All Party Parliamentary Group on Rare, Genetic and Undiagnosed Conditions

Access to rare disease medicines

28 April 2021 via Zoom — the recording of the event can be found here.

Parliamentarians in attendance:

Liz Twist MP (Chair)	Andrew Lewer MP	Marie Rimmer MP
Nickie Aiken MP	Lord Bethell	Baroness Pauline Neville Jones
Jeremy Hunt MP	Lord Lucas	Tom Randall MP
Jim Shannon MP	Baroness Masham of Ilton	Bambos Charalambous MP
Christina Rees MP	Tim Farron MP	

Minutes

Liz Twist MP, Chair, APPG on Rare, Genetic and Undiagnosed Conditions
Liz Twist MP welcomes attendees to the meeting and pays her respects to the late Dame Cheryl Gillan MP.

The NICE methods and process reviews, the upcoming Innovative Medicines Fund (IMF) and the new Innovative Licensing Access Pathway (ILAP) provide opportunities for positive change. However, there is a concern that unintended consequences may arise if these systems are not viewed holistically and developed to align with each other.

Nick Meade, Interim Chief Executive and Director of Policy, Genetic Alliance UK
Nick made a declaration of interest on behalf of Genetic Alliance UK, the APPG's secretariat. The charity receives funding from a wide range of sources, including the pharmaceutical industry and Genetic Alliance UK's ethical collaboration policy and working with industry policy which ensure the organisation is independent from its funders are available online.

Kye Gbangbola, Sickle Cell Society
Sickle cell is a condition that affects the red blood cells and is more common in people with an African or Caribbean family background. People experience severe pain events and life expectancy is often limited. Access to mental health support is an issue that should also be included in the access to medicines debate. Access to medicines affect the sickle cell community in four ways:

- People ration their medicines due to a lack of support to afford prescriptions.
- There has been no new treatment for sickle cell in the UK for 30 years, however there is a treatment currently being reviewed by NICE after an interim negative decision, but in the meantime, people are still struggling with their condition.
- People living with sickle cell conditions are battling to receive pain medication from healthcare professionals due to stigma and a lack of awareness.
- Covid-19 has highlighted health inequality issues as clinically extremely vulnerable people have struggled to receive their medicines or transfusions.

Kate Learoyd, National Society for Phenylketonuria
Phenylketonuria (PKU) is a metabolic condition that affects the brain via a build-up of an amino acid called phenylalanine. This amino acid is found in all proteins and therefore in most foods. For many years the only treatment available was a very restrictive diet until Kuvan was licensed for treating PKU in 2008. Kuvan has been available in almost all European countries for the majority of the last 13 years apart from the UK, where earlier this year, NICE approved its use in children only. Currently it is still not available for adults in the UK. There are ongoing gene therapy clinical trials for treating PKU in the UK, however, given recent experiences, it is difficult to see that this research will benefit people in the UK as it is unlikely to be available in the UK in the future.

Lord Bethell, Parliamentary Under Secretary of State (Minister for Innovation)
The UK Rare Disease Framework was published earlier this year with clear objectives for improving diagnosis, increasing awareness of rare conditions amongst healthcare professionals, improving coordination of care and access to innovative medicines.

Few rare conditions have a treatment and, where there is one, challenges do exist in accessing it. The government looks to embrace every opportunity to improve access to medicines such as the Accelerated Access Collaborative and the upcoming Innovative Medicines Fund (IMF), which is an extension of the very successful Cancer Drugs Fund (CDF).

The Rare Disease Framework outlines underpinning themes; maximising the use of digital tools and collaboration, ensuring policy alignment across government, translating research into clinical care, and putting patient voice at the centre of decision making.

All four nations have agreed to publish action plans for the framework within two years of its publication with clear deliverables and progress will be tracked with annual updates. It is essential that patient voice plays a role in the development of these action plans.

Emily Crossley, Duchenne UK
Duchenne muscular dystrophy is a progressive muscle wasting condition with childhood onset. In 2014, a medicine for the condition was licensed but it took NICE two years to approve it. It was only approved for children five years and older who could walk, in the time it took for this positive opinion children lost the ability to walk and therefore were no longer eligible for this treatment. In light of this, project HERCULES was created (Health Research Collaboration United in Evidence Synthesis). The idea was to bring competing pharmaceutical companies together to develop tools that can generate the type of data required for health technology assessments, to make it easier for bodies like NICE to make decisions about Duchenne drugs. Hopefully, HERCULES can be a model for other conditions.

Jess Hobart, The UK Mastocytosis Support Group
The UK Mastocytosis Support Group (UK Masto) supports people living with mast cell diseases which are vital cells of the immune system that help fight off infections. The European Medicines Agency (EMA) approved the first targeted therapy in 2017; however, it is still not funded in the UK whereas it has become the standard of care in many other countries around the world. This treatment is being assessed via a Single Technology Appraisal (STA) which struggles to cope with uncertainty, a concern that surrounds most rare disease medicines.

In addition to this, medicines that have multiple indications face further challenges. The rules around multiple indication pricing mean that negotiations for the rare disease medicines are affected by decisions made about the price of the drug in the other more common indication.

Roanna Maharaj, UK Thalassaemia Society
Thalassaemia is an inherited condition that affects the production of haemoglobin, the main component of red blood cells. The condition is often life limiting, the treatment is burdensome and can cause secondary conditions. A recent therapy for treating thalassaemia was assessed via the STA route rather than the Highly Specialised Technology pathway. The appraisal systems in place are trying to compare a current treatment to a potential cure and not capturing the qualitative data fully. The implications of a treatment and the quality of life it can bring cannot be measured solely through quantitative data. The thalassaemia community also share similar health inequality and stigma concerns that were mentioned by the sickle cell community.

Meindert Boysen, Deputy Chief Executive and Director of the Centre for Health Technology Evaluation, NICE
NICE is an evidence-based organisation, it tries to translate patient experiences into a quality of life assessment and is working to improve how it does this through its methods and processes reviews. Uncertainty around rare disease medicines is a common theme across the conditions represented today and NICE has tried over the years to improve how it looks at uncertainty, for example with the introduction of the Managed Access Arrangements (MAA,) but there is more to do.

There is a desire to assess new innovative technologies and to do so more quickly. In order to achieve this, the innovative regulation and access pathway must be connected, to speed processes up there needs to be more proactive engagement with partners much earlier in the pipeline. This way, potential barriers could be identified and more progress could be made. One example of collaborative working is the development of the Innovative Licensing Access Pathway (ILAP) that hopes to assist technologies find their way through the health technology assessments with fewer hurdles.

NICE's ambition is to develop an integrated end to end regulatory and access approach to assess health technologies, allowing early access while collecting real world evidence to determine value.

Nina Pinwill, Head of Commercial Operations, Commercial Medicines Directorate, NHS England and NHS Improvement
The upcoming Innovative Medicines Fund (IMF) is a type of managed access approach. A lack of evidence is often the challenge for rare disease medicines and previously some new cancer drugs had similar issues. This led to the development of the Cancer Drugs Fund (CDF) where patients were able to access treatments that didn't have enough evidence for NICE to make a decision, with the data needed to make that decision being collected in the real world for a review at NICE down the line. The CDF has been in effect for over four years now and the IMF will build on its success for treatments outside of cancer.

It is essential for NICE and NHS to work in partnership in the development and operation of the IMF, as it already does in the CDF. While the IMF is a part of the puzzle to improve access to rare disease medicines, it is unlikely to resolve all issues.

Stakeholder engagement is important and therefore a public engagement exercise is expected to take place around the principles behind the IMF. This exercise will also look at ensuring the NHS is prepared for the delivery of these innovative treatments and the extra data collection required. The timeline of the consultation exercise is to be confirmed, as the NHS continues to prioritise the Covid-19 vaccination roll out and wider response.

Discussion
Comments were raised about health inequality and the need for improvements in education amongst healthcare professionals on rare conditions that have a predominant prevalence in ethnic minority groups. Questions were raised asking how people from ethnic minority groups will be engaged in developing the action plans to the rare disease framework. Additionally, suggestions were raised around decision making bodies, like the NICE committees, becoming more representative of the population.

There is an understanding amongst the patient communities that there is a limited resource and they appreciate the need for bodies such as NICE, however, there is a need for a system that can reliably make fair and timely decisions. Suggestions were made to increase collaboration at earlier stages of the process to help increase the speed of decisions and overcome future hurdles.

There was a discussion around the uncertainty problems that rare disease medicines experience. There is likely to always be some uncertainty, as some rare conditions don't have a standard of care to be a consistent comparator for clinical trials. There is hope that the IMF will be able to address most of the uncertainty by providing a systematic, robust and clear approach to deal with it, and therefore will speed up the process. However, there is an acknowledgement that it will not solve all uncertainty issues. In tandem to this, there needs to be flexibility on how these processes consider uncertainty.

Comments were made around the difficulty of gathering the appropriate evidence required for decision makers and questions were raised as to who is responsible for ensuring data is collected in a way that is suitable for these bodies. Collaboration is a potential solution, with mention of the HERCULES project but for many patient organisation resources are often limited.

The need for collaboration and flexibility to ensure the system fits together and medicines don't fall between the gap of a Highly Specialised Technology Appraisal and Single Technology Appraisal were also mentioned. The quality of life assessments also need to be flexible as severity of a condition can be difficult to quantify.