Cross Party Group on Muscular Dystrophy meeting on Wednesday 28th June

Attendees

MSPs

Jackie Baillie
Maree Todd
Annie Wells

Guests

Michael Armstrong
Dr Anthony Bateman
Mark Chapman (with PA), DMD Pathfinders
Jessie Csere
Marina di Marco, Chair of Scottish Muscle Network
Jonathan Kingsley, Muscular Dystrophy UK
Anne Lee, Chief Pharmacist of Scottish Medicines Consortium
Dr Alan MacDonald, Chair of Scottish Medicines Consortium
Sheonad Macfarlane, Muscular Dystrophy UK
John McDermott
Liz McDermott
Robert Meadowcroft, Chief Executive, Muscular Dystrophy UK
Oona Miller
Jackie Munro, Muscular Dystrophy UK
Dr Richard Petty
Yvonne Robb
Justin Young
Michelle Young

Summary of meeting

AGM business

- Jackie Baillie, Maree Todd and Annie Wells to be co-conveners of the group.
- Jackie Baillie will be the group's registered contact.
- Muscular Dystrophy UK to continue as the secretariat of the group.

Information and support

Jackie Munro, Advocacy and Information Officer for Muscular Dystrophy UK, gives some background about her role and increased support in clinics and forthcoming peer support Muscle Groups.

Access to new medicines and the Montgomery Review

- Background to SMC by Dr MacDonald: function, rationale of approval decisions, engagement with pharmaceutical company, Patient and Clinician Engagement (PACE) process and starting to make changes after the Montgomery Review.

- With an increasing number of treatments emerging for muscular dystrophy and other rare diseases, does the Scottish Medicines Consortium have sufficient capacity to undertake a growing amount of appraisals?
- SMC - can cope with capacity and are confident that we will continue to meet deadlines; welcome new treatments and if there is need for prioritisation, conditions with an unmet need will be prioritised.

- What is the balance of economic and clinical factors in the decision?
- SMC – cost and efficacy are both considered. There is potentially a point – for low or high cost treatments – where it's not economically beneficial, but there's no upper limit on cost per QALY. It's difficult to have a completely open debate with confidentiality around cost and any discounts discussed. Changes to the process haven't met the intention for medicines for very rare conditions so working on further changes.

- What is the influence of patient groups in the process?
- SMC – we are keen to include the understanding of patients' experiences, clinical experts and patient groups.

- How has the Montgomery Review been received by the SMC?
- SMC – recommendations have been accepted in full by the Scottish Government. We have prioritised changes for extremely rare conditions in the process and revised definitions for ultra-orphan conditions. An announcement on changes to decision-making is expected soon. We are looking again at the role of the Public Partner which already has a seat around the table with the committee from this month onwards.

- What is the horizon scanning process within the SMC?
- SMC – there is a comprehensive horizon scanning in place but there is confidentiality around drugs in the pipeline. There are patient group events and horizon scanning is built in.

- Lack of early engagement with pharmaceutical companies a common concern and not producing a case that fits the requirements of the SMC.
- SMC – we have a criteria meeting with the company. The quality of submissions have improved but there is a push for more early engagement. When companies don't make a submission, it's not helpful. Our work is looked at internationally.

- What are the implications of Brexit on treatment appraisals?
- SMC – there seem to be two potential outcomes. One is that European Medicines Agency licences will be rubberstamped in the UK but we won't have influence over the EMA's decision-making. The other is that the UK regulatory bodies will be enhanced but there is concern that companies would come to the UK later and that if all regulation took place in the UK, the process will inevitably be slower due to capacity level compared with the EMA.

- What is the awareness of the SMC? With Translarna there seemed to be a lack of understanding about the SMC process.
- SMC – always trying to build understanding and awareness amongst pharmaceutical industry and hold events with companies.

- What evidence is gathered apart from PACE?
- SMC – there is uncertainty about future projections of treatment impact but we do have a pharmaco-economic model. There have been 80 PACE meetings so far. PACE process at its best when it fits with and adds to the evidence presented.

- Robert Meadowcroft joined Michelle Young at the Translarna PACE process but there was very limited opportunity to engage – including the template document being too short and Michelle only being allowed 5 bullet points to summarise complex issues. Robert – pleased to hear steps being taken to improve patient involvement in decision-making.
- SMC – take points on board and still learning.

- Limited information on outcome measures.
- The Montgomery Review recommends an additional decision option of: "recommend for use subject to ongoing evaluation and future reassessment." What action is the SMC and NHS Scotland taking to adopt this recommendation and learn from the MAA process which is being increasingly used in England? Is there the opportunity to undertake pricing negotiations with the pharmaceutical company at an early stage in the process?
- SMC – this still needs to be worked out across the agencies. It's a work in progress but a change isn't imminent. It's not in anyone's interest for medicines not to be used so there is an opportunity for a conditional yes in the system.

Hospice and respite provision

- Robert Meadowcroft – pay tribute to Robert Watson's campaigning and work on this issue. Life expectancy for Duchenne is increasing and therefore there is a growing population of young disabled adults. In the CHAS transition programme, many have been settled in new provision but some still haven't been transitioned yet. There needs to be collaboration and commitment from many different sources – Scottish Government, Local Authorities, Health Boards, hospices and charities, with a roundtable discussion proposed for the autumn.

Discussion

- CHAS holding an update meeting to showcase positive case studies in the autumn, but it's not a positive picture for everyone.
- Nowhere to transition to in respite settings.
- Needs to be Scottish Government co-ordination and we need to hear from those not transitioning well.
- Neuromuscular Complex Care Centre at Queen Square in London – could be explored in Scotland
- CHAS – structure of support is a Health Board lead with input from COSLA
- Roundtable to be arranged in the autumn

Priorities for the Cross Party Group

- Hospice and respite provision for both adults and children and complex care unit
- Psychology support
- Travel insurance
- PAs – recruitment and pay
- Accessible housing
- Cardiac care