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Pàrlamaid na h-Alba

Official Report

# HEALTH AND SPORT COMMITTEE

Tuesday 29 January 2013

Session 4

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## HEALTH AND SPORT COMMITTEE
**3rd Meeting 2013, Session 4**

**CONVENER**

*Duncan McNeil (Greenock and Inverclyde) (Lab)

**DEPUTY CONVENER**

*Bob Doris (Glasgow) (SNP)

**COMMITTEE MEMBERS**

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

*attended

**THE FOLLOWING ALSO PARTICIPATED:**

Marion Ferguson (Ivacaftor Patient Interest Group)
Joan Fletcher (Association for Glycogen Storage Disease (UK))
Professor Keith A A Fox (University of Edinburgh)
Professor Tim Goodship (Newcastle University)
Nancy Greig (Health and Social Care Alliance Scotland)
George Grindlay (Health and Social Care Alliance Scotland)
Professor Peter Hillmen (PNH Alliance)
Alastair Kent (Genetic Alliance UK and Rare Disease UK)
Lesley Loeliger (PNH Alliance)
Richard Lyle (Central Scotland) (SNP) (Committee Substitute)
Ian Mackersie (aHUSUK—A Patients and Families Support Group)
Professor David Newby (NHS Lothian and University of Edinburgh)
Dr Mark Eldon Roberts (Salford Royal NHS Foundation Trust)
Len Woodward (aHUSUK—A Patients and Families Support Group)

**CLERK TO THE COMMITTEE**

Eugene Windsor

**LOCATION**

Committee Room 2

# Scottish Parliament

## Health and Sport Committee

*Tuesday 29 January 2013*

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

## New Medicines (Access)

**The Convener (Duncan McNeil):** Good morning. I welcome members and the public to the third meeting in 2013 of the Health and Sport Committee. As usual, I remind you to switch off all mobile phones, BlackBerrys and other wireless devices, as they can interfere with our sound system.

We have received apologies from Aileen McLeod and Drew Smith, who are unable to be with us, and I welcome Richard Lyle, who is a committee substitute.

Before we discuss access to new medicines, it will be useful to introduce ourselves. I am the MSP for Greenock and Inverclyde and the convener of the committee.

**Professor David Newby (NHS Lothian and University of Edinburgh):** I am British Heart Foundation professor of cardiology at the University of Edinburgh.

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

**Professor Keith A A Fox (University of Edinburgh):** I am also a professor of cardiology at the University of Edinburgh.

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

**Professor Tim Goodship (Newcastle University):** I am a professor of renal medicine from Newcastle University.

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

**Nancy Greig (Health and Social Care Alliance Scotland):** I am network development officer for the Health and Social Care Alliance Scotland.

**Mark McDonald (North East Scotland) (SNP):** I am an MSP for North East Scotland.

**George Grindlay (Health and Social Care Alliance Scotland):** I am lead facilitator for Angus long-term conditions support groups and a non-executive director of the Health and Social Care Alliance Scotland.

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

**Ian Mackersie (aHUSUK—A Patients and Families Support Group):** I am secretary of aHUSUK—A Patients and Families Support Group.

**David Torrance (Kirkcaldy) (SNP):** I am the MSP for the Kirkcaldy constituency.

**Len Woodward (aHUSUK—A Patients and Families Support Group):** I am a trustee of aHUSUK.

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

**The Convener:** I thank you all.

With the formalities over, Richard Simpson will ask the first question in this round-table discussion.

**Dr Simpson:** As you know, the committee has been investigating access to new medicines for some time. Indeed, we did so in the previous session, and that resulted in the changes that created the individual patient treatment request system. Obviously, the two areas for investigation are the Scottish Medicines Consortium and the IPTR. Since we started the investigation, there have, of course, been the announcements of the Routledge, Scott and Swainson reviews of the whole process. We are therefore in an iterative process in which things are already changing, or appear to be changing. Most recently, there has been the announcement of £21 million for orphan medicines.

What do the panellists hope to get out of the Routledge, Scott and Swainson reviews that will take us further down the line of creating a robust, fair and transparent system in which there is not a postcode lottery? What revisions to the system would you like to see?

**Professor Fox:** One of the guiding principles is that evidence that is based on the risk and the risk-benefit balance for individuals should be applied locally. If we have a consistent way of applying that principle that can be interpreted locally, we could avoid some of the problems that we have had in the past.

**Nancy Greig:** I should mention that the Health and Social Care Alliance has not thus far been invited to contribute to the Scottish Government's on-going review of new medicines. We have written to both Professor Philip Routledge and Professor Charles Swainson to offer assistance with both sections of the review. We will be interested to see how the findings of this inquiry feed into the Government's review and vice versa.

**Professor Goodship:** As someone who has an interest in a very rare kidney disease, I would like

there to be a rapid, transparent and equitable process in which patients who are proven to have a disease for which there is an effective treatment can rapidly get access to that treatment, because some such diseases need to be treated as soon as possible.

**The Convener:** Does anyone else have a comment?

**Dr Simpson:** Can I ask some further specific questions, convener?

**The Convener:** Yes.

**Dr Simpson:** I have a couple of questions that arise from a quotation in the Health and Social Care Alliance Scotland submission by one of its members, who said that consultants

"actively discourage IPTRs…. Patients were also told that to make an IPTR which was refused would not be looked on favourably in future."

One of the concerns seems to be that consultants do not feel that the IPTR system is functioning adequately. Another point is that the IPTR should be rapid, because many patients are in the late stage of their illness, so a new drug might extend their life. We have some consultants present. Are you aware, from what colleagues have said, that people are discouraged or that they discourage patients because the system does not work properly?

**Professor Fox:** I disagree with that; I think that it is possible to use the system. It is cumbersome, but it has been possible to use it.

**The Convener:** Have you experienced that?

**Professor Fox:** Yes. When there are new treatments for something for which old treatments have failed, there is a real case for justifying consideration of the new treatment under the IPTR.

**The Convener:** Is that the common experience around the table?

**Professor Newby:** I think so. The IPTR can be and has been used appropriately. For me, the disconnect often happens when we have treatments for which there are international guidelines, and treatments that are endorsed by the SMC and the National Institute for Health and Clinical Excellence which are still not being used in a clinic or are being used variably. That is the big issue for me.

**The Convener:** Why would that happen?

**Professor Newby:** It would usually happen because of local decision making over drug budgets.

**Dr Simpson:** I wonder whether Professor Newby would give us a specific example.

**The Convener:** I was going to let Professor Goodship respond to your original question, Dr Simpson.

**Professor Goodship:** My experience of treating the rare disease that I am interested in is that there are two patients in Scotland who have been successfully treated with eculizumab under the IPTR system. When it has worked, it has worked really well. One patient who was given approval to use the drug within 24 hours has had a dramatic response to it. What is difficult is the variation of the application process; it varies from IPTR to IPTR, which I think is what my colleagues sometimes find difficulty with.

**George Grindlay:** I think that consultants appreciate the IPTR, but it is, as has been identified, a large and cumbersome document that takes a lot out of consultants' clinical time. They must go through a large committee at local level and then they have to go to the pharmacist; it is a long drag through for a consultant to take that time, which is possibly why the IPTR is not being used effectively.

**Ian Mackersie:** In observing the scene in Scotland, we were very interested in the Cabinet Secretary for Health and Wellbeing's award of £20 million for IPTR and his comment that

"It is only right that Scottish patients with rare conditions have access to innovative medicines which are clinically justified and that they are not disadvantaged due to the very high cost of these treatments."

Four days later he said that he would issue guidance to health boards that clinicians should be allowed to prescribe a certain drug

"to ensure that ... the IPTR process does not present a barrier to accessing it."

What concerned me about that was the admission by the health secretary that the IPTR system was a barrier and that he was able to override the entire system for the sake of one drug.

**Nancy Greig:** It is worth noting that many staff and volunteers from the third sector organisations to whom we spoke in our engagement exercise did not know what an IPTR is. They simply had the perception that some consultants find it easier to access off-licence medicines than others, without understanding how that process operates. If members of the public do not know that the system exists, how can it possibly be used effectively?

**Len Woodward:** I have knowledge of one person who has atypical haemolytic uraemic syndrome who should be making an IPTR but is being put off doing so because of the difficulty for her and her clinician. As a small charity, we struggle to find resources to help such people with the process because they cannot do it individually.

**Bob Doris:** It is important that we maximise the time that the witnesses rather than MSPs get to speak, so they should just let us know when they need to come in.

I have spoken to many people at committee meetings, and interest groups have contacted me as an individual MSP when they have become aware of the inquiry. It is clear to me that there is a lack of understanding about what IPTRs are for. I will elaborate a little bit on that and see whether any of it chimes with the witnesses.

In some parts of the country, there is almost a conveyor belt of IPTRs: someone who has been told that they cannot get a medicine is automatically told to do an IPTR. However, my understanding is that the system was never designed for that, but was intended for cases in which there is specialist evidence to show that the patient would receive a benefit over and above their peer group. We are back to exceptionality.

Has that been lost somewhere down the line through IPTRs being used in some parts of the country as an automatic means to get a drug that otherwise would not have been approved? In other parts of the country, clinicians are more selective because they understand the exceptionality argument. Do we have to reinforce what IPTRs are for in the first place?

**Professor Goodship:** For rare diseases, every patient, almost by definition, will be exceptional, so to compare such patients with their peer group is almost nonsensical. In Scotland, three patients a year will present with aHUS. I would say that every one of them is exceptional, but if you compare them with their peer group, they would not be exceptional.

**George Grindlay:** As an individual, it is difficult to find out from any health board's site what an IPTR is. It is difficult for the layperson to understand how they can get an IPTR, how it is supported and what it is for. On some websites, it is quite hidden.

**Professor Newby:** I am not aware of the IPTR system being abused as a routine approach within my realm, if that is the question that is being posed. I have used it in selected exceptional cases. That is not to say that it is not abused in certain areas.

**Bob Doris:** You used the word "abused". I was saying that we have seen evidence to show that it is used far more frequently in some parts of the country than it is in others. I would not say that that is an abuse in the slightest. Some parts of the country may have a different perception of the purpose of an IPTR from other parts of the country. I am trying to dig beneath the surface of that point.

**Professor Fox:** It is fair to say that, even among consultants, there is not complete clarity of information. Those who have used the system and gone through it know how it works. However, not every consultant does and they can be put off by the process.

**Len Woodward:** In preparing for this meeting, I found out that the drug in which we are particularly interested is being used for another disease. I also found that the IPTR process for that disease has been quite successful because it is defined by a specific genetic defect. It is no less applicable to atypical HUS, in which a specific genetic defect determines a patient's place in the cohort.

**Bob Doris:** There are 14 area drug and therapeutics committees across the health boards. We are trying to make IPTRs more appropriate and ensure that approvals are processed more speedily. Do we need 14 committees? Is there a way of streamlining that system and making it more effective?

10:00

**Professor Goodship:** For very rare diseases, such as the one in which I am interested, it would make more sense for one body to deal with applications for a specific drug to treat that disease, because the expertise could be readily accessed. Of the 14 different committees, none might have the expertise to make a judgment on a drug that might be applicable to a particular rare disease. I think therefore that there should be fewer committees to deal with rare diseases.

**The Convener:** I might be picking you up wrong, but are you suggesting that we should have a committee for each particular disease?

**Professor Goodship:** No. You keep all the committees, but one becomes responsible for and has the expertise to deal with a variety of diseases.

**The Convener:** The evidence that we have received and my constituency experience suggest, however, that there might be a general issue in that respect. Surely the experts would be the ones supporting the application, while the generalists—if I can use that term—would be the ones discussing whether it had merit. I do not know how you would overcome that, but we might have an opportunity this morning to discuss that.

**Professor Fox:** Given that, by definition, the process will involve new therapies, and given that most people will not be familiar with the treatments, the best judgment will come from an informed committee that recognises the context and appreciates where existing treatments might not work. We should therefore have better expertise and an expedited process.

**George Grindlay:** I note from NHS Tayside's website that the IPTR that the clinician has to fill in is six pages long. That document goes to the principal pharmacist or the pharmacist in the clinical area and is then passed on for approval to a decision-making panel comprising, among others, the associate medical director, lead specialty consultants, a lead general practitioner, a lead or principal clinical pharmacist, a clinical service manager and the head of patient care and nursing. That is the process in NHS Tayside and, I assume, Scotland-wide.

**The Convener:** Do you have another comment, Bob?

**Bob Doris:** I will come back in later, if I may, convener.

**The Convener:** Absolutely. Do you have a question, Gil?

**Gil Paterson:** I wanted to ask about the point that Bob Doris raised about the 14 ADTCs.

**The Convener:** That is fine.

**Gil Paterson:** I will come back to that after I follow up Mr Grindlay's comment.

I believe that Mr Woodward talked about difficulties and that someone else used the term "cumbersome"; I have to say that the process that has been described by Mr Grindlay sounds cumbersome to me. Leaving aside the question of who actually makes the decision, I wonder whether there is a need to make all the decisions separately. Surely if they were all taken together, it might truncate the whole process and bring things together a bit better, although I have no idea how long the process takes. How could we overcome such barriers?

Everyone who comes before the committee—even those who are critical of the system—says that the handling of the SMC is really good but that things fall down somewhere down the line. How do we improve the system and address some of the points that Mr Grindlay has made?

**George Grindlay:** I read the IPTR process in NHS Tayside. Before the IPTR form is submitted, the consultant must give some tenuous figures. For example, the form asks for treatment cost, annual cost, anticipated duration of treatment and dose—including strength, form and frequency—of the medication. The consultant has to give a reason for the request. Is it a

"Continuation of medicine initiated in primary care"?

The form asks whether the treatment will continue only in hospital or in hospital and then primary care, and it asks about previous treatments. It is a cumbersome document. However, because the consultant is the expert who provides information to the ADTC, I do not think that the process can be shortened.

**Len Woodward:** I have experience of submitting the English equivalent of an IPTR; I presented to the local lead pharmacist a case for eculizumab. It took me two days to write two pages to get the essence of the quality of life of the individual, clinical effectiveness and exceptionality. When I submitted it, the pharmacist said that nobody at the hospital could ever have done that. The problem is when there is no expertise for a specific disease. Professor Goodship could probably have done a better job than me.

**Nancy Greig:** The issue of expertise does not apply just to rare diseases. There may be specialisms in the area drug and therapeutics committee, but there will not necessarily be specialist knowledge on every condition for which somebody might require an IPTR.

Proof of exceptionality applies to other long-term conditions and it is sometimes difficult to prove why somebody requires a drug. They might have tried all the treatment options and all the standard drugs that are available for their condition but none has, for whatever reason, been suitable. However, that does not necessarily mean that the person is an exceptional case; it simply means that the portfolio of drugs that is available to the consultant, and which are licensed by the SMC for that particular condition, has not been enough.

**Professor Fox:** I will raise a bigger issue. We have been talking about rare conditions. Those are important, but there are also much more common conditions for which drugs that have been approved and licensed are not available. Our patients come to us and tell us that they cannot manage with the existing therapy. I am talking about the specific instance of the difficulty in managing anticoagulants. For some patients, that is a real problem because of the interaction with foods and other medicines. Those patients swing in and out of the therapeutic range, putting themselves at risk of haemorrhage and major complications.

We are facing a situation in which there is substantial large-scale evidence, guideline approval, NICE approval and approval in Scotland but no local approval. Do we face the prospect of IPTRs being submitted for drugs for which they are not really intended—drugs that are proved but which are not available?

**Dr Simpson:** Could you name such a drug in your field? Is ticagrelor such a drug?

**Professor Fox:** My colleague Professor Newby might speak to ticagrelor. I can speak to novel anticoagulants. I want to do so because we have a serious issue in Scotland—and in Britain as a

whole—with under-recognition of a major rhythm disturbance called atrial fibrillation. Only about half of cases are picked up. About 125,000 people in Scotland have atrial fibrillation. It is much more common in the elderly—about 15 per cent of people over 80 have it. Why is it important? It is because a quarter of those people will have a stroke within five years. The existing treatment is warfarin anticoagulation, which fails in about half of cases because people cannot manage with it. They swing in and out of range and have little bleeds.

For the first time, we have novel anticoagulants that would fall into that category. If someone is failing on the existing treatments, we have options with novel anticoagulants and good guidance and risk scores to know for whom the benefit-risk balance is favourable. Our patient comes to us and says, "Look—I cannot manage with that treatment. Why can't I get something that is approved for my long-term care?"

**Dr Simpson:** Would that decision be a local decision? I want it to be clear, for the record.

**Professor Fox:** That would be a local decision.

**Dr Simpson:** The fourteen area drug and therapeutics committees—or the combinations of south, north, east and west—make different decisions about drugs that are approved and in the guidance the provide.

**Professor Fox:** Exactly.

**Dr Simpson:** How do clinicians feel about that?

**Professor Fox:** Clinicians are absolutely frustrated about it.

**Bob Doris:** Please correct me if I have the procedure wrong. Once a drug is approved by the SMC, each health board will decide how best to place it on what I think is called its formulary.

**Professor Fox:** That is right.

**Bob Doris:** The drug can sit there and it will be classified as standard treatment for some conditions, but not for others. However, the committee took evidence that individual clinicians can still prescribe—an IPTR need not be requested because there might be another option in the system to prescribe the required drug.

I want it to be clear for the committee that the decisions on whether to use a medicine as standard are different from the decisions on whether individual clinicians are able or unable to prescribe a certain drug. My understanding was that the problem has been the lack of ease of going through the process, which can be unwieldy, bureaucratic and time consuming. The committee would appreciate an answer on whether or not the process is cumbersome or acts as a barrier.

**Professor Fox:** The IPTR is a barrier because of lack of approval. The process is so cumbersome that it is a barrier to patients' getting treatments that are proven in evidence and which are accepted by the regulatory bodies and the profession.

**The Convener:** We have concentrated a lot of our evidence on the SMC and its role, and the IPTR. However, although we are aware of the 14 area drug and therapeutics committees, it would be interesting to tease out how the process applies at local level, so what Bob Doris is asking is what is stopping you from prescribing a drug for patients who would, in your clinical opinion, benefit greatly?

**Professor Fox:** We cannot systematically use such drugs for groups of patients; we must go through the process individually. That is the issue.

**The Convener:** Ah. What is the scale of individual cases? Do they amount to large numbers?

**Professor Fox:** The numbers would make it impossible.

**Dr Simpson:** I want to ask Professor Newby to come in on that because I have raised the issue of ticagrelor before. I should declare that I used to be a consultant to AstraZeneca, which produces that drug.

The SMC approved ticagrelor. The east group approved it for use with one type of heart problem, and the west group approved it for use with another. I understand that the west has revised its approval, so it has now gone ahead of the east and covers a greater range of conditions. The public cannot understand—I doubt that clinicians can either—a situation in which a condition can be treated in one area but not in another. The difference is that the costs are about 10 times as great for a new drug—

**The Convener:** We will see if we can get an answer.

**Professor Newby:** I share that frustration. The disconnect is that ticagrelor is licensed for the treatment of heart attacks. It is in the European and American guidelines and it has been approved by NICE and the SMC. However, to be brutal, the issue is the cost. In the east, we have selected a group of individual patients for treatment, although there is no rationale for why that particular group was chosen, because the drug is effective across the whole range. To be clear, the drug has a 1.4 to 1.5 per cent mortality benefit, which means that for every 70 people treated, one life will be saved.

The cost is relatively high, but at £60 a month—we are talking about between three and 12 months of treatment—it is not a huge amount of money on

an individual basis compared with the cost of many of the other drugs that we have been talking about, and it is not dissimilar to what the previous drug, clopidogrel, cost when it was initiated 15 years ago. That cost £40 a month, and we used it. However, we are now in a different financial climate.

10:15

What happens is that we get approval and guidance, but people at the local level have to deliver all the services, and they have to make harsh choices. I think that, if ticagrelor was the same price as clopidogrel, it would be used universally across Scotland. The issue is that local people are struggling with the constraints that they have on their budgets. Personally, I believe that a mortality benefit is well worth spending money on, because it is generally young people that we are talking about. The trials were done in people under 75. We invest in other treatments that are much more expensive, often at the extremes of life and with the elderly—not that that is in any way not a good thing to do, but we need to think about where we invest and what we prioritise.

**Professor Fox:** I entirely support what Dave Newby said about ticagrelor, and the cost of the novel anticoagulants is of the same order. The impact is not just that they are a more convenient treatment that people do not have to monitor. If we prevent stroke and multiembolic stroke, we are preventing dementia and the need for long-term institutional care among an elderly population. Our frustration is that we have treatments that can change that process and the proportion of individuals who will need long-term institutional care, but they are not being implemented.

**Nancy Greig:** The alliance calls for greater public debate about what taxpayers are and are not willing to fund, and further research on and wider understanding of the social costs of not managing long-term conditions effectively. If somebody does not get effective medicines along with support to self-manage, there will be greater cost implications in relation to welfare benefits because they cannot work and they might develop dementia and require social care support.

The public and decision makers need to consider that not all medicines are about saving or prolonging life. Many are about controlling symptoms and effectively managing conditions so that people have a certain quality of life and can remain economically active. Decisions about quality of life need to involve the voices of people with long-term conditions, as only they can give a true perspective on what matters to them, their levels of pain and fatigue, their mental health and the burden of unpaid care on their families.

**Mark McDonald:** I have a few questions on a different theme. First, how confident can we be that the SMC, NICE or any other regulator sees all the relevant trial data on new medicines when it makes a decision?

**Professor Fox:** A number of us have served as external experts to either NICE or the SMC. When there is a new medicine, it is a requirement that all the available data are made available. All the published data and preliminary data have to be made available for NICE or the SMC to make the decision. I am confident that that is not the stumbling block.

**Mark McDonald:** So we can always trust that, if a trial shows unfavourable results, that will at least be reported on in all circumstances.

**Professor Fox:** The issue of unpublished trials is really important. There may well be related or other agents on which there are unpublished data, but if an organisation is submitting a dossier, it is bound by law to provide all the evidence relating to the particular agent.

**Mark McDonald:** In terms of how information on new drugs is published or communicated—this is probably my last question on this, so I would welcome others' views—are you satisfied that the current system is effective and that, for example, there is no need to insist on systematic review of all trials of new medicines?

**Professor Fox:** The NICE process goes through systematic review. The difficulty is that it may not capture everything if information on related drugs that have never reached the market has never been published. That could be an issue.

**The Convener:** Does Professor Newby also want to respond?

**Professor Newby:** I can only reinforce what Keith Fox has said. Most people acknowledge the robustness of the NICE and SMC process, in which I also know there is access to information that is not necessarily in the public domain. Publishing all the information is a requirement. That class effect, where information exists on a drug related to the one being considered, is a very small point. Overall, most people are very impressed by the process. In fact, I know that many countries that also face the need to prioritise medicines are starting to adopt similar processes and use very similar methodology. In North America, people have looked for a long time at the NICE approach because they see it as a fair way of critically appraising things.

**Professor Goodship:** I agree with the comments of both my colleagues. In addition, new medicines go through another process with the European Medicines Agency, which also requires rigorous review of trial documents. Even before

some medicines get to NICE, they have already undergone one rigorous review.

**The Convener:** Does anyone else want to contribute on that point before we move on to Bob Doris's next question?

**Nanette Milne:** Convener—

**The Convener:** Sorry, I have been concentrating on this end of the table, where things are very busy.

**Nanette Milne:** I have a slightly different line of questioning. We know that there is a pretty robust system in place for assessing new drugs, but it has been pointed out to the committee—by the British Heart Foundation, I think—that there is not the same sort of robust assessment of non-pharmacological interventions. Is similar pressure put on such interventions, which can be equally beneficial to quality of life and length of life?

**Professor Fox:** NICE and the SMC also tackle devices, but you are right that pharmacological agents previously had the greatest scrutiny, whereas a device that was a technological bit of kit, such as a new valve, did not go through quite the same process. However, that has now changed. For example, NICE treats a new stent in the same way that it would treat a new drug.

**Nanette Milne:** Other interventions in the NHS, such as cardiac rehabilitation and specialist nursing, may not be scrutinised particularly robustly. Have you any comments on that?

**Professor Fox:** You raise a very big issue. Traditional treatments that have been in place for a long time may not have been reviewed recently in the context of current care. That needs to be tackled, but that is a long-term process.

**Nanette Milne:** Presumably, that could lead to the withdrawal of certain interventions and free up resource for other or new treatments.

**Professor Fox:** Yes, I agree.

**The Convener:** Are there any other comments on that? How we assess the value and outcomes of current procedures and medicines is a recurring theme in the context of the challenge of introducing new and innovative medicines and procedures.

We will move on to Bob Doris's next question.

**Bob Doris:** I want to explore various questions, but let me stay on IPTRs for the moment. If an IPTR is successful, is any work done to see what the impact was on the individual patient who received the medicine? For example, if one can demonstrate that the drug has had a dramatic effect on the patient, is that fed back into the process? Does someone say to the SMC, "You might want to look at this again"? Conversely, if the benefits of a successful IPTR are—unfortunately—at the margins, does the same thing happen? Are we led by the results for the patients who receive the medication from the IPTR process?

**Professor Newby:** It depends somewhat on the treatment. When a drug is used a lot with apparent benefits, it just adds fuel to the fire to get it mainstreamed and accepted. The mechanism of auditing outcomes is very important and, indeed, many health boards are investing in that more and more to find out how best to use and align their resources.

I am not sure that what you are suggesting is happening systematically, but it is certainly something that we could think about. Again, however, it all comes down to time and the challenge itself. Having worked well into the night to fill them in, I agree that IPTRs are cumbersome for clinicians but I can see no way around the system. The fact is that if you want the medication to be assessed and the panel to reach a good value judgment you need to provide all the information.

You are talking about giving feedback, and I certainly think it important to feedback the results of an intervention. Of course, we do that all the time in the clinic: we treat patients and see whether they get better or not.

**Professor Fox:** I should sound a note of caution. Many of our treatments are tested on large numbers of patients, but idiosyncratic responses from a few could actually swing the interpretation when, in fact, a large experience provides the most robust response with regard to the overall effect—in other words, the net benefit and hazard. Although feedback and audit are certainly useful, we could be misled by idiosyncratic responses.

**Bob Doris:** When you talk about "idiosyncratic responses" and being "misled", are you suggesting that the results might not be as positive as you thought they would have been ahead of the IPTR being granted?

**Professor Fox:** Let me give an example. Someone could have a dramatically positive response while someone else could have a dramatically negative response, but neither will give the whole picture about the population's behaviour.

**Bob Doris:** This is a very interesting line of questioning. We sometimes get the impression that if the IPTR is not granted, some dramatic ill outcome will befall the person who does not get the drug involved. However, at the other end of the spectrum, the medication might have no benefit at all. Are we analysing why there might have been no benefit? After all, we need to get these

decisions right, which means that the situation needs to be audited.

**Professor Fox:** But we have to consider the effect that we might expect from the treatment. If, say, a certain treatment reduces the risk of stroke by 30 per cent, that will be very important to an individual patient. However, they might have gone through a period in which they did not have a stroke at all. It is very difficult to show whether there has been a benefit on the basis of one patient, which is why having a much bigger experience is, in a scientific sense, the most robust approach.

**Bob Doris:** I accept that but I have to say that I find it a bit frustrating, given that the committee will want to be led by the evidence and that that is an obvious question to ask.

I have other questions, convener, but I am aware that other members have questions of their own.

**The Convener:** I think that other witnesses want to respond to your question.

**Professor Goodship:** Mr Doris raises a very important point. The difficulty with rare diseases is that there might be only one patient in a country and if they do not do well on a treatment, that might bias what happens in the future.

It is mandatory for a registry to be kept of all patients with a rare disease who are treated with a novel agent. Perhaps it would be better if the overall results were fed back to individual boards so that they could get a big picture and see what has happened to their one patient and to the other 99 patients who were treated elsewhere.

10:30

**Dr Simpson:** I want to take the issue a little further. We have dealt with the rare diseases where there must be a registry, but what about diseases that are less rare? Is there adequate follow-up on the new drugs that come out? It seems to me that we get through the fairly complex business involving licensing, the SMC, NICE and local approval, but we need to be certain that your experience as clinicians in using new drugs defines the drug—as in the case of anticoagulants—where you say that it works well or is necessary for a particular group. To give a recent example, an anti-cancer drug worked for 70 per cent of people with bowel cancer who had a particular genetic marker but it did not work as well for the 30 per cent who did not have that marker. Are we collating and collecting information on diseases that are a bit rarer in Scotland, across the United Kingdom, and, indeed, across Europe in a way that allows us to better determine how we should use those drugs?

**Professor Fox:** When the European Medicines Agency looks at the approval of a new agent, it can provide a requirement and put it into a category. If the confidence bounds for the effect and hazard are such that the agency still has a worry about it, it can require so-called phase 4 studies, which are, in fact, large registries. They may well be required by the licensing authority if there is uncertainty.

There are a number of other treatments. There is, for example, a novel treatment for resistant high blood pressure that does not respond to drugs, which involves ablating the nerves to the renal arteries. It is really important to know the long-term impact of that. Therefore, in the UK, the profession has set up a registry for every single case, but it would be impossible to do that for all drugs. We have to be guided by the European Medicines Agency saying, “There’s uncertainty and a grey area, so let’s require some post-marketing surveillance.”

**Professor Newby:** We also have fantastic electronic record systems in Scotland. The chief scientist, Andrew Morris, is certainly addressing a lot of the issue. There have been quite a few examples across Scotland of people having done non-intrusive patient records research to address post-marketing surveillance. Indeed, industry has collaborated in some of those examples, as it keen to use the research. We are very lucky to have that potential resource in Scotland.

**Mark McDonald:** Something has niggled me since I heard the responses to my questions, and I will kick myself if I do not put it on the record. In response to one of my questions, Professor Fox said that there may be an issue relating to drugs that have been trialled but never submitted for approval, which means that there is no obligation for the trial data to be published. That might be an issue in the consideration of other drugs or related drugs for trial and approval. Does Professor Fox think that it would be beneficial if all trial data, whether or not the drug ever makes it to the market, were published as a matter of course in order to inform future trials and approval decisions on new medicines? It is possible that we in Scotland do not have the relevant powers to require that.

**Professor Fox:** I would be very much in favour of all that information being in the public domain. I think that the solution is having an electronic record of the results of studies, because a study may be submitted that the journal may turn down because it is not very interesting. There must be a depository so that any independent person can look at the data and say that they agree or disagree with the interpretation. The information would be in the public domain, and that would be an advance.

**Mark McDonald:** I appreciate that. Does anyone else have a comment on that point?

**Professor Newby:** Increasingly now, there are open-access journals, which tend to get round the problem. However, people still have to pay to publish. Publishing is generally free if the paper is of interest and the publishers want to sell the journal. However, with open-access journals, it is about ensuring that the methodology is robust and that there has been some peer review. The danger is that a lot of rubbish will be published, to put it bluntly, unless there is some oversight and review of data by independent experts in the field, which is a necessary element.

With the increasing availability of open-access journals, more data will come out. It would potentially be good to put some onus on industry to publish more of its possibly uninteresting data in an open format, without compromising commercial opportunity or intellectual property. Such publishing could be encouraged and perhaps taken forward in association with the Association of the British Pharmaceutical Industry.

**Mark McDonald:** Does the ownership of the data from trials affect whether it would be possible to have data published?

**Professor Newby:** I am not quite sure what you mean.

**Mark McDonald:** Is who owns the data from a trial—the academic conducting the study or the company that is funding it—an issue?

**Professor Newby:** A lot of research is done purely by the company, which the company would obviously own. When the research is done in collaboration with an academic, ownership depends on the setting of the study and the relationship; sometimes the study is completely led by the academic and sometimes it is led jointly. There are different models.

**Professor Fox:** But in the establishment of any large study, one of the requirements is that it will be submitted for publication irrespective of the result.

**Professor Newby:** I have one final point, just to reassure you. All trials now have to be registered. Therefore, even if the results are not published, we know that the trial has happened, so we can go knocking on the door.

**Len Woodward:** An additional point is that data from trials should be available to patients and patients organisations in a language that they understand.

**Professor Goodship:** It is obvious that all trials must undergo a rigorous governance procedure before they are allowed to commence. There is no reason why part of that procedure should not be a guarantee that any results will be published, which would be part of the process of industry getting approval for trials.

**Nancy Greig:** Some of our members highlighted that, because the ABPI has tightened up its code of conduct a bit, some organisations find it difficult to access information about a drug, such as a briefing or a patient leaflet about a trial, that is in plain language and which they can use as a basis for their submission. There needs to be a bit of work on that. Obviously, there should be a balance between pharma companies being able to solicit patients organisations and encourage them to make submissions, perhaps spoon feeding them information, and organisations getting information that they need for the basis of their submissions. The information is not always readily available for every new drug in Scotland.

**Dr Simpson:** Do the patients organisations feel that there is adequate patient representation in the IPTR groups?

**Ian Mackersie:** I have no direct experience of IPTRs and the IPTR groups but, as far as patient representation is concerned, I can tell you how the system worked extremely well in England. We contributed to a submission that was presented to AGNSS, the advisory group for national specialised services. We were allowed by the NHS to present information about the patient experience, which comprised evidence from 10 of our members who had direct experience of a disease. In addition, the NHS supplied us with consultants to help us to elicit the information and present it to AGNSS. In the end, we produced a 16,000-word document, which was an extremely powerful piece of work.

That document went to AGNSS, which also took the clinical submission and recommended to the Department of Health that eculizumab—the drug that we have been speaking about—be centrally funded in England for the treatment of aHUS. The Department of Health overruled that advice from AGNSS just last week when it put the case to NICE for further consideration of the drug's affordability. That does not detract from the consideration and assistance that the patient group was given in providing the patient voice and patient experience for the process.

**George Grindlay:** When an IPTR is put to the local ADT committee, the patient to whom the request relates has an opportunity to have a say at the decision-making committee meeting. They can choose whether to appear at that meeting.

**Dr Simpson:** Can they take an expert or a patient advocate with them?

**George Grindlay:** Yes.

**Dr Simpson:** That is helpful.

**The Convener:** What is the level of participation in that process? The patients are present at the meetings as witnesses, not as participants, are they not?

**George Grindlay:** The patients are there to support their submissions for the drugs that are on their IPTRs and to give their personal view of the impact.

**The Convener:** I am thinking back to a constituency case of mine. That experience was very difficult for the patient involved, as I recall. She was not allowed to participate or speak and there was a dispute about whether her nominated expert was allowed to make representations. It was just the basic presentation.

Does practice vary throughout the country from one board area to another? There is obviously greater participation in Tayside. Is it the same in the NHS Greater Glasgow and Clyde area or Edinburgh? Can anyone speak to that?

**Professor Newby:** I do not recall the patient being allowed to be present in Lothian. They certainly were not in my case.

**George Grindlay:** I will clarify what is meant by "advocate". An advocate is someone who is able to speak for the patient rather than a professional speaking on their behalf.

**Len Woodward:** I do not know much about the Scottish IPTR process but, in the one in which I was involved in England, we were not allowed to participate. We could only give written statements. I would have liked to have the opportunity to participate. We had such an opportunity for the AGNSS process, in which a patient gave evidence about what aHUS is like.

In Wales, patients are not allowed to talk to the decision-making forum; they have a layperson to talk for them.

**The Convener:** We can write to the health boards and ask about the level of patient participation in the process.

**Bob Doris:** We heard talk of the social care cost of a drug not being provided to patients. I am reminded that the UK Government, under its reserved powers, is considering value-based pricing and the holistic cost of medicines as they come through the system. The committee has been trying to find out a little bit about that, but how it is all going to shake down seems to be cloaked in mystery. Once it all shakes down, will the IPTR system, the pharma companies or the approvals system more generally consider in more detail the on-costs to society, as well as the cost to the individual, of not prescribing certain medicines? Should we hope that that will have an impact on the SMC approval process and the IPTR process?

10:45

**Professor Fox:** You have put your finger on something that is fundamental. If we take the anticoagulants as an example, the assessment that NICE did was to say, "What is the cost of the new drug? What is the cost of warfarin anticoagulation? What is the cost of running the clinics?" We add that up, and that is it. The cost came within a boundary that NICE would approve. What it did not consider was how many strokes were prevented and what the impact was on the long-term care of people who have suffered strokes. I absolutely agree that we need to look at the long-term implications of prescribing and not prescribing a medication.

**Nancy Greig:** I agree that there needs to be more consideration of the societal benefits of medicines. The alliance accepts that the quality-adjusted life year system is robust, but it is not clear to the alliance or—from reading the *Official Report* of your previous discussion on the subject—to many others how the plans for value-based pricing will be interpreted in Scotland and how it will interact with the current SMC process. There needs to be more research both in Scotland and more widely, as it is a Department of Health plan for value-based pricing, about the burden of illness and about systems that take into account the long-term effect of prescribing on society.

In our written submission, we reference the socio-technical allocation of resources—STAR—approach, which was developed by NHS Sheffield in partnership with the Health Foundation and the London School of Economics. That is a value-for-money analysis with stakeholder engagement that uses a deliberative process involving stakeholders, patients and the public. Perhaps we need to look at systems such as that.

**Bob Doris:** I just wanted that to be put on the record. I think that it was the alliance that mentioned it during earlier conversations.

**Nanette Milne:** I understand that there is a right of appeal against IPTR decisions. Is that operating effectively across Scotland? How does it work?

**George Grindlay:** Not having been through the IPTR process personally, I have only taken on reference information. There is a process that people can go through, but I cannot say how effective it is. The committee might wish to seek information on that from the boards.

**The Convener:** There is a general area there about patient knowledge and indeed clinical experience. I am sure that we can pick that up.

Going back to the issue that Richard Simpson raised, I note that there has been some movement in the area during the inquiry. A review is under way of general access to new medicines, we have

a review of individual patient treatment requests, and a fund has been announced for medicines for rare diseases. I presume that everyone supports that movement and the fact that the issues are being addressed. There will be no dissent there. I suppose that the next question is to ask what priorities should come out of that for the various interests that are represented round the table. Does anyone wish to comment on that before I close the session?

This is also an opportunity for you to cover any areas that committee members have not raised in their questions, and any issues that you want to leave with us for consideration when we conclude the inquiry and proceed to produce our report. You have that opportunity at this point if you wish to take it up. We encourage you to keep an eye on our next round-table session, and if there is anything that was not picked up today that you wish to cover in a more informal way after the meeting, we encourage you to email the clerks to inform the committee about that.

Would anyone like to add anything or pick up on any points?

**Len Woodward:** Going back to Nancy Greig's point about societal costs, I should point out that NHS Scotland is already developing financial models in that respect. Indeed, it has used quite robust data to determine medical interventions and health programmes for our sister disease, HUS.

**Ian Mackersie:** There should be more consistency between England and Scotland in the treatment of rare diseases and, indeed, the funding of that treatment. There are various anomalies between the two and I am sure that, when it finishes its deliberations and in view of the various investigations that have been carried out, the committee will recommend a system that will be effective and acceptable in Scotland. We want England, Scotland, Wales and Northern Ireland to have the same system.

**Professor Fox:** As Dave Newby has pointed out, Scotland probably has one of the most robust record linkage systems linking long-term outcomes with individual patients. A particular block, however, is the issue of confidentiality and anonymous records. If we could apply pressure by suggesting that, for the purposes of patient safety, bodies that collate this sort of data be able to access truly anonymised records that do not compromise the patient, it would be a big advance.

**The Convener:** That is useful.

**George Grindlay:** I want to acknowledge the SMC's work and, in particular, the establishment of a public involvement officer, which has enabled patients to become more involved in decisions about bringing new medications on board.

That said, I will make one constructive criticism. Angus long-term conditions support group has 100 members with varying and different conditions but, because we are not a constituted organisation—we have no committee, no papers, no statutory basis and no company status—we are not allowed to make any input to the SMC. As a result, the 100 people we represent do not have a voice. Moreover, the SMC does not take individual information, so again they go unrepresented.

**Nancy Greig:** In addition to George Grindlay's point, I note that the Health and Social Care Alliance Scotland works very closely with the SMC and want to acknowledge the public involvement project's positive impact over the past two years in increasing the number of submissions. However, although that demonstrates a really positive commitment to public engagement, we still have an opportunity to enhance the system and the transparency of the SMC's decision-making process. Our members have raised a number of issues about the process, including the inability of certain third sector organisations to make a submission because they are not a constituted group or uncertainty over the importance of their submission in the decision-making process. The briefing notes on the SMC website for drugs that have been passed simply state that a patient interest group made a submission and do not go into any detail about the submission's contents. Enhancing the transparency of the decision-making process can be only a positive move that will encourage more submissions.

**Ian Mackersie:** Finally, I thank the committee for allowing us to have our say. As a small charity that is run entirely by volunteers and which represents a tiny group of people with a serious but obscure disease, we rarely get the opportunity to voice our concerns.

**George Grindlay:** I echo those comments.

**The Convener:** Thank you all very much. In drawing this first evidence-taking session to a close, I want on the committee's behalf to express our appreciation for the time and the evidence that you have given us this morning.

10:54

*Meeting suspended.*

10:59

*On resuming—*

**The Convener:** We continue with agenda item 1, which is on access to new medicines. I welcome our new panel of witnesses. As we did previously, it would be useful if all of us around the table could introduce ourselves.

I am the MSP for Greenock and Inverclyde and I am convener of the Health and Sport Committee.

**Alastair Kent (Genetic Alliance UK and Rare Disease UK):** I am the director of Genetic Alliance UK, which is an alliance of 156 different patient organisations that support families across the spectrum of genetic disorders. I am also the chair of Rare Disease UK, which is a multistakeholder coalition that has been created for the express purpose of supporting the improvement of services and support for families with rare diseases.

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

**Professor Peter Hillmen (PNH Alliance):** I am a haematologist from Leeds with an interest in paroxysmal nocturnal haemoglobinuria—PNH for short—on which I lead the national service in England. I also chair the two international committees on PNH. I support the Scottish clinic on PNH, which is led by one of the Scottish haematologists in Monklands.

**Dr Simpson:** I am an MSP for Mid Scotland and Fife.

**Lesley Loeliger (PNH Alliance):** I am the founder and chairman of PNH Scotland, which is a charity that was set up to support PNH patients throughout Scotland. I am also a patient with PNH.

**Gil Paterson:** I am the MSP for Clydebank and Milngavie.

**Dr Mark Eldon Roberts (Salford Royal NHS Foundation Trust):** I am a consultant neurologist in Salford and Manchester. I lead the neuromuscular services in Manchester, I have a special interest in metabolic disorders and I have seen all the Scottish patients with Pompe disease, which is a rare condition that we will be talking about today.

**Mark McDonald:** I am an MSP for North East Scotland.

**Joan Fletcher (Association for Glycogen Storage Disease (UK)):** I represent the AGSD and my role is to support families who suffer from Pompe disease.

**Nanette Milne:** I am an MSP for North East Scotland.

**Marion Ferguson (Ivacaftor Patient Interest Group):** I am chair of the ivacaftor patient interest group, which is a group for patients with the G551D mutation, which is a rare form of cystic fibrosis.

**David Torrance:** I am MSP for the Kirkcaldy constituency.

**Richard Lyle:** I am an MSP for Central region.

**The Convener:** With those formalities now over, I hope that we can have a good exchange. Mark McDonald will ask our first question.

**Mark McDonald:** In today's earlier session, I asked a number of questions on whether we can be confident that the regulatory bodies see all the available trial data that relate to new medicines. Following on from that, we had a discussion on unpublished trial data on related medicines. How can we ensure that the best available data are made available to regulatory bodies so that we get the most robust decisions on new medicines coming on to the market? How do we ensure that new medicines are approved on the back of the most robust data available? Where new medicines are not approved, can we be confident that all the trial data are made available to allow the regulators to make the best decisions? I throw that one out there.

**Professor Hillmen:** I should have mentioned that I also sit on the Medicines and Healthcare products Regulatory Agency advisory board for haematology and oncology. That means that I see the submissions coming through for new therapies across oncology, so I also see the issue from the other side of the fence, if you like.

For new therapies that are just being approved, only a limited amount of trial data is available. Virtually all those trials are sponsored by the pharmaceutical industry and the data are required to be used in the submission for approval of the drug. At that stage, virtually all the trial data will be from that domain. As was alluded to in the earlier evidence session, part of the ethical approval of trials is a requirement that the data should be published and presented at international meetings and in peer-reviewed literature. There is an obligation on the companies to have the data peer-reviewed further down the line, although that does not always happen.

Another point to make is that, as is the case with the drug for PNH that I have been involved with for more than 10 years, data evolve after the trial and the approval. For example, we publish our own data on survival. At the time of the drug's approval, there may not be survival data, but we now have very convincing survival data on PNH. Even after the approval, the data continue to accumulate. The data should be available.

**The Convener:** Does anyone else want to respond to Mark McDonald's question?

**Marion Ferguson:** Professor Elborn, who is professor of respiratory medicine at Queen's University, Belfast, said about the data:

"These data showing the consistent and sustained benefit of this medicine confirm that ivacaftor has the potential to make a significant difference to the lives of children"

and young people with cystic fibrosis G551D. He added:

"The data don't capture the full benefit for the patients. It's been very noticeable in the patients I look after that they are able to do things they previously couldn't after starting treatment with ivacaftor. They feel better and more able to plan for the future."

Data are important, but many more case studies need to be brought forward to the SMC—through patient interest groups, for example—so that people can see the difference that the medicines make to quality of life, rather than just looking at the data alone.

**Alastair Kent:** When we look at clinical trials for rare diseases, it is important to recognise that often they are carried out on very small populations and that, although the data may be of high quality, there is not always great depth. Often, marketing authorisations are granted by the European Medicines Agency on a conditional basis, with further evidence being required. The evidence that is submitted to the European Medicines Agency for the granting of the marketing authorisation is available, but it is much more difficult to get the real-world data based on application of the drug in practice.

In the previous evidence session, one of the witnesses referred to open-access journals and the publication of information in journals that are freely available. In that situation, the cost of publication is met by the institution that generates the data rather than by the sale of the journal in which the data are published. Although that may open up the readership, there is a danger that the submitting institutions—the universities, the hospitals, and the companies—may choose to not incur the expense of publishing data that could be of limited quality if that will impose an additional cost on strained resources.

In principle, the idea of open access and the publication of all information is a good, positive development. However, we need to be careful to factor in resources to make that a realistic possibility and not to put institutions that are already under financial pressure in a cleft stick over deciding what they can and cannot afford to publish.

**Dr Roberts:** I echo what Mr Kent was saying. There is obviously a risk of publication bias towards positive data in clinical trials. To give you a flavour of how small those clinical trials are, the biggest ever trial in the world on Pompe disease had just 90 patients. They are very small trials, but it is a very rare disease.

On a positive note, because the condition is so rare, observational data that might not otherwise pass a threshold for inclusion in a trial are quite frequently published. For example, a large countrywide study of Italian patients, a Spanish paper and a German paper have been published—and an English paper is about to be published—on all of the patients with Pompe disease.

The German paper was especially interesting because it showed many patients deteriorating on the treatment that is now available, but in the patients who showed the biggest benefit, there seemed to be an additional benefit from exercise. That study has been powerful in, for some, reaffirming and, for others, enhancing our understanding of the benefit of potential additional therapies. However, I agree entirely that the data on patients who do very badly probably do not get published, unfortunately.

**Professor Hillmen:** I want to follow up on Marion Ferguson's point. Therapies for rare diseases tend to be targeted treatments that are specific to that disease. The early results from the trials are dramatic in stopping the process of the disease.

However, the data on deaths associated with conditions such as PNH, renal failure and thrombosis come later. We had the data for PNH with eculizumab in 2007, when the drug was licensed. In England, it was funded from 2008, but it was only from 2010 that we started to demonstrate survival advantages in relation to thrombosis and renal failure.

Our experience is that, if there is a therapy that effectively treats the disease, the evidence that can be gathered from patients across the United Kingdom is powerful and is useful several years later.

**Mark McDonald:** That is helpful. I recognise that, by definition, rare conditions do not lend themselves to large trials. At the same time, I think that some of the information has been beneficial.

I take on board Mr Kent's point around the resource constraints, but Professor Hillmen said that there is an ethical obligation to publish but that does not always happen. Do we need a legal requirement to publish?

**Professor Hillmen:** The primary trial data that are used for the approval of drugs are almost always published, because they come from the key trials. On the ethical issues, when a drug is approved, the primary end point of the trial involves the publishing of the data. However, the important data come out later and it is hard to ensure that there is an obligation to publish that, because the trials might not be followed up in the long term.

I have concerns about the open-access journals. Academic groups are under intense pressure to publish data, but in journals that are

peer-reviewed and have high impact factors, because that is where our funding comes from. The open-access journals are just as difficult to create manuscripts for but are of little value to the academic institution. It is a good concept, but I do not think that it works in reality.

**Nanette Milne:** I take it that there are no sanctions that can be applied when publication requirements are not met.

**Alastair Kent:** If you think about the real-world situation of clinicians working with patients with a condition, under pressure of time and resources, it is difficult to add additional requirements on them to record and publish data. If there is to be an ethical or legal obligation to publish this kind of data, you must ensure that the clinicians are supported, with the infrastructure to enable data to be collected systematically and examined realistically to reach conclusions, otherwise there is a danger of a mass of unstructured information coming out, which will be difficult to interpret and make use of for policy and planning decisions.

**Professor Hillmen:** There is an obligation to publish data, but I do not think that there is a process to ensure that that obligation is carried out. I do not have any experience of publications on rare diseases not being presented publicly. However, recently, with a more common disease, one of the more negative trials was not published. I was the chief investigator for the UK in that regard and insisted on getting the report. It was decided last week that the data would be published, as it is extremely valuable data for the patient group.

I do not know whether there should be a legal obligation to publish.

**Dr Roberts:** At the moment, in real world clinical practice, it is difficult to collect the kind of robust scientific data that would be collected in a trial. However, it would not surprise me if, with NICE taking on some of the roles that AGNSS holds in England, that became an obligation.

Given that AGNSS has been so transparent in making its report system available, that data could eventually become available. Given the sums involved in treating these rare patients, I, for one, feel that that would be a reasonable request. However, I would very much echo Alastair Kent's comment that if it becomes a legal requirement, we must be given the time, resources and administrative support to do it.

11:15

**Mark McDonald:** I am not familiar with the acronym AGNSS. I wonder whether we could get that clarified, just for the record.

**Dr Roberts:** You will be aware that, very much like the SMC, NICE deals with many disorders and looks at the cost-effectiveness of treatments for those. However, a decision was taken to have a specialist group to look at very rare conditions whose treatment might be very expensive and where centrally commissioned services might be required. The process has been very successful in the past five years. It is now coming back under NICE, but Michael Rawlins has already stated that he is keen that the attributes of AGNSS continue with the NICE process.

**Mark McDonald:** Would that process be similar to the process for the £21 million rare medicines fund that the cabinet secretary recently announced?

**Dr Roberts:** Yes.

**Alastair Kent:** Not entirely. The acronym AGNSS stands for the advisory group for national specialised services, which was set up to advise the national specialised commissioning team—or NSCT—in England. AGNSS was mandated to make recommendations to the secretary of state on conditions affecting fewer than 500 patients in England. If AGNSS made a positive recommendation to the secretary of state, the secretary of state had the power previously to instruct the health service to provide the service—or not as the case may be.

When the Health and Social Care Act 2012 came into force, the secretary of state had removed from himself the power to make demands upon the national health service, and the NHS Commissioning Board was given responsibility for making those day-to-day allocations. AGNSS no longer had a route under the law to implement its recommendations. It is being dissolved with effect from 31 March and responsibility for making decisions that were formerly the province of AGNSS has been passed to NICE.

NICE has not yet determined the procedure that it will adopt for those very rare conditions. Historically, it looked at whether its existing procedures would work for very rare conditions and decided that although there was no theoretical barrier to the NICE process delivering a result, the evidence—the population of the equation to determine cost and clinical effectiveness—would be so thin on the ground as to invalidate the process.

AGNSS did not have a separate fund to disburse; it had the power to recommend to the secretary of state, and the secretary of state, if his decision was to implement, had to determine where the necessary resources would come from. The situation is slightly different.

**Lesley Loeliger:** Mark McDonald mentioned the extra £21 million. The issue that we have with the £21 million fund is that it will make no difference to PNH patients because it has been stated that the fund can be accessed only if you have a successful individual patient treatment request. PNH patients and other rare disease patients are not getting through the current IPTR system.

**Dr Simpson:** That takes us on neatly to the central problem with rare diseases, which is that the group that is used for the research to produce the licence is very small. The requirement for IPTR is that the individual is different from the general population covered by the medicine licence. For the people represented by this group of witnesses, that seems to me to be a total catch-22 situation, which the IPTR system cannot resolve. Am I misreading the situation?

A patient with PNH, for example, is unlikely to be exceptional within the licence group. That might not be true of other conditions of which panel members have experience—they might have experienced situations in which, even though the condition is rare, there is a sub-group of patients that would benefit from a particular treatment. However, there seems to me to be a fundamental flaw with the IPTR system. Am I correct?

**Professor Hillmen:** Absolutely. We had approval to treat 195 patients—35 of which were in the UK—with eculizumab. Every patient benefited from the trials and patients have subsequently benefited since we have had a nationally funded service. In fact, most of the Scottish patients eventually got through the IPTR system and they have all benefited from treatment. The trials are designed to take patients with a specific abnormality and treat them with a specific drug that interferes with that abnormality, so you would expect all patients to benefit. To then select patients separately is impossible.

The other issue with the IPTR system concerns patients who have been declared not to be exceptional from the group and who are therefore not funded, even though the rest of the United Kingdom will fund patients with similar conditions. If, for example, a thrombotic complication occurs, the patient can die within 24 or 48 hours. There is no time to go back through the IPTR system before the patient can be rescued with treatment. We have treated people around the UK the day that the complication has happened because we do not need to go through a bureaucratic process to get approval. The IPTR system disadvantages not only patients with rare diseases generally, but patients in extremis, because there is no funding mechanism for patients in that situation.

**Dr Roberts:** I very much echo the points that have been made. With a rare condition, which NICE would define as an ultra-orphan condition, or one with a prevalence of fewer than five in 100,000—in the case of Pompe, there are only 111 patients in the UK—it is difficult to show exceptionality and that one patient is radically different from another. Clearly, as we have referenced already, the clinical trials have been small and have sought to recruit most of the affected patients; to show that one individual patient is different from the trial patients is extremely unlikely, if not completely impossible.

The IPTR system, which is difficult to put into practice, has several potential problems. First, clinicians might be put off the process because they feel that they might not succeed on behalf of their patient, which would be tremendously upsetting for the patient and would damage the doctor-patient relationship. Secondly, the patient might feel that it is a rigorous and difficult process to go through. Thirdly, many of the conditions that we are hearing about are progressive and, in the time that the process takes, the patient's condition might become much worse.

Although the SMC has a world-wide and enviable reputation in judging evidence, rare diseases are a difficult area, and a different approach might be required, as the committee recognises.

**Alastair Kent:** Dr Simpson is absolutely right: the IPTR system is a catch 22. The notion that underpins the IPTR is that there is an on-off switch that can demonstrate difference. For example, there is a specific genetic mutation and people who have it respond to ivacaftor, but the people who do not have the condition do not respond to that drug. However, that is a rare situation.

With most rare diseases, like most other diseases, there is a continuum of response and, given the amount of evidence on effectiveness at the point at which the drug becomes available, it is not possible to say that patient A will respond better than patient B. Therefore, you cannot make a case for exceptionality, because all the patients in the group—we are talking small numbers—will respond to a greater or lesser extent. Over time, you may be able to gather evidence that determines that some patients respond better than others and kind of parse the patient community to focus on those who do better. However, in the absence of an alternative, most patients would rather have the opportunity to benefit, because 10 per cent of not a lot is better than 100 per cent of nothing, if nothing means that you progress to die.

If you do not allow patients to have access to medication in the first place, you never get to the position of being able to make further decisions and determine whether something was as useful in practice as clinical trial data indicated it would be.

**Dr Simpson:** I would like to ask specifically about cystic fibrosis, which seems to be in a catch-22 situation par excellence. There is a new drug—I do not know it; it is completely new—specifically for the G551D group, so presumably the licence says that. Therefore, proving exceptionality would be absolutely impossible. If the drug has not been approved and it cannot be applied for under IPTR, we do not have a system with which that drug can be used in Scotland—or do we?

**Marion Ferguson:** You are absolutely right. In our situation, ivacaftor was 100 per cent beneficial for 100 per cent of the patient cohort, which was very small: between 53 and 72 patients. Some patients might not be genotyped and we do not have an exact figure because of data protection.

We could not prove exceptionality. We had perhaps one child who more urgently required intervention, maybe through an IPTR, but who is to say that that patient would benefit the most? It was a very difficult situation. The patient who is healthier, or appears healthier, might in fact benefit most.

**Professor Hillmen:** That is true, but many of the other rare diseases are genetic. PNH and glycogen storage disease are genetic and their therapies are specific to those genetic problems. Every patient with PNH has the same genetic abnormality and responds to the therapy. To pick out one disease and say that it is different because it so obviously targets a sub-group of patients is probably incorrect, because all the other diseases have largely genetic causes that are specifically treated.

**Lesley Loeliger:** The current IPTR recommendations state that each health board must bring in a recognised expert. To take PNH as an example, if a health board decides to bring in a haematologist who has never met a PNH patient, their opinion will be less informed than that of a recognised PNH expert. In Scotland we have 30 PNH patients, of which only 12 have been recommended for the drug. The reason why 12 have been recommended is that we have consultants such as Professor Hillmen and Dr Lindsay Mitchell in Monklands, which is the Scottish centre of excellence for PNH. They understand the drug and the conditions so well that they can say that, for those 12 patients, Soliris will be 100 per cent effective.

The point is that if there is a Scottish centre of excellence for a condition, expert opinion must be sought there. Among those with my condition, PNH, 12 patients out of 30 have been recommended for the drug. The difficult decision to decide who the drug will work for has already been made before it goes to an IPTR situation.

**Dr Simpson:** That is very helpful.

11:30

**Bob Doris:** I am curious about the new arrangements that the cabinet secretary has introduced as a result of the interim findings of the Government's review. Will the IPTR methodology be amended to take into account, say, Ms Ferguson's point about patients suffering from one particular strand of CF? Has there been any movement in that respect?

**Marion Ferguson:** I am not an expert on the IPTR system, but I think that it probably works very well for a much bigger population. However, rare diseases that have such a small cohort of patients need to be taken out and dealt with not through IPTR but in a separate process. Some kind of group IPTR system, in which the whole cohort could apply for funding and be dealt with as a group rather than as individuals, would be ideal. After all, having expert clinicians sit down and prove exceptionality in 18 applications must be a great waste of resources; even if they spend only an hour and a half or two hours on each, they would be taking 36 to 40 hours—a full week's work—to deal with only 18 patients.

**Professor Hillmen:** Supporting those comments, I point out that a more common disease would give rise to 10 or 20 IPTR applications, which would allow the committee to consider the different merits for patients. However, a rare disease might give rise to only one application every three years. How does the committee then decide that this particular patient, rather than the other 12 or 30 patients in the country, is exceptional? It is a real issue with regard to the IPTR.

**Dr Roberts:** I echo the comments made by Ms Ferguson and Professor Hillmen, particularly in view of what has happened with Pompe disease in Scotland. Initially, there were 11 patients, two of whom moved across to England to get treatment. Of the nine patients left, two were children—and it was a battle to get the treatment even for them. Two of the seven adults have been treated, which demonstrates the mismatch in treatment between health boards. Every two weeks, one patient comes all the way to anything-but-sunny Salford to see me for a clinical trial, which leaves five patients not receiving any treatment at all. As Ms Ferguson has suggested, the current IPTR system does not seem fit for purpose, at least for this rare patient group, and a more modified group approach—with, I would agree, the pursuit of a very good evidence base—would seem fairer.

It all depends on body mass but in England it can cost up to an eye-watering £250,000 a year to treat a patient with Pompe disease. However, there are stop criteria. In other words, if, after a good period of assessment on treatment, it is clear

that individual patients are not benefiting, we might consider phasing out their treatment.

**Professor Hillmen:** I echo those comments. From my recollection, PNH Alliance has had 12 IPTR applications submitted in Scotland, nine of which have been approved; those patients are now receiving therapy. The three applications that have not been approved are all in the same health board area, so it is clear that there is a difference across Scotland in the IPTR process's effectiveness or ineffectiveness.

**The Convener:** I know that other committee members want to ask questions either on this issue or on others, but Bob Doris has not finished his own questions and other witnesses want to respond to them.

**Alastair Kent:** Following on from Mark Roberts's comments, I think that one alternative to using IPTRs as a gateway to accessing a particular therapy would be the system of coverage with evidence development that is operated in Australia and the Netherlands and in which the presumption is that the drug will be licensed but with stop criteria in place. In a sense, you say, "Right, we don't know how effective this drug is going to be but on the basis of the clinical trials it looks like it's going to work. We will fund it for patients who need it for however long it takes, depending on the nature of the condition."

Those who are allowed to prescribe and monitor the drug have an obligation to report according to a set of predetermined criteria so that we can establish whether the intervention is as good as we thought. That means that individual health boards or funding authorities are not forced to make decisions between apparently equally deserving patients based on where they live but are put under an obligation to generate the evidence that allows a proper decision to be made on the benefits of the real-world application of a therapy.

**Lesley Loeliger:** I will follow up what Professor Hillmen said. The one thing that we really must have is equality between the health boards because we do not have that with the IPTR system at the moment.

Last Thursday, I held my PNH patient group at Monklands. Two patients sat beside each other. One was a man who is on eculizumab and had taken a day off work to come for his appointment; the other was a woman who had to be brought in by her husband because she is so physically and emotionally ill with her condition. The woman asked the man who is on the drug, "Why did you get the drug and I didn't?" His answer was, "Oh, I'm not in your health board, so I was fine."

The concept that there is a difference between health boards is unfair. Having a slightly different process and considering requests more centrally would take that impression away.

**Bob Doris:** I will ask a brief question about the idea of stopping a treatment. Can you envisage there being a situation Scotland in which you prescribe a drug to a cohort of 10 people and then you decide that it is not working for five of them and that you will stop it? There would be a heck of a hue and cry about that, would there not? Would it be practical to do that, or is it an idea that just sounds good but is unworkable in reality?

**Joan Fletcher:** We have spoken about AGNSS and so on, but the system will definitely happen in England—there is no question about that. Clinicians will have to give evidence that a drug is working for patients on a limited timescale, and I cannot see why the same system could not work in Scotland.

**Alastair Kent:** It is what happens in medicine. Patients are taken off medicines all the time, whether they are rare or common. With some of the drugs that we are talking about, the issue is being clear that the criteria for evaluating whether a particular intervention is effective take account of the patients' experience and views about the salience of various elements of the condition in the impact that it has on the quality and quantity of their lives.

With some conditions, some elements are a nuisance but can be managed; other things really screw up a patient's ability to enjoy a reasonably normal quality of life. As long as the appraisal process for effectiveness takes account of the elements that the patients consider to be important to the intervention's effectiveness, there is no reason why a transparent process should not work in practice.

As patients, we have no interest in taking things that do not work, but we have an interest in accessing treatments that will change our lives for the better. If you are going to have such a coverage-with-evidence-development scheme, the important thing is to ensure that the end points are clear to, and accepted by, all the stakeholders.

**Professor Hillmen:** There are two issues. The first is whether the drug is effective and whether we stop it because it is not effective. With the diseases that we are talking about, I do not know of a case in which a drug has not been shown to be effective in the long term if it has been effective in the short term. In that situation, if the drug works, we will not want to stop it.

I guess that we will discuss this a little later, but the role of the expert service is critical. We have stopped and plan to stop some patients with PNH on eculizumab. For example, we had a lady in Scotland who was pregnant. She had treatment through the pregnancy and stopped three months

afterwards because we knew that that was the best way to manage her.

We have more experience than anybody else in the world of managing pregnancy in PNH. It is a complex issue. We have stopped two or three patients with PNH in England because their disease got better. The expert service can do that.

We therefore do look at patients individually and treat them appropriately. It is not really about the expense. We have no interest in treating people with drugs if, for example, there is a risk of infection from having intravenous therapy every two weeks. We do not want to expose patients to that if it is not working.

**The Convener:** Gil Paterson wants to come in, but it must be a clear intervention, Gil, because two other committee members are waiting to come in.

**Gil Paterson:** Sure. It is just on the request to consider having a small group on its own and removing the need for individual applications. Would there not be the danger of the whole group being excluded and its individual members having no redress? I have heard about all the flaws of the IPTR, to which we should pay attention, but at least some people who provide evidence benefit. I would be very uncomfortable with a committee making a decision about a whole group. Can anyone help me with that and explain their views on it?

**Marion Ferguson:** If we were to use a group system, I do not think that it would exclude an individual from coming back and saying "Well, actually, I'm different from that cohort and I want to make an IPTR." I think that there is room for both systems.

**Gil Paterson:** You think so?

**Marion Ferguson:** Yes.

**Gil Paterson:** Okay.

**Bob Doris:** On the stop criteria and the evidence base around that, I have to put it on the record that the question is of course not about whether a pharmaceutical intervention works: it is the degree to which it works, how it impacts on the QALY and what the modifiers are. It is not enough to say that, if we treat someone and it works a bit, we should keep on using that intervention. It is not as straightforward as the discussion around this table might suggest. I wanted to put my concerns about that on the record—I will be interested to see what happens in England.

What I am more concerned about, though, is the 14 health boards interpreting current systems differently, which is not acceptable. Yes, they are entitled to make their own informed decision, but if they interpret the guidelines differently, that is not acceptable. One of the questions that I asked the previous panel was on getting all 14 health boards to be more aligned in how they interpret information, but it would be easier to align fewer than 14. Would it be helpful to have fewer than 14? We would be looking not at health boards but ADTCs.

**Lesley Loeliger:** I think that the wording of the guidelines needs to be changed so that it clearly states who will be asked for their expert opinion. For me, if we had the right wording in the guidelines, it would not really matter how many divisions there were.

**Bob Doris:** So 14 teams are okay.

**Professor Hillmen:** I am not sure that it is a very efficient way of proceeding. As I said previously, the teams, particularly for the smaller health boards, might never have seen an application for an ultra-orphan disease. In such cases, it would be difficult for them to judge the application.

An effective way of proceeding would be to have a single entity such as the national services division, with which we have worked, to oversee the application for the use of a therapy for a particular disease. That is not necessarily a funding issue, but the expertise is important.

The differences between health boards' IPTR processes were mentioned. I have not been permitted to see the IPTR processes, but we have supported local haematologists in generating evidence for them. It is hard to understand the processes without having been through them, but they appear to take the same evidence for patients with the same disease and come up with completely different decisions.

Our experience of the appeal process has been difficult. Our patients are so ill that they cannot go to represent themselves to a committee such as this one. They are lucky to get to the clinic, which can happen only if their husband or wife carries them in. It is impossible for them to come to this sort of committee and argue their case.

11:45

**Marion Ferguson:** Bob Doris said that 14 different health boards is perhaps too many, and that is a good point. My son attends hospital in Glasgow, so it would be the Glasgow health board that would make his application, but we live in Lanarkshire, so it would then have to go back to NHS Lanarkshire as well. It is a roundabout process.

If there was something more centralised—especially for specialised, rare diseases—it would make the process much simpler and avoid the need to go round different boards. NHS

Lanarkshire does not have a specialist in the particular area, so it has to rely on the Glasgow board to make the decision. Ultimately, NSD in Edinburgh is providing the funding with the rare medicines fund, but the application has to go through a lot of hoops. That could be cut out by reducing the number of people who need to make decisions.

**Bob Doris:** Thank you.

**Nanette Milne:** We know of the plans to move to a value-based pricing system, but we have not had much detail on it so far. Do you have any views on the effects that such a system might have on the appraisal system in Scotland, or is it too early to say?

**Lesley Loeliger:** We do not know the details, but whatever form the system takes it is essential that it takes into account the cost offsets. For example, I no longer require blood transfusions every six weeks and I do not face the possibility of kidney dialysis. Other conditions involve things such as plasma exchange. Those treatments cost a lot, so it is essential that they are taken into account. We just need the details.

**Dr Roberts:** We do not know what the system will involve. We all feel strongly that a QALY-based assessment would not work for rare conditions where treatments are inherently expensive because of the development costs. It is important to have a thorough socioeconomic model. For example, many patients with Pompe disease are unable to work because of their disability. If they had an intervention earlier, they could generate income, as well as wellbeing, for themselves. All those things need to be factored into the assessments.

Particularly with conditions that could require acute hospital interventions such as ventilation, if we could treat earlier and delay the period of or requirement for ventilation, there might be hidden benefits. I appreciate that the argument that we should spend money to save it is always a difficult one, but it might have some validity with these rare diseases.

**Alastair Kent:** The rhetoric about value-based pricing is superficially attractive. If something delivers value, perhaps it ought to be able to attract a higher price in a commercially focused environment. However, in the negotiations that have been going on between the UK Government and the pharmaceutical industry, the opposite of transparency seems to have been introduced into the process. A miasma has sprung up around the issue.

It is not that, as a patient and a patient representative, I want to have a say in the price of a drug. What a company seeks to charge for its product is a commercial decision for the company. However, I want to have a say on the value of the drug to me and patients like me.

Part of the problem with the rhetoric around value-based pricing is that it has proved to be singularly difficult to engage in the negotiations about the elements that will determine the value of a drug. The fear is that a simplistic definition of value will be adopted, partly because of the speed of the process—it has to be negotiated, agreed, and up and running in time to be implemented from 1 January 2014, which is 11 months away—partly because of the confusion between value and price and the commercial nature of the discussions that are going on between the ABPI and the Department of Health, and partly because there has been some simplistic interpretation of some potentially complicated issues.

For example, how do you judge the relative value of a drug that will give a child with a formerly life-limiting condition a normal life expectancy compared with the value of a drug that will add a few extra months to a terminal patient with cancer? How can those two situations be put through the same mill? For many patients, the fear is that they will be excluded from the discussions around value. The fear is that the elements that make up the calculation of the value of a particular intervention will be made on the basis of an oversimplistic model because of the necessity to achieve a result under the legislation that has been enacted.

**Professor Hillmen:** Let me support that by giving another example, of which there are many.

When a young patient with two children is disabled, that has an effect on the family and on the children's upbringing because that mother or father cannot participate in the children's care and becomes the cared-for person rather than the carer. Therefore, the issue goes much wider than whether we should provide dialysis or other medical interventions.

As Alastair Kent pointed out, the great concern is that those wider aspects of treating the patient effectively will not be taken into account. The advantage of value-based pricing for drugs is that it works for everybody. However, some drugs may work for only 10 per cent of patients but have no benefit for 90 per cent of patients, whereas other drugs may benefit everybody, although some people may be more severely affected and therefore receive greater benefit.

**Mark McDonald:** A couple of questions that I had intended to ask have already been dealt with, but one of my concerns when I first joined the committee for this inquiry was that we would find ourselves in a situation where illness was pitted against illness, if you will, with different groups claiming a more deserving call on funding. Luckily,

we have not got to that stage, but I have long been concerned that in health issues generally, and for rare conditions in particular, all the attention is given to those who have the numbers behind them, whether that be the number of individuals who have the condition or the strength of the lobbying organisations that have the ability to put across their case publicly.

In our decision making, is there a concern that, because rare conditions affect such a small number of individuals, there is not enough of a voice to advocate publicly on behalf of those individuals, with the result that they are not thought about first? For example, some would argue that the cancer drugs fund south of the border puts cancer above other conditions largely due to the successful lobbying efforts of cancer charities and other organisations. In Scotland and elsewhere, are rare conditions perhaps not given the necessary level of exposure to allow decision makers to take appropriate decisions in favour of those conditions?

**Dr Roberts:** All those points are well taken. Many who work in the rare disease field feel that it is appropriate that cancer and cardiovascular diseases are prioritised given the frequency of those conditions, but there is a concern that—by inadvertent default, to be fair—the rare diseases may become disadvantaged. The very fact that the committee is looking into those issues bodes extremely well, because that is a concern that we have. I think that there is a need for greater representation.

There are practical problems. If the number of patients is small, the number of members of the charity is intrinsically small, which makes it potentially a relatively weak lobbying group. That is why Alastair Kent's group is so important in bringing together those involved with rare diseases. We need almost a champion or tsar for rare disease management. Perhaps Mr Kent would like to comment on that—if I am allowed to suggest that to another participant, convener.

**Alastair Kent:** I am second in line, I think. Professor Hillmen had his hand up first.

**Professor Hillmen:** I agree entirely with Dr Roberts. This process is evidence that rare diseases are being taken seriously. Those of us who have been involved in rare diseases for many years have not had this sort of access before, and patients in some health communities have not had any real voice. Even the patient group has not been able to access the committees that make the decisions.

We are very much focusing on drugs and the cost of drugs, but we cannot underplay the importance of specialist services. The patient needs to understand their disease and know that they are getting the best advice. Sometimes the therapy is not a drug, but physiotherapy or surgery. That all needs to be considered as a whole service to get the best treatment for patients in Scotland.

**Alastair Kent:** I am flattered by the recognition from Dr Roberts. Thank you.

I wish to make a number of points in response to Mr McDonald's question. Yes, there is a risk that the absence of advocacy could prejudice the effectiveness of the case for a particular intervention. Patient support organisations vary in their effectiveness. I am fortunate in that it is my job to make myself available to you. I do not have to take time off work to be here or to represent and support a patient making a case for a particular intervention. However, many of our 156 member groups are made up entirely of volunteers. If one of them needs to support a family member seeking a particular intervention, that is done at some personal cost, including financial cost, because they have to take a day off work and, if necessary, make arrangements for their own children or the person they are caring for to be looked after.

The people who are directly concerned face institutional barriers in making an effective case. We need to find ways to reduce or eliminate those barriers so that patients with very rare conditions, their families and support groups—where they exist—that are made up entirely of people with the conditions and those caring for them can have their say before the committees and the powers that be.

The importance of that advocacy is growing. Given the direction in which biological research is taking us—which is, increasingly, towards a fragmentation of common conditions into rare conditions because of the existence of genetically distinct subsets of those conditions, with increasing recognition of the relationship between some very rare conditions and the biological processes underpinning more complex conditions—patient-led advocacy in making the case will become an ever increasing factor in enabling policy makers, planners and people who have to make the resource allocation decisions understand the totality of the situation in which they make their decisions.

We need to be careful. I would never argue against seeking the patient perspective. Having a patient and end-user perspective, and knowledge of the impact of the condition on the lives of those who are affected by it, is absolutely fundamental to a fair decision-making process. I think that it was you who asked a question during the previous discussion, Mr Doris, about individual patient input to IPTR requests. Yes, there absolutely should be the opportunity for patients to make a case about the impact of their condition.

However, we need to be hugely careful that we do not turn patients into supplicants before the committee, beseeching it to grant them their treatment. The treatment should be available as a matter of right, as a matter of good medicine and as a matter of good practice, not because someone happens to be able to make a case that melts the committee's heart and is therefore made an exception. Patient advocacy, patient evidence and patient input are central to the process; making people beg for their own therapy would be a completely retrograde step and should not be permitted.

12:00

**Mark McDonald:** I take the point. Of course, that was not where I was going when I mentioned advocacy.

**Alastair Kent:** I was not for a minute suggesting that you were—I simply wanted to make the point.

**Mark McDonald:** Professor Hillmen touched on the interesting issue of specialism. Given that the conditions have such small patient numbers, one has to ask how specialised an individual clinician can be.

Moreover, I—and, I am sure, other members—have dealt with constituents who face geographical problems, as Ms Ferguson so eloquently highlighted, of having to cross health board areas to access specialist treatment. It is all about ensuring continuity. Although there might be specialists in certain conditions at the moment, they will not be there for ever; eventually, they will retire from practice. Following my point about information on some rare conditions not being publicly available, I wonder whether we can be confident that those who are coming through the system will make a career choice to specialise in this or that rare condition and ensure that patients of the future can be treated by specialist clinicians who are able to offer not only treatment, but advocacy on their behalf.

**Professor Hillmen:** I know from experience that the English national commissioning group, which is part of AGNSS, funds services not drugs; PNH specialists, specialist nurses and support staff are funded through the process. Given the size of England and the number of patients, those costs are small compared to the costs of the drugs that we use, but that funding gives us complete independence and allows us appropriately to manage patients who need either those therapies or other supported treatments. Moreover, it allows us to have three part-time haematologists who are also specialists and who therefore not only help to manage that rare disease. After all, only 120 of the 250 PNH patients we see across England, Scotland, Wales and Northern Ireland are receiving treatment.

In Scotland, we have supported Lindsay Mitchell, the haematologist in the clinic at Monklands, who is now an expert in her own right and sees all the Scottish patients. However, we have worked out models that allow English consultants to provide support when she is away. As a result, not only the consultants but the nurses are pivotal to the process—they educate patients, provide home care and so on. The fact that we provide a service and not just one or two consultants adds huge value with regard to effective use of the therapies.

**Alastair Kent:** Mr McDonald has raised a hugely important point about sustainability. There are examples of services that have been founded by enthusiastic practitioners and which have provided valuable support to particular groups of patients, but which have withered on the vine when the practitioner died, moved on or whatever.

The developments that are being encouraged through the European Commission and the European Union Committee of Experts on Rare Diseases with regard to centres of expertise and the clustering of similar groups of rare diseases in order to create a critical mass of expertise and to increase the volume of patients coming through will, in themselves, perpetuate research, which will be made easier as a result. The developments will attract interested clinicians into careers in the field, which will make it sustainable, and they will allow the economically efficient and effective use of expertise and resources, which will be used to full capacity instead of standing idle some of the time because of the small volume of patients. By designating centres of expertise, it is possible to address sustainability. As we heard from Professor Hillmen, who gave us an example of arm's-length support for expertise through professional networks, it is possible to ensure that local Scottish centres of expertise are plugged into the forefront of UK-wide, European and global thinking about what constitutes good and effective practice for a particular cluster of rare diseases.

**Dr Simpson:** I have a question that will lead on from something that Professor Hillmen said. It seems to me that we think of the drugs as being separate from the services, but the drugs are an integral part of the services. Do we have the model for rare diseases completely wrong? Should the model look at the service as a whole? We have the NSD in Scotland and we have the AGNSS system in England. Perhaps we need to adopt a slightly different approach.

We also have a system of reinsurance in Scotland. I am not sure whether it operates across the whole UK for very rare diseases but, in Scotland, rather than one health board funding a

Pompe case, it is reinsured at Scotland level or, perhaps, UK level. Have we got that system—which operates on a Scotland, UK or Europe level, depending on the rarity of the condition—right? There are some conditions of which there are only half a dozen cases in the whole of Europe. Do we need to have discussions about finding a better system that would take the issue away from our 14 area drug and therapeutics committees, which do not have the necessary expertise in most of the conditions? We should recognise that some conditions will be dealt with in Scotland if the number of cases is big enough, but that with other conditions, cases need to be reinsured at UK level, which is where the decision should be made about who gets the service. I am just thinking on my feet.

**Professor Hillmen:** As my previous point indicated, I agree entirely.

The two issues—the drug and the service—are not necessarily entwined. If a drug is to be funded, it is necessary to ensure that the national services division can cope with that appropriately. As Lesley Loeliger pointed out, two thirds of PNH patients do not need therapy, but they need support and might need other interventions that are delivered locally. It is extremely important that those patients can access the service rather than just the drug. The model can vary depending on the disease.

The role of the national services division here or the national commissioning group in the UK is extremely important. There needs to be risk sharing across health boards, and husbandry—if that is the right word—by a Government body such as the NSD is a highly effective way of managing a rare disease service.

England is unlike almost any other country in the world. I have advised a number of countries about approval, including Australia, Canada and the Netherlands. It is very important that the service is funded. That makes commercial sense, because it represents a small amount of money compared with the cost of the drugs.

**Dr Roberts:** I strongly echo the comments about the need, in addition to drugs, for centres of excellence to develop services for patients. By "services", I mean things such as cardiac support and physiotherapy, all of which are vital.

Patients come a long way, so I think that the main role of centres is to provide direction with regard to services, and to work with local services. Although those elements are often just as important as the drugs, it is the drug cost that falls on the centres and which needs to be funded centrally through some mechanism.

I cannot comment on the financial risk-sharing scheme and what would be the best model, but I well understand that an individual health board that had four Pompe patients, for example—which is the situation that one health board in Scotland faces—might feel that it had a very difficult financial dilemma compared with other health boards in Scotland. That is inequitable, so some mechanism at national or UK level needs to be thought through.

**Alastair Kent:** From a patient and family perspective, we need a sense of what the NHS can reasonably be expected to provide, and of what constitutes good practice that reflects current scientific understanding and current clinical possibility. If that includes a drug, that drug should be seen as part of the service, not a bolt-on that is added if someone can get it through their health board. That way, patients and families will have a clear expectation about their interaction and relationship with the health service and with health and social care and other services.

To separate out a drug and put it in a different pot puts uncertainty into patient expectation and leads to anxiety and a disproportionate risk that silo budgeting will creep in. If we identify one little pot for that drug and other little pots that contribute to elements of the service to the patient and family, when those who are responsible for administering the separate pots are under financial pressure, they will be tempted to ask why they should spend their money on a certain patient. If there is an integrated view across the nationally and locally commissioned integrated provision, and across from health to social care and so on, there is a positive incentive for people to do the best that they can for the patient, rather than simply take a short-term approach and ensure that their budget balances at the end of the financial year.

**Lesley Loeliger:** From a patient perspective, a centre of excellence for a condition is absolutely imperative. For patients in my patient group, those who have been told by an expert that they are not eligible for a drug are fine with that, because they have that level of trust and they accept it, but those who are told that they cannot get a drug and that it comes down to whether they are lucky enough to get it, based on the financial situation, are not happy—they are upset and they find it depressing. A magnificent trust is built up in a centre of excellence that gives patients some sort of security.

**Professor Hillmen:** I want to clarify my previous statement in the light of those further comments. I firmly believe that the role of the specialist centre is to work with local specialists—in my case it is local haematologists. We very much share care with local haematologists throughout the country. I do not want it to be thought that we are trying to take over the

patients, because we are not; we are supporting the patients and the service.

**The Convener:** That is helpful.

**Marion Ferguson:** I want to return to Richard Simpson's suggestion that medicines should perhaps be lumped in with services. The people with the condition that we deal with are a sub-group of a larger population of cystic fibrosis patients. I, for one, would not support all the money being put into one pot, because clinicians—or, indeed, managers—might decide that one patient requires medicine, but a G551D patient does not require it, so they will not give them it. That would not be fair. Because our sub-group is within a much larger group, I would like the money for the drug to be ring fenced, which goes against Mr Kent's point.

**Alastair Kent:** To come back on that, we would need to define what constitutes a good-quality service. Marion Ferguson has a clear indication that a good-quality service should include access to appropriate medication. When such a service specification exists, it would not be a matter of robbing Peter to pay Paul within a disease group. There would be the opportunity to have a nuanced discussion and to state what is clinically possible, what is clinical good practice and what is reflected by current scientific understanding, and what responds to patient needs. It is not about playing off subsets of a population against one another; it is about ensuring that there is a shared understanding of what it is reasonable, appropriate and feasible to expect for patients who fall within defined groups.

**The Convener:** Members have no further questions. I thank our witnesses for attending. There might be issues that you feel have not been covered or an important point that you wish to put on the record. When travelling away from the meeting, people often think, "Oh, I wish I'd said that." You still have the opportunity to interact with the committee through the clerks. If you want to add anything or give us a submission for further consideration, feel free to do so.

At this point, do any of our witnesses wish to make brief points on issues that they feel it is important for the committee to consider?

**Professor Hillmen:** We briefly discussed the £21 million fund for rare diseases. That is a welcome recognition of rare diseases, but the clarity will come when we see how the fund is administered. We have highlighted the difficulties in accessing funding for rare diseases. Under the current wording, patients who get funded through the IPTR anyway will have access to the funding, so is it really additional money or is it just rebadging of the money that is already being spent on rare diseases?

**The Convener:** The committee is interested in that too, and will seek clarification on it. As the results of the review on access to medicines and the IPTR become clearer, we are planning engagement in order to get clarity. I am sure that we will retain your attention and that you will observe developments closely. We welcome your support in that process.

It just remains for me to thank you all on behalf of the committee for attending and for your evidence. We appreciate it very much.

12:17

*Meeting suspended.*

12:19

*On resuming—*

# Subordinate Legislation

## General Pharmaceutical Council (Amendment of Miscellaneous Provisions) Rules Order of Council 2012 (SI 2012/3171)

**The Convener:** Agenda item 2 is consideration of subordinate legislation. As members have seen from the papers, the first instrument is a UK statutory instrument that is subject to annulment by resolution of the UK Houses of Parliament or by the Scottish Parliament. That is unusual, but the procedure is the same as it would normally be for a Scottish statutory instrument.

As members have no comments, do we agree that the committee has no recommendations to make on the instrument?

**Members** *indicated agreement.*

## Sports Grounds and Sporting Events (Designation) (Scotland) Amendment Order 2013 (SSI 2013/4)

**The Convener:** Members have no comments on the order. Do members agree that the committee has no recommendation to make on it?

**Members** *indicated agreement.*

# European Priorities

12:20

**The Convener:** We come to agenda item 3. Paper HS/S4/13/3/10 is by the clerks and our Europe reporter, Aileen McLeod. Aileen is not with us today, but members have the paper and I invite comments on it.

**Bob Doris:** I thank Aileen McLeod for her input and for taking on the role of reporter. I am keen to have active and healthy ageing on the agenda. I attended a conference on that in Canada, and I know that the European Commission is doing a lot of work on it. It is obviously relevant for this committee to explore that, so I support that recommendation.

**The Convener:** The basic decision that we have to make is on the priority topics that Aileen McLeod has suggested. Those are the e-health action plan for 2012 to 2020 and the European innovation partnership on active and healthy ageing, which Bob Doris referred to. We also need to consider—although this is not an either/or option—whether to monitor the UK Government's review of the balance of competences and ascertain how the Scottish Government intends to respond before considering our approach; and to keep under review any developments concerning the two main priorities from 2012, which were revision of the tobacco products directive and a package of innovation in health and medical devices.

Do members agree with the recommendations, as laid out in the paper?

**Members** *indicated agreement.*

**The Convener:** That concludes our meeting. I thank members for their participation and patience.

*Meeting closed at 12:23.*

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