HOUSE OF COMMONS LIBRARY

DEBATE PACK

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NICE appraisals of rare diseases

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Contents

Summary

This House of Commons Library Debate Pack briefing has been prepared in advance of a debate entitled "NICE appraisals of rare diseases". This will be led by Liz Twist MP and will take place in the House of Commons on Thursday 14 March 2019. This Pack contains background information, parliamentary and press material, and further reading suggestions which Members may find useful when preparing for this debate.

A rare disease is generally considered as one affecting fewer than 5 people in 10,000. However, large numbers of people are affected by rare conditions. The *UK Strategy for Rare Diseases* (2013) estimated that in the UK alone, more than 3 million people will suffer from a rare disease at some point in their life. Due to the fact that relatively small numbers of people are affected by a particular rare disease, there are specific challenges in ensuring speedy diagnosis and access to appropriate services and treatments. The National Institute for Health and Care Excellence (NICE) provides evidence-based information for the NHS on the effectiveness and cost-effectiveness of healthcare interventions. It publishes technology appraisal guidance on treatments which must be funded by NHS commissioners in England, including a process for the assessment of treatments for rare conditions. These have a higher cost effectiveness threshold than standard technology appraisals.

1. Background on rare diseases

A rare disease is generally considered as one affecting fewer than 5 people in 10,000. However, large numbers of people are affected by rare conditions. It is estimated that in the UK alone, more than 3 million people will suffer from a rare disease at some point in their life.[1]

A significant proportion (around 80%) of rare diseases have a known genetic cause but other causes include infections and cancer. Due to the fact that relatively small numbers of people are affected by a particular rare disease, there are specific challenges in ensuring speedy diagnosis and access to appropriate services and treatments.

In November 2013 the UK Department of Health and the devolved Governments published the *UK Strategy for Rare Diseases*. This was the UK's first strategy to help build understanding of rare diseases and boost research to find effective treatments and therapies. It set out 51 commitments to be delivered by 2020, addressing 5 key areas:

- empowering those affected by rare diseases;
- developing better methods of identifying and preventing rare diseases;
- improving diagnosis and earlier intervention for those with a rare disease;
- developing better coordination of care for those with a rare disease, including joined up consultation and treatment schedules; and,
- building on research to improve personalised approaches to healthcare for those with a rare disease.
- The strategy also highlighted the importance of collaborative working with patients, their families, industry, research and international partners.

The UK Rare Disease Policy Board was established in October 2016, and is responsible for the coordination of action to meet the commitments within the UK Strategy for Rare Diseases. The Board is made up of representatives of the UK departments of health, and rare disease charities such as Genetic Alliance UK.

A larger group, the UK Rare Disease Policy Forum has membership from wider stakeholder groups. This group is responsible for providing insight and advice on the strategy and ensuring that those with a range of rare conditions are represented.

The first progress report of the Rare Disease Strategy, *Delivering for patients with rare diseases: Implementing a strategy*, was published by the UK Rare Disease Policy Forum in February 2016. The report

[1] Department of Health and Social Care, UK strategy for rare diseases: implementation plan for England, January 2018

highlighted that there had been significant progress made since the publication of the strategy.

Implementation plans by NHS England and the Department of Health and Social Care (DHSC) were published jointly in January 2018 and describe the actions and framework in place to deliver this commitment to improve the lives of those affected by rare disease, as defined in the UK Strategy for Rare Diseases.[2]

Implementation plans have been published in Scotland, Wales and Northern Ireland. Links to these implementation plans are included below:

- Scottish Government, It's not rare to have a rare disease:The Implementation Plan for Rare Diseases in Scotland (2014)
- Welsh Government, Rare diseases implementation plan (2016)
- Northern Ireland Government, The Northern Ireland Implementation Plan for Rare Diseases (2015)

[2] Department of Health and Social Care, UK strategy for rare diseases: implementation plan for England; and NHS England, Implementation Plan for UK Strategy for Rare Diseases, January 2018

2. NICE appraisals

The National Institute for Health and Care Excellence (NICE) provides evidence-based information for the NHS in England on the effectiveness and cost-effectiveness of healthcare interventions. It publishes mandatory technology appraisal guidance (stipulating clinical interventions – mainly medicines – which must be funded by NHS commissioners (primarily clinical commissioning groups (CCGs) and NHS England), as well as advisory clinical guidelines and public health guidance (which commissioners are not obliged to implement).

The technology appraisal process involves looking at:

- evidence from clinical trials and peer reviewed research showing how well a medicine or treatment works, including its likely impact on mortality and quality of life (such as pain or disability);
- economic evidence on how much it costs the NHS; and,
- the views of clinicians, patients and other stakeholders.

NICE also undertakes separate appraisals for specialised treatments or treatments for rare conditions, known as Highly Specialised Technology (HST) evaluations. These have a higher cost effectiveness threshold than technology appraisals.

NICE describes the appraisal process as follows:

> We are asked [by the Department of Health and Social Care – DHSC] to look at particular drugs and devices when availability varies across the country. This may be because of different local prescribing or funding policies, or because there is confusion or uncertainty over its value. Our advice ends the uncertainty and helps to standardise access to healthcare across the country.

An appraisal or evaluation can classify a treatment, drug or device as 'recommended' (to be routinely commissioned), 'optimised' (to be commissioned in certain circumstances), 'only in research' or 'not recommended'. For cancer treatments, a fifth classification, 'recommended for use in the Cancer Drugs Fund' (CDF) is also available (this gives access to treatment for a fixed period of time to gather more clinical data, before a reappraisal is carried out).

Under the NHS Constitution, the relevant commissioning body (NHS England in the case of specialised treatments, CCGs for most other treatments) is legally obliged to fund treatments recommended by NICE within three months of the publication of the technology appraisal or HST evaluation (or longer, if a different time period is specified).

As well as looking at the clinical effectiveness of a treatment, technology appraisals and HST evaluations also assess its cost effectiveness. This is usually measured in terms of the cost per additional quality-adjusted life year (QALY). This is assessed by looking at how many extra months or years of life, of a reasonable quality, a person might gain as a result of treatment. When combined with information about the prices of different treatments, these techniques can be used

to guide decisions on how to maximise health benefits from the available resources.

Each treatment is considered on a case-by-case basis. Generally, however, if a treatment costs more than £20,000-30,000 per QALY, then it would not be considered cost effective. The £20,000-30,000 threshold has been in place since 2004.[3] Following changes introduced in April 2017, NICE set a maximum additional QALY threshold of £300,000 for highly specialised treatments, under which they will automatically be approved for routine commissioning. Further information on NICE Highly Specialised Technology appraisals can be found in the section below.

2.1 NICE Highly Specialised Technology appraisals

NICE carries out evaluations for selected high-cost low-volume drugs under its Highly Specialised Technologies programme. As with other NICE technology appraisals NHS England is required to fund treatments that have been evaluated and recommended by this programme within three months of the guidance publication.

The methods used to develop NICE's highly specialised technology (HST) guidance acknowledges that, given the very small numbers of patients living with these very rare conditions, establishing value for money is not straightforward. In particular, the HST guidance recognises the particular circumstances of these very rare conditions –the vulnerability of very small patient groups with limited treatment options, the nature and extent of the evidence, and the challenge for manufacturers in making a reasonable return on their investment because of the very small populations treated. In evaluating these drugs, NICE takes into account a greater range of criteria about the benefits and costs of highly specialised technologies than is the case with its appraisals of mainstream drugs and treatments.

In relation to HSTs, NICE states:

> We only consider drugs for very rare conditions. The majority of our topics are identified by the National Institute for Health Research Innovation Observatory. They aim to notify the Department of Health and Social Care of key, new and emerging healthcare technologies that might need to be referred to NICE against the following timeframes:
>
> •new drugs, in development, at 20 months to marketing authorisation
>
> •new indications, at 15 months to marketing authorisation.

A single HST evaluation can only cover a single technology for a single indication.

The NICE website provides further information on the HSTs development process (there are 11 stages):

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[3] Centre for Health Economics Research Paper 81, Methods for the Estimation of the NICE Cost Effectiveness Threshold, November 2013, pvii

- DHSC produces a list of provisional evaluation topics
- NICE identifies consultees and commentators
- NICE and DHSC develop a scope to define the disease, the patients and the technology
- DHSC refers evaluation topics to NICE
- NICE invites the manufacturer to submit evidence, and other consultees to submit statements on its clinical effectiveness and value for money
- NICE commissions an independent academic centre -Evidence Review Group (ERG) – to review the submissions
- ERG prepares a report
- An independent Evaluation Committee considers the report and hears evidence from clinical experts, patients and carers (in public)
- The Committee makes it provisional recommendation. If the recommendation is restrictive (that is, the recommendation is more limited in scope than the instructions for use of the technology), an evaluation consultation document is put out to consultation for health professionals and members of the public
- A Final Evaluation Determination (FED) is produced. Consultees can appeal against these recommendations.
- If there are no upheld appeals, the final guidance is issued.

Under the NHS Constitution, the relevant commissioning body (NHS England in the case of specialised treatments) is legally obliged to fund treatments recommended by NICE within three months of the publication of the HST (or longer, if a different time period is specified).

As well as looking at the clinical effectiveness of a treatment, HSTs also assess its cost effectiveness. This is usually measured in terms of the cost per additional Quality-Adjusted Life Year (QALY) that the treatment provides. QALYs are a measure of the years of life remaining for a patient, weighted on a quality of life scale. One QALY is equal to one year of life in perfect health.

Following changes introduced in April 2017, NICE set a maximum additional QALY threshold of £300,000 for highly specialised treatments, under which they will automatically be approved for routine commissioning. This is ten times higher than the standard NICE threshold of £30,000 for non-specialised treatments. The upper limit will vary according to the lifelong impact of the technology on the patient, varying from £100,000 per quality-adjusted life year for treatments that deliver less than 10 QALYs to the patient in their lifetime, up to a maximum of £300,000 for treatments that deliver more than 30 additional QALYs to the patient in their lifetime.

Although HSTs assess cost effectiveness, they do not assess affordability in terms of overall cost to the NHS of routinely commissioning a treatment. However, following changes introduced in April 2017, NICE can now allow for a phased introduction of any new treatment that

may have a substantial impact on the NHS budget, for a period of usually no more than three years (as opposed to the three month standard set out in the NHS Constitution).

Even where NICE does not produce TAs or HSTs on a treatment, commissioners are still able to commission the treatment. Information on NHS England's processes for deciding on commissioning treatments not evaluated by NICE can be found in section 2.3 of the Commons Library briefing paper, NHS commissioning of specialised services (CBP 7970, June 2017).

Although the recommendations of NICE technology appraisals are automatically adopted within NHS Wales, this is not the case for NICE highly specialised technology (HST) evaluations. For HSTs, the All Wales Medicines Strategy Group (AWMSG) will liaises with the Welsh Health Specialised Services Committee (WHSSC) to consider the applicability of the advice within NHS Wales. AWMSG then advise the Welsh Government on whether the highly specialised technology should be adopted within NHS Wales. The policy not to automatically adopt NICE HST evaluations followed concerns from WHSSCs about differences in accountability between England and Wales. NICE recommendations do not automatically apply in Scotland and Northern Ireland. Further information on specialised commissioning in the devolved administrations of the UK can be found in section 5 of the Library briefing paper, NHS commissioning of specialised services (CBP 7970, June 2017).

2.2 Further background to the NICE review of process for evaluating drugs from April 2017

NICE announced on 15 March 2017 that it was introducing a new approach to evaluating drugs, and that this would apply to those treatments that are reviewed from 1 April 2017 onwards.

The introduction of the changes followed a joint NHS England and NICE public consultation. The consultation sought view on a number of proposed changes, these included a speeded up process of evaluating drugs, and the introduction of a 'budget impact threshold' of £20 million. This threshold would mean that where any drugs that receive a positive NICE decision, but would exceed this cost in any of the first three years of use, NICE would indicate the need for a commercial agreement between the company and NHS England. NHS England could also apply for an extended period to introduce the treatment- this could mean that rather than the current three month deadline for NHS England to follow the technology appraisal guidance, this could be extended to three years. The consultation document highlighted that only a small number of new treatments would exceed this threshold- and stated that between June 2015 and June 2016, around 80% of all recommended treatments fell below the threshold.

The final changes that would be introduced were outlined in a press release:

> The changes will see the introduction of a new fast-track option for appraising treatments which offer exceptional value for money. The aim is for them to be available to patients a month after they are licensed.
>
> The fast track appraisals, which will be rolled out to more drugs in time, are part of several changes to NICE processes approved by the Board on Wednesday.
>
> Others include:
>
> - A budget impact test, where drugs that will cost more than £20m in any one of their first three years of use will trigger commercial discussions between the company and NHS England to mitigate the impact on the rest of the NHS.
> - Where agreement to minimise the impact of those drugs cannot be reached, NHS England will be able to choose to apply to NICE for an extended period in which to introduce the drug in a phased way. This will usually be for no more than three years.
> - Drugs for very rare diseases will be evaluated against a sliding scale, so that the more the medicine costs the greater the health benefit it must provide in order to be approved for routine NHS use by NICE.

The consultation document outlined that NICE evaluates a small number of drugs for rare diseases, but NHS England also considers these treatments through its specialised commissioning prioritisation process. It stated that these processes should be more linked.

The proposals also included that NICE highly specialised technology appraisals (HST) would use a funding requirement of £100,000 per QALY, which is 5 times higher than lower end of the standard threshold range for medicines. If a treatment was above this QALY value, it could be considered under the NHS England process for considering highly specialised technologies. Responses to the consultation on this subject expressed concerns about this funding requirement from patient charities and pharmaceutical companies.

The final NICE announcement on the new approach confirmed that a variable threshold would be used. NICE also announced that those treatments that are deemed to have significant benefits could be assessed against a maximum threshold of £300,000 per QALY rather than the £100,000 per QALY limit in the original proposal.

2.3 Responses to the changes to the NICE assessment process

Following the consultation publication, and the NICE announcement of the final decision regarding changes to its assessment process, concerns were expressed by organisations representing patients with rare diseases.

Genetic Alliance UK sent a letter to the then Secretary of State for Health, Jeremy Hunt, signed by 198 patient organisations:

> I am writing to you in your role as Secretary of State for Health to ask for a halt to the implementation of NICE and NHS England's

> proposed dynamic upper-limit for highly specialised technologies (HST), which will have a devastating impact on patients and work against the Government's goal of accelerating access to innovative medicines.
>
> We oppose these proposed changes because:
>
> - England already has extremely slow and limited access to treatments for rare genetic conditions. Further narrowing of access routes will in practice shut the door to innovation for our community of patients and families.
> - It is unacceptable to implement such drastically damaging proposals just 18 days after they are announced. We call for a pause in implementation, for an impact assessment on the proposals and for a comprehensive consultation with stakeholders.
> - These changes are contradictory to the positive recommendations made in the Accelerated Access Review, and will limit the impact of any attempt through the Industrial Strategy to position the UK as a centre for the development of innovative medicine.
>
> Fourteen days ago, NICE announced measures to limit recommendations by its Highly Specialised Technologies Evaluation programme. These proposals will halt future access to innovative treatments for rare genetic conditions in England, and send a chilling message to the life sciences sector, that runs contrary to messages from other parts of Government. We are calling for a pause in the implementation of these proposals, planned for 1 April 2017, to ensure that NICE's and NHS England's approaches are in step with what UK patients and families affected by rare genetic conditions need.[..]

Genetic Alliance UK provides more information about the criticisms of the new evaluation approach for highly specialised technologies on its website. In its response it states that whilst it welcomes the changes made since the consultation, these changes are not enough to prevent the proposals from having a significant effect on access to drugs for people with rare diseases.

3. Further reading

Commons Library debate pack, *Access to treatment, support and innovative new medicines for Phenylketonuria patients*, 25 June 2018

Commons Library briefing paper, *NHS Commissioning of Specialised Services*, 7 June 2017

Commons Library debate pack, *Implementing the UK Strategy for Rare Diseases*, 24 March 2017

The Westminster All Party Parliamentary Group on Rare, Genetic and Undiagnosed Conditions, [Accessed: 11 March 2019]

> On 3 December 2018, the APPG held a third hearing on access to rare disease medicines in England. The hearing provided attendees with the opportunity to hear from individuals and organisations with experience of working in the field of rare diseases. An update was also provided on Genetic Alliance UK's Resetting the Model project. Please see the Minutes from the hearing on Monday 3 December.

UK strategy for rare diseases: implementation plan for England, 29 January 2018

UK Rare diseases strategy, 22 November 2013

4. News

Brexit could harm my sons' access to treatment for rare 'black bone' disease, says father who quit job to find cure, Independent, 29 March 2018

FT Health: Orphan diseases move into the spotlight, Financial Times, 02 March 2018

Brexit risks for UK rare disease sufferers must be addressed, Financial Times, 28 February 2018

Niche drugs market looks set to balloon, Financial Times, 28 February 2018

'Diagnostic odyssey' nears its end for many families, Financial Times, 28 February 2018

5. Parliamentary Material

5.1 Health and Social Care Committee

The Health and Social Care Committee is undertaking an inquiry on the availability of the cystic fibrosis medicine Orkambi on the NHS in England. The Committee's inquiry into this area is currently concentrated on the provision and pricing of Orkambi, and other drugs developed by Vertex Pharmaceuticals for the treatment of cystic fibrosis. The Committee is considering the positions taken in the ongoing negotiations between Vertex and NHS England, and NICE's assessment of the cost-effectiveness of these drugs. Further information about the inquiry is available on the Committee website here.

5.2 Oral PQs

Business of the House HC deb 07 Mar 2019 volume 655 c1138

> As ever, I am grateful to the hon. Gentleman for giving me prior notice of upcoming debates. He mentions the Back-Bench debate proposed for next week on rare diseases. I have two young people
>
> in my constituency with cystic fibrosis who are both desperate for access to the Orkambi drug, so it is my very dear wish that that debate go ahead. It will not surprise him to know that I am also extremely keen that it go ahead because that will mean that the House will have passed a previous motion. I will take careful account of what he is asking for.
>
> **Member:** Andrea Leadsom | **Party:** Conservative Party

Topical Questions HC deb 28 Feb 2019 volume 655 cc518-640

> **Asked by:** Liz Twist (Blaydon) (Lab) | **Party:** Labour Party
>
> Can the Secretary of State guarantee that in the event of a no-deal Brexit, the UK will have stocks of the essential dietary products for people with the rare disease phenylketonuria, or PKU?
>
> **Answered by:** Chris Heaton-Harris | **Party:** Conservative Party | **Department:** Exiting the European Union
>
> Probably better than me confirming that is for me to point the hon. Lady to the written ministerial statement laid before the House earlier this week, which goes into great detail. I will happily give her a copy afterwards.
>
> **Date answered:** 28 Feb 2019

Rare Diseases and Cancer HC deb 15 Jan 2019 volume 652 c1006

> **Asked by:** Daniel Zeichner (Cambridge) (Lab) | **Party:** Labour Party
>
> Given that the number of people suffering from rare diseases in any one country is always likely to be small, and given our changing relationship with the European Medicines Agency and the European medicines market, what is the Minister doing to

ensure that the future development of orphan drugs in this country is safeguarded?

Answered by: Steve Brine | **Party:** Conservative Party | **Department:** Health and Social Care

The hon. Gentleman will know that the draft withdrawal agreement hopefully sets us on a relationship with the EMA, but the UK's strategy for

rare diseases, which was published in 2013, sets out our commitment to improve the diagnosis and treatment of patients with rare diseases and to end the diagnostic odyssey that has been referred to throughout the past few years.

Date answered: 15 Jan 2019

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Given that the number of people suffering from rare diseases in any one country is always likely to be small, and given our changing relationship with the European Medicines Agency and the European medicines market, what is the Minister doing to ensure that the future development of orphan drugs in this country is safeguarded?

Oral questions - Supplementary

Answering member: Steve Brine | **Party:** Conservative Party | **Department:** Health and Social Care

The hon. Gentleman will know that the draft withdrawal agreement hopefully sets us on a relationship with the EMA, but the UK's strategy for

rare diseases, which was published in 2013, sets out our commitment to improve the diagnosis and treatment of patients with rare diseases and to end the diagnostic odyssey that has been referred to throughout the past few years.

Date answered: 15 Jan 2019

Rare Diseases and Cancer HC deb 15 Jan 2019 volume 652 c1006

Asked by: John Howell (Henley) (Con) | **Party:** Conservative Party

What steps he is taking to improve the diagnosis and treatment for patients with rare diseases and cancer.

Oral questions - Lead

Answering member: The Parliamentary Under-Secretary of State for Health and Social Care (Steve Brine) | **Party:** Conservative Party | **Department:** Health and Social Care

Our much-mentioned new plan sets out the clear ambition to diagnose three quarters of all cancers at an early stage—up from half today.

Date answered: 15 Jan 2019

NHS Long-Term Plan HC deb 07 Jan 2019 volume 652 c74

Asked by: Jim Shannon (Strangford) (DUP) | **Party:** DUP

I, too, congratulate the Secretary of State on the NHS 10-year long-term plan. There are between 6,000 and 8,000 rare diseases. One in 17 people, or 6% of the population, will be affected by a rare disease in their lifetime—that is 3.5 million people in the United Kingdom. Will he confirm a commitment to assisting those with rare diseases, and can the NICE process for new life-saving drugs be urgently speeded up so that more lives can be lightened and saved?

Answering member: Matt Hancock (West Suffolk) (Conservative) | **Party:** Conservative Party

I strongly agree with the hon. Gentleman. For those who have rare diseases, diagnosis takes seven years, on average, and genomics can bring that down to a matter of seven days, in the best cases. We are the world leaders in genomics and we are going to stay that way. We have reached the 100,000 genome sequence and we are going to take it to 1 million, with 500,000 from the NHS and 500,000 from the UK Biobank. He is absolutely right. This is one of the bright shining stars of the future of healthcare, and Britain is going to lead the way.

5.3 Written PQs

Medicine: Research PQ HC226213 06 Mar 2019

Asked by: Moore, Damien | **Party:** Conservative Party

To ask the Secretary of State for Health and Social Care, what recent assessment his Department has made of the potential effect on health research of the UK leaving the EU without a deal.

Answering member: Stephen Hammond | **Party:** Conservative Party | **Department:** Department of Health and Social Care

The Government has stated its aim to establish a far-reaching science and innovation pact with the European Union, facilitating the exchange of ideas and researchers, and enabling the United Kingdom to participate in key programmes alongside our EU partners.

The Government is committed to securing a Withdrawal Agreement with the EU. However, as a responsible Government, we continue to prepare for all scenarios, including 'no deal'. As part of these preparations, the Government is working across a range of workstreams to understand the potential effect of the UK leaving the EU without a deal, and to ensure that the UK has the best possible environment for basic, biomedical, health, and life sciences research. This includes access to and mobility of research leaders and the technical and research delivery workforce; access to EU research funding; the regulation of clinical trials, data and devices; rare diseases research; and clinical trials supplies in a 'no deal' scenario.

Rare Diseases: Drugs PQ HC223060 26 Feb 2019

Asked by: Lamont, John | **Party:** Conservative Party

To ask the Secretary of State for Health and Social Care, if he will make an assessment of the potential merits of implementing a UK-wide scheme for the purchase and distribution of expensive drugs and medical supplies that are used to treat rare conditions.

Answering member: Steve Brine | **Party:** Conservative Party | **Department:** Department of Health and Social Care

We have no plans to assess the potential merits of implementing a United Kingdom-wide scheme for the purchase and distribution of expensive drugs and medical supplies that are used to treat rare conditions. It is for the National Health Service in England and the devolved administrations to make decisions in the best interest of their patients, including those with rare diseases, to ensure access to the medicines and medical supplies they need.

Cancer: Screening PQ HL13271 12 Feb 2019

Asked by: Lord Warner | **Party:** Crossbench

To ask Her Majesty's Government what steps they are taking, or plan to take, to mitigate the possibility of variation in patient access to cancer genomic testing in England resulting from Clinical Commissioning Group decision-making.

Answering member: Baroness Blackwood of North Oxford | **Party:** Conservative Party | **Department:** Department of Health and Social Care

The *Annual Report of the Chief Medical Officer 2016: Generation Genome*, made clear that in order to achieve equitable access to genomic testing the National Health Service will need to implement a first-class genomic medicine service that is scalable, future proof and delivers value for money. The report also noted the work undertaken as part of the 100,000 Genomes Project. A copy of the report is attached.

Therefore, NHS England announced in October 2018 that over the next 18 months work to mobilise the NHS Genomic Medicine Service (GMS) would get underway. The aims of the GMS are to:

- Provide consistent and equitable care for the country's 55 million population;

- Operate to common national standards, specifications and protocols;

- Deliver to a single national genomic testing directory – covering use of all technologies from single gene to whole genome sequencing initially for rare disease and cancer;

- Give all patients the opportunity to participate in research (for individual benefit and to inform future care); and

- Build a national genomic knowledge base to provide real world data to inform.

A key element of the GMS is the National Genomic Test Directory which outlines the entire repertoire of genomic tests for cancer and rare and inherited disease – from Whole Genomic Sequencing to panel tests, to tests for single genes and molecular markers – that are available as part of the NHS clinical service in England.

The NHS GMS will be supported by an informatics system, that is being developed in partnership with Genomics England. When fully operational the National Genomics Informatics Service will

enable NHS England to monitor the number of genomic tests being carried out across the country and benchmark activity.

Attachment: CMO Annual Report 2016 Generation Genome

Orphan Drugs: Regulation PQ HC206025 16 Jan 2019

Asked by: Frith, James | **Party:** Labour Party

To ask the Secretary of State for Health and Social Care, when his Department plans to publish its consultation on the regulation of orphan drugs after the UK leaves the EU.

Answering member: Jackie Doyle-Price | **Party:** Conservative Party | **Department:** Department of Health and Social Care

The Government is committed to the safe and effective regulation of medicines in the United Kingdom; ensuring patients and the public have fast access to new, innovative medicines, including medicines for rare diseases.

The Medicines and Healthcare products Regulatory Agency (MHRA) launched in October 2018 a consultation on the regulation of medicines in the event of a 'no deal' exit, which included the proposed UK approach to the regulation of orphan medicines post-exit, including on incentives to encourage such medicines onto the UK market. Following this consultation, the MHRA, on 3 January 2019, published guidance on the UK proposed arrangements for the regulation of orphan medicines, in the event of a 'no deal' exit. This can be accessed at the following link:

https://www.gov.uk/government/publications/further-guidance-note-on-the-regulation-of-medicines-medical-devices-and-clinical-trials-if-theres-no-brexit-deal

Rare Diseases: Drugs PQ HC205211 14 Jan 2019

Asked by: Tami, Mark | **Party:** Labour Party

To ask the Secretary of State for Health and Social Care, what proportion of the rare disease medicines that do not meet the Highly Specialised Technology Criteria have been approved for access by NICE.

Answering member: Steve Brine | **Party:** Conservative Party | **Department:** Department of Health and Social Care

The National Institute for Health and Care Excellence has advised that it has published 48 technology appraisals of orphan medicines for rare diseases, of which it has recommended 75% for some or all of the eligible patient population.

Rare Diseases PQ HC206027 15 Jan 2019

Asked by: Frith, James | **Party:** Labour Party

To ask the Secretary of State for Health and Social Care, what steps he is taking to ensure the availability of treatments for patients with rare diseases since the introduction of the Orphan Medical Product Regulation 2000.

Answering member: Steve Brine | **Party:** Conservative Party | **Department:** Department of Health and Social Care

The Government is committed to improving the lives of all those affected by a rare disease. Implementation plans by NHS England and the Department were published jointly in January 2018 and describe the actions and framework in place to deliver this commitment to improve the lives of those affected by rare disease, as defined in the UK Strategy for Rare Diseases.

Through its technology appraisal and highly specialised technologies programmes, the National Institute for Health and Care Excellence (NICE) plays an important role in ensuring that patients, including patients with rare diseases, are able to benefit from effective new treatments. NICE has recommended a number of drugs for rare diseases through these programmes that are now routinely funded by the National Health Service for eligible patients.

Rare Diseases PQ HC206026 10 Jan 2019

Asked by: Frith, James | **Party:** Labour Party

To ask the Secretary of State for Health and Social Care, what measures he is taking to utilise UK genomic medical research to improve outcomes for patients living with rare diseases.

Answering member: Caroline Dinenage | **Party:** Conservative Party | **Department:** Department of Health and Social Care

The Government's 2013 UK Strategy for Rare Diseases aims to improve the lives all those with a rare disease and is currently being implemented across England. The Strategy covers five areas including the role of research, with significant support from the National Institute of Health Research.

In October 2018, the Secretary of State announced his ambition to sequence 5 million genomes in the next five years including 1 million whole genome sequences. This builds on the success of the transformative 100,000 Genomes Project which recruited people with rare, undiagnosed genetic diseases, and with cancer, and which reached 100,000 sequences in December 2018.

The NHS Long Term Plan published on 7 January sets out the National Health Service's commitment to research and innovation to drive outcome improvement, including through targeted investment in genomics. It confirmed that the new NHS Genomic Medicine Service will sequence 500,000 whole genomes by 2023/24 as part of the NHS's contribution to the Government's ambition for genomics. The NHS has become the first national health care system to offer whole genome sequencing as part of routine care. Another 500,000 whole genomes will be sequenced through UK Biobank as was announced in the 2017 Life Sciences Sector deal. Together, these activities will significantly increase the volume of high-quality genomic data for research to advance our knowledge to diagnose and treat rare diseases and other conditions to complement the research dataset already generated by the 100,000 Genomes Project.

Rare Diseases: Medical Treatments PQ HC202091 20 Dec 2018

Asked by: Day, Martyn | **Party:** Scottish National Party

To ask the Secretary of State for Health and Social Care, what steps he is taking to ensure that the UK remains a world leader for the treatment of rare diseases after the UK leaves the EU.

Answering member: Jackie Doyle-Price | **Party:** Conservative Party | **Department:** Department of Health and Social Care

The Government is committed to the safe and effective regulation of medicines in the United Kingdom; ensuring patients and the public have fast access to new, innovative medicines, including medicines for rare diseases.

The agreement of an implementation period will ensure that access to medicines continues, and patient safety is maintained, in both the UK and European Union markets. Beyond that, the Political Declaration sets out a plan for a free trade area for goods, underpinned by deep regulatory co-operation, as well as a joint commitment to explore close cooperation with the European Medicines Agency.

The Government also continues to prepare for the unlikely outcome that we leave the EU without any deal in March 2019. The Department has been engaging with all pharmaceutical companies that supply the United Kingdom with pharmacy or prescription-only medicines from, or via, the EU/European Economic Area, on their contingency plans in the event of a no deal EU Exit.

Whatever the exit scenario, we will work to ensure that the UK will remain a world leader for the treatment of rare diseases after the UK leaves the EU, that UK patients are able to access the best and most innovative medicines including medicines for rare diseases and that their safety is protected. The UK life sciences industry has much to offer in creating, developing, trialing and commercialising medicines that will benefit UK patients and strengthen the ability of the UK to compete internationally.

Rare Diseases: Drugs PQ HC203975 07 Jan 2019

Asked by: Frith, James | **Party:** Labour Party

To ask the Secretary of State for Health and Social Care, pursuant to the Answer of 19 December 2018 to Question 201214 on Rare Diseases: Drugs, on how many occasions NICE has made a conditional recommendation for a rare disease treatment that do not qualify for conditional recommendation through the cancer drugs fund; and what criteria NICE uses to determine whether such treatments are eligible for conditional recommendations.

Answering member: Steve Brine | **Party:** Conservative Party | **Department:** Department of Health and Social Care

The National Institute for Health and Care Excellence (NICE) is able to take into account the commercial terms offered by a manufacturer during a NICE appraisal through a mechanism known as a commercial access agreement. On two occasions NICE has issued optimised recommendations to make rare disease medicines routinely available for the appropriate group of National Health Service patients that are conditional on it being supplied in accordance with the terms of the commercial access arrangements. These were Sorafenib, for advanced hepatocellular carcinoma, and ibrutinib for relapsed or refractory mantle cell lymphoma.

This is different from the process of a conditional recommendation for use within the Cancer Drugs Fund (CDF) for oncology drugs, where there is significant remaining clinical uncertainty which needs more investigation through data collection. In these cases funding is provided from the CDF for a time-limited period to allow patient access whilst this data is collected, before guidance is then reviewed again for routine commissioning.

NICE does not have any particular criteria to determine whether treatments are eligible for conditional recommendations. However, the process guide for patient access schemes and commercial access agreements and the CDF can be found at the following link:

https://www.nice.org.uk/process/pmg19/chapter/patient-access-schemes-commercial-access-agreements-and-flexible-pricing

Orphan Drugs PQ HC202090 20 Dec 2018

Asked by: Day, Martyn | **Party:** Scottish National Party

To ask the Secretary of State for Health and Social Care, whether he is taking steps to introduce a revised definition for ultra-orphan medicine.

Answering member: Jackie Doyle-Price | **Party:** Conservative Party | **Department:** Department of Health and Social Care

Currently, rare diseases are defined as conditions affecting no more than five in 10,000 people in the European Union. The new European Clinical Trial Regulation (Regulation (EU) No 536/2014) will introduce an 'ultra-rare' condition and states that clinical trials for the development of orphan medicinal products and those of medicines affecting no more than one person in 50,000 in the EU (ultra-rare diseases) should be fostered, and in such areas, a rapid yet in-depth assessment is of particular importance. The new clinical trials regulation is expected to be implemented in the EU in late 2020. The Government has confirmed that United Kingdom law will remain aligned with the parts of the new EU clinical trials Regulation that are within the UK's control.

DEBATE PACK
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