

Cross Party Group on Rare Diseases

18th November 2014 (5.45 – 7.30pm)

The Scottish Parliament

MINUTES of MEETING

- **Welcome and introductions**

Malcolm Chisholm welcomed all in attendance and invited those around the table to say who they were and what organisation they were representing.

Apologies were noted from Yvonne Hughes (Cystic Fibrosis Trust) and Lindsay Lockhart (Carer's Trust)

- **Access to New Medicines for Rare Diseases**

Malcolm Chisholm noted that in December 2013, we had held a Cross Party Group meeting which focused on the Scottish Government and Scottish Parliament's Health and Sport Committee review of new medicines for rare diseases. It was acknowledged that a number of wide-ranging recommendations had emerged from the review and that decision making with regards to access to medicines would now be subject to greater transparency and patient involvement.

Ann Lee and Alan Macdonald, representing the Scottish Medicines Consortium (SMC) were introduced as invited speakers. Ann Lee provided a presentation explaining that there had been some significant changes at SMC, including meetings open to the public, changes to the criteria for assessing orphan, ultra-orphan and end of life treatments and most notably, the introduction of the Patient and Clinician Engagement (PACE) step-change process. For orphan and ultra-orphan medicines, patient groups and clinicians will be invited to take part in a PACE meeting to produce a statement on the impact of the medicine on the patient's quality of life. This could include information such as whether the patient requires less care, needs to attend fewer hospital appointments, suffers fewer side effects. The SMC has a dedicated Public Involvement Team that supports this work and can advise and support patient groups through this process. It was noted, that although there had only been a small number of meetings to date, that this improved patient involvement process had brought new light to SMC meetings and the insight from patients has proved valuable to those making decisions at SMC.

Kathryn Fergusson, Head of Medicines for the Scottish Government, was then invited to speak and to share information on the New Medicines Fund. Kathryn explained that, unlike other parts of the UK, the Scottish Government had created the New Medicines Fund to reinvest money generated by the Pharmaceutical Price Regulation Scheme (PPRS). The New Medicines Fund represents a dedicated £40million and expands, and replaces, the Rare Conditions Medicines Fund set up in 2013. It was noted that in 2013/14 the Rare Conditions Medicines Fund supported the cost of 45 different medicines benefiting more than 200 patients. These include Ivacaftor for cystic fibrosis, eculizumab for atypical hemolytic-uremic syndrome and PNH.

Kathryn noted that the interim arrangements that had been put in place for Individual Patient Treatment Requests (IPTRs) were working extremely well and that there was an increase in the number of patients accessing medicines for rare diseases. It was noted that the Scottish Government were still committed to rolling out the Peer Approved Clinical System, which will ultimately replace IPTRs, however this would be subject to a pilot system and phased introduction to ensure that there no disruption to access to medicines for rare disease patients was caused by a rushed change over. Kathryn noted that the Scottish Government were also keen to ensure pharmaceutical companies submitted medicines for assessment through the SMC and encouraged patient groups to call for this too. It was also noted that should any patients or families have difficulties in obtaining a medicine through the interim IPTR arrangements, that patient groups should contact Kathryn who would be more than happy to look into the matter.

During discussions patient groups made it clear that the changes at SMC had been welcomed and that patient groups and patients could have more confidence in this system. It was acknowledged that the Public Involvement Team at SMC were incredibly helpful and had been a welcome addition to SMC. Patient groups were keen to see the introduction of the new Peer Approved Clinical System (PACS), but were also keen to ensure that patients were not adversely affected during the transition from the interim IPTR arrangements. Patient groups noted that clear guidance on PACS and the New Medicines Fund would be welcomed by the rare disease community, including clinicians.

- **AOB**

Lothian Immunology

Rae McNairney, representing PID UK (Primary Immunodeficiency UK), provide an insight to current immunology services in NHS Lothian. She explained that there was not an adult immunology service in NHS Lothian, despite a relatively high number of patients being present in the Lothian area. NHS Lothian is the

only health board in Scotland to not have an adult immunology service, and a clinical immunologist. At present, many patients have to access clinical services in Newcastle, which it is suggested, is not in the best of interests of patients. There were a number of patients in attendance at the meeting that shared their experience of care at NHS Lothian and shared their concerns about the lack of an adult immunology service. Clinical staff in attendance also shared their concerns. It was noted that this had been an ongoing issue for a number of years, and despite many campaigns from interested patient groups, the issue remains unresolved and patients with primary immunodeficiency in Lothian are disadvantaged by this.

It was agreed that Mr Chisholm would write to the NHS Lothian to share the concerns raised and to ask for an update on what NHS Lothian are doing to resolve the matter.

Rare Disease Day 2015

Natalie Frankish shared information about Rare Disease Day 2015 and explained that Rare Disease UK would be holding a Parliamentary Reception on February 24th 2015, chaired by Malcolm Chisholm. Group members were invited to share any information about events that they would be undertaking to mark Rare Disease Day with Natalie via email.

Date of Next Meeting

Due to the date of the Rare Disease Day reception, the arrangements for the next CPG meeting had not yet been made. Natalie will circulate information and post on the Scottish Parliament website when the date becomes available.