That is perfectly acceptable. I thank all the witnesses for attending, for their time and for the evidence that they gave.

We now go into private, as agreed earlier in the meeting.

12:54

Meeting continued in private until 13:07.

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