

ALL PARTY PARLIAMENTARY GROUP ON RARE, GENETIC AND UNDIAGNOSED CONDITIONS



Action for Access Report Launch

Date: 21 October 2019

Venue: Churchill Room, House of Commons

Parliamentarians

Catherine West MP (Chair)

Jim Shannon MP

Baroness Redfern

Baroness Neville-Jones

Guest Speakers

Nick Meade, Genetic Alliance UK

David Ramsden, Cystic Fibrosis Trust

Kate Learoyd, National Society for Phenylketonuria

Dr Chris Kingswood, Consultant Nephrologist and Head of Research Strategy, Tuberous Sclerosis Association

1. Welcome from Catherine West MP

2. Introduction by Nick Meade, Director of Policy, Genetic Alliance UK

Nick Meade outlined Genetic Alliance UK's Action for Access report, which was officially launched at this All Party Parliamentary Group and lays out recommendations for moving forward the dialogue on access to rare disease medicines.

The Action for Access report is a result of the work undertaken following the 2017 meeting of the All Party Parliamentary Group for Rare, Genetic and Undiagnosed Conditions that discussed access to rare disease medicines. It has been written and produced by Genetic Alliance UK following contributions from a wide range of stakeholders, including many attendees of this meeting.

Around 3.5 million people in the UK will be affected by a rare disease at some point in their lives. There are licensed medicinal products for a small minority of rare diseases. Despite this, it is incredibly difficult for patients to access potentially life-changing treatments.

In the UK, health technology assessment (HTA) decides whether or not medicines will be made available on the NHS. But the path between licence and HTA decision is governed by a system that is difficult to navigate.

Genetic Alliance UK

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Genetic Alliance UK's Action for Access report outlined five systemic problems that restrict access to rare disease treatments: fragmentation, inflexibility, challenges with capturing the value of treatments, delays and a lack of transparency. One of the report's key conclusions is that behind these systemic problems are two fundamental problems: uncertainty and money.

To address the uncertainty problem, Genetic Alliance UK recommends that the UK make rare disease medicines available in the NHS as soon as they receive marketing authorisation through a period of managed access. Once introduced, evidence should be gathered from within the NHS in relation to their value for an eventual HTA. This should be on terms set as the period of evidence gathering begins. The funds to cover the costs of these medicines should be provided through flexible schemes arranged between government and industry.

To address the systemic problems, Genetic Alliance UK recommends that NICE develop a single flexible approach for making decisions about access to rare disease medicines, ensuring all rare disease medicines are assessed by an appropriate pathway with realistic expectations for evidence and outcomes.

To address the money problem, Genetic Alliance UK recommends both industry and government clearly explain the process for setting rare disease medicine prices, and how the price that the government can afford is set. The benefits of the recently agreed Voluntary Pricing and Access Scheme for Branded Medicines to all stakeholders should be set out. This dialogue should guide the rare disease patient community and the public to a greater understanding of reference pricing and the voluntary pricing access scheme for branded medicines.

3. Guest Speaker: David Ramsden, Chief Executive of the Cystic Fibrosis Trust

Over 10,400 people in the UK have cystic fibrosis, representing 12% of the world's cystic fibrosis sufferers. The median age of death for sufferers in the UK is 32.

David Ramsden provided insight into the struggle that patients and families affected by cystic fibrosis have encountered in attempting to access Orkambi, a medicine that can slow the decline in lung function – the leading cause of death amongst patients affected by cystic fibrosis – by 42%. Though NICE recognised Orkambi as an 'important treatment' in 2016, it did not recommend the NHS fund the drug on the grounds of cost effectiveness and lack of long term data.

There have been Parliamentary debates, Parliamentary questions, and two petitions that have each attracted over 100,000 signatures calling for the British Government to call for a resolution between NHS England, NICE and Vertex Pharmaceuticals – the manufacturer of Orkambi. David Ramsden explained the campaign for access to Orkambi relies on the tenacity of those affected by cystic fibrosis and that many deaths have occurred while the medicine has not been available.

The Scottish Government reached an agreement with Vertex Pharmaceuticals on 12 September 2019 to provide Orkambi to patients in Scotland affected by cystic fibrosis.

4. Guest Speaker: Kate Learoyd, Campaign Manager, the National Society for Phenylketonuria

Phenylketonuria affects 1 in 12,000 people in the UK. It was stated that phenylketonuria is a rare condition that can result in brain and nervous system damage, which, if left untreated, can lead to learning difficulties and seizures. Phenylketonuria is diagnosed by new-born screening.

Kate Learoyd provided insight into the struggle that patients and families affected by phenylketonuria have encountered in attempting to access Kuvan. Though Kuvan was licensed in 2008, 11 years later it has not been funded by the NHS. This means that no patients in the UK affected by phenylketonuria have access to Kuvan.

The alternative to Kuvan is a strict low-protein diet that harms the quality of life of patients affected by phenylketonuria.

5. Guest Speaker: Dr Chris Kingswood, Consultant Nephrologist and Head of Research Strategy, Tuberous Sclerosis Association

Tuberous sclerosis affects over 8,000 people in the UK. Tuberous sclerosis can affect patients at various rates of severity, but most patients affected by tuberous sclerosis in the UK are on the severe side of the spectrum. The leading cause of death amongst children who suffer from tuberous sclerosis is brain tumours.

Dr Kingswood provided insight into the struggle that patients and families affected by tuberous sclerosis have encountered in attempting to access Everolimus. Though Everolimus became licensed in 2001, it was only routinely funded for tuberous sclerosis patients with kidney tumours in 2016 and for tuberous sclerosis patients with brain tumours in 2017. Everolimus became available to tuberous sclerosis patients in other countries immediately after it became licensed.

Dr Kingswood highlighted that fragmentation and cost effectiveness are significant obstacles for the funding of rare disease medicines in the UK.

6. Discussion

A mother of two children affected by Batten disease, described the struggle that the Batten disease community have endured since Brineura was licensed in May 2017. It was stated the current system is unfit for purpose and highlighted that two children in England affected by Batten disease have died waiting to access Brineura.

A mother of a child affected by phenylketonuria described that her daughter has been tested and is responsive to Kuvan, but the medicine is not funded on the NHS. It was noted that other European countries fund Kuvan, but the UK does not.

David Ramsden responded by noting that the process for accessing drugs for rare diseases is harmful to patients and their families. David Ramsden highlighted that the process for accessing

drugs for rare diseases often involves a lack of transparency from the NHS and pharmaceutical companies, leaving patients and families isolated.

An attendee from industry stated that Genetic Alliance UK should be commended for the Action for Access report. It was suggested that campaigners review NICE methods every two years, as delays in funding medicines are not acceptable to patients.

A campaigner from the UK Mastocytosis Support Group noted that a medicine for patients affected by mastocytosis is provided by the manufacturer to patients who participated in trials, but not funded by the NHS for new patients. It was also noted that there was a lack of transparency regarding the reasoning as to why a mastocytosis drug was assessed via an inappropriate Single Technology Appraisal (STA) route.

An attendee from industry noted that despite world class research, the access problem means that the UK may not be selected as a clinical trial site for research into new treatments. It was also suggested that investment in diagnosis could be used as a lever to fund medicines and access.

David Ramsden responded by noting that the transparency of the Action for Access report makes it easier to map the process for rare disease medicines to gain NHS funding and allows pharmaceutical companies to be held to account.

Kate Learoyd suggested that it is unethical that pharmaceutical companies are allowed to test medicines here but the UK Government fails to fund medicines.

Dr Kingswood responded by highlighting that collectively rare diseases are not that rare, stating that one in 17 people in the UK will be affected by a rare disease at some point in their lives.

An academic what barriers exist to managed access and what impact that devolved departments of health will have on the recommendations from Action for Access.

A campaigner from the MPS Society questioned if NICE is capable of evaluating rare disease medicines or whether a completely new system is required.

Kate Learoyd responded by expressing disappointment at the lack of ambition in the NICE review.

Dr Kingswood offered that NICE can be fit for purpose but is constrained by lack of resources.

7. Closing Remarks

Catherine West MP thanked speakers and attendees for their contributions. Catherine West closed by recommending patients and their families continue to communicate with their MPs, either in writing or meeting with them, and explain the processes and the struggles of accessing rare disease medicines to them to collectively move the dialogue forward.