

Cross Party Group on Muscular Dystrophy meeting on Wednesday 27th February 2019

Co-Conveners: Jackie Baillie MSP and Annie Wells MSP

Other MSP attendees: Maurice Corry MSP

Guests

Mark Chapman (with PA)

Clare Eadie

Gerry Kelly

Sheonad MacFarlane

Ann Marie Penman

John Miller

Oona Miller

Stuart Townhill

Prof Alison Strath, Pharmacy and Medicines, Scottish Government

Rob Burley, Muscular Dystrophy UK

Jonathan Kingsley, Muscular Dystrophy UK

Jackie Munro, Muscular Dystrophy UK

Meeting summary

MSPs and guests introduce themselves.

Muscular Dystrophy UK update:

- Three pillars – research, care, living well
- Strategy – Fast Track, Mental Health Matters, assistive technology, sport and leisure
- **Action – share MDUK Strategy document by email (Jonathan)**

Alison Strath on the new ultra-orphan medicines pathway and access to Spinraza:

- Support and input from the Cross Party Group on processes and experiences which has informed policy development
- Updated definition of ultra-orphan medicine
- Validation from sub group within Healthcare Improvement Scotland
- After validation, submission to Scottish Medicines Consortium (SMC)
- SMC report including any uncertainties relating to the data
- Patient Access Scheme – as cost effective way as possible
- 3 years to collect real world data and wider societal benefits
- Flexibility to extend 3 years if informative to do so
- Data collection – small sample from clinical trial data
- Advice for routine use at the end of 3 years
- Pointless for Spinraza to start from beginning with full submission as appraised so recently
- Rest of medicines in pipeline to be appraised from April onwards in a 12 week assessment
- Instead of PACE process, company will organise workshop with academic partner – what data is robust, and discussion with patients and families about most helpful measures
- **Action – Written description of Ultra-Orphan Pathway with lay language (Alison)**
- Spinraza validated as ultra-orphan medicine in October
- Biogen putting together a data package
- Data collection process is the last piece of information needed
- NHS Boards and extra resources to administer Spinraza – working through that

Discussion on pathway and Spinraza:

- At end of 3 years of Patient Access Scheme, if SMC doesn't recommend, clinicians could still provide Spinraza if they think it will be beneficial
- When considering new therapies, clinical and cost effectiveness will be considered
- New advanced therapies challenge the current provision of treatment
- No priority to one type of SMA over another
- Information required – one to the clinicians and the service, and another to patients
- Conversations with Biogen about patient information which is required
- Significant discussions taking place with clinical and spinal teams
- Health Boards are there to implement decision reached by SMC
- Discussions on support for NHS resources
- Not unusual to see disparity in decisions between SMC and NICE
- It is a choice of the patient if they wish to move from England to Scotland
- Confident that Biogen are progressing well on data collection to meet the timeframes
- Tweaking Patient Reported Outcome Measures if not already in place
- Focused on resources to undertake data collection
- Work with NHS Boards on finance and data collection

Psychological support discussion:

- Background about psychological support and written submission from Scottish Government Directorate for Mental Health
- Access needed as part of multi-disciplinary team (MDT) support
- 11 young adults at Prince and Princess of Wales Hospice (PPWH) have access to support through counselling service in last few years
- Everyone needs psychological support including siblings
- No support from diagnosis onwards
- Clinicians aware that psychological support would be beneficial
- Social model as part of PPWH support
- Matrix in submission is out of date
- No mention of neuromuscular conditions in Neurological Conditions Action Plan
- Where is the 66% capacity mentioned in submission
- Very little support for families – steep learning curve and a balancing act
- Educational route should be explored as a means of provision
- Anxiety for family members and exploration of genetic counselling
- **Action – FOI to every Health Board about psychological support and MDT, and how many need mental health support (Jonathan/Sheonad)**
- Generic counselling services don't have any knowledge of neuromuscular conditions
- Upskilling care advisors for signposting and MDUK virtual psychologist pilot project
- Overstretched care advisor support and some people don't have access to it

Hospice and respite collaboration:

- CPG collaboration – already reached out to Rare, Genetic and Undiagnosed and Palliative Care CPGs
- **Action – make contact with Disability CPG and bring MND to the collaboration (Jonathan)**
- PPWH – lack of funding for bed space
- Difficulties with SDS – not easy option at the cost for bed space

Housing:

- Inappropriate inaccessible housing – no options and no clear route
- Housing Options and Stirling University report due in a couple of months
- Budgets for adaptations for Scheme of Assistance insufficient
- Announcement of 50,000 new homes
- **Action – FOI to local authorities on availability of accessible housing and how many on waiting list (Jackie Munro/Jonathan)**

AOB:

- Mark Chapman – issue with procurement of equipment in Lothian – reliable equipment being taken away and replacement not necessarily as good
- **Action – letter to NHS Lothian from Jackie Baillie on equipment procurement (Jonathan to draft)**

Action list:

- **Action 1 – share MDUK Strategy document by email (Jonathan)**
- **Action 2 – Written description of Ultra-Orphan Pathway with lay language (Alison)**
- **Action 3 – FOI to every Health Board about psychological support and MDT, and how many need mental health support (Jonathan/Sheonad)**
- **Action 4 – make contact with Disability CPG and bring MND to the collaboration (Jonathan)**
- **Action 5 – FOI to local authorities on availability of accessible housing and how many on waiting list (Jackie Munro/Jonathan)**
- **Action 6 – letter to NHS Lothian from Jackie Baillie on equipment procurement (Jonathan to draft)**