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DEBATE PACK

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Access to medicines for people with cystic fibrosis and other rare diseases

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Contents

Westminster Hall debate, Tuesday 8 December 2015 at 9.30am

This debate pack has been compiled ahead of the debate on Access to medicines for people with cystic fibrosis and other rare diseases, to be held on Tuesday 8 December 2015 at 9.30am in Westminster Hall. The Member in charge of the debate is Ian Austin.

The Cystic Fibrosis Trust's "Stopping the Clock campaign" aims to ensure that as many people as possible will have access to "precision medicines", which treat the underlying genetic causes of cystic fibrosis, not just the symptoms.

Prior to the Westminster Hall debate, the Cystic Fibrosis Trust and Parliament's Digital Outreach Service have organised a digital debate on Twitter. The debate starts at 5pm on Monday 7 December and you can take part in this discussion using the hashtag #CFdebate.

The idea for digital debates linked to debates in Westminster Hall came from the Speaker's Commission on Digital Democracy.

1. Background

Cystic Fibrosis

Cystic fibrosis is a genetic condition which results in an increase of mucus in the body. It is caused by a genetic mutation in the CFTR gene. This gene normally works to control the levels of salts in the cells but when this is not working, it results in a build-up of thick mucus.

Mucus commonly builds up in the lungs of patients with cystic fibrosis, leading to inflammation and recurrent infections. Shortness of breath, cough and wheezing are common symptoms. The pancreas is also commonly affected in cystic fibrosis. This can affect its ability to produce the enzymes necessary to break down food and can lead to malnutrition. It can also impact on the production of insulin and lead to diabetes. Other organs that can be affected include sinuses, bones and joints, the reproductive system and bladder.[1]

It is estimated that 1 in 2,500 babies will be born in the UK with cystic fibrosis and there are more than 9,000 living with this condition in the country. It most commonly affects white people of northern European descent and is much less common in other ethnic groups.

Babies are screened for cystic fibrosis at birth using a heel prick test as part of the NHS newborn screening programme.

Treatment for cystic fibrosis is not curative but seeks to manage symptoms. Medications including steroids, antibiotics, insulin and bronchodilator inhalers are often used. Nutritional advice and physiotherapy for airway clearance are commonly part of management. Cystic fibrosis patients may also be suitable for lung transplants. NICE provide a number of guidelines on specific treatments for cystic fibrosis and they are currently updating their cystic fibrosis guidelines. This is due to be published in 2017.

Prognosis is variable but much improved in recent years. Most people with cystic fibrosis will still have a shorter than average life expectancy. The cystic fibrosis trust reports that half of all patients with cystic fibrosis will live longer than 41 years.[2] It is now estimated that, on average, children born in the 21st century with cystic fibrosis will live for more than 50 years.

Rare diseases

A rare disease is generally considered as one affecting fewer than 5 people in 10,000, ultra-rare diseases have been described as affecting 1 person in 10,000 or fewer.[3] However, overall about 3 million people in the UK will be affected by one of these conditions. The UK Rare Diseases Strategy, published in November 2013, and to be implemented across the UK, seeks to address some of the specific challenges relating

1 NHS Choices, Cystic fibrosis
2 Cystic Fibrosis Trust, Is there a cure for cystic fibrosis?
3 European Commission, Rare diseases

to these conditions, such as improving diagnosis, and investing in research for these conditions.

A significant proportion (around 80%) of these conditions have a known genetic cause. Due to the fact that relatively small numbers of people are affected with a particular rare disease, there are specific challenges in ensuring speedy diagnosis and access to appropriate services and treatments.

Drugs for rare diseases (orphan medicines) are usually granted marketing authorisations through a centralised procedure at the European Medicines Agency (EMA) and services for rare conditions are the direct commissioning responsibility of NHS England in England.

In order to allow access to drugs to meet an unmet demand and in the interest of public health, where less than complete data is provided on a product, a conditional marketing authorisation may be granted by the EMA.[4] The EMA also provide incentives to encourage the development of these medicines, these include fee reductions and ten year marketing exclusivity from the EMA.

The UK Government has launched the Early access to medicine scheme in 2014. This aims to allow access to medicines that do not yet have a full marketing authorisation for patients with severely debilitating or life threatening conditions, where there is a clear unmet medical need. The Medicines and Healthcare Products Regulatory Agency is responsible for the scheme and provides a scientific opinion on the benefit/risk balance of the medicine.[5]

In November 2014, a new Innovative Medicines review to consider how access to new medicines can be accelerated was also announced by the UK Government.[6] The Accelerated Access Review (AAR) will focus on new types of products such as medicines based on a stratified approach, new diagnostics, and digital health technologies.[7] The AAR published an interim report in October 2015.

Further information can be found in the Library Debate Pack Briefing Access to drugs for ultra-rare diseases (CDP0011, 15 June 2015)

4 EMA, Presubmission guidance, FAQs

5 HC Deb 28 November 2014 | PQ 215820

6 GOV.uk, *News story: Major investment in life sciences*, November 2014

7 Gov.uk website on Accelerated Access Review

2. Press releases

Cystic Fibrosis Trust

Stopping the Clock

We are campaigning to ensure that as many people with CF as possible will have access to precision medicines by 2020.

Precision medicines treat the underlying cause of the condition, not just the symptoms, meaning a future where everyone with CF can live a life unlimited is more achievable than ever.

We are determined to ensure people with CF have access to transformational therapies that can change lives.

There are challenges ahead: precision medicines are expensive and it's difficult to predict the cost-effectiveness of new treatments. The Government is currently considering how we can speed up access to innovative treatments, including proposals to provisionally approve new drugs while using real-world data to assess the benefits.

The UK CF Registry captures data that demonstrates the real-world impact of CF care; we believe this makes CF a unique testing ground for assessing the value of innovative medicines.

We look forward to engaging closely with the NHS, pharmaceutical companies, clinicians and – of course – people who are affected by cystic fibrosis, over the next few months to explore how this will work.

We want to hear the views of people with cystic fibrosis and promote their voice throughout the process. Our upcoming Digital Parliamentary Debate is the next opportunity to add your voice to the campaign and share your experiences with the people in power.

Join the debate

We have teamed up with Parliament to hold a Digital Parliamentary Debate, focussing on accelerating access to precision medicine for people with cystic fibrosis.

You can directly share your experiences and opinions with the Ian Austin MP, who will be raising the parliamentary debate, in a digital discussion on Twitter just before the debate itself. We will also be encouraging people to ask their MP to take part in the debate.

He will use these powerful stories in the debate to try and persuade the government to help people with cystic fibrosis. The debate will be broadcast live and we will be live tweeting the proceedings.

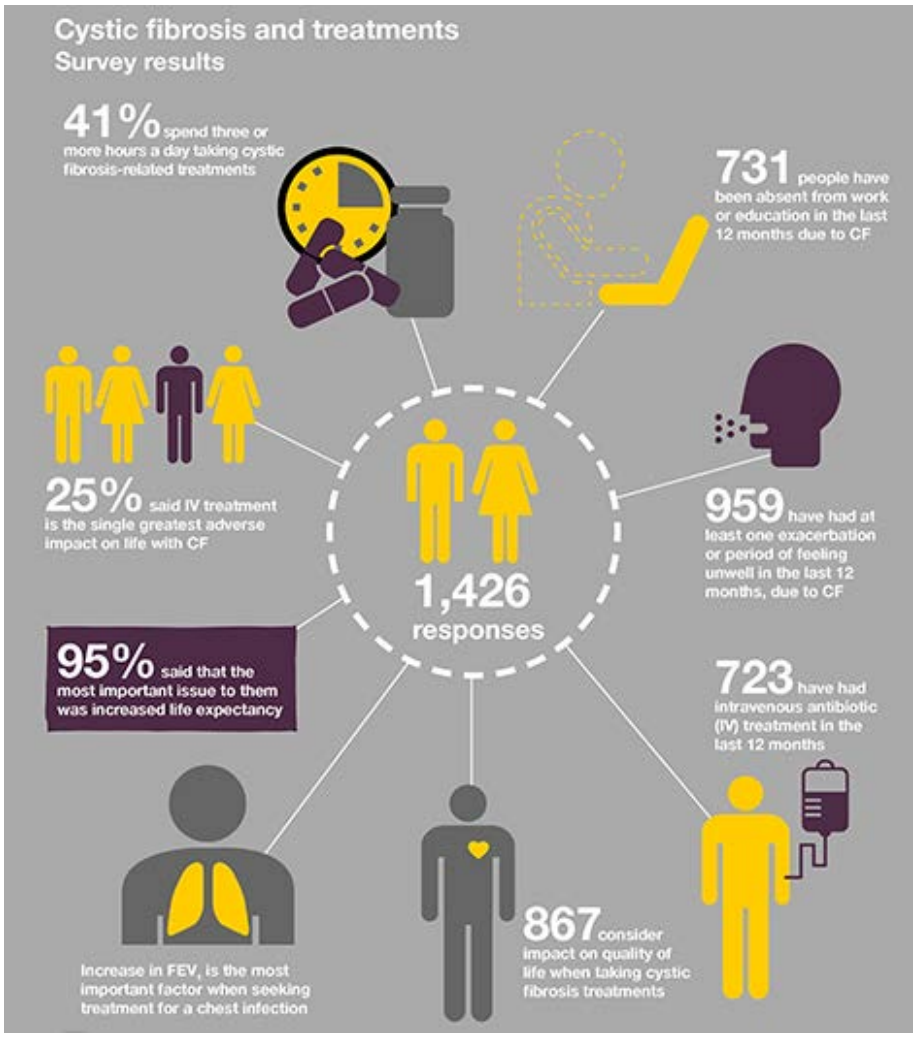
What next?

Register your interest or find out more by emailing PublicAffairsTeam@cysticfibrosis.org.uk. The date of the debate is subject to Parliamentary business; we'll confirm soon and share some simple guidelines to help you make the most of the opportunity. Easy!

The community raises its voice!

We asked people with cystic fibrosis what precision medicines mean to them and received nearly 1,500 responses! This feedback will be used as evidence for the NHS consultation on Orkambi, a promising new precision medicine, and for future treatments too, giving a voice to those who matter most: the people who live with cystic fibrosis.

A snapshot of the key information from the survey is below. More news on Stopping the Clock, including the Digital Debate and the Orkambi consultation will be coming soon.



Join the call for precision medicine

13 October 2015

Use your voice to support access to transformational treatments.

The Cystic Fibrosis Trust is committed to ensuring people with cystic fibrosis have access to therapies that can improve and transform their lives.

Fill in the survey **and help people with cystic fibrosis get access to the most effective medicines, sooner.**

The latest therapy recommended for approval by European regulators is Orkambi, a combination therapy that is one of many potential 'precision medicines', which target particular CF mutations. Orkambi seeks to correct the basic underlying genetic defect in 50% of people with CF in the UK.

The decision as to whether the therapy can legally be made available in the UK will soon be made, and if it can the NHS will then have to decide whether or not to pay for it in each of the four regions of the UK.

To help it make this decision, NHS England have asked a body called NICE to assess Orkambi both in terms of its clinical effectiveness and, importantly given Orkambi's likely high price, its cost effectiveness.

NICE will start the appraisal of Orkambi in February 2016, and have asked the Cystic Fibrosis Trust to submit evidence to them on the issue.

While the Trust has concerns about the NICE process for appraising drugs of this sort, it is vital that the voice of the CF community is heard loud and clear throughout, so please fill in the survey to have your say today.

This process and the arrival of similar innovative therapies in the future will challenge the NHS to look at what 'value for money' really means in the context of CF care. The whole community must speak up and make sure these decisions are guided by the unique knowledge and experience of people living with CF.

If you would like to speak to the Trust directly about supporting this campaign with your story, please contact pressoffice@cysticfibrosis.org.uk.

3. Parliamentary questions and debates

PQs

Cystic Fibrosis

Asked by: Green, Kate

To ask the Secretary of State for Health, if he will make an assessment of the implications for the NHS of the paper by Stephenson et al, A contemporary survival analysis of individuals with cystic fibrosis, published in the European Respiratory Journal, Volume 45, March 2015; and what steps he is taking to ensure that the NHS is adequately resourced to cope with an increase in cystic fibrosis patients over the next 10 years.

Answering member: Jane Ellison | Department of Health

Since 1 April NHS England has been responsible for securing high quality outcomes for patients with cystic fibrosis (CF) as part of its remit to deliver specialised services. The CF service specifications (one for adults and one for children) NHS England has published sets out what providers must have in place in offer high quality CF care and support equity of access to services for patients wherever they live. Both specifications can be viewed at the following link:

www.england.nhs.uk/commissioning/spec-services/npc-crg/group-a/a01/.

NHS England's CF Clinical Reference Group, which advises on the development of services for patents, keeps relevant published literature under review. Moreover, it is recognised that the number of adults living with CF is gradually increasing over time, because of improvements in diagnosis and treatment. The growth in numbers is taken into account by NHS England as part of its annual commissioning process and will be considered in the ongoing development of its five year specialised commissioning strategy.

26 Mar 2015 | Written questions | 228708

Cystic Fibrosis

Asked by: Jarvis, Dan | **Party:** Labour Party

To ask the Secretary of State for Health, what steps the Government is taking to support research into new treatments for people with cystic fibrosis.

Answering member: George Freeman | **Party:** Conservative Party | **Department:** Department of Health

The National Institute for Health Research (NIHR) Evaluation, Trials and Studies Coordinating Centre manages the Efficacy and Mechanism Evaluation programme. With funding from the Medical Research

Council, this programme is currently supporting a £3.3 million trial of repeated application of gene therapy in patients with cystic fibrosis. The report of this trial is expected to be published in August 2015.

A range of commercial and researcher-led cystic fibrosis treatment trials and studies are hosted by NIHR research infrastructure including the NIHR Clinical Research Network, NIHR biomedical research centres and units, and NIHR clinical research facilities for experimental medicine.

27 Oct 2014 | Written questions | Answered | House of Commons | 211584

Cystic Fibrosis

Asked by: Jason McCartney

To ask the Secretary of State for Health (1) what steps he is taking to ensure that high-cost drugs are delivered to cystic fibrosis patients on time;

(2) what recent assessment he has made of the effectiveness of the commissioning of homecare services by the NHS.

Answering member: Dr Poulter | Department of Health

It is important that patients, including those with cystic fibrosis, get those high cost drugs provided through homecare services on time.

The Department commissioned a review of homecare medicine supply arrangements to ensure they deliver the best value for patients, the National Health Service and the provider market. The review report, Homecare Medicines: Towards a Vision for the Future, was published in December 2011 and is available at:

http://media.dh.gov.uk/network/121/files/2011/12/111201-Homecare-Medicines-Towards-a-Vision-for-the-Future2.pdf

The outcome of subsequent improvement work is summarised in the further report, Homecare Medicines: Towards a Vision for the Future—Taking Forward the Recommendations, published in May 2014 and available at:

www.uhns.nhs.uk/AboutUs/NHSHomecareMedicinesinEngland.aspx

NHS England issued a patient safety alert on minimising the risks of omitted and delayed medicines for patients receiving homecare services on 10 April 2014. This recommended that all health care organisations that commission clinical homecare services:

establish if medicine homecare services were used by their organisation and if incidents of omitted and delayed medicines had occurred;

consider whether immediate action needed to be taken locally and, if required, develop an action plan, to reduce risk and the potential risk to patients;

disseminate the alert to all medical, nursing, pharmacy and other staff involved in the care of homecare patients; and

report patient safety incidents concerning homecare to the National Reporting and Learning Service.

A copy of the patient safety alert is available at:

www.england.nhs.uk/wp-content/uploads/2014/04/psa-omitted-delayed-meds.pdf

The Department continues to work with NHS England, homecare companies, pharmaceutical suppliers and the NHS to ensure that homecare arrangements are safe and deliver value for the NHS and improved outcomes for patients.

16 Jun 2014 | Written questions | 582 cc489-490W

Debates

Drugs: Ultra-rare Diseases

HC Deb16 Jun 2015 | 597 cc29-55WH

Cystic Fibrosis

HC Deb 10 Apr 2014 | 579 cc501-514

4. Further reading

Library Debate Pack Briefing Access to drugs for ultra-rare diseases (CDP0011, 15 June 2015)

Department of Health, The UK Strategy for Rare Diseases, November 2013

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