Cross Party Group on Rare, Genetic and Undiagnosed Conditions
Tuesday 11th September 2018
6pm-7.30pm

(DRAFT) MINUTES

1. Welcome and introductions

Apologies were received from John Scott MSP and Miles Briggs MSP. Apologies were also received from Arlene Smyth (TSSS), Lynn Shields (TSA) and Sandra Thoms (Fragile X Society)

Bob Doris welcomed guest speakers for the meeting:

- Alison Strath, Principal Pharmaceutical Officer and Angela Morgan from Scottish Government
- Anne Lee, Chief Pharmacist, SMC
- Nick Meade, Director of Policy Genetic Alliance UK

2. Access to medicines in Scotland

Access to medicines has been a regular theme for the CPG and the topic has been subject to review over a number of years. This meeting of the CPG has been convened to consider recent developments in medicine policy.

A new ultra-orphan framework for decision making was announced by Scottish Government in June 2018 . Alison Strath explained that the Montgomery review had highlighted that there needed to be a rethink of the way in which medicines for extremely rare conditions and small patient populations are considered.

The Scottish Government have announced that it will be introducing a new definition of 'ultra-orphan medicines' for very rare conditions. If the medicine meets the new definition of an ultra-orphan medicine and the SMC consider it clinically effective, then it will be made available on the NHS for at least three years while information on its effectiveness is gathered. The SMC will then review the evidence and may make a final decision on its routine use in NHS Scotland.

Anne Lee explained that the SMC were working closely with the Scottish Government to meet the ambitious timeline of introducing the new framework for 1st October 2018.

During discussion, the proposed new framework was widely welcomed by CPG members as were the improvements to patient engagement methods at SMC (including the PACE process).

The process of gathering evidence whilst a medicine is in use was considered a improvement in the process, however some concerns were raised over how such data would be collected. Alison Strath explained that Scottish Government were in discussion with a Scottish university to develop a system for this. Patient groups present raised the importance of ensuring there was a mechanism for patient reported outcomes to be collected and that patients should be involved in the design of the methodology for the gathering of this evidence.

Nick Meade, Directory of Policy at Genetic Alliance UK, introduced the Resetting the Model Project. The project recognises the underlying problems of existing models of assessment- for example that traditional health technology assessment methodology is designed to compare treatments, in large populations, in well studied indications. Rare disease treatments are often the first ever treatment, or a major paradigm-shift, for small, poorly understood patient populations and typically, methodology cannot cope with the enormous value of innovative rare disease treatments such as saving a child's life or preventing a learning disability.

In the UK there are fifteen different access routes for rare disease medicines, three separate ring-fenced funding schemes and only one pharmaceutical price regulation scheme for the UK. It was noted that Germany, France, Italy and Spain all have faster systems in place. The future challenges of gene therapies, combination therapies and multiple indications for orphan products will make it necessary for medicines appraisal processes to be fit for purpose.

The Resetting the Model project will involve a workshop and discussions with key stakeholders (including patient groups from Scotland and representatives from Scottish Government and Scottish Medicines Consortium) and will aim to deliver a multi stakeholder vision for the future of access to rare disease medicines in the UK that is: effective, innovative, flexible, proportionate, pragmatic, sustainable, attractive, transparent, quick and fair.

Agreed actions:

- Scottish Government will write to the Cross Party Group in December 2018 to provide an update on the implementation of the Montgomery Review recommendations and, in particular the progress relating to the introduction of the ultra-orphan framework.
- The CPG will revisit the topic of access to medicine in one years time. Scottish Government and SMC will be invited to attend and provide an update on progress. Nick Meade will also be invited to attend to present findings from the Resetting the Model project.

3. AOB

No other business was raised.

4. **Date of next meeting**
   The next meeting will take place on Tuesday 6th November, 6.30pm-8pm, Committee Room 4. This meeting will focus on raising awareness of genetic, rare and undiagnosed conditions amongst the clinical community.

   Attendance List

Bob Doris MSP	CPG Member (Chair)
Anas Sarwar MSP	CPG Member
Natalie Frankish	Genetic Alliance UK (Secretariat)
John Miller	Action Duchenne
Harriette Campbell	Sickle Cell Support Group
Susanne Shanks	PCD Family Support Group
Sue Rees	UK PIPS
Nick Meade	Genetic Alliance UK
Sam Graham	Biogen
Amy Comrie	EDS/HSD Awareness Scotland
Arlene Smyth	TSSS
Liz Dougan	Office for Rare Conditions
Lynn Stewart	MyAware
Tracey Bowden	Pfizer UK
Amy Caffrey	British Liver Trust
Anne Lee	Scottish Medicines Consortium
Angela Morgan	Scottish Government
Alison Strath	Scottish Government
David Newman	Alexion