Progress towards improving access to new medicines for patients at the end of life, or with very rare conditions

1. Background

On January 31 this year the Cabinet Secretary for Health welcomed the Scottish Medicines Consortium’s proposals on how to improve access to new medicines for patients at the end of life, or with very rare conditions.

The proposals included:

- The introduction of a new Patient and Clinician Engagement (PACE) meeting to give patient groups and clinicians a stronger voice in SMC decisions for end of life and very rare conditions.
- Pharmaceutical companies will also have an additional opportunity in the new process to put forward a Patient Access Scheme, which makes their medicines more cost-effective.

The SMC believes that these changes could benefit up to 1,500 patients in the first year through access to these new medicines.

2. Progress

The timeframe announced in January outlined that the changes would begin to take effect from May 2014. Since then SMC has been working extremely hard making preparations to put those proposals into practice for the benefit of patients in Scotland.

2.1. Meetings to be held in public from May 2014

As proposed in 2013, our meetings will be held in public from May 2014 and plans for this change are well underway. Two new public involvement officers have been recruited to support the transition to meeting in public and improving engagement with patients and the public in the SMC process. Work is well underway to ensure that a wide range of people are aware the meetings will be in public and that they run smoothly. We will provide more information on public access to meetings on our website in the next few weeks.
2.2. Access to medicines for end of life care and very rare conditions

We have worked hard to find a way to enable access to medicines for end of life care and very rare conditions in Scotland.

We have established the definitions SMC will use for end of life, orphan (for very rare conditions) and ultra-orphan medicines (for extremely rare conditions). These definitions are broader than those currently used by NICE and EMA. We will ask pharmaceutical companies to state in their submission whether the medicine is in one of these categories.

We have outlined plans to introduce a new Patient and Clinician Engagement (PACE) group meeting to give patient groups and clinicians a stronger voice in SMC decisions for end of life and very rare conditions. We are involving representatives from patient groups, clinicians, and the pharmaceutical industry as we develop the detail.

We are engaging with groups such as the Scottish Cancer Coalition and Rare Disease UK to explore the best way of securing Patient Interest Group representation and also with the NHS Managed Clinical Networks for clinician representatives.

The PACE meeting will be a new step in the process that comes in when the New Drugs Committee view is that the medicine cannot be recommended for use in NHSScotland. The PACE meeting will give patients and clinicians a voice on the benefits of the medicine and will take into account aspects of the medicine’s ‘value’ such as disease severity, unmet need and impact on carers. There will also be an additional opportunity at this point for the company to put forward a Patient Access Scheme, to make the medicine more cost-effective.

For ‘ultra-orphan’ medicines that are used to treat extremely rare conditions, affecting up to 100 people in Scotland, we will introduce a new type of assessment that considers more than the direct health impact on the patient, including: the nature of the condition, the impact of the medicine, the impact of the technology beyond direct health benefits, the strength of the case and value for money. We also plan, where required, to capture patient and clinician views on the value of the medicine in a PACE meeting. The company will still be asked for the cost per QALY but this will not be the focus of the decision.

2.3. Timetable

We now wish to put the changes for end of life medicines and medicines for rare conditions into practice as soon as possible so that patients can benefit at the earliest opportunity. The Cabinet Secretary’s announcement on January 31 stated
that SMC will assess end of life and orphan medicines using the more flexible approaches from May 2014. Broadly, the timetable is as follows:

Mar 2014  Communications sent to stakeholders on new arrangements
6 May 2014  SMC meetings held in public
            Submissions received from companies in May onwards will be able to use the new process
Jun 2014  SMC’s New Drugs Committee considers May submissions
Aug 2014  First PACE meeting(s) to consider medicines for end of life care and very rare conditions
Sep 2014  SMC decision with input from PACE

This would mean the first decisions with the new processes in place would be published in October 2014.

3. Making the changes happen

For the benefit of patients and the NHS in Scotland SMC processes are extremely robust. The new processes we are implementing are a step change in increasing access to medicines for end of life care and very rare conditions and we know they need to be equally effective, robust, transparent and able to withstand scrutiny. At the same time SMC is extremely keen to make these changes happen without any undue delay for patients.

Scottish Medicines Consortium
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