

Cross Party Group on Rare, Genetic and Undiagnosed Conditions

Tuesday 27th June 2017 – 1pm to 2.30pm

Scottish Parliament, Committee Room 3

DRAFT MINUTES

1. Welcome and Introductions

1.1 Bob Doris MSP welcomed all in attendance and invited attendees to introduce themselves. Apologies were received from Miles Briggs MSP, John Scott MSP, Arlene Smyth (TSSS), Hazel McLachlan (Bechet's Syndrome Society), Lesley Loeliger (PNH Scotland), Lynn Stewart (MyAware) and Prof Jonathan Berg.

2. The CPG on Rare, Genetic and Undiagnosed Conditions Overview and Proposed Work Plan

2.1 The Cross Party Group will meet a minimum of three times per year. Meetings are likely to take place on Tuesday lunchtimes, depending on availability of committee rooms in the Scottish Parliament. Attendees were reminded that the purpose of the group is to look at the challenges that cross cut the large number of rare, genetic and undiagnosed conditions rather than to focus on specific conditions.

2.2 Bob Doris explained that this is a new Cross Party Group which will consider various themes concerning rare, genetic and undiagnosed conditions. Attendees were invited to suggest themes for the future workplan and Bob explained that consideration would then be given to the suggestions and the workplan communicated at the next meeting.

2.3 Suggested themes include:

- **Support for families with Autism** – it was noted by several attendees that for many rare, genetic conditions, autism is also a factor and that families and support groups were struggling to access appropriate support services. It was noted that there are perhaps other Cross Party Groups (such as the CPG on Disability and CPG on Learning Difficulties) that would be a more appropriate forum to explore this topic. Natalie Frankish (Secretariat) will investigate appropriate Cross Party Groups and contact the Secretariats to explore options for joint working.
- **Education** – several attendees agreed that education providers and local authorities were not aware of many rare, genetic and undiagnosed conditions and were not adequately equipped to support children, teens and families. It was noted that education was a key focus of the Scottish Government.
- **Transition** – it was noted that there was little support for families at transitional milestones in education and clinical care.
- **Specialist Nursing** – it was noted that for many conditions specialist nursing does not exist or there is under provision. It was noted that the allocation of funds from a specialist nursing fund in 2015 had not been communicated. Bob agreed to enquire about this. Specialist nursing was considered an important topic for the CPG to consider.

- **Sport and Activity** – the issues of inequity in access to, and participation in, sport and related activities was raised. It was noted that this was across the spectrum, from school and local clubs to Paralympic level. It was suggested that those with rare, genetic and undiagnosed conditions were at a disadvantage. It was noted that there are other CPGs (such as the CPG on Disability and CPG on Sport) that may be better placed to advance this work – Natalie will contact the Secretariat of these groups to discuss possible partnership working opportunities.
- **Benefits and Social Welfare** – patients and patient groups noted that the existing processes for obtaining benefits (and including things as Motability) were not appropriate for those with rare, genetic and undiagnosed conditions. The Scottish Government are currently consulting on these matters and it was noted that the CPG may wish to contribute to this work.
- **Data Management and Sharing** – issues surrounding collecting patient data and the sharing of information between clinical systems was raised. There was discussion of the inadequacies of IT infrastructure across the NHS and how the population size in rare conditions would enable piloting of new initiatives. Data management is a key feature of the Rare Disease Plan.
- **Cross border healthcare** – pathways for accessing services in other parts of the UK were raised, as was the desire for a greater number of services to be held in Scotland. Specialist services were of importance to the CPG.

2.4 It was noted that it would be helpful to understand upcoming work of the Scottish Parliament to help shape the priorities of the CPG. Bob Doris explained he would raise this with the Scottish Parliament's Conveners Group.

2.5 Attendees and members were informed that there would be the opportunity to raise further potential topics by email and that a specific email address cpgscotland@geneticalliance.org.uk had been set up.

3. Access to medicines for rare conditions in Scotland – Montgomery Review

3.1 The discussion on the Montgomery Review recommendations was cut short due to business in the Scottish Parliament. It was agreed that the slides for the meeting would be shared and that further comment could be made via email.

3.2 The Scottish Government published Dr Montgomery's Review of Access to New Medicines in Scotland in December 2016 with 28 recommendations on how to further improve access to medicines. The review recognised that whilst access to medicines for orphan and end of life had seen a marked improvement in availability and access, this had not been the case for ultra orphan medicines for very rare conditions.

3.3 Natalie explained that work had already begun to implement the recommendations and that many of those in attendance at the meeting had participated in various consultation events. Natalie shared some of the examples that she was aware of. For example, a workshop was held to tighten definitions around what constitutes an ultra orphan medicine and that a new decision making body was to be established to make a final decision on ultra orphan medicines. Also, the Scottish Medicines Consortium's Patient Involvement Network has been involved in implementing several changes to improve patient involvement in the process –

including giving patient groups a seat at the SMC table (started in June) and modifying the role of the Public Partner. The Scottish Government have also established a scoping task force to consider the recommendations regarding improving data collection and analysis.

3.4 There are a number of outstanding recommendations yet to be implemented that the Cross Party Group can contribute to. These include recommendations around NHS Scotland's role in negotiation, conditional acceptance of a medicine and managed access schemes. Those in attendance were invited to contribute views at the meeting, or after via email. Points raised at the meeting included:

- Perceived inflexibility of NHS Procurement process to consider complex managed access schemes. It was suggested that this may be improved by NHS Procurement being given additional resources to accommodate more complex schemes.
- Early engagement was seen to be key – with patient groups very supportive of this. Patient groups were also interested in being involved in developing what patient outcomes should be identified. It was suggested that experience of early engagement from England should be gathered and considered when developing a process for Scotland.
- Businesses modelling relating to identifying preventative spend will be important. The need to balance large upfront costs against longer term future costs and savings needs, and how to measure and evaluate this should be addressed – and there was support of payment by results (patient outcomes).
- Regarding ongoing evaluation and data capturing, patient groups recognised that the NHS in Scotland is currently ill equipped to capture patient outcomes. There was discussion around patient reported outcomes and advancements in technology that could be explored to pursue this. Patient groups also felt that clinicians would be best placed to capture data, although there was recognition that this would put additional pressure on clinician's time. There was a wariness of placing the responsibility of capturing data with pharmaceutical companies – issues around ownership of data and trust were raised. It was also suggested that patient groups may have a role to play in this, however this was balanced by the recognition that many groups are very small and possibly not able to do this.
- With regard to complex managed access schemes, it was noted that there was greater scope for this in England. However, it was suggested that there is a lack of consistency in approach. A suggestion for development of complex access schemes in Scotland is that it is accompanied by guidance and applied in a consistent and equitable manner.

3.5 Further comment was invited by email.

4. Adults with Incapacity (Scotland) Act 2000 – Upcoming consultation

4.1 There was insufficient time to discuss this matter in full. It will be picked up at the next meeting to be discussed ahead of consultation on the Act that is due later in the year. Natalie will circulate information ahead of the next meeting.

5. Date of next meeting - 5th September 2017 in Committee Room 5 between 1 and 2.30pm

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Attendees and Apologies

Clare Adamson	MSP	Attendee
Jonathan Berg	Dundee Clinical Genetics	Apologies
Miles Briggs	MSP	Apologies
Amy Caffrey	British Liver Trust	Attendee
Harriette Campbell	Representing Sickle Cell	Attendee
Bob Doris (Chair)	MSP	Attendee
Elizabeth Dougan	Officer of Rare Conditions	Attendee
Michelle Erskine	Aarskog Syndrome Foundation	Attendee
Jane Ferguson		Attendee
Karen Ferguson	Funny Lumps	Attendee
Natalie Frankish (Secretariat)	Genetic Alliance UK	Attendee
Simon Flynn	Genzyme	Attendee
Yasmin Fraser	Biogen	Attendee
Yvonne Hughes	Cystic Fibrosis Trust	Attendee
Lesley Loeliger	PNH Scotland	Apologies
Hazel McLachlan	Bechet's Syndrome Society	Apologies
Rae McNairney	PID UK	Attendee
John Miller	Action Duchenne	Attendee
David Newman	Alexion	Attendee
Catherine Orr		Attendee
Susan Rees	UK PIPs	Attendee
John Scott	MSP	Apologies
Arlene Smyth	TSSS	Attendee
Rebecca Stewart	Teddington Trust	Attendee
Margarita Sweeney-Baird	BBSUK and Inclusive Skating	Attendee
John Wallace	NORP12	Attendee