ALL PARTY PARLIAMENTARY GROUP ON RARE, GENETIC AND UNDIAGNOSED CONDITIONS

Annual General Meeting and access to medicines second hearing: minutes

Date: **11 October 2017**

Venue: **Committee Room 9, Houses of Parliament**

Parliamentarians

Stephen Twigg MP (co-chair, and chair of meeting)
Vicky Ford MP (co-chair)
Rt Hon Baroness Neville-Jones (vice-chair)
Catherine West MP (officer)
Alex Sobel MP (officer)
Baroness Hollins (officer)
Lord Rogan (officer)
Rt Hon George Howarth MP
Daniel Zeichner MP

Guest Speakers

Dr Jayne Spink, Genetic Alliance UK
Sally-Anne Tsangarides, Santhera (UK) Limited
Mark Bell, Sobi
David Lewis, Bio Products Laboratory

1. **Welcome from Dr Jayne Spink, CEO, Genetic Alliance UK**
   Annual General Meeting – election of the officers.
   Election of co-chairs: Vicky Ford MP & Stephen Twigg MP
   Election of vice-chairs: Rt Hon Baroness Neville-Jones, Lord Patel and Lord Turnberg, Rt Hon Cheryl Gillan MP
   Election of officers: Catherine West MP, Alex Sobel MP, Norman Lamb MP, Baroness Hollins and Lord Rogan

2. **Introduction from Stephen Twigg MP and Vicky Ford MP**

3. **Update from Dr Jayne Spink, CEO, Genetic Alliance UK**

   The APPG's previous hearing on access to rare disease medicines in October 2016 delivered broad agreement that the key issues affecting the current system for access to rare disease medicines included:

   - poor integration and communication between NICE and NHS England;

- lack of transparency of processes managed by NHS England;
- scarcity of clinical expertise at critical stages of medicines' reviews in NHS England's process;
- differences between the systems in the devolved nations, which many argued were more streamlined than those in place in England.

Genetic Alliance UK were disappointed that representatives from NICE and NHS England were unable to send representatives to this meeting.

4. Guest speaker 1: Sally-Anne Tsangarides, Santhera

Santhera representative Sally-Anne Tsangarides outlined her company's experience of trying to achieve access for a drug for Leber's Hereditary Optic Neuropathy (LHON), which has been within NHS England's remit for the majority of the treatment's progress through the system. Idebenone is currently only accessed through a clinical trial, on which ten patients are enrolled in England. The delay to a commissioning decision for this treatment will have a serious clinical impact, as without treatment 80% of patients will become severely sight impaired, or registered blind, within a year of showing symptoms.

In the two years since the European Medicines Agency (EMA) licensed the product, Scotland is the only country in the UK that has completed an evaluation of the treatment, deciding to routinely commission the medicine. In contrast, complex bureaucratic processes within NHS England have at least another 12 months to continue before patients will access the medicine through the NHS.

Guest speaker 2: David Lewis, Bio Products Laboratory

Bio Products Laboratory (BPL) produces purified coagulation factor X for the treatment of hereditary factor X deficiency. This is currently the only treatment available for the condition, one of the most severe clotting disorders. There are only 230 diagnosed patients in the UK, just 35 of whom require treatment. BPL grew out of public ownership, until it was partially privatised in 2013. While still a subsidiary of the Department of Health, the UK government made the decision to invest in the development of this therapy.

A number of issues with the current commissioning policy known as the 'Clinical Prioritisation Advisory Group process' were also identified. These were primarily related to poor communication and failures from NHS England on the process, and the use of the new NICE commissioning support programme which does not yet have a finalised set of methods. David Lewis called for the process to put the patient first and be more transparent.

5. Guest speaker 3: Mark Bell, Sobi

Sobi's case study described the company's attempts to deliver patients access to glycerol phenylbutyrate for the treatment of urea cycle disorders (UCDs). In the UK these conditions affect approximately 1,000 patients, with 150 needing treatment. The number of patients needing treatment would be higher, but currently, without treatment, about half die in the first few months of life. There is currently one other licensed treatment available for UCDs, also produced by Sobi, sodium phenylbutyrate, but this has serious side effects and is difficult to take.

Sobi have been in discussions with NHS England for over a year, with a view to working towards an agreement to replace the current treatment with glycerol phenylbutyrate without changing the current commissioning agreement, i.e. at no additional cost to NHS England.
Mark Bell asked what message these delays send to bioindustry, when simple negotiations can take over a year, delaying the delivery of innovation and improvements to patients' quality of life.

6. Discussion

The large amount of bureaucracy governing access to rare disease medicines in England was discussed, with guest speakers agreeing with criticism of the 'unnecessarily long processes'. Attendees called for a much clearer explanation of what NHS England wants in terms of evidence, and the pathway to a decision.

The importance of patient voice to commissioning decisions was strongly endorsed by attendees and guest speakers. Patient organisations voiced their frustrations over slow and limited access to medicines for life threatening conditions, stressing the impact on families, and explaining the real impact of a delay in a commissioning decision.

Concerns were raised that amendments and additions made by NICE and NHS England to their decision-making processes in the last 12 months were not in the best interest of patients.

The current system for delivering access to rare disease medicines is highly complex, with multiple systems leading to possible patient access, governed by NICE and NHS England. This complexity makes defining a route to improving the environment difficult, to the point where attendees suggested a blank sheet of paper as the best starting point for developing a solution.

7. Next steps

The chair thanked speakers and attendees for their contributions to discussion.

The secretariat of the APPG (provided by Genetic Alliance UK) were challenged to work with a range of stakeholder groups starting from a conceptual blank page to propose a method of making decisions about rare disease medicines that is ambitious, effective, transparent and fair. A new solution needs to be delivered to health ministers, NHS England and NICE. The APPG will reconvene to consider this issue as progress is made towards a new model for access to rare disease medicines.

8. Close