HOUSE OF COMMONS

Health and Social Care Committee

Oral evidence: Future Cancer: NICE Medicines Appraisal (Enhertu), HC 739

Wednesday 8 May 2024

Ordered by the House of Commons to be published on 8 May 2024.

Watch the meeting

Members present: Steve Brine (Chair); Paul Blomfield; Chris Green; Rachael Maskell; James Morris.

Questions 1-70

Witnesses

I: David Brocklehurst, Head of Oncology, AstraZeneca UK; and Haran Maheson, Vice President and Head of Oncology, Daiichi Sankyo UK.

II: Baroness Delyth Morgan, Chief Executive, Breast Cancer Now.

III: Dr Sam Roberts, Chief Executive, National Institute for Health and Care Excellence (NICE); and Helen Knight, Director of Medicines Evaluation, National Institute for Health and Care Excellence (NICE).

Written evidence from witnesses:

- [Add names of witnesses and hyperlink to submissions]

Examination of witnesses

Witnesses: David Brocklehurst and Haran Maheson.

Q1 **Chair:** Good morning. This is the Health and Social Care Committee for the second day in a row—we just cannot get enough. This is our public evidence session on the morning of Wednesday 8 May as part of our Future Cancer inquiry. As an addendum to that, we are looking at the NICE medicines appraisal process. We are drafting our report on Future Cancer, which is the culmination of a long and complex inquiry into that subject. Today we will explore NICE's approach to appraising medicines for use in the NHS in England, which obviously has implications across the United Kingdom, with a particular focus on NICE's revised evaluation system, which came into effect two years ago.

The aim of the session, for those watching and listening, is to understand the implications of the changes, notably of the introduction of what is called the severity modifier on novel cancer medicines. This sounds complex—it is—but there are real people's lives at stake here. This is an opportunity for our cross-party Committee to explore the system through high-profile cases, not least that of Enhertu, a breast cancer drug we will be discussing throughout the session. The session is not specifically about that—it is about NICE medicines appraisal, with various examples.

We have three panels and we have to crack on, because we have a fairly tight day here, including Prime Minister's questions downstairs, which Members need to get to. Our first panel is in place and that is David Brocklehurst, who is head of oncology at AstraZeneca UK, and Haran Maheson, who is vice president of oncology at Daiichi Sankyo UK. Thank you very much for coming.

I will kick off, if that is okay. You said in your joint note to us that you are "keen to explain, as transparently as possible, why this outcome"—in respect of Enhertu—"has happened." Broadening it out, as I have said we want to try to do, why do you think that NICE reached the decision not to recommend Enhertu for use on the NHS on England? What do you think that decision says about the state and the status of the appraisal process for cancer medicines in England today?

David Brocklehurst: Sure. Thank you so much for the question, and thank you for convening this session—these are such critical topics. First, as you mentioned, there is the pressing issue of the recent NICE appraisal of Enhertu for HER2-low metastatic breast cancer. Clearly, we are highly disappointed with that outcome and it is intertwined, as you rightly recognise, with the recent change in NICE methods and the introduction of the severity modifier system, which has been central to this appraisal.

Q2 **Chair:** Bearing in mind that there are people watching this who may not be literally living and breathing this every day, in terms of the details of the NICE severity modifier, how would you explain, in the best layman's terms, what the severity modifier does or attempts to do?

David Brocklehurst: Sure. It is essentially an algorithm used by NICE within their appraisals for new medicines to assess the severity of different diseases, and it classifies them as "high severity", "medium severity" or "not severe". That determines the weighting and the outcomes of the appraisal, and essentially the willingness to pay for new medicines in those settings. It was introduced in 2022, replacing what was previously called an end-of-life modifier, which was predominantly used for metastatic cancer. The intent of the severity modifier was good because it was intended to enable more conditions beyond cancer to benefit from a modifier. Unfortunately—NICE has acknowledged this itself in recent board papers—in reality the utilisation of the modifiers has been a little bit lower than expected. That is really why we believe that the Enhertu case resulted in a negative outcome, and why we believe that the modifier system needs to be evolved so we do not end up in the same situation with future treatments.

Q3 **Chair:** Okay. But what do you think it then says about the status of the appraisal process?

David Brocklehurst: Like I said, I think the intentions were good. In reality, because of that under-utilisation, there has been a de facto degradation of willingness to pay for new cancer treatments without the broader benefit that was envisioned. There needs to be an adjustment to the qualification criteria for these modifiers.

Q4 **Chair:** Okay. Mr Maheson, what do you think, in answer to my initial question?

Haran Maheson: I would agree with Mr Brocklehurst's comments. It is important to state as well that, up until 2022, for over a dozen years NICE had a special criteria and funding mechanism to recognise the severity of disease. In particular, with metastatic breast cancer, it was the fact that patients have a relatively short life expectancy. Essentially, metastatic breast cancer was considered to be a disease of "high severity". If we fast-forward to 2022, NICE evolved its methodology with good intent, as David said. However, one of the impacts of those changes in methodologies has been that diseases are essentially segmented according to severity using this algorithm. Metastatic breast cancer—a disease that is essentially terminal and has a very short life expectancy—has been downgraded from being a disease of "high severity" to a disease of "medium severity". I think that anyone who is a patient, carer or has signed the petition that Breast Cancer Now has in place—with 240,000 signatures—would argue, from looking after a patient with metastatic disease or suffering from it, that it is certainly not a disease of "medium severity". It is a disease of "high severity", as NICE recognised up until 2022, and as Scotland still recognises to this day.

Q5 **Chair:** What do you think the likelihood is of a change? This is barely two years old, so logic would suggest that these are early days and there are many more examples and case law—for want of a better expression—to flow under the bridge. What do you think the chances are of change?

Haran Maheson: Mr Chairman, I think it is fair to say that NICE has recognised that there is a challenge with the implementation of the severity modifier. Indeed, at committee meetings, it has mentioned that there is a challenge with its utilisation, and that can be borne out in the number of appraisals that have taken place since the severity modifier was introduced around January 2022. Of the 39 or so appraisals that have taken place, only 2 have reached the highest level of severity. Everything else has essentially been downgraded to either "medium severity" or "not severe", in terms of disease state. It is important to recognise that the severity modifier first of all recognises the disease state rather than the product. The product is then essentially dovetailed into that disease state.

Q6 **Chair:** Yes, that makes sense. It is important to say, for those watching outside, that NICE will be talking to the Committee this morning as well. We are not talking about them without them. No decision about me without me, as they say. Mr Brocklehurst, what do you think the chances of a change are?

David Brocklehurst: Haran rightly mentioned that NICE has recognised the underutilisation. It has committed to a review of the severity modifiers within the 2024-25 year and we welcome that. In that sense, we will have to wait and see the outcome. There is really some urgency around the higher severity modifier in the short term. The reality now for the case with Enhertu is that we have four months from Wednesday next week to have an evolution in the system that would allow us to utilise what is called the rapid review process, which really is the only way we could see Enhertu being made available for this patient population.

Q7 **Chair:** Correct me if I am wrong, but why have you decided not to launch an appeal against the decision?

David Brocklehurst: The appeal process with NICE really is just to appeal whether the methods have been applied appropriately.

Q8 **Chair:** Which they have.

David Brocklehurst: Which they have—absolutely. That is why we chose not to appeal. We recognise and commend Breast Cancer Now and the UK Breast Cancer Group for appealing, but we did not feel that was likely. Also, really, for the medicine to be available for the patients who need it, it is going to require a change in the methods.

Q9 **Chair:** Which is why you're here, because you are asking us as the scrutiny Committee of the health and social care sector to scrutinise not whether the system has worked correctly, because it clearly has, but whether it is fit for purpose in this instance.

David Brocklehurst: Absolutely.

Q10 **Chair:** Finally from me, before I bring in Rachael Maskell. As I said at the start, we are writing our report on our Future Cancer inquiry and this is clearly very much Future Cancer. If you were writing recommendations for that report, what would you recommend to us?

David Brocklehurst: There are two things. Thank you for the question. You rightly said that it is quite a technical, complex issue. The first answer and the first thing I would recommend is quite a technical answer, so please forgive that. Ultimately, we would recommend an evolution to the qualification criteria for the severity modifier. Quite simply, based on our analysis, we believe that if you reduce what is called the proportional shortfall threshold for the higher modifier from 95% to 90%, that would enable all medicines that previously would have qualified for the end-of-life criteria to also benefit from the 1.7 modifier. That would essentially remove the unintended inequity that has been created through the methods change and assure that there is no retrograde step for cancer patients. That is the first thing that can happen, maybe quickly, while the second thing is ongoing. That is the fuller review of the methods, which NICE has already committed to.

Q11 **Chair:** People listening will think, "Okay, 2024-25 review," but bluntly, there will be people listening who do not have 2024-25. Therefore, for a longer-term solution, maybe, but a short-term solution would be qualifications to the security modifier threshold. Does that make sense?

Haran Maheson: I would agree with David's comments. Essentially, it is increasing the bandwidth for qualification for the different grades of severity—in this case, high severity.

Chair: Okay, that makes sense. A good start. Rachael Maskell, over to you.

Q12 **Rachael Maskell:** Thank you, Chair, and thank you, both, for coming in today. I will start by asking about the efficacy. Clearly, NICE recognised the efficacy of the drug we are talking about in this instance, but also its mechanisms seem robust in that area; in other words, the analysis around cost, essentially, and how that comes together. In the light of the modifier brought in, should metastasised disease automatically be included in high severity? Should that just be a criteria that is recognised to then move those drugs into that category in order to reach the thresholds? Is that the solution?

Haran Maheson: Up until 2022, that was the case. Metastatic diseases, such as lung cancer and breast cancer, were classified as diseases of high severity, so, de facto, all treatments or most treatments for those conditions would be within that banding. It's just that it was previously called end-of-life criteria.

David Brocklehurst: It is certainly something that would be valid methodologically. When you look at this specific patient population, we are talking about a subset of metastatic breast cancer, typically affecting women younger than 60 years of age, who on average have 18 months of life remaining. The algorithm is declaring that as medium severity, and we believe that the algorithm must be broken to reach that conclusion.

To show what the scale of the issue could be beyond this particular case, when we look into our own pipeline, the AstraZeneca pipeline, we have planned, in the next three years, 13 NICE submissions that would have

qualified for the end-of-life criteria—the previous methodology, which Haran mentioned. Based on our analysis, right now none of those 13 will qualify for the 1.7 severity modifier. So it is clear something needs to be done. Whether that is that all metastatic cancers are deemed severe—I think that would be valid, but there are other changes to the qualification criteria that I have mentioned and which could be a short-term solution as well.

Q13 **Rachael Maskell:** Thank you, Mr Brocklehurst. That takes me on to my next question, about the implications this has for research and development. Clearly, licensing drugs and NICE approving drugs provide an opportunity to build greater clinical evidence as to the effectiveness of drugs, but also can push the science to ensure that a future generation of drugs can be even more effective, prolong life longer or, indeed, provide a curative effect, which is obviously what we long and hope to see. What are the implications for science in this new mechanism for drug approval?

David Brocklehurst: That is a really important question; thank you for raising it. AstraZeneca is the largest private R&D investor in the UK across any sector—pharmaceuticals and beyond. We invest £2.5 billion per annum in R&D in the UK. We have more than 70 clinical trials ongoing within the UK, which recognises the amazing science that we have within this country and the environment we have for doing world-leading R&D. That's great. In order to do that and continue doing it, standards of care need to represent the latest standards of care, and what we are concerned about is that without a methods change, standards of care in the UK could fall behind those in other countries.

If we take the case in hand, there are 16 other European countries where this medicine is already available for HER2-low metastatic breast cancer. One of them is just a few hours north of here—Scotland—as you know. We need to make sure that UK patients are able to benefit from the latest treatments, many of which would be researched within the United Kingdom, in order for standards of care to keep pace and for us to be able to continue to do science and world-leading R&D in this country.

Chair: Is that all? All right—fine. Paul Blomfield.

Q14 **Paul Blomfield:** I just want to be absolutely clear, not least for the subsequent part of the session with NICE, about the problem. Is the problem the movement from the end-of-life modifier to the severity modifier? Is it what was described, I think, by the ABPI as the severity modifier being applied on a more conservative basis? Indeed, the NICE board noted that there was "lower than expected use of the severity modifier" and that indicated the problem. Or is it, as you seem to have very clearly said to us, simply that metastatic disease is wrongly classified as medium rather than high? I just want to be absolutely clear on the nub of the problem.

Haran Maheson: The two problems essentially have the same core, which is that the thresholds for severity are being applied very, very conservatively. As a result, most disease areas—as evidenced by the fact

that only two out of 39 applications in the last two years have achieved high severity—are not even achieving that threshold of high severity. That includes metastatic diseases.

David Brocklehurst: My only additional point—because I fully agree with that—is that there is an interim modifier as well, of medium severity. That is also being under-utilised versus what was originally envisioned by NICE. So it is not just a metastatic cancer problem; it is all diseases. The medium severity modifier is essentially being utilised around half the amount that it was expected to have been by NICE.

Q15 **Paul Blomfield:** That is helpful. Given that you have identified the problem so clearly, what discussions have you had with NICE on that issue, because clearly it is leaving England as an outlier in Europe? What discussions have you had, and what response has there been?

David Brocklehurst: The first thing is, obviously, that we submitted Enhertu to NICE for assessment and we worked through, in partnership with NICE, the methods to bring the case to resolution. We wanted to be a constructive partner in that regard. By the way, we see an emerging trend where the number of appraisals that are being withdrawn or terminated and do not reach a conclusion has doubled in the last three years, to 20%. So we wanted to make sure that we were constructively submitting to give every chance of this reaching a positive conclusion.

Medicines in the UK are made available typically with a confidential discount versus what is publicly available, in terms of price. We already have a commercial agreement in place for other uses of this medicine, so there is a confidential discount in place. There is also a confidential discount in place in Scotland, which was deemed to be cost-effective in this patient population. We have offered a further discount to that in this setting, which was deemed not cost-effective under current methods, because of the application of the medium severity modifier for these patients. We feel we have really been constructive in the process and gone as far as we can, actually.

Haran Maheson: When the severity modifier was originally consulted on by NICE prior to its starting in 2022, all of the major cancer charities objected, or at least challenged, the idea of a severity modifier and certainly the application of it, in terms of how rigidly or how conservatively those thresholds could be applied. That is on the record, in terms of Cancer Research UK and many other charities and patient groups.

On top of that, as David said, from the get-go we have tried to work in partnership with NICE, both to share our concerns about the potential application of the severity modifier in this particular indication, but also Enhertu—the product that we are talking about—has had two successful NICE appraisals over the last three years. It is important to note that we are not anti-NICE; we're just anti the application of the methods as they currently stand, because they have disenfranchised a whole swathe of deserving patients.

Q16 **Paul Blomfield:** On the issue of pricing, which you have highlighted—and I appreciate that you will not conduct negotiations publicly—campaigners have said that NICE, NHS England and the pharmaceutical companies need to get together to sort this. That suggests that there is a responsibility on all parts for movement. How does that play in terms of the space for movement for your companies?

Haran Maheson: We are willing to engage with NICE as an alliance—AstraZeneca and Daiichi Sankyo. What we need, though, is a commitment to relook at the thresholds for the different bandwidths of a severity modifier, and also to enter into a rapid review, which, according to NICE's methodology, starts from next Wednesday and lasts for four months. So time is really short to create some sense of short-term change for this particular indication. However, the question is also broader than that. It is around NICE methodology and recognising all deserving diseases for patients within those disease segments that could be in a highly severe state.

David Brocklehurst: I fully agree with that. The only thing I would add is that the price that we offered would have been cost-effective under the old methods. The end-of-life methods would have been cost-effective had a 1.7 severity modifier been applied. It is absolutely correct to say that if the methods were to evolve, so that they broaden the criteria so severe diseases such as metastatic breast cancer are recognised as such by the severity modifiers, I think there is a pretty clear conversation to be had by all parties.

Paul Blomfield: Thank you very much.

Chair: Rachael, do you want to come in?

Q17 **Rachael Maskell:** Yes, I just want to come in for a quick question on your concern about this. Are you more concerned about the pipeline of medication that is awaiting appraisal rather than about just Enhertu itself, in the light of the implications for other things in the pipeline? And, therefore, are you using this as leverage to change the mechanism on this occasion by not renegotiating on your own costs, because you know that the implications for the pipeline could be so significant and you therefore need this mechanism to change, rather than just this one pharmaceutical product to be approved?

Haran Maheson: There are 2,000 oncology products in the pipeline across pharma in general that are due to come to market—due to get a licence—in the next three to five years. That is a huge number of products. In many ways, trastuzumab deruxtecan in HER2-low is just the tip of the iceberg. It is the canary in the coalmine, in terms of what could happen with non-appraisals or failed appraisals. This goes way beyond one company's individual pipeline or one particular product and one particular indication. It is an industry-wide issue.

Q18 **Rachael Maskell:** If I can press further, knowing that 2,000 products will be coming into this space—

Haran Maheson: In oncology.

Q19 **Rachael Maskell:** In oncology. Is that of greater significance than Enhertu itself?

Haran Maheson: Absolutely.

Rachael Maskell: Okay. That is interesting.

Chair: That is a good clarification.

Q20 **James Morris:** Perhaps I can build on what Rachael Maskell asked. You talked about 2,000 oncology products, and with Mr Brocklehurst, we talked about 13 drugs in the pipeline. If the NICE criteria that we have been discussing were not to change and were to stay as they are today, what would be the implications for your business?

David Brocklehurst: Thank you for the question. Like I said, it is clear that there are 13 NICE submissions that would have got the end-of-life criteria, and there are many more planned. As Haran said, we have concerns that we will be unable to secure the usage of these medicines for the patients who need them.

Ultimately, we are pushing the boundaries of science to develop new medicines. We are bringing forward those medicines because they are going to extend life and maybe one day help to eliminate cancer as a cause of death. That is why we exist as a company, and that is what enables us to continue the reinvestment in R&D. Yes, this is an issue that needs to be solved for the 1,000 HER2-low breast cancer patients who are not getting the standard treatment that 16 other countries are getting, and also for the many other new medicines that are going to come through from AstraZeneca, the Daiichi Sankyo alliance, or other sources or academia.

Q21 **James Morris:** This is a global market, so is there anything we can learn? You are saying that other jurisdictions have allowed this particular drug and approved it. What can we learn from those jurisdictions?

Haran Maheson: Probably the nearest analogies are markets that have what we call cost-effectiveness thresholds—like NICE in the UK. In Scotland, for example, the SMC has a similar system. Other countries such as the Netherlands, Norway and Canada will have broadly similar systems, but all of them have a broader definition of severity. That is the key thing. Having a more liberal, broader definition of severity means that the value of the treatment and the value of survival within that treatment is properly recognised.

James Morris: Thank you.

Q22 **Chair:** You say that this is why you exist as a company, David. You exist as a company, of course, to make profit for your shareholders. That is why you exist as a company, right? You do good things as a result of being a successful company. There was quite a big increase in the salary and bonus package of your chief executive recently, which hit a lot of headlines. A lot of people struggling to make ends meet while battling cancer right now might think about your chief executive being on a

maximum remuneration of £18.7 million. Do you see how that jars a bit? Is that unfair?

David Brocklehurst: Thank you for the question. I feel, actually, that that particular topic doesn't have any determination on whether a disease is classified as medium severity or high severity. I am proud that I work for a company that is successful and has the ability—because of the profit it makes—for the shareholders to give us, essentially, the permission to invest £10 billion a year in research and development. Like I said, a quarter of that is in the UK. Our success is what enables us to continue to reinvest, and that is the fundamental premise—

Q23 **Chair:** I appreciate that it doesn't impact the severity modifier, but my point is that you could argue that that success allows you to be more generous in the way that you make this drug available to what is a relatively small number of patients, in the case of Enhertu, through the NHS. Discuss.

David Brocklehurst: I understand the question and I thank you for it. I would just, essentially, reconfirm that we feel we have already gone a long way and been quite generous in what we have already put on the table.

Chair: That is what I was trying to get at.

David Brocklehurst: It is lower than what is already deemed cost-effective in Scotland. It is lower than what the NHS is currently paying for other uses of this medicine.

Q24 **Chair:** Finally—you touched on this a bit with Rachael Maskell, in thinking about our work and our report—there is the wider possible impact of this decision, or these decisions, on pharmaceutical companies looking to launch in England. What would you say on that score to Ministers?

Haran Maheson: One of the statistics that David mentioned at the beginning was that around 20% of medicines that have been launched in other markets have not been launched in the UK. Quite often, that is because of the perception of a prohibitive funding environment, so I could see that figure at least staying stable, if not growing, because companies have to make decisions around investment and launching. So it is a really pressing issue that we want to get right.

David Brocklehurst: I fully agree. We have been able to invest heavily in R&D in this country specifically because of the quality of the science that exists here, and because it is obviously a home country for AstraZeneca. But as you recognised, it is a really competitive global market. We would like to see the evolution to the method so that the products of the R&D will become available to UK patients that deserve them.

Chair: Okay. Thank you very much David Brocklehurst from AZ and Haran Maheson from Daiichi Sankyo UK.

Examination of witness

Witness: Baroness Delyth Morgan.

Q25 **Chair:** Now we have Baroness Delyth Morgan, who is the outgoing chief executive of Breast Cancer Now. It is very nice to see you again, Delyth. You were sitting in the audience listening to the discussion we have just had. Before we get to that, you responded a couple of days ago—as did I—to the loss of our good friend Kris Hallenga, who set up CoppaFeel! and lost her battle with breast cancer at 38 years of age on Monday, which is just heartbreaking. I just wanted to give you a chance to talk about your memory of Kris.

Baroness Morgan: I wouldn't know where to start, really. She was so fabulous, inspiring and energetic—someone who was so full of life—and I think it's really hard to believe that she is no longer with us. She was absolutely incredible in the way that she took her diagnosis of incurable secondary breast cancer at such a young age and started the charity, CoppaFeel!, which is all about raising awareness and the need to check your chest—that was her message. So if we would all, in tribute to her, just remember to continue to be breast aware and think about her family and her friends, who will be missing her very sorely at the moment. It has been a privilege for Breast Cancer Now to work with CoppaFeel! on so many campaigns, and we will continue to do so.

Q26 **Chair:** Well said. I would just to add to that that shortly after I was elected, when I was chairing the all-party group on breast cancer—when we first met—I met Kris and asked her if she would come to Winchester and speak to some children in secondary school about the "Check your boobs" campaign. She said "Yes, but only if we can do all three secondary schools in one morning," which was exhausting for anybody, and we did. We went to Kings', Westgate and Henry Beaufort School in Winchester city centre. She spoke to school assemblies one after the other and you could have heard a pin drop—it was extraordinary, the way she held the room. So RIP Kris, and best wishes to Maren, her twin; the family are finding it very difficult today.

That is sort of why we are here, right? We are talking about Enhertu. Why is it such a promising treatment for women with HER2-low secondary breast cancer? Explain to people specifically how lethal HER2 metastatic breast cancer is.

Baroness Morgan: It is true. We have already heard how, in theory, this affects a small number of women—about 1,000—but we need to remember that they are women living with incurable secondary breast cancer, who really do not have long to live. Enhertu can offer an additional six and a half months of life—for some it could be longer—and that makes a huge difference. That is the opportunity to stay around for important life events, maybe see your children make it to nursery school or secondary school—these life events are really important, creating memories for loved ones and giving some hope for the future.

At Breast Cancer Now, we are hearing from women with HER2-low breast cancer who are absolutely devastated about this news, this result. They were holding out for that next line of treatment, and the delay for some women will mean that they lose their life. It is so hard for people to hear

that and to hear today about the severity modifier. As a charity keen to influence policy in a responsible way, we are very supportive of the work that NICE does. It is a vital part of our health system, but the change to the severity modifier from the end-of-life criteria—since 2017, we have seen 20 new breast cancer drugs come on line. They are really changing the future for people with incurable breast cancer. For those patients to be left out of that is really tough—it is heartbreaking.

Q27 **Chair:** Is it accurate to say that this is the first breast cancer drug that has not made it?

Baroness Morgan: For many years. There was a dry period—if I can call it that—when not many new treatments were coming through, but since 2017, as I said, we have had real progress. Kris, for example, lived for 15 years with secondary breast cancer—with really gruelling treatment, but she did survive. If we are going to realise our dream, which is that everyone diagnosed with breast cancer can live—there is reason to hope for that in the future, but we will not achieve it if things like this keep holding us back. This is a really important treatment. It is there for a small number of people, but for those people it is absolutely everything, and to think that it might be classified as a medium-severe disease feels wrong.

Q28 **Chair:** I guess it is bad enough when we have conditions that, as a human race, we cannot touch, because we are just not there yet on the science, but to have it on the shelf but unable to reach it is heartbreaking.

May I ask you about inequalities within the UK, which make not being able to reach the shelf even more frustrating? Some parts of the UK have a ladder that does reach the shelf. Scotland, of course, made a different decision. Wales and Northern Ireland will traditionally follow the recommendations of NICE, but I think a divergence is going on. Will you tell us about that?

Baroness Morgan: It is available in Scotland, so obviously it is hard for many people to understand that where you live in the UK will affect access to this life-extending treatment. Until this, the decisions around the UK have been quite similar. Wales and Northern Ireland will follow NICE, so although at the moment some patients in Northern Ireland will have access to Enhertu through a limited access scheme, that will not persist once the final decision from NICE is read across the UK. Yes, we have a situation now where your postcode or location will affect access, which is very tough.

Chair: We will come back on some of this. As I said when I introduced you, you are the outgoing chief executive of BCN, and we will come back with some reflections just before we finish with you. It will probably be the last time that you will be before us, you will be relieved to know. In the meantime, we will continue on this subject. Rachael Maskell is going to ask you a question.

Q29 **Rachael Maskell:** Obviously, we have just heard that this is the tip of the mound on top of the iceberg with regard to the supply of oncology drugs

that are awaiting approval, but this is a specific drug that impacts women. Are we seeing an inequality that impacts women's health in particular being built into the system? I appreciate that men can also get breast cancer, but it is obviously predominantly women.

Baroness Morgan: The history of how the science of breast cancer has unfolded has been that, because breast cancer is so common, it has been possible to see quite a lot of research being done in the breast cancer space over the years, so there have been lots of exciting developments. What I am worried about is that we will get to a point when people think, "Breast cancer is done—we've done that," yet there is all this potential still to come down the track—that we could be world leading but we are putting the anchors on.

That is what I am worried about, and I think there is a long way to go. We are still seeing 11,500 women dying of breast cancer a year, and about 100 men, too. That number has not changed massively. The number of women diagnosed with breast cancer has gone up, so survival rates are improving, but we still have that hard number that we have to grapple with. There is an inequality unfolding, and there is an opportunity to stop it in its tracks with this review of the severity modifier process that NICE is looking at.

Q30 **Rachael Maskell:** Certainly, from our Future Cancer inquiry, we know that the world looks to the UK when developing its science. It clearly will have a dumbing-down impact on people wanting to invest in that science if they know that they cannot get the necessary approvals. Is it just the severity modifier that needs to be reviewed, or are there other elements that could impact the decisions that NICE makes? Only 46% of approvals are coming through, and we are clearly seeing much higher rates in other countries. Are there other factors within that, or is it just the modifier?

Baroness Morgan: Thinking about it from the patient perspective, I would say that the whole process needs to be speeded up. We have seen an enormous gap between licensing and drugs becoming accessible, and then you have the implementation through the NHS, which always takes time. From a patient point of view, the whole thing needs to be speeded up, and that has got be good for industry as well. I note that NICE is part of that, and there are capacity issues as well. The whole thing can take months and months, and if you are living with an incurable disease, you do not have that time. It has to be good for UK plc to have that whole system really well oiled and meeting the needs of patients as quickly as possible.

Q31 **Rachael Maskell:** You mentioned pace, which is certainly something we have picked up within the processes. Is there anything within the mechanisms that you would want to see change as well, for those approvals?

Baroness Morgan: We need to look again at the severity modifier, which everyone has talked about. We were concerned about it because of the effectiveness we had seen of the end-of-life criteria for people with metastatic cancer. We had seen those breast cancer drugs coming through

the NICE process, getting into clinics and really changing lives. We have all talked for years about the benefits of personalised medicine and targeted treatment. Here they are coming along, so we need to get them into clinics as quickly as possible for the benefit of patients. I am not sure that has answered your question, actually.

Q32 **Rachael Maskell:** It is helpful. I want to push a little bit further. The valuation of the QALY from the Treasury Green Book is at £70,000, yet NICE is still working to around £20,000 to £30,000. Do you have any comments on why that is disproportionately downgraded in the pharmaceutical space?

Baroness Morgan: I find it hard to understand why that feels so out of date.

Q33 **Rachael Maskell:** It was 1999.

Baroness Morgan: Yes, that feels like quite a long time ago. I have seen, over the years, the benefits of having a vibrant science environment; we do some of the best breast cancer research in the world in this country. I believe in the benefits of helping to get the system to work really efficiently for patients with the NHS, speeding it up and making new treatments more accessible. You will have thought about clinical trials; we need to improve access to clinical trials for patients across the UK. We did used to be better at that, so I worry about that too.

Chair: Paul Blomfield, did you want to ask a quickie?

Q34 **Paul Blomfield:** I have a brief follow-up. I would think anybody watching this session would find it difficult to understand why metastatic disease is not seen as high severity, and would also think that it seems like a reasonably easy fix to get that reclassification. You described yourself as an admirer of NICE. From your perspective, why do you think they have got this so wrong? Have you had any opportunity to engage with them in providing patient voice on the issue?

Baroness Morgan: We will, and do, talk to anyone and everyone about this. We have been campaigning on access to new medicines for a long time, so we were very excited about the prospect of Enhertu becoming available. We have spoken to NICE and played our part, supporting patient advocates to be involved in the process. We will continue to do that to make sure the patient voice is heard.

Maybe you can ask NICE this, but they have a process that they have to stick to. That is their approach; they have got to do that, haven't they? I suppose we are hoping that the review will come along quickly and that there will be the opportunity to say, "This isn't right. We will think again and do that really quickly." We hope that the scope of that review will be wide enough so that there is the opportunity to put this right really quickly. We have to believe that they are doing the right thing for the NHS and for our health system, which is under a lot of pressure. We have to hope that that will happen really quickly and that they will do the right thing.

Q35 **James Morris:** I have one brief question. Coming back to the issue about clinical trials, which you just alluded to, are there any particular challenges around clinical trials for breast cancer drugs?

Baroness Morgan: There are challenges for the system generally. Breast cancer services are particularly under pressure in terms of time, with new treatments coming through, and staffing requirements and so on. That puts pressure on the system. There are also questions about how you can set up clinical trials. I am not an expert, but the standard of care in this country needs to keep pace with the rest of the world's leading countries in order for here to be an attractive place. You also need access to enough people with enough different sub-types so that you can get a critical mass for these kinds of studies. Patients really want to be a part of this and are really ready to get involved.

Q36 **James Morris:** Are there particular barriers in this country compared to others around the world?

Baroness Morgan: I think infrastructure is a challenge, as is joining up data and research nurses. So I would say all of those things, but none of that is insurmountable, because for me one of the most exciting and valuable things that our leading clinicians have to bring is that research leadership. We have the most amazing clinical leaders in this country, and to be able to support them to crack some of the challenging questions in breast cancer is really important.

Q37 **Chair:** We heard in the last panel how we have two excellent, world-leading companies that have come up with an amazing product. What is the money shot for you in terms of when NICE next takes the seat you are sitting in now? Nobody is questioning anyone's motives here, because everybody wants to do the best thing for people with breast cancer, but what is the key question you would ask of them?

Baroness Morgan: I think I would ask, "What is the quickest and fairest mechanism for fixing this, which is so obviously a mistake, and how can we do that with integrity?" That is the important thing. As you say, no one is questioning anyone's motives, but we need to have checks and balances in our system; otherwise, there lies ruin. We have to find a practical and processed way of dealing with this.

Q38 **Chair:** That is helpful. Just by way of reflection, when we were in Singapore on this inquiry, somebody said, "Our mission with cancer is to prevent it. When we cannot prevent it, we want to find it early, and when we find it, we want to treat it with precision." I just loved that—what a mantra. If only we had a cancer strategy in this country that we could put that at the top of.

I have a funny feeling that we won't see the last of you, because we are all signed up to life on this one, aren't we? Looking back at your time in this space, what are you optimistic about in the breast cancer space?

Baroness Morgan: I am optimistic about making a reality the stopping of women dying of breast cancer, and doing that in a really tangible way. Going back to the '90s, I have seen the improvements there have been

since the new innovations around hormone treatments and early diagnosis and new surgeries, and the introduction of targeted treatments. Because of all that, we have seen real progress in survival rates for breast cancer.

The really interesting challenge is that we now have a situation where, as we have heard, people cannot understand why it is that metastatic disease is not recognised as being serious. What has happened as a product of all this success is that there are now more women living with secondary breast cancer who feel that they are invisible and do not have the same access to, say, clinical nurse specialists, and they worry that they will not get access to the future treatments they should have and that they will not be taken seriously or counted, because the data that is being collected around metastatic breast cancer is really incomplete.

At a service level, breast units do not know how many metastatic breast cancer patients they have to look after, so how can they plan their service? That sounds like a terrible picture, but as someone who has their glass half full, I see it as a huge opportunity to make real change, to improve the quality of life for all people living with incurable breast cancer and to give them the support they need and the certainty that they will be well looked after. We will continue to take steps one by one—such as Enhertu and access to new modern treatments coming through quickly—and then we will achieve the day when everyone diagnosed with breast cancer will live. That will come. I feel very optimistic, but there is a lot of work to do.

Chair: That is a lovely note to finish on. Thank you for all you have done, all the people you have supported and the lives you have saved.

Baroness Morgan: Thank you very much for having us today.

Chair: Not at all; thank you for coming. Please exit stage right, and the final two guests will enter stage left.

Examination of witnesses

Witnesses: Dr Sam Roberts and Helen Knight.

Q39 **Chair:** As if by magic, the shopkeeper appeared—otherwise known as Dr Sam Roberts, the chief executive of NICE, which, for those watching, stands for the National Institute for Health and Care Excellence, and Helen Knight, the director of medicines evaluation at NICE. It is great to see you both. Thank you for giving up your time to sit and listen to the first two panels.

We will start with Delyth's question: what is the quickest and fastest mechanism to fix the Enhertu issue with integrity? But I will preamble that by asking: does it need fixing?

Dr Roberts: We both believe that it needs fixing. The quickest fix is to reduce the price of the medicine. That does not take months or weeks. It could be a matter of days for NICE to approve the medicine. We agree that it is very effective, but it is not being offered at good value for the taxpayer in general. That is the quickest fix.

Why do we say that, rather than a fundamental root-and-branch review of our methods? Let's zoom out and then zoom in. We, like everybody in this room, understand the burden that cancer puts on society, individuals and families, so we take very seriously our responsibility to provide access to these medicines. Over the last 10 years we have said yes to 88% of the cancer medicines we have looked at. If we look at whether the rate of time it takes to say yes is getting worse since we implemented severity, the answer is no. Of the breast cancer medicines that we have looked at, we have said yes to 21 since 2018, and Enhertu is the first that we have said no to. In fact, we said yes to a similar medicine, talazoparib. It was for a similar disease: HER2-negative metastatic breast cancer. We said yes because the value offered by the company was right.

We think this is an effective medicine. We absolutely think that patients should get it. The way to get it is to reduce the price at which it is being offered.

Q40 **Chair:** The first panel told us that in the short term the qualification for entry to the severity modifier needs to change, and in the longer term there needs to be a review. You are saying that the qualification for entry to that is right, but the price is wrong.

Dr Roberts: Yes, and—let me explain some more. There is no mechanism available for us, just on a case-by-case basis, to say, "For this medicine, we changed the rules."

Q41 **Chair:** No, not case by case, but is there a moment in time—a line in the sand—where you would change the qualification criteria to the severity modifier? Is this that moment? That is the nub of this, isn't it?

Dr Roberts: That could be the nub of it. To answer that question, we have been monitoring the use of the severity modifier, and I would like to correct some of the numbers presented earlier.

Chair: Please do.

Dr Roberts: I think the claim was that only two cancer medicines had received the highest severity. That is not true. There have been five, one today. The claim was that we never say that metastatic cancer is severe. Again, that is not true. We have, in a number of cases, given that the highest weighting.

What we are currently doing is monitoring every single medicine that goes through. Did we say yes? At what level? If so, why not? And so on. In that monitoring, we are asking two questions; I think you made this point when we started. These are small numbers of medicine, so it is hard to draw conclusions, but the first conclusion we can draw is: are committees not understanding the process, and prosecuting it wrong or calculating it wrong? We have had a detailed look at that and that seems not to be the case. Committees are following the rules to the letter. The next question is the one that you asked.

Q42 **Chair:** I said that, didn't I?

Dr Roberts: That is exactly what you said, yes. The next question we asked is: are there big enough numbers here for us to draw conclusions on whether the rule is right? That is the process that we are busy monitoring at the moment because, as you say, the numbers are small with this kind of thing, so you get lots of variability.

Helen Knight: Obviously, the introduction of the severity modifier came after a lengthy methods review that NICE undertook, and we published that in 2022. I just wanted to remind everybody of the reason why we changed to a severity modifier. During the methods review, we found evidence to say that people valued the health benefits in more severe diseases more highly than in non-severe diseases, whereas our previous approach was just focused on what was an end-of-life criteria, which was very much limited to cancer and was only about extending life at the end of life. There was support. The evidence was there for the severity modifier.

Obviously, we very carefully looked at how we would be able to implement that. One of the benefits that we saw from moving to a severity modifier was that we could attribute a higher weighting for benefits outside of cancer; both non-cancer medicines and cancer medicines that did not meet the end-of-life criteria would then start to have a severity modifier attributed to them. We gave a lot of careful consideration to how we could apply that.

We came up with the two steps: the 1.2 and the 1.7. We are hearing that this treatment would have got through with end of life, but previously we did not have an automatic 1.7 modifier for end of life, so we did actually see over the years that the maximum weight of 1.7 was not always applied for end of life.

I think what I am trying to say is that it is not going to be as simple as just changing those modifiers. The fact that we have an exceptionality for severity is right—that is what the evidence said. What we do not know is how much more weight you should give in those circumstances; that is where we have to be quite careful, because these are exceptional.

What is happening in the NHS is that every time we say yes and apply a severity modifier, we are displacing more health elsewhere in the system than we get for the treatment that we are looking at. That is the really difficult and challenging decision that sits with NICE: how do we allocate a fair approach to considering value for an NHS that has a limited budget?

Q43 **Chair:** Yes, because resources are not infinite. The customer here ultimately is NHS England, which we did invite to come and be part of this discussion today on the stand.

I don't want to go all Bob Geldof here and bang the desk, but in the words of Mr Geldof, people are dying now. We have to try to get to a better place on this now, and that means meeting in the middle. That is what compromise ultimately is between you, the companies and the customer. We have to get to a better place on this. Delyth is retiring—

help her out here.

Dr Roberts: Indeed, and I think it is fair to say—I am not breaking any confidences on the side of either the company or Breast Cancer Now—that we have absolutely tried to get around the table and find that middle ground, but the value proposition offered is still just too high. If there is movement on it, we absolutely stand ready to take it back into our process in a matter of weeks. We don't need to wait months.

Q44 **Chair:** Okay, so there is no door closed and no process—

Dr Roberts: Absolutely not.

Chair: The company is quite right in the note it sent to us that there is no point in appealing it because the process has been followed correctly. Whether our casework is about local housing cases or national policy issues, as MPs we are interested in whether the process—the law—has been applied correctly. It has been here, and therefore we are looking at the law and the process. That is what we are doing now.

I am going to bring in Rachael, and maybe we can return to the QALY point that you were just discussing with Baroness Morgan.

Q45 **Rachael Maskell:** I will do, but first I want to put the proposition to you that this should not be a case of either/or for people at the end of their life—this is their lifeline. Surely we should look at the criteria that make accommodation for metastasised cancers. This crucial pharmaceutical should be accommodated within the modelling to ensure it is taken on board, more than just as the severity modifier that it is currently. Do you agree?

Dr Roberts: Severity values the length and quality of life—not just length, but length and quality. As Helen was saying, that sometimes allows us to apply it to cancers earlier in the pathway, so you get more health and less length, and to non-cancer medicines. We do not currently have evidence to suggest that it is not fulfilling that broader aim. That does not mean that this specific case was not a really regrettable decision that we were surprised we came to.

As I say, with equivalent medicines, we have got to a yes without having the maximum severity for metastatic cancer. I don't think it necessarily follows that because this decision was a no, the entire rulebook is incorrect, given that it has worked in other cases. I suppose that is our claim.

Q46 **Rachael Maskell:** Can I explore that a bit further? This drug is approved in Scotland and 15 other countries. Why do we have criteria that mark it as not reaching the appropriate severity? That disproportionately affects women in England.

Dr Roberts: Scotland has the Scottish Medicines Consortium, which looks at medicines, and it had a set of rules that it could apply to this that were not at our disposal—they are basically not in our rulebook. We look very carefully at variability between England and Scotland. If you look at the

last set of data on that—the EFPIA WAIT data—England is consistently ahead of Scotland, both in time to availability and in number of cancer medicines available. In aggregate, we are performing better overall. Again, this is a really regrettable case and we hope we can resolve it.

Q47 **Rachael Maskell:** I want to come on to the pricing. As we have heard from the pharmaceutical companies, they are not just looking at Enhertu; they are looking at the pipeline—perhaps 2,000 different drug applications. Clearly, there will be a dumbing-down effect on science if people say, "We can't get our medicines to treatment." You obviously understand those concerns.

It seems that there is gridlock over this, and some leverage is being applied to ensure that pipeline is not blocked in the way Enhertu is perceived. What scope do you have for negotiation to ensure that confidence can be built within the pharmaceutical industry that they will be able to get their pharmaceuticals approved?

Dr Roberts: What we do is look at our performance. Is it getting worse or staying the same? Then we look at our performance relative to the rest of the world and how we are doing.

In terms of our performance, we have not seen an increase in the number of medicines that we say no to, and we have not seen an increase in the number of medicines that were withdrawn, as our industry colleagues reported. Those have bumped along within the same kind of parameters for 10 years or so. So that is thing one: we see our performance on how often we say yes.

Q48 **Rachael Maskell:** But something has changed in your criteria in 2022, so we need to look from that point rather than from 10 years.

Dr Roberts: Absolutely, and we monitor it year on year. If you think about the number of medicines we look at, it is about 100 a year, so two on either side can bump things up and down. We roughly see things in a 10% window.

On all medicines, we roughly say yes between 75% and 85% of the time and over 10 years, it is bumped in between those and continues to do so. We have had medicines pull out of the process roughly between 15% and 25% of the time, and again, that tends to bump between those. That is the first thing. We keep an eagle eye on our performance and ask whether it is degrading year on year.

The second thing we keep a really eagle eye on is our performance relative to the rest of the world. Are we getting worse? On cancer medicines, the UK remains second in OECD countries in terms of cancer medicine launches and we have incremented to third on the time to launch. That has actually improved. We were sixth and we have got quite a bit faster. But yes, we do look at that data.

Q49 **Rachael Maskell:** Although that is the point, clearly, that Breast Cancer Now is pressing you on.

Dr Roberts: Absolutely, and we can see that there is much more to do on the speed and agility of the process.

Q50 **Rachael Maskell:** I will just move on to a question about the QALY. The Treasury Green Book evaluation of the QALY has £70,000 compared with that set in 1999 for NICE. More money than ever is going into the NHS. What needs to be done to ensure that the value of the QALY keeps pace with the value of the economy and the market?

Dr Roberts: Maybe Helen and I will do a double act on this, because the threshold that we use—you said the £20,000 to £30,000—measures something different from what the Green Book measures. We are basically saying that there is a fixed amount of money in the NHS. Every pound we put in can only be spent once, so it is displacing some care that somebody is already getting.

Q51 **Rachael Maskell:** This is 25 years old.

Dr Roberts: Yes. Our threshold is based on what is the health being delivered and how much it costs of the care that is already in the NHS. There has been widespread debate on that for many years about what is the health delivered and how you value that. That is something that is not NICE's sole prerogative to opine on. Traditionally, it is part of a negotiation every five years called the voluntary pricing and access agreement, which has recently been concluded, where, as you say, that was a topic of debate. The final conclusion was to leave it at the existing threshold.

Q52 **Rachael Maskell:** So does the VPAS need to be revisited? Would you recommend that?

Helen Knight: Obviously, NICE is not part of that voluntary scheme. We are not a signatory but we are affected by what we see. Without knowing the exact value of health displaced, it is very difficult, and we feel that the £20,000 to £30,000 does represent and it is and has been working well.

Q53 **Rachael Maskell:** Was it overvaluated in 1999?

Helen Knight: It is difficult to say whether it should have increased with inflation; that is not what we are using it for. We are talking about health displaced, so current health being displaced. In 1999, it would have been health displaced in 1999. So it is a constant that we have taken over the years. As we said, health provisions have changed over those years. You are seeing improvements, so it does not necessarily mean that you should be increasing the cost per QALY threshold over time with inflation.

There has been evidence to show that the health opportunity cost is actually much lower than NICE's £20,000 to £30,000 that we currently have. There were academic estimates around £13,000 to £15,000 per QALY. We feel that the £20,000 to £30,000 is reflective and works well for the types of products that we evaluate. As we were saying, every decision that we take, if we were to increase that, we are potentially overall removing more health than we are gaining for these new medicines.

Q54 **Rachael Maskell:** But clearly not working for women wanting to see their

lives extended by a number of months.

Helen Knight: For this particular one. We have recommended another treatment for metastatic breast cancer that received a 1.2 modifier recently. It is only with Enhertu that we have seen this challenge.

Chair: Paul, do you want to ask a question?

Paul Blomfield: I do, though I am conscious we are pursuing the same themes but probably coming at them differently.

Chair: Repetition is not a crime in this place.

Q55 **Paul Blomfield:** I have discovered that. The Chair made an important point at the outset. Everybody recognises that you are trying to do the right thing, and that is a challenge. You were clearly distressed at the decision that you have made in relation to Enhertu. In doing the right thing, there seems to have been a broad welcome when you introduced the severity modifier. We have heard—if you would confirm—that under the previous end-of-life modifier, Enhertu would probably have been approved. Is that the case?

Dr Roberts: I think Helen touched on it; I'll start and then you can take over. If the committee concluded that it got the maximum end of life, that might well have been the case. What Helen was saying was that the committee often did not conclude that. So, we cannot know. In the most optimistic scenario is the scenario that industry was painting.

Helen Knight: Yes, I think that Sam's covered that.

Q56 **Paul Blomfield:** You said that under the new system, other metastatic cancers have got the highest ratings. What is the factor here? Is it, as you said in your opening remarks, price?

Dr Roberts: Severity ratings are a combination of the number of years, or indeed months, that one lives and the quality of one's life during those years or months. That is one part of the calculation. The second part is price. On the previous metastatic breast cancer medicine, which we recommended in February, it was again given a 1.2 severity. Not the maximum, but the price was in a window where it was still considered to be cost effective.

Q57 **Paul Blomfield:** In a general sense, you clearly have a really important job in ensuring value to the NHS. But, when looking at cost, how far is that affected by the resources available? I'm conscious that, as a proportion of GDP, we spend far less on health in this country than most comparable countries. That is 9% from the latest figures I saw, compared with 14% in the US. How far are the resources available to the NHS a factor in you reaching your decisions?

Dr Roberts: I would say—and come in again, Helen, if you wish—that on a day-to-day basis it is not. That is why we are so pedantic about having rules about how we make our assessments, which are published, we

consult on, and are based on research, so that we cannot be buffeted around by the media about no ambulances or this, that and the next thing.

I think that it is an important part of our independence that everybody knows, when they come to us, that their medicine and their condition will be valid in exactly the same way as every other person's—that we are going to consider whether the length and the quality of life is greater, and we are going to pay more for that, in a consistent way.

Q58 **Paul Blomfield:** On the impact, I think that Helen made the point that you have to recognise that, if we are paying more for any particular drug, it has a consequence on the other services that the NHS is able to deliver, so the kind of resource available to the NHS must be a factor at play, at some level, in your decisions.

Helen Knight: The plain answer to your question is that the available resource does not impact our decisions. When we are actually looking at how well a treatment works and its value for the NHS, we take account of the current standard of care for that patient population, how much additional benefit you get with the new treatment, and how much more expensive it is to deliver that treatment. That is what is factored in, so the available resources within the system are not factored into the committee's decision making on the value-add of that specific treatment. It is important to be aware, and we are obviously aware, that there may be health displace—you know, the moneys spent in one place cannot be spent twice—but we do not definitively know what services or populations would be impacted by that decision.

Dr Roberts: I will have one more go because I think we are edging towards an answer. What we are seeing is that, in the day-to-day running—in the individual decisions—we do not consider it because it is absolutely important that we have a consistent set of rules applied everywhere. However, as you say, in the creation of the rules, we create rules because we are a tax-funded health system that has finite resource, so we know that, every single time that NICE says yes, it is pushing something else out.

Q59 **Paul Blomfield:** So if we spent 14% of GDP on health, the rules to provide consistency may be different.

Dr Roberts: They may be different, indeed.

Q60 **Paul Blomfield:** Can I just pursue another point, which Rachael has raised, about international comparisons? Clearly, we are an outlier on Enhertu, and we have heard this morning that the concerns about the application of this approach to other new cancer drugs will affect the appetite for investment in the UK and the availability of treatments for patients. You quoted some current international comparisons, but we are at an early stage of your new approach. Are you confident that we are going to maintain our position, going forward?

Dr Roberts: I think it is tricky to be entirely confident, because the net position on medicines access is a result of very many factors, of which

NICE severity is one small one. We take our role in this very seriously, so, as I say, we monitor quarterly and annually, and we monitor internationally as well. Therefore, we will absolutely know within the month, the quarter, or the year if things are starting to move downwards. But I think that our industry colleagues would agree that we are part of a big system that gets medicines to patients—we have a very important regulator, a very important payer, and a very pressurised NHS. Therefore, I think that we take our own role very seriously, but we also understand that we are in some ways a small part of a much bigger system that affects the global attractiveness of the UK.

Paul Blomfield: Thank you very much.

Chair: That is a very interesting line of questioning.

Q61 **James Morris:** This is just a quick general question, Doctor Roberts. On the international comparisons, I think that you said that we are third on time to launch for drugs, but then you said that you recognise that we could get better on speed and agility. How can we get better?

Dr Roberts: Exactly as Baroness Morgan says, we think that every day that we delay is a day without patients having that care. We have been looking closely at every medicine that goes through us and what the main sources of delay are. The first job we did last year was to figure out that about one in five of the medicines we see are pretty straightforward to evaluate. We were giving them the full-fat NICE appraisal and they just did not need it. We now put those medicines through a much more slim-lined approach.

Q62 **James Morris:** When did you implement those changes?

Dr Roberts: We implemented those last year. In 2023-24, about 20% of medicines went through that, and the average time from when MHRA said yes to when we said yes was 38 days. That was tech. Now we have to look at what is happening with that other 80%. We look at what the biggest sources of delay are for every medicine. Currently, those are either that we did not know the medicine was coming in time—we weren't notified—and we are doing lots of work with industry, the regulator and overseas regulators to rectify that, or that the industry said, "Sorry, we don't have the evidence available. Can we delay it?"

We always worry about NICE capacity. Two years ago, exactly 20% of NICE appraisals were delayed because we did not have capacity.

Q63 **James Morris:** When you say capacity, do you mean people?

Helen Knight: It was resource to be able to deliver the evaluations.

Q64 **James Morris:** Is that mainly people?

Helen Knight: It includes NICE staff, committee time and other people who are involved in the evaluation.

Dr Roberts: Having said that, we have just seen our end-of-year numbers for 2023-24 and NICE capacity has led to zero delays. We have dealt with

our internal staff issues and are fully staffed and functioning. Now we need to get the handover between regulator, industry and NHS England really slick so we can deal with that last 80%.

Q65 **James Morris:** How does that become slicker?

Dr Roberts: We have five things we are doing this year. Do you want to take this?

Helen Knight: Before I do, I just wanted to comment on how important information sharing is, which means understanding when products are going through the regulatory process and how we can align NICE's process to help with that. As Sam said, the majority of delays we have seen are because we did not know they were coming. We have an important role to understand the information in terms of timing and also to start understanding the types of technical data that is going through the regulatory process that can help NICE be better prepared for evaluating those treatments.

The first workstream that we are looking at is alignment of the timing of the NICE process with regulatory programmes. We are also benchmarking our current process to understand with more depth what is causing deviations from processes. It should take between 36 and 50 weeks, but we are seeing topics take an average of longer than that. We want to have a better picture of what is happening and what is causing those delays, so we can respond to them.

Q66 **James Morris:** Is that going to be implemented as a series of initiatives in this business year?

Helen Knight: Yes. In this business year we are baselining everything. We want to have a much better picture of what is happening in practice and what is driving those deviations from what we expect, so that we can address what those issues are. We are also working closely with NHS England, which is updating its commercial framework. Obviously, we understand the importance of the commercial negotiations, but we are asking whether that can happen at a much earlier point in our process. We often see that we get to a committee meeting and we do not have those final commercial ranges in place. Is there a way that we can get that done in advance so that we can make positive recommendations at a first committee meeting?

We are working with our external assessment groups, which are academic groups that support the NICE process, to ensure that they are giving us exactly what we need at the time we need it, and we are quality checking their performance for the work they do for NICE. We are also looking to shorten the back end of the process, where appeals happen. The majority of our recommendations are positive, and therefore adding a three to five-week end part of the process when we have actually reached a positive decision may not be required. We are looking at a number of different factors this business year. Once we have collated all that information, we will be able to change some of it immediately; for some, we will need to do a bit of a deeper dive to understand how can rectify it.

Q67 **Chair:** Finally, as we conclude our Future Cancer inquiry, it sounds like in this instance it is not a ministerial decision—clearly, it is not a ministerial decision—but is there anything you would like to say about the process? Perhaps you sit there and think, "Yeah, we've got independence, we've sorted our internal staffing challenges, and we've got the five-point plan." But when it comes to the recommendations that we make to Ministers about the operation of NICE and the environment in which you thrive, is there anything that you would like to put on the record?

Dr Roberts: Yes. Notwithstanding the fact that we have kind of pored over the medicines appraisal process today and looked at its strengths and weaknesses, NICE medicines access is often recognised internationally as a pretty good thing that England has done. For the future of cancer, if you look at things such as early diagnosis, genomics, digital technologies and devices, we do not really have an equivalent process for those where you have very clear reimbursements and procurements. Yes, we could absolutely tinker with the NICE medicines access and we will continue to improve, but there is a big gulf when it comes to things that could be helping people care for themselves at home, and that could be helping them to get faster diagnosis and earlier diagnosis.

Q68 **Chair:** As tech develops, that opens a whole new can of worms on devices, and what are the devices?

Dr Roberts: Digital technologies, genomic diagnostics—

Q69 **Chair:** Artificial intelligence technologies?

Dr Roberts: Indeed.

Q70 **Rachael Maskell:** I just want to ask one last question. We have heard what you believe the industry needs to do now with regards to Enhertu. We have heard from Breast Cancer Now about the longing for women to be able to access the medication that is needed. What steps will you be taking when you leave the room to ensure that the hope that women have can be fulfilled?

Dr Roberts: Following today, we will get back in touch with our industry colleagues and understand if perhaps a new deal is on the table, which could be quickly taken back to committee. Then, to ensure that this is indeed an exception, not the rule, we will continue—day by day by day by day—to monitor severity, and then once we have a portfolio of severity, and medicines have gone through the process that we can make a decision on, we will take a decision on whether the calculation needs to be modified.

Chair: Thank you. Suffice it to say, you know us well enough to know that we will be watching closely and continue our great interest in this issue. To you from NICE and to everyone else who has spoken this morning: please make this the continuation, not the end, of the conversation. People's lives depend on it. Helen Knight and Dr Sam Roberts from NICE, thank you. That concludes the session.