ALL PARTY PARLIAMENTARY GROUP ON RARE, GENETIC AND UNDIAGNOSED CONDITIONS

Access to medicines third hearing: minutes

Date: **3 December 2018**

Venue: **Committee Room 12, Houses of Parliament**

Parliamentarians	Guest Speakers
Stephen Twigg MP (Chair)	Dr Jayne Spink, Genetic Alliance UK
Rt Hon Baroness Neville-Jones (Vice-Chair)	Dr Gerry Coghlan, Royal Free London NHS Foundation Trust
Catherine West MP (Officer)	Julia Wilkins, Imperial College Health Partners
Baroness Masham of Ilton	Tina Taube, Association of Research-Based Pharmaceutical Companies (Germany)

1. Welcome from Stephen Twigg MP

2. Update from Dr Jayne Spink, Chief Executive, Genetic Alliance UK

At the previous All Party Parliamentary Group (APPG) hearing on access to rare disease medicines, Genetic Alliance UK was challenged to work with a range of stakeholders to propose a new model of making decisions on rare disease medicines that is fair, transparent, effective, and would provide improved access for people living with rare conditions. This project is underway. The initial focus has been on building a strong evidence base to outline problems with the current system of access; and researching alternative commissioning approaches to identify best practice. An initial theme identified in Genetic Alliance UK's analysis is that none of the current processes for decision-making adequately address the inherent uncertainty in value of treatments for rare conditions.

This uncertainty is due to small population numbers and participants for clinical trials, and the acceleration of marketing authorisation decisions - which brings treatments forward for a funding decision with small portfolios of evidence.

Genetic Alliance UK is also developing communications tools to raise awareness of the challenges affecting access to rare disease medicines and to empower stakeholders to disseminate the new model.

3. **Guest speaker one: Julia Wilkins, Imperial College Health Partners**

Imperial College Health Partners collect and analyse health and social care data to provide information which can be used to make informed decisions when developing new models of care. Imperial College Health Partners collected data to show the impact of living with a rare disease and the interactions that took place with the healthcare system prior to diagnosis. They looked at patients diagnosed with a coded rare condition in 2017/18 and examined all of that patient's interactions across the health and social care system over the previous 10 years. This method can show the impact and cost of rare conditions on the NHS before they are diagnosed. The study found the average cost of services that patients with rare diseases consume was 416% higher than the rest of the inpatient population.

4. **Guest speaker two: Tina Taube, Association of Research-Based Pharmaceutical Companies (Germany)**

Tina Taube introduced attendees to the access approach for orphan medicines used in Germany. Currently the healthcare spend on medicines is 16.4% of health expenditure, and of this 3.7% is for orphan medicines. In 2011 the German Government introduced AMNOG (the Pharmaceuticals Market Reorganisation Act), a health technology appraisal (HTA) procedure. Under this Act, 94 orphan medicines have been regulated, reimbursed, and are accessible for patients. This procedure is mandatory for all new products. There are two parts to the appraisal: the medical benefit and the price negotiation. Medicines are available for patients and reimbursed for the first 12 months after licensing, after which they undergo an assessment to determine benefit. AMNOG includes a rule for orphan medicines that proof of added benefit is not required during the HTA process. Instead, approval by the European Medicines Agency is seen as evidence of this. This rule is only applied for a medicine that accrues less than €50 million actual sales per year. For a medicine with a higher cost, proof of additional benefit is required.

5. **Discussion**

The absence of consistency involved in appraisal routes across the UK was identified as a key issue. There are currently 15 appraisal routes and no consistency in the time taken for post-licensing decisions, in the evidence used or methodology being followed. This causes problems for those submitting the evidence.

Genetic Alliance UK will undertake a discrete choice experiment to examine the preferences of stakeholders in relation to appraisal routes and identify compromises they may be willing to make. The discussion also highlighted the importance of raising awareness of data sources such as those collected by Imperial College Health Partners, which are key for collecting real world evidence for the launch of new medicines and highlighting the subsequent impact across the health and social care system.

6. **Guest speaker three: Dr Gerry Coghlan, Royal Free London NHS Foundation Trust**

Pulmonary arterial hypertension (PAH) is the progressive narrowing of the lung arteries, leading to heart failure and death, affecting fewer than 1 in 20,000 people in the UK. Without treatment the average survival is two and half years. Current commissioning guidelines mean patients do not receive treatment until they are severely breathless. Combination therapy increases quality

of life and slows the disease progression, however, guidance requires at least two single therapies to fail before moving on to combination treatment – this often comes too late. During the appraisal process for selexipag Dr Coghlan provided evidence on the morbidity-mortality endpoint however, the clinical effectiveness team at NICE declined to include this evidence in their presentation to the Clinical Priorities Advisory Group (CPAG). Evidence provided by patients was also excluded.

This had been allowed in the process within the devolved nations, and may have contributed to these nations of the UK granting access to the treatment. Dr Coghlan concluded that three years after approval of the new medicine there is no mechanism for reimbursement or access for patients in England. The system is not transparent and the general assessment process in place for all medicines cannot work for rare diseases.

7. Discussion

Representatives of PHA UK raised frustrations that within a specialist centre based in Sheffield patients from Wales, Scotland and Northern Ireland can all access the medicine but within the same ward those from England cannot. CPAG's processes are not seen as valuing patient evidence which is often disregarded. There is no consistency in the types of evidence being accepted by NHS England and NICE. There is a need to include patient evidence, voice and experience at the heart of the commissioning process. This will go some way to capturing the full economic impact of new medicines as well as informing the decision makers on the rare condition in question. Attendees agreed that improved communication throughout the process is needed to ensure the correct evidence is being collected and will be accepted by both NHS England and NICE.

8. Next steps

Attendees agreed that the topic of access to rare disease medicines should be brought to the attention of the Health and Social Care Committee by the APPG.

Genetic Alliance UK indicated that it would be possible to present the results of the work to develop a new model for access to rare disease medicines to the APPG in Spring 2019.

9. Close