ALL PARTY PARLIAMENTARY GROUP ON RARE, GENETIC AND UNDIAGNOSED CONDITIONS

RARE DISEASE UK

Hearing on the implementation of the UK Strategy for Rare Diseases

Date: **15 November 2016**

Venue: **Room M, Portcullis House**

Attendees

- *Ben Howlett MP (Chair)*
- Farhana Ali, Genetic Alliance UK
- Lucy Barnes, NHS England
- Lara Bloom, The Ehlers-Danlos Society
- Rosie Collington, Genetic Alliance UK
- Sameena Conning, Biogen
- Chris Goard, Genetic Alliance UK
- Sam Graham, Biogen
- Alastair Kent OBE, Genetic Alliance UK
- Jerome Ma, Department of Health
- Fiona Marley, NHS England
- Nick Meade, Genetic Alliance UK
- James Palmer, NHS England
- Rupert Purchase, Wilson's Disease Support Group UK
- Caroline Saunders, guest of Ben Howlett MP
- Valerie Stevenson, Genzyme
- Sheela Upadhyaya, NICE
- Lindsay Weaver, CLIMB

Minutes

1. **Welcome and introductions from Ben Howlett MP**

2. **Presentation from Sheela Upadhyaya, the National Institute for Health and Care Excellence (NICE)**

NICE is responsible for implementing the commitment to 'ensure that there are appropriate procedures for evaluating the costs and benefits of treatments for patients'. NICE currently evaluates medicines for rare orphan conditions through its Highly Specialised Technology (HST) route. Other rare disease medicines are evaluated by NHS England. HST evaluations must balance the potential value of a product with the uncertainties associated with its evidence resulting from the small population of patients trialling the product; and whether or not NICE is supportive of NHS England to take responsibility for the risks associated with commissioning that product.

NICE has recently launched a joint consultation with NHS England to assist decisions about commissioning. Two elements of this are relevant to rare diseases. Firstly, the consultation aims to look at developing a more flexible adoption of cost-effective technologies into the NHS England. The second relevant element concerns automatic funding for routine commitments for medicines that cost over £100,000 per quality-adjusted life year (QALY) for rare conditions. Unlike other technology appraisal programmes, NICE must take into account what impact commissioning a medicine will have on the entirety of the specialised commissioning budget once clinical and economic evidence has been considered. This is because the technologies evaluated through the HST programme are of a very high cost and are often taken for long-life use. NICE and NHS England have successfully arranged for two treatments to be commissioned through 'managed access agreements'. Managed access agreements

allow NICE to enable access without potentially committing NHS England to funding a treatment that may not work.

NICE has also established mechanisms for incorporating patient and clinical expertise into evaluations. Patients and patient groups are involved in HST committee meetings, and are also able to submit evidence on paper. Although NICE has not worked directly with the devolved nations around how evaluations should take place, the NICE HST team works closely with the Scottish Medicines Consortium to advise on decisions and evaluate its processes. Wales and Northern Ireland usually implement the decisions that NICE recommends.

3. Presentation from James Palmer and Fiona Marley, NHS England

NHS England commissions services for rare disease patients by providing service specifications and defining access policies for individual treatments. NHS England's Rare Disease Advisory Group (RDAG) advises NHS England and devolved services on highly specialised technologies and rare diseases. NHS England and NICE have developed horizon scanning methodologies for both pharmaceutical products and diagnostic services, where companies can promote new products. The 100,000 Genomes Project is a flagship project run by the DH and NHS England. Rare diseases form the most productive area of the project, with over 17,000 samples from rare disease patients collected to date. NHS England's Specialised Services team has worked with the Royal Colleges and professional associations to look at ways to better identify conditions that are not only hard to diagnose, but have a detrimental impact if they remain undiagnosed. It also plans to refine its annex for highly specialised services. The Specialised Services team is looking at ways to develop a prompt for use in primary services to encourage GPs to consider rare disease diagnoses. They are also looking at how the UK can continue to be part of European Reference Networks in future, and use the experience and principles of those in UK rare disease networks. The mental health needs of rare disease patients are considered as part of the development of service specifications.

4. Discussion

- NHS England believes the DH is responsible for overseeing implementation of the Strategy.
- The Five Year Forward View, published a year after the UK Strategy for Rare Diseases, has delayed the development of an implementation plan for NHS England.
- NHS England is keen to embed plans for implementing the Strategy across all its work.
- NHS England will present RDAG with information about how the commitments will be implemented in 2017.
- NHS England does not believe it is responsible for communicating how it is implementing the Strategy to patients.
- NHS England and NICE are interested in suggestions from stakeholders on all proposed new processes to manage the introduction of new technologies without displacing what is currently on offer.
- NHS England's investment plan is balanced by a cost efficiency programme that saves approximately £200million per year.