Scottish Parliament

Cross Party Group on Rare Diseases

Minutes of Meeting: December 1st 2013

1. Introduction:

Bob Doris MSP welcomed all present and invited those in attendance to introduce themselves.

2. Liz Porterfield: Update on Scotland's Plan for Rare Diseases

Liz Porterfield updated those in attendance on the work that the Scottish Government has been doing to develop the UK strategy for rare diseases and explained that work was in progress internally to gather information to produce Scotland's Plan for rare diseases. The UK Strategy for Rare Diseases was launched on November 23rd 2013, outlining 51 commitments. Liz explained that the Scottish Government team working on the Plan was currently pulling together information and to assist, Natalie Frankish agreed to circulate the 51 commitments outlined in the strategy to CPG members for comment, and forward to Liz. Liz explained that the 51 commitments outlined in the UK strategy would be covered by the Scottish Plan, but noted that a degree of flexibility in how these commitments will be achieved may be necessary and that each devolved nation was likely to do things a bit differently.

Liz explained that she hoped her team would have a draft plan in place soon and would then expect to consult with RDUK on a strategic level.

3. Access to New Medicines for Rare Diseases in Scotland

Bob Doris explained that he had been involved in the Health and Sport Committee's work on access to new medicines and welcomed Joan Fletcher (AGSD UK) and Angela Timoney, Chair of the Scottish Medicines Consortium, to the meeting.

Joan Fletcher, Family Support Officer, AGSD UK

Joan Fletcher provided background to the Public Petitions for Access to New Medicines submitted to the Scottish Parliament. AGSD UK, in conjunction with Rare Disease UK, PNH Alliance and PNH Scotland lodged public petitions, highlighting the difficulties in accessing medicines for patients with rare diseases in Scotland. Joan explained that medicines for very rare diseases were less likely to receive an SMC 'Yes' decision, as the cost of a medicine to treat very few patients is likely to be very high. Were a medicine is not recommended for use by the SMC, it can still be accessed through an Individual Patient Treatment Request (IPTR) lodged by a clinician who believes the patient will benefit from the medicine. Joan explained that to access a medicine through an IPTR, a patient must demonstrate 'exceptionality' and that it was extremely difficult for a patient to meet the criteria. The petitions lodged by the three organisations called for a thorough and independent review of the processes by which rare disease medicines can be accessed in Scotland. The Health and Sport Committee of the Scottish Parliament, undertook a series of round table meetings to gather evidence from a wide range of stakeholders. A report was published by the Committee in 2013 and Joan noted that the report provided a number of welcomed recommendations, including that the term 'ultra orphan' be recognised by the SMC, that the SMC should consider alternative methods to the QALY when assessing medicines for rare diseases and the requirement to show 'exceptionality' during the IPTR process be removed.

Joan explained that the Scottish Government had also undertaken a review and that the recommendations from the reports by Professor Routledge and Professor Swainson were also encouraging. The announcement of a £21 million rare conditions medicines fund was also welcomed, although concern was noted that many patient groups and clinicians were unsure how the fund operated and could be accessed. Joan noted that the work that had been undertaken had been

encouraging and that it was hoped that the reviews would lead to improved access to medicines for rare disease medicines in Scotland.

Angela Timoney, Scottish Medicines Consortium

Professor Timoney provided an overview of the process by which medicines for rare diseases (and for more common diseases) are currently assessed. The Quality Adjusted Life Year (QALY), is the current tool used to assess medicines in Scotland and the SMC looks at information supplied by the medicine manufacturer on the clinical and cost effectiveness of the medicine being assessed. Information from clinical experts and patient groups are also involved in the decision making processes.

Professor Timoney explained that following the recent reviews undertaken by Professor Routledge and Professor Swainson, the SMC had been tasked with undertaking a review of the appraisal process of medicines for end of life or rare conditions. The SMC established a Rapid Response Task and Finish Group and is expected to produce a report by December 20th. Professor Timoney explained that the Group had looked at ways to increase patient and clinician involvement in the decision making process and explored the possible different approaches to appraising medicines for rare diseases. Professor Timoney also noted that the Task and Finish Group included patient group representation, including Alastair Kent from Rare Disease UK.

Professor Timoney also provided an overview of the work of SMC's Patient and Public Involvement Group for the benefit of patient groups present. The role of PAPIG is to promote public awareness of the work of the SMC and to ensure that the patient and carer perspective is prominent in all SMC assessments. The PAPIG group will work closely with patients/patient groups to develop a patient interest groups submission, which a member of PAPIG will present at SMC meetings to ensure the patient view is reflected in the decision making process.

4. AOB

- Patricia Osborne (Brittle Bone Society) shared news of the launch of the BBS educational schools campaign and the BBS' participation in the 'RUDY' Database, working in conjunction with a research team at Nuffield University developing tests for patients with rare bone diseases.

- Cathy Watt provided a brief update on the registration process for genetic counsellors. Cathy explained that there is currently no statutory register for Genetic Counsellors and that there had been a move towards Assured Voluntary Registration. As part of this process, the Professional Standards Authority had outlined specific criteria which have to be met including, sustainable financial reserves, a website suitable for public,patients and professionals, and a robust process to handle complaints and disciplinary actions.

5. Arrangements for next meeting

It was agreed that, due to the high level of attendance, future CPG meetings should be held at 5.30pm on Tuesday evenings. The proposed date for the next meeting, pending room availability, is Tuesday 15th March.

Natalie Frankish will explore the suggestions for topics for the next meeting and approach potential speakers for availability.