HOUSE OF COMMONS LIBRARY

DEBATE PACK

Number 2016/0242, 9 December 2016

Implications of the Accelerated Access review on cystic fibrosis and other conditions

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Summary

This Debate Pack has been prepared ahead of the debate on the Implications of the Accelerated Access Review for cystic fibrosis and other conditions, to be held in Westminster Hall on Tuesday 13 December at 9.30am.

The subject for the debate has been chosen by the Backbench Business Committee, following a representation made by Ian Austin, Ben Howlett and Kerry McCarthy.

Contents

1. Implications of the Accelerated Access review on cystic fibrosis and other conditions

The final report of the independent Accelerated Access Review was published on 24 October 2016. This makes a number of recommendations aimed at improving access for patients to new and effective innovative products. These include an accelerated access pathway for designated transformative products, and involving patients at all stages along this pathway. It has been reported that the implementation of these recommendations could lead to patient access to medicines being brought forward by 4 years.[1]

The recommendations in the report have been welcomed by the Government, and by a number of medical and charitable organisations.

The Cystic Fibrosis Trust have been campaigning for access to two medicines that target specific genetic mutations in some patients with the condition- Kalydeco and Orkambi. The National Institute for Health and Care Excellence has recently published its final decision on Orkambi- that it is not recommended for use on the basis that it could not be considered a cost-effective use of NHS resources. The Cystic Fibrosis Trust states that the Accelerated Access Review made several recommendations which could enable access to this drug. The organisation has an ongoing campaign, Stopping the clock, and has been encouraging people to write to their MP on this issue- it has been reported that over 6,000 people had written at the end of November.

Access to medicines for a number of conditions could be affected by the implementation of the recommendations in the AAR. The report itself highlights examples of approaches to developing and accessing new medicines in myeloma, melanoma and dementia. Muscular Dystrophy UK have stated that accelerating access to new treatments, would be important for people with muscle wasting conditions.[2] Alzheimer's Research UK[3] have reported that the review recommendations came at a crucial time for people with dementia- a number of new treatments are currently in clinical trials, and if these are positive the recommendations could ensure they get to patients quicker.

The Accelerated Access Review

Background

In November 2014 the then Minster for Life Sciences, George Freeman, announced "*an external review of the development, assessment and adoption of innovative medicines and medical technologies*". He said

1 Department of Health, Getting patients quicker access to innovative healthcare, 24 October 2016
2 Muscular Dystrophy UK, Major report on speeding up access to treatments, 24 October 2016
3 Alzheimers Research UK, New report sets out plan to accelerate access to new medicines at 'crucial time' for dementia, 24 October 2016

that recent technological advances have the potential to transform prevention and treatment. He said that the independent review would consider how to speed up access to cost-effective diagnostics and treatments; it would focus on innovative products.

The main themes and questions of the review were:

1. establishing need, priorities and principles for innovation – how can we find a transparent way for innovators to make sure that innovation is based on patient need, and that industry, the NHS, research charities and academia collaborate to understand and respond to patient need?
2. new development pathways – how can we make sure that the existing safety and efficacy process is more efficient and simple, while maintaining safeguards for patients, and that there is a clear, and quicker way to have access to particularly innovative products?
3. affordable national funding models to drive innovation – how can we integrate and speed up national reimbursement processes, and fund clinically and cost-effective innovation across the pathway?
4. local adoption and diffusion – how can we speed up how clinically and cost effective innovative products are commissioned by the local NHS, and get to patients?

The final report

The final report of the Accelerated Access Review (AAR) was published on 24 October 2016. The report made a number of recommendations, including that more streamlined processes could allow for quicker access to drugs and ensure patients benefit sooner. The report recommended that those innovations with the greatest potential could be designated as 'transformative products', and enter an accelerated pathway to patient use.

The Department of Health highlighted the issues relating to accessing innovative treatments, and the AAR recommendation of a new accelerated access partnership*"to speed up and simplify the process for getting the most promising new treatments and diagnostics safely from pre-clinical development to patients:"*

> The review says that accessing innovation in the NHS has become increasingly challenging. This creates frustration for clinicians and patients who often have to wait for life-saving treatments, and for innovators who must navigate multiple processes before their products can be used.
>
> Through the new partnership, innovators would be able to access joined-up help for clinical development, regulation, and assessment of cost effectiveness. It is recommended that the partnership includes NHS England, NHS Improvement, the National Institute for Health and Care Excellence (NICE) and the Medicines and Healthcare Products Regulatory Agency (MHRA).
>
> Patient access to drugs could be brought forward by up to 4 years if a scientific opinion from the early access to medicines scheme is used (saving 12 to 18 months) and there is no delay during the technology appraisal (which can take up to 2 years) or during the process for NHS commissioning and adoption (which can take 2 years or more).

> The review recommends a simpler process for digital technologies which are often developed by smaller companies, such as healthcare apps for managing long-term conditions.[4]

Box 1: AAR recommendations

1. The NHS should develop an enhanced horizon scanning process and clarify its needs to innovators.
2. A new transformative designation should be applied to those innovations with the potential for greatest impact.
3. Patients should be involved in horizon scanning and prioritisation, and this involvement should continue along the whole innovation pathway.
4. An Accelerated Access Pathway for strategically important, transformative products should align and coordinate regulatory, reimbursement, evaluation and diffusion processes to bring these transformative products to patients more quickly.
5. A new strategic commercial unit should be established in NHS England.
6. The accelerated access pathway should be suitable for medical technologies, diagnostics and digital products as well as medicines and emerging forms of treatment.
7. There should be a single set of clear national and local routes to get medical technologies, diagnostics, pharmaceuticals and digital products to patients.
8. National routes to market should be streamlined and clarified.
9. Many products will benefit from regional and local routes to market, which should be enhanced to operate consistently across the NHS.
10. The route for digital products should build on the Paperless 2020 simplified app assessment process.
11. The digital infrastructure should enable the system to capture information on the use of innovations and associated outcomes.
12. The process of assessing emerging technologies should be evolved so that it is fit for the future.
13. A range of incentives should support the local uptake and spread of innovation, enabling collaboration and with greater capacity and capability for change.
14. AHSNs, tertiary academic teaching hospitals and clinical leaders across the NHS should drive and support the evaluation and diffusion of innovative products.
15. Improved accountability and transparency around uptake of innovation should be supported by NICE.
16. An Accelerated Access Partnership should align national bodies around accelerating innovation.
17. The Accelerated Access Partnership should be established immediately.
18. Implementation of the report's recommendations should be led by the Accelerated Access Partnership and clinicians.

Responses to the report

In a Written Statement, the Secretary of State for Health said that the Government welcomed the recommendations in the report:

> The report provides us with a strong basis to make the right decisions about how the health system can be adapted to meet the challenges of the future, attract inward investment, grow our thriving life sciences industry and use innovation to improve patient outcomes in the context of the financial pressures on the NHS. It will be important to implement this report in a way that is affordable for the NHS. The Government will now consider the proposals in detail with our partners and will provide a fuller response in due course.

[4] Department of Health, Getting patients quicker access to innovative healthcare, 24 October 2016

> The Government remains strongly committed to the life sciences and to building a long-term partnership with industry. It is determined to help the UK become the best place in the world to produce new drugs and products that can transform the health of patients, where the research, development, regulatory, commercialisation and investment infrastructure enable innovation to flourish and thrive while improving patient's lives.[5]

A number of medical and charitable organisations[6,7] have also welcomed the recommendations in the report. The CEO of the Association of Medical Research Charities, Aisling Burnard said that the review offers patients the opportunity to have a say in what innovations are important to them:

> "We welcome the publication of the Accelerated Access Review and its recommendations for getting the best technologies for patients more quickly. We know patients and their families want early and fast access to new life-changing and life-enhancing innovations.
>
> "The review offers patients the opportunity to have a say about what innovations are important to them, this is a good outcome for patients. As the beneficiaries of new health technologies, patients have important experiences to share in developing routes to access innovations.
>
> "The recommendations are a step in the right direction in supporting the UK's position as a leader in the life sciences, as we prepare to leave the European Union. We need to continue to work together to ensure the UK remains not only a great place to do research, but also enables patients fast and effective access in the NHS to the best technologies as they emerge."

5 HC Written statement HCWS209: Accelerated Access Review, 24 October 2016
6 Alzheimers Research UK, New report sets out plan to accelerate access to new medicines at 'crucial time' for dementia, 24 October 2016
7 AMRC, AMRC's response to the Accelerated Access Review, 24 October 2016

2. News articles

BBC news online

Thousands campaign for cystic fibrosis drug Orkambi to be made available on the NHS

30 November 2016

http://www.bbc.co.uk/news/uk-england-berkshire-38141663

The Guardian

NHS access to innovative drugs could be cut by four years, says review

24 October 2016

https://www.theguardian.com/society/2016/oct/24/nhs-access-to-innovative-drugs-could-be-cut-by-four-years-says-review

The Independent

Jeremy Hunt backs plan to cut four years from NHS scrutiny of new medicines

24 October 2016

http://www.independent.co.uk/news/uk/politics/nhs-jeremy-hunt-four-years-accelerated-access-review-a7377696.html

British Medical Journal

NHS must find ways to speed access to new drugs and technologies

25 October 2016

http://www.bmj.com/content/355/bmj.i5760

BBC news online

Cystic Fibrosis drug Orkambi decision 'a death sentence'

24 March 2016

http://www.bbc.co.uk/news/uk-england-leeds-35892531

FT

Drug prices: Tweaking the formula: UK regulators are under pressure from Big Pharma as well as patients

Andrew Ward 25 April 2016

https://www.ft.com/content/b034a38e-0abf-11e6-9456-444ab5211a2f

3. Press releases

Cystic Fibrosis Trust

Green light for Kalydeco

5 December 2016

NHS England gives the go-ahead for transformational treatment that can halt the progression of cystic fibrosis for two-to-five-year-olds.

After a sustained campaign by the Cystic Fibrosis Trust, young children in England will receive Ivacaftor (Kalydeco), a life-changing drug for cystic fibrosis (CF). Kalydeco is proven to significantly improve lung function and slow the progression of CF, reducing time spent on intravenous antibiotics from five weeks to less than five days a year. It has also been found that the earlier Kalydeco is provided, the better the outcome for individuals. Data also suggests for the first time that people receiving this treatment could have a near normal life expectancy.

Kalydeco has been provided in England for people over the age of six since 2014. It was licensed for two-to-five-year-olds in November, and today NHS England have announced that it will be one of the drugs offered on the NHS to eligible two-to-five-year-olds with CF in England following the 're-prioritisation process'. Kalydeco is already being offered to children in Scotland, and it is expected that Northern Ireland and Wales will now follow suit.

The first precision medicine in CF, Kalydeco targets a group of rare mutations that affect about 5% of people with the condition in the UK. Emma Foord, from Wiltshire in Southwick, has a 22-month-old son, William, who could benefit from the drug:

"I'm over the moon to hear that William will be given access to Kalydeco - this drug could have a huge impact on my son's future. I have watched him fight for his life on three occasions as a result of cystic fibrosis. I have nearly lost him to a perforated bowel, a bowel obstruction and septic shock as a result of a ruptured bowel abscess. I have also seen him go to an operating theatre on four occasions, two of which were lifesaving, so to know that he will be given access to this life-changing drug is a huge relief. My biggest worry is CF winning and my son losing. This gives me great hope that it will help normalise William's life now and in the future."

James Barrow, Head of External Affairs at the Trust, said:

"After a year of waiting we are pleased that this life-changing treatment is now available to young children in England. It will transform lives and give hope for the future. The drug is also available in Scotland and we urge Wales and Northern Ireland to follow suit."

The Trust is campaigning hard to ensure innovative drugs like Kalydeco are provided to those who need them. We are funding vital work into clinical trials to help unblock the pipeline of exciting new treatments that could help improve the lives of people with cystic fibrosis.

Please donate now to help us continue our vital work.

Cystic Fibrosis Trust

Add your voice to the Orkambi debate

24 November 2016

Your story could inform a parliamentary debate that aims to put pressure on the Government to help ensure access to Orkambi.

A parliamentary debate on 13 December, organised by the Cystic Fibrosis Trust in collaboration with MPs Ian Austin, Kerry McCarthy and Ben Howlett, will encourage the Government to take action using stories from the CF community submitted to MPs via an e-action launching today.

Write to your MP now!

Since NICE was unable to recommend Orkambi for use on the NHS in England, negotiations between Orkambi manufacturer Vertex, the Government and the NHS have reached a deadlock. Last month, the Accelerated Access Review – an influential report into speeding up access to drugs like Orkambi – made many recommendations to the Government that could be key to breaking this deadlock, such as flexible pricing arrangements and the gathering of real-world data to prove how effective the drug is.

The Cystic Fibrosis Trust is urging all parties to embrace these recommendations and get Orkambi to those who desperately need it without delay. The debate will put pressure on the Government to help apply the flexibility recommended in the Accelerated Access Review to the Orkambi negotiations.

What can you do?

Ask your MP to share your story at the debate to show the Government how important this issue is. To help, the Trust has produced a template; please personalise the letter as much as possible, this will have a stronger impact on your MP and ensure that if they cannot attend the debate they still raise the matter on your behalf. If you have previously contacted your MP about Orkambi, please remember to mention this too.

You can also watch the event online via a livestream from 9.30am on 13 December, and stay up-to-date with the Trust's Stopping the Clock campaign.

Not from England?

If you're from Wales or Northern Ireland, it's still worth writing to your MP. This is because although both nations have their own processes for drugs like Orkambi, they tend to follow England's lead.

However, if you're from Scotland, then your MP cannot assist with this. This is because Scotland has an entirely independent process. We're

pursuing a separate campaign that reflects that; look out for updates, coming soon.

Why is it so important?

Orkambi is a precision medicine that treats the underlying genetic cause of cystic fibrosis rather than just the symptoms. Studies have shown that it significantly reduces hospital admissions and slows the decline in lung function in people with the genetic mutations it targets. With the power to lift so many of the limits cystic fibrosis can place on people with the condition, it's vital that access is granted without delay.

Department of Health

News story: Getting patients quicker access to innovative healthcare

24 October 2016

New recommendations set out how patients could get quicker access to innovative new diagnostic tools, treatments, and medical technologies.

The Accelerated Access Review: final report, commissioned by the government and led by an independent chair, was published today (24 October). The review aims to make the UK a world-leader in healthcare innovation, with an NHS that embraces the new drugs and technologies that patients need and supports work with local areas to develop solutions to their specific healthcare needs.

The report says streamlined processes could bring forward patient access to drugs by up to 4 years and patients will benefit from quicker access to medical technologies too. The report will help the NHS to provide the best care to patients, use funds more effectively, and create the conditions to help the life sciences industry continue to thrive.

Chair of the Accelerated Access Review, Sir Hugh Taylor, said:

This ambitious plan will prepare the health system for an exciting era in medical innovation.

We've listened to the views of the NHS, patients, clinicians, the life sciences industries and academia – and it is clear we need to act now to make the most of the tidal wave of new drugs and technologies that are being developed.

The review, developed in partnership with the Wellcome Trust, recommends the creation of a new accelerated access partnership to speed up and simplify the process for getting the most promising new treatments and diagnostics safely from pre-clinical development to patients. Patients expect the NHS to provide life-changing innovations as soon as they become available – but evidence has shown that the UK sometimes lags behind other countries.

The review says that accessing innovation in the NHS has become increasingly challenging. This creates frustration for clinicians and patients who often have to wait for life-saving treatments, and for

innovators who must navigate multiple processes before their products can be used.

Through the new partnership, innovators would be able to access joined-up help for clinical development, regulation, and assessment of cost effectiveness. It is recommended that the partnership includes NHS England, NHS Improvement, the National Institute for Health and Care Excellence (NICE) and the Medicines and Healthcare Products Regulatory Agency (MHRA).

Patient access to drugs could be brought forward by up to 4 years if a scientific opinion from the early access to medicines scheme is used (saving 12 to 18 months) and there is no delay during the technology appraisal (which can take up to 2 years) or during the process for NHS commissioning and adoption (which can take 2 years or more).

The review recommends a simpler process for digital technologies which are often developed by smaller companies, such as healthcare apps for managing long-term conditions.

The review also suggests that a new strategic commercial unit should be created within NHS England to enter into commercial dialogue to create flexible arrangements with innovators who are working on transformative new products. The unit would aim for "win-win" scenarios where innovators benefit through earlier access to the NHS market and increased sales. In return innovators would offer better value to the NHS and patients.

Health Minister Lord Prior said:

> This government has a strong commitment to the life sciences and to building a long-term partnership with the life sciences industry. We are determined to make the UK the best place in the world to develop new drugs and other products that can transform the health of patients.
>
> The report provides us with a strong basis to make the right decisions about how the health system can be adapted to meet the challenges of the future, attract inward investment, grow the thriving life science industry and use innovation to improve patient outcomes and tackle the financial pressures on the NHS.

Read Lord Prior's full statement on the Accelerated Access Review.

Patients are central to the review. Under the recommendations of the review, patients are offered a greater say in determining what innovations are important to them, so that real experiences of conditions such as diabetes or cancer can be used to shape priorities for new drugs, techniques and treatments.

The government will now consider the proposals and respond more fully in due course, mindful of the need to ensure affordability.

Association of the British Pharmaceutical Industry

Response to publication of the Accelerated Access Review

24 October 2016

The Association of the British Pharmaceutical Industry (ABPI) has responded to the publication of the Government's Accelerated Access Review (AAR).

First launched by the government in March 2015, the aim of the AAR was to make the UK "the fastest place in the world for the design, development and widespread adoption of medical innovations and stimulate new investment, jobs and economic growth to support the NHS".

The ABPI believes the Review is a significant first step in addressing poor patient access to new medicines in the UK and reflects the Government's manifesto commitment to increase the use of innovative new medicines for NHS patients.

Chief Executive of the ABPI, Mike Thompson, said:

"The Accelerated Access Review is an important foundation for building a Life Sciences Industrial Strategy and opens the door to greater collaboration between innovators, patients and the NHS to make the UK a world leader in researching, developing and using new treatments and technologies.

If we work together to deliver the necessary step-change in getting innovative medicines to British patients we then could see growth in research and development, manufacturing and employment in a post Brexit UK. Turning this into reality now relies on a positive government response to the Review, and a clear implementation plan."

Association of the British Pharmaceutical Industry

Accelerated Access Review

The Accelerated Access Review of Innovative Medicines and Medical Technologies (AAR)

The Accelerated Access Review (AAR) officially launched on 23 March 2015 when its terms of reference and Chair were announced by the Government.

The Review will make recommendations to Government on reforms to accelerate access for NHS patients to innovative medicines and medical technologies. This is the widest ranging review of the environment for the industry since 2009/10 when an incoming government finalised and launched the Life Sciences Strategy and Innovation, Health and Wealth. As its scope covers the whole medicines lifecycle, it touches on all our members' interests.

The Review published its interim report on 27 October 2015. Click here for the full ABPI comment. The AAR will deliver a final report by April 2016.

The ABPI has fully engaged with the Review and its advisers on behalf of our members. We have been working closely with the Review team to ensure that the views of the innovative medicines sector are taken on board.

This has involved, agreeing an industry secondee into the Review team, arranging a series of roundtable meetings between ABPI member companies and the Review workstream leads and champions, one on one meetings with senior members of the Review team and preparing the ABPI submission to the Review: ABPI Submission to the AAR.pdf.

The ABPI has now submitted its final comments to the AAR: ABPI AAR Final Submission 4 Jan 2016.pdf

Alzheimer's Research UK

New report sets out plan to accelerate access to new medicines at 'crucial time' for dementia

24 October 2016

A new report launched today (24 October), commissioned by the government, puts forward a number of recommendations aimed at speeding up the process by which the NHS implements newly developed drugs and medical procedures. The *Accelerated Access Review Final Report* was led by an independent Chair and developed in partnership with the Wellcome Trust.

The report includes dementia as a case study, given the likelihood of new treatments for diseases like Alzheimer's coming through final stage clinical testing in the coming years. The report highlights the work of Alzheimer's Research UK and the Organisation for Economic Co-operation and Development (OECD) in looking for an integrated solution to the drug development challenges in dementia.

The review recommends the creation of an Accelerated Access Partnership to speed up the translation of new treatments from the lab to patients. The report also highlights the need for simple processes for the development and implementation of digital technologies, such as healthcare apps, and suggests that a Strategic Commercial unit be created within NHS England to work with innovators who are developing new medicines.

Hilary Evans, Chief Executive of Alzheimer's Research UK, said:

"The Accelerated Access Review provides an ambitious blueprint for a new era of rapid adoption of innovative treatments. This Review comes at a crucial time for the 850,000 people with dementia, as trials of a number of potential treatments are due to report results soon. If these are shown to be positive then the recommendations in this report, including the creation of a new Strategic Commercial Unit, could help ensure these treatments get to patients more rapidly, while ensuring that appropriate reimbursement to innovators is balanced against good value to the NHS.

"We urge the government to consider these proposals carefully and work with patients, charities and innovators to implement the best of the recommendations. It can often take many years from the point at which a treatment is shown to be effective to actually reaching patients. If we want a truly world class NHS that is a hotbed for drug

development and the highest quality care, then we need to accelerate this process. Alzheimer's Research UK will be working hard to ensure these recommendations will work to speed up access to future dementia treatments."

Association of Medical Research Charities

AMRC's response to the Accelerated Access Review

24 October 2016

The Accelerated Access Review: final report, commissioned by the government and led by Sir Hugh Taylor, was published today. The report can be viewed in full here.

Commenting on the publication of the Accelerated Access Review, Aisling Burnand, CEO, Association of Medical Research Charities said:

"We welcome the publication of the Accelerated Access Review and its recommendations for getting the best technologies for patients more quickly. We know patients and their families want early and fast access to new life-changing and life-enhancing innovations.

"The review offers patients the opportunity to have a say about what innovations are important to them, this is a good outcome for patients. As the beneficiaries of new health technologies, patients have important experiences to share in developing routes to access innovations.

"The recommendations are a step in the right direction in supporting the UK's position as a leader in the life sciences, as we prepare to leave the European Union. We need to continue to work together to ensure the UK remains not only a great place to do research, but also enables patients fast and effective access in the NHS to the best technologies as they emerge."

Muscular Dystrophy UK

Major report on speeding up access to treatments

24 October 2016

A UK Government commissioned report has been published today (24 October) on access to new medicines and drug treatments.

The Accelerated Access Review (AAR) was set up to address how the UK can speed up access to medicines for patients – and ensure the country is not lagging behind other nations in the approval of new treatments.

MDUK took part in the review and you can read our evidence here.

In its 2015 Manifesto, the Conservative Party committed to implementing the findings of the review once they were published.

The report's final recommendations include:

- Streamlining approvals processes and allowing NICE to make more use of flexible approvals – for example, conditional approval
- Setting up an Accelerated Access Partnership which would include NICE, NHS England, NIHR and the Department of Health

- Giving patients more involvement in discussions on which treatments should be developed and prioritised
- A new commercial unit at NHS England to help support pricing discussions with companies and enable flexible funding arrangements
- An Accelerated Access Pathway for drugs which receive a 'transformative designation'
- £20-£30million of funding should be set aside to support drug companies who are making their medicines available on the Early Access to Medicines Scheme. Currently companies have to meet all costs themselves – which could prevent some from applying
- Clearer details on the processes for approving new treatments – and clarity on the timescales involved

What does the review mean for people affected by muscle-wasting conditions?

Accelerating access to effective new medicines is crucial for people affected by muscle-wasting conditions, and research into treatments is moving fast. Translarna for Duchenne muscular dystrophy is an approved drug in the UK. Exondys 51 – also for Duchenne – has recently been approved in the United States. And Nusinersen for spinal muscular atrophy is set to be made available through an expanded access programme.

MDUK welcomes the report and believes that its recommendations can play an important role in accelerating access to emerging treatments.

Ensuring faster assessments at NICE – and flexible approval arrangements – is crucial, as is ensuring that NHS England has the resources to negotiate price and flexible commercial deals at an early stage. We also welcome the recommendation to attach funding to the Early Access to Medicines Scheme – which we have pressed for as part of our Fast Forward campaign on access to new drugs.

Although the Accelerated Access Pathway and 'transformation designation' is an important proposal, it is likely it would apply to a small number of medicines per year. It will be essential to ensure that drugs which are more incremental in their effect – for example, slowing down rather than transforming the progression of a condition – are not held up or disadvantaged.

There are also other important aspects which the report didn't address – such as the need for protected, ring fenced funding for drugs for rare conditions.

However, overall the review has produced an important set of recommendations which if implemented can help speed up patient access to treatments.

If you're passionate about speeding up access to treatments, join MDUK's Fast Forward campaign to push the Government to take action.

> Paul Kelly, whose son, Patrick, has spinal muscular atrophy, says:
>
> As a family, we do all we can to support research into SMA, which could lead to treatments for Patrick's condition. We need

to ensure that should treatments come through, they can be licensed and funded quickly so that they can reach people like Patrick as fast as possible.

Emma Morgan, whose son, Oliver, has Duchenne muscular dystrophy, says:

Oliver is a lively and outgoing character who brings joy to all who meet him. He truly is a special boy.

Our big fear is that if treatments for Duchenne muscular dystrophy do become available, we will face an endless wait for the NHS to actually deliver them. Oliver's condition is affecting him more and more each day. The sooner treatments are licenced the less irreversible damage will be done. We must make sure not a moment is wasted if a therapy is shown to work.

NICE

23 March 2016

NICE consults on draft guidance for new cystic fibrosis treatment

NICE – the National Institute for Health and Care Excellence – has issued draft guidance which does not recommend Orkambi (lumacaftor-ivacaftor marketed by Vertex Pharmaceuticals) for treating cystic fibrosis.

The independent appraisal committee concluded that the cost of Orkambi was considerably higher than the current standard of care and it could not be considered a cost effective use of NHS resources.

Cystic fibrosis is an inherited condition where a gene defect causes a person's lungs and digestive system to become clogged with sticky mucus. This leads to blockages that damage the body's tubes and passages. Symptoms include persistent cough, recurring infections and malnutrition.

There is currently no cure for cystic fibrosis and current treatments aim to manage individual symptoms.

Orkambi is licensed to treat people who have a specific genetic defect known as the F508del mutation. There are around 2,750 people in England with this genotype. It costs £104,000 per patient for every year of treatment. Most people suitable for Orkambi would also still remain on the current standard of care whilst taking it.

The committee acknowledged that Orkambi does offer benefit because it reduces instances where people experience a sudden worsening of symptoms that requires hospitalisation. However, the benefits to lung function – the test to see how people with cystic fibrosis are improving overall – were modest.

Commenting on the draft guidance, **Professor Carole Longson, director of the NICE Centre for Health Technology Evaluation said:** Orkambi is a new treatment option and it is disappointing that we are not able to recommend it. However our independent committee found that when compared to the current standard of care, the benefit it offered was modest and comes at a considerable cost. We have to recognise that the NHS is a finite resource and we can only recommend

treatments for routine funding that are both clinically effective and represent good value for money."

Consultees, healthcare professionals and members of the public, are now able to comment on the preliminary recommendations which are available for public consultation until Friday 15 April 2016. Comments received during this consultation will be considered by the committee at a further appraisal meeting before the next draft guidance is issued.

Until final guidance is issued, NHS organisations should make decisions locally on the funding of specific treatments.

Ends

For more information call the NICE press office on 0300 323 0142 or out of hours on 07775 583 813.

Notes to Editors

Further info

- According to NHS Choices around 1 in every 2,500 babies born in the UK will have cystic fibrosis and there are thought to be more than 9,000 people living with the condition.
- The draft guidance will be available at /guidance/indevelopment/gid-tag530.
- The committee discussed the clinical evidence from the TRAFFIC, TRANSPORT and PROGRESS trials. The acute improvements in ppFEV1 seen with lumacaftor–ivacaftor were modest and unlikely to be clinically significant. The reductions in pulmonary exacerbations seen with lumacaftor–ivacaftor treatment were clinically significant and important for the management of cystic fibrosis.
- The committee considered the company's economic model but concluded that the price reduction applied to lumacaftor–ivacaftor after 12 years was not appropriate. The committee acknowledged that when the company's arbitrary price reduction was removed, the company's base-case ICER increased from £218,000 to £349,000 per QALY gained for lumacaftor–ivacaftor plus standard of care compared with standard of care alone. The committee concluded that, even without including all of its preferred assumptions, the estimated ICERs were considerably higher than what is normally considered a cost-effective use of NHS resources.
- The cost of lumacaftor–ivacaftor is £8,000 per 112-tablet pack (excluding VAT; company's evidence submission). The cost of a 1-year course of treatment is £104,000 (excluding VAT).
- The Scottish Medicines Consortium is due to issue it guidance on this drug in May: https://www.scottishmedicines.org.uk/SMC_Advice/Forthcoming_Submissions/lumacaftor_ivacaftor_Orkambi.

About NICE

The National Institute for Health and Care Excellence (NICE) is the independent body responsible for driving improvement and excellence in the health and social care system. We develop guidance, standards and information on high-quality health and social care. We also advise on ways to promote healthy living and prevent ill health.

Our aim is to help practitioners deliver the best possible care and give people the most effective treatments, which are based on the most up-to-date evidence and provide value for money, in order to reduce inequalities and variation.

Our products and resources are produced for the NHS, local authorities, care providers, charities, and anyone who has a responsibility for commissioning or providing healthcare, public health or social care services.

To find out more about what we do, visit our website:www.nice.org.uk and follow us on Twitter: @NICEComms.

4. Parliamentary material

PQs

Engagements

Asked by: Ben Howlett (Bath) (Con)

Last week's announcement on the report on accelerated access to medicines will have a positive impact on the lives of children and adults with a rare genetic or undiagnosed condition. For decades, patients have struggled to get access to medicines in a timely fashion, and the work of my hon. Friend the Member for Mid Norfolk (George Freeman) made massive progress. Will the Prime Minister confirm that if the programme is successful with the first five to 10 drugs in the first year, it will be extended to further drugs in following years?

Answering member: The Prime Minister | **Department:** Prime Minister

My hon. Friend is right to welcome the accelerated access review and to pay tribute to my hon. Friend the Member for Mid Norfolk (George Freeman), who has done so much to place life sciences in the UK on the agenda and to ensure that the UK develops as the best possible place to develop new drugs, which is exactly what we want to see. The Department of Health will look at the review's recommendations and respond to them shortly. This is an important development in our ability to accelerate access to drugs, which is to the benefit of patients.

HC Deb 02 Nov 2016 | vol 616 c885

Engagements

Asked by: George Freeman (Mid Norfolk) (Con)

Every year, hundreds of people are diagnosed with, suffer and usually die prematurely from rare diseases such as cystic fibrosis and rare cancers, for which there has been no treatment, or for which the latest drugs are prohibitively expensive. This week sees the final report of our accelerated access review, which sets out a new model for the NHS to use its genetic and data leadership to get quicker access and discounted prices. Will the Prime Minister join me in welcoming the review, which is strongly supported by patients, charities and the life sciences sector, and in encouraging the National Institute for Health and Care Excellence and NHS England to implement it speedily?

Answering member: The Prime Minister | **Department:** Prime Minister

I certainly join my hon. Friend in welcoming the publication of the review. This is important in enabling patients to get quicker access to drugs and treatments. The United Kingdom has established a leading role in life sciences, and I pay tribute to my hon. Friend for the role he has played in that. I know that the Department of Health will be looking very closely at the report's specific recommendations, recognising that where we can take opportunities through the national health service to

encourage the development of new drugs to benefit patients, we should do so.

HC Deb 26 Oct 2016 | vol 616 c281

Orkambi

Asked by: Austin, Ian

To ask the Secretary of State for Health, with reference to the NICE press release of 17 June 2016, entitled Cost of cystic fibrosis treatment too high for benefit offered, if his Department will meet with NICE to discuss NICE's decision on the provision on the NHS of Orkambi for the treatment of cystic fibrosis.

Answering member: Nicola Blackwood | Department: Department of Health

There are no plans to meet with the National Institute for Health and Care Excellence (NICE) to discuss its technology appraisal guidance on Orkambi (lumacaftor-ivacaftor) for treating cystic fibrosis homozygous for the F508del mutation.

NICE is the independent body which makes decisions on the clinical and cost effectiveness of products based on thorough assessment of the best available evidence. NICE's guidance is based on a thorough assessment of the available evidence and is developed through wide consultation with stakeholders.

HC Deb 07 Sep 2016 | 44580W

Orkambi

Asked by: Austin, Ian

To ask the Secretary of State for Health, if he will make an assessment of the potential merits of alternative mechanisms to appraise the use of Orkambi in the NHS.

Answering member: Nicola Blackwood | Department: Department of Health

The National Institute for Health and Care Excellence (NICE) published final technology appraisal guidance on 27 July 2016 which does not recommend Orkambi (lumacaftor-ivacaftor) for treating cystic fibrosis homozygous for the F508del mutation.

In the absence of positive NICE technology appraisal guidance, any funding decisions should be made by National Health Service commissioners, based on an assessment of the available evidence and on the basis of an individual patient's clinical circumstances.

HC Deb 07 Sep 2016 | 44520W

Medical Treatments

Asked by: Zeichner, Daniel

To ask the Secretary of State for Business, Innovation and Skills, what engagement his Department has had with the Accelerated Access Review (AAR); and whether the AAR plans to investigate expediting the delivery of new drugs and devices to tackle MRSA and antimicrobial resistance.

Answering member: George Freeman | Department: Department for Business, Innovation and Skills

The Accelerated Access Review team is a joint unit between the Department of Health and the Department for Business, Innovation and Skills. The Accelerated Access Review is intended to reform the landscape for the assessment, adoption and reimbursement of innovative drugs, devices and other treatments across all therapeutic areas.

HC Deb 08 Jul 2016 | 41572W

NHS: Drugs

Asked by: Cunningham, Mr Jim

To ask the Secretary of State for Health, what the most expensive drugs bought from pharmaceutical companies by the NHS were in the last five years; and if he will make a statement.

Answering member: Alistair Burt | Department: Department of Health

Based on National Health Service list prices at launch notified to the Department between 2009 and 2015 under the rules of the Pharmaceutical Price Regulation Scheme and statutory regulations, the 10 most expensive medicines in terms of list price per pack are shown in the table.

In considering the impact of a medicine, it is important to take into account both the product's total net costs and the net health benefits it generates.

Product	Manufacturer
Strensiq (asfotase alfa) 80mg injection	Alexion
Translarna (ataluren) granules for oral suspension	PTC Therapeutics Ltd
Sirturo (bedaquiline fumarate) tablets	Janssen-Cilag Ltd
Lojuxta (lomitapide) capsules	Aegerion Pharmaceuticals
Provenge (sipuleucel-T) dispersion for infusion	Dendreon Coporation

ChondroCelect (characterised viable autologous cartilage cells expanded exvivo) implantation suspension	Swedish Orphan Biovitrum (SOBI)
Yervoy (ipilimumab) vials	Bristol-Myers Squibb UK
Revestive (teduglutide) injection	NPS Pharma International
Kalydeco (ivacaftor) tablets	Vertex Pharmaceuticals
Kalydeco (ivacaftor) oral granules	Vertex Pharmaceuticals

Source: Department of Health

HC Deb 13 Jun 2016 | 40024W

Orkambi

Asked by: Godsiff, Mr Roger

To ask the Secretary of State for Health, if his Department will meet with NICE, NHS England and Vertex to discuss the provision on the NHS of the drug Orkambi to treat cystic fibrosis.

Answering member: George Freeman | Department: Department of Health

The National Institute for Health and Care Excellence (NICE) is currently consulting on its draft technology appraisal guidance on the use of Orkambi (lumacaftor in combination with ivacaftor) for the treatment of cystic fibrosis in people who are homozygous for the F508del mutation. Stakeholders, including the Department, NHS England and the manufacturer, Vertex Pharmaceuticals, now have an opportunity to comment on this draft guidance.

Departmental officials remain ready to discuss any proposals from the company that could enable National Health Service patients to access the drug at a cost-effective price.

NICE expects to publish its final guidance in July 2016.

HC Deb 12 Apr 2016 | 32606W

NHS: Technology

Asked by: Anderson, Mr David

To ask the Secretary of State for Health, what steps he plans to take to increase the availability and uptake of innovative technology available to patients on the NHS; and if he will make a statement.

Answering member: George Freeman | Department: Department of Health

The Government is actively supporting a number of initiatives to accelerate access to innovative treatments. These include the Early Access to Medicines Schemes which supports access in the United Kingdom to unlicensed or off-label medicines and represents a significant advance in treatment in areas of unmet medical need and the Accelerated Access Review, which aims to speed up access to innovative drugs, devices, diagnostics and digital products for National Health Service patients, and to make the UK the best place to develop these innovations.

NHS England supports the invention and adoption of transformative healthcare technologies. This includes existing technologies, where the benefits are already proven but wider adoption is critical to benefit all patients, and new technologies, which require larger scale trials to test out their impact individually and in combination. Current initiatives include the sponsorship of 15 Academic Health Science Networks (£48 million core funding in 2015-16), the Small Business Research Initiative (£20 million in 2015-16), and the Test Bed programme which is providing funding for frontline health and care workers to evaluate the use of novel combinations of interconnected devices such as wearable monitors, data analysis and new ways of working.

NHS England has been tasked under section 7.1 of the NHS Mandate to "Implement the agreed recommendations of the Accelerated Access Review including developing ambition and trajectory on NHS uptake of affordable and cost-effective new innovations".

HC Deb 23 Mar 2016 | 31061W

NHS: Drugs

Asked by: Madders, Justin

To ask the Secretary of State for Health, what assessment his Department has made of the potential effect of the Access to Medical Treatments (Innovation) Bill on reducing the length of time it takes to bring a new drug to market.

Answering member: George Freeman | Department: Department of Health

The purpose of the Access to Medical Treatments (Innovation) Bill is to promote access to innovative medical treatments. It is not specifically designed to reduce the length of time it takes to bring a new drug to market. To which end work is being done in parallel, notably through the Accelerated Access Review, which explores options to speed up access to innovative drugs, devices and diagnostics for National Health Service patients. Sir Hugh Taylor, the independent chair of the review will make his recommendations in the spring.

HC Deb 28 Jan 2016 | 23994W

NHS: Drugs

Asked by: Dakin, Nic

To ask the Secretary of State for Health, what plans his Department has to hold NHS bodies accountable to the recommendations of the Accelerated Access Review.

Answering member: George Freeman | Department: Department of Health

The Accelerated Access Review, which was set up to look at speeding up access to innovative drugs including those for rare diseases, devices and diagnostics for National Health Service patients, has been making strong progress since it was first announced in November 2014.

The review is independently chaired by Sir Hugh Taylor and has been engaging stakeholders extensively since the summer to gather evidence on the big questions the review is considering, and has developed a number of emerging solutions.

Sir Hugh published his interim report of the review on 27 October 2015. The report is high-level, direction-setting and grounded in evidence gathered through the review's stakeholder engagement to date. Included in the report were proposals for a National Innovation Partnership of key bodies in the innovation pathway who would be party to a Concordat committing them to the ambitions set out in the review. The review has used a second phase of engagement to work closely with industry stakeholders, Government, the NHS and arm's length bodies on these and other proposals set out in the interim report.

In April 2016 the review will conclude with Sir Hugh making his final recommendations to Government. The Department will review these recommendations and consider how best to respond.

HC Deb 28 Jan 2016 | 23928W

Prescription Drugs

Asked by: Ms Margaret Ritchie

To ask the Secretary of State for Health, what assessment he has made of the effectiveness of the processes undertaken by the NICE for approving and commissioning new medicines for cancers and ultra rare diseases; and if he will make a statement.

Answering member: George Freeman | Department: Department of Health

No such assessment has been made.

The National Institute for Health and Care Excellence (NICE) is the independent body that makes recommendations to the National Health Service on the use of selected drugs and treatments through its technology appraisal and highly specialised technologies programmes. NICE does not commission new medicines and treatments.

NICE is responsible for its own processes and methodology, which it periodically reviews in consultation with stakeholders. Further information on NICE's processes is available at:

www.nice.org.uk.

The Accelerated Access Review, chaired by Sir Hugh Taylor, is looking at speeding up access to innovative medicines and technologies for patients. NICE has been working closely with the review as it develops its recommendations which are due to be published in Spring 2016.

HC Deb15 Jan 2016 | 21207W

NHS: Drugs

Asked by: Sharma, Mr Virendra

To ask the Secretary of State for Health, pursuant to the Answer of 23 November 2015 to Question 16399, what discussions he has had with the Accelerated Access Review team on biosimilars medicines; and whether he has been given assurance that the final report will make specific recommendations on their use.

Answering member: George Freeman | Department: Department of Health

In accordance with the terms of reference the Accelerated Access Review is not excluding any particular type or class of medicine, but is creating a framework through which to accelerate access for National Health Service patients to innovative health technologies including biosimilar medicines where appropriate.

HC Deb 18 Dec 2015 | 20066W

Written Ministerial Statements

Accelerated Access Review

This morning Sir Hugh Taylor has published the final report of the Accelerated Access Review (AAR). The AAR was tasked with making recommendations to government on reforms to accelerate access for NHS patients to innovative medicines, medical technologies, diagnostics and digital products. The report sets out a framework of recommendations to streamline and accelerate the pathway for new products from development to their use with patients and to enable widespread adoption across the NHS. A copy of the report is attached.

The Government welcomes Sir Hugh's final report and are grateful to him, Sir John Bell, the External Champions and the External Advisory Group for their excellent work, which draws upon contributions from many individuals and organisations from patient groups, the NHS, industry, academia and clinicians. We are grateful for the important

input that this review has had from NHS England NICE, the MHRA and NHS Improvement.

The report provides us with a strong basis to make the right decisions about how the health system can be adapted to meet the challenges of the future, attract inward investment, grow our thriving life sciences industry and use innovation to improve patient outcomes in the context of the financial pressures on the NHS. It will be important to implement this report in a way that is affordable for the NHS. The Government will now consider the proposals in detail with our partners and will provide a fuller response in due course.

The Government remains strongly committed to the life sciences and to building a long-term partnership with industry. It is determined to help the UK become the best place in the world to produce new drugs and products that can transform the health of patients, where the research, development, regulatory, commercialisation and investment infrastructure enable innovation to flourish and thrive while improving patient's lives.

HC Deb 24 Oct 2016 | WMS209

Debates

Backbench Business debate in Westminster Hall on Cystic Fibrosis
HC Deb 8 Dec 2015 | vol 603 cc255-279WH

EDM

CYSTIC FIBROSIS TRUST - ACCESS TO ORKAMBI

That this House notes that the National Institute for Health and Care Excellence (NICE) has found Orkambi to be effective and important for managing cystic fibrosis; regrets that uncertainty around its long-term effect and its high cost means NICE cannot yet recommend the drug to the NHS; commends the initiative of the Cystic Fibrosis Trust, consistent with the Government's own Accelerated Access Review, to accelerate access to the drug while informing assessment of longer-term effects through use of its UK Cystic Fibrosis Data Registry; and urges cooperation between the NHS and drug manufacturer Vertex to ensure that the 3,000 patients who could see huge health benefits from Orkambi are able to do so.

11 Apr 2016 | EDM 1330 (session 2015-16)

Primary sponsor: McDonald, Stuart

5. Useful links and further reading

Accelerated Access Review: Final Report – *Review of innovative medicines and technologies*

https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/565072/AAR_final.pdf

ABPI final comments on the Accelerated Access Review, January 2016

http://www.abpi.org.uk/our-work/value-access/Documents/ABPI%20AAR%20Final%20Submission%204%20Jan%202016.pdf

NHS England: Clinical Commissioning Policy: Ivaftor for Cystic Fibrosis

https://www.england.nhs.uk/commissioning/wp-content/uploads/sites/12/2015/10/a01pc-ivacftr-cystic-fibrosis.pdf

National Institute for Health and Care Excellence (NICE): Final appraisal determination: Lumacaftor–ivacaftor for treating cystic fibrosis homozygous for the F508del mutation, published June 2016

https://www.nice.org.uk/guidance/TA398/documents/final-appraisal-determination-document

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