

Cross Party Group on Muscular Dystrophy meeting on Wednesday 14th November 2018

Attendees

Co-Conveners: Jackie Baillie MSP and Annie Wells MSP

Guests

Lindsey Armstrong

Mark Chapman (with PA), DMD Pathfinders

Marina di Marco, Scottish Muscle Network

Clare Eadie

Kim Kemp

Jonathan Kingsley, Muscular Dystrophy UK

Martyn McDonald, Pharmacy and Medicines, Scottish Government

John Miller, Action Duchenne

Jackie Munro, Muscular Dystrophy UK

Gillian Sherwood, Prince and Princess of Wales Hospice

Prof Alison Strath, Pharmacy and Medicines, Scottish Government

Ben Tichler, Biogen

Stuart Townhill

Robbie Warner

Fiona Wylie, Prince and Princess of Wales Hospice

Michelle Young

Meeting summary

MSPs and guests introduce themselves.

Muscular Dystrophy UK update:

- Spinraza latest developments across the UK
- Mental Health Matters campaign and All Party Parliamentary Group for Muscular Dystrophy Psychology Inquiry Report being launched on 21st November in Westminster

Alison Strath on the new ultra-orphan medicines pathway:

- Cross Party Group has stressed the need for this to be introduced quickly
- Implementing Montgomery Review recommendation
- Work has been taking place over the summer with the Scottish Medicines Consortium (SMC) on the criteria for the pathway
- Recognition of designating ultra-orphan status
- Engaging companies early with the SMC
- Looking at Patient Access Scheme and data collection mechanisms
- Complex and time consuming Managed Access Agreement in England
- Trying to put in place a pragmatic agreement and to establish what data is important
- Exploring ways to engage patients, carers, families and clinicians – for half a day or a full day workshop – to establish Quality of Life aspects
- PACE meeting at endpoint and after the workshop
- For Spinraza, it is already available for SMA Type 1, and it would Types 2 and 3 under consideration in the new pathway

- Legacy medicines, such as potentially Spinraza, will help with testing the system out
- Testing legacy medicines likely to start just after Christmas, with the pathway fully live from April 2019 onwards
- Send guidance out to clinicians
- Work with patient groups to disseminate information

Discussion on Spinraza and pathway:

- Some room for flexibility on the number of patients
- Registries – not always accurate information on patient numbers
- SMC has ownership over the process as system is reformed
- Improve Public Patient Partner aspects
- There would need to be commissioning protocols in place in terms of English patients potentially seeking access in Scotland
- Need to look at clinical and admin time to administer Spinraza and NHS preparedness
- Electronic prescribing just starting out
- Fewer patients in Scotland compared to England, but fewer clinicians to administer the treatment
- If rejection from SMC, could still be made available through the PACs system
- Need to have the right structure in place in terms of access schemes, and impact on families if scenario of treatment being taken away
- Healthcare is changing with different procedures and adapting to technology
- Working closely with regional and local planning
- Last thing we want is approval without being able to administer it

Hospice and respite discussion:

- Changing criteria in children's hospice care with CHAS changing its service model
- Focus on greatest need from 0-6 from a study carried out
- 4 out of 5 hospices in England not providing respite services
- Alternative hospice and respite options needed – CHAS a victim of their own success in some ways
- Short breaks and respite are what is wanted
- Can't exclude a cohort who are needing respite
- Can't offer something that isn't sustainable
- Health and Social Care integration
- Mental health support – relying on families but need a peer support respite structure
- Mental wellbeing impacts on physical health
- **Action: explore possibility of CPG summit across relevant CPGs on this issue**
- **Action: Write to Government about Working Group**

Trial centre points:

- Potential for trial centre funding
- Future self-funding mechanism – Alison to speak to Clinical Priorities Team