

Cross Party Group on Muscular Dystrophy meeting: 16/06/2015

Present:

Graeme Dey MSP
Jim Eadie MSP
Rhoda Grant MSP
Hanzala Malik MSP
Mary Scanlon MSP

Jo-anna Allen, PTC Therapeutics
Mark Chapman (plus PA)
Kathryn Fergusson, Scottish Government
Jonathan Kingsley, Muscular Dystrophy UK
Sarah Fidelo
Eileen McCallum
John Miller, Action Duchenne
Mariana Pierotti, Muscular Dystrophy UK
Aaron Revel, Action Duchenne
Peter Sutton, Muscular Dystrophy UK
Dean Widd, Muscular Dystrophy UK
Justin Young
Michelle Young

Apologies:

Jackie Baillie MSP
Nanette Milne MSP

Minutes:

Jonathan Kingsley welcomes attendees and gives apologies.

Access to suitable Hospice and Respite Facilities:

Peter Sutton spoke on behalf of MDUK to update the group on the campaign for access to suitable hospice and respite facilities for young disabled adults in Scotland. CHAS are asking regions to list suitable facilities and hope to have a comprehensive list to share. CHAS have appointed a transition team for 3 years which was launched in January this year to ensure all young people who will be affected by the CHAS age restrictions will be appointed a suitable respite facility.

CHAS are carrying out research at the University of York to understand numbers in the UK as a whole effected by muscular dystrophy, beyond Duchenne.

Claire Turnball from the transition team at CHAS attended our meeting on 22/04/2015 and will report back in 6 months on the progress CHAS are making.

Summary of discussion on Translarna:

Jo-Anna Allen from PTC Therapeutics answered questions on the company's plans for their submission to the Scottish Medicines Consortium (SMC). PTC has most of their submission complete,

this includes all the patient data, however they are still developing the pharmaco-economic model. The SMC met with PTC in March, prior to a submission, on a new process that has come about due to the changes in the SMC submission process. SMC are working closely with PTC to ensure the best possible chance of a successful submission.

Kathryn Fergusson stated that the government and SMC are awaiting the submission from PTC. She also mentioned the ring fenced fund for rare disease drugs, the New Medicines Fund, which was doubled last month to £80million, could pay for Translarna. The patient access scheme (Patient and Clinician Engagement) will be available for families to have their say.

Michelle Young, mother of 8 year old Michael who has Duchenne and is on the Translarna trial spoke from a parent's perspective and about the impact of Michael being on the trial. She urged PTC Therapeutics and SMC to work together to help keep her son and other boys eligible for the treatment walking.

Priorities over the coming year:

The group agreed the main priorities for the coming 12 months for the Cross Party Group should be:

- fast access to Translarna in Scotland
- the lack of hospice and respite facilities in Scotland
- the lack of care advisor support in Scotland in the north of Scotland
- the impact of welfare reform on people with muscle-wasting conditions
- dedicated neuromuscular time for professionals