PACE (Patient & Clinician Engagement) Overview Document

Process Changes for End of Life and Very Rare Conditions (orphan and ultra-orphan medicines)

Introduction

The Scottish Medicines Consortium (SMC) is changing the way it evaluates end of life medicines and medicines to treat very rare conditions. This is as a result of an extensive review of how new medicines are introduced in Scotland. One important change is the introduction of a Patient and Clinician Engagement (PACE) group which will give patient groups and clinicians a stronger voice in SMC decision making.

The document outlines:

- the background to why the changes are happening
- how they are being introduced
- a proposal for how the process will work
- timelines for introducing the new process

Background

In October 2013, the Scottish Government published its response to the Health and Sport Committee inquiry into access to new medicines. The response states that the Health and Sport Committee recognised that existing cost-effectiveness thresholds are not always appropriate for end of life medicines or for medicines to treat very rare conditions. The Cabinet Secretary directed SMC to apply more flexible approaches in the evaluation of these medicines, as a first step in a wider process to determine Scotland’s requirement for a value-based approach for the health technology assessment of new medicines.

SMC set up a Task and Finish Group to undertake a rapid review of processes and a report was submitted to the Cabinet Secretary in December 2013. On 31st January the Cabinet Secretary for Health and Wellbeing welcomed the report and asked SMC to have the recommendations in place by May 2014. The recommendations included the concept of a Patient and Clinician Engagement Group to give patient groups and clinicians a stronger voice in SMC decisions for medicines used at the end of life and for very rare conditions.

Consultation

This is a challenging timescale. SMC will include as much input from both a patient and clinician perspective as possible in the development of how the new process operates.

Patient and Clinician Engagement (PACE) Information

The definitions SMC will use for end of life, orphan (for very rare conditions) and ultra-orphan medicines (for extremely rare conditions) are stated below.
End of life medicine: “A medicine used to treat a condition at a stage that usually leads to death within 3 years with currently available treatments.”

Orphan medicine: “A medicine with European Medicines Agency (EMA) designated orphan status (i.e. conditions affecting fewer than 2,500 people in a population of 5 million) or a medicine to treat an equivalent size of population irrespective of whether it has designated orphan status.”

Ultra-orphan medicine: “A medicine used to treat a condition with a prevalence of 1 in 50,000 or less (or around 100 people in Scotland”).

These definitions are broader than those currently used by the National Institute for Health and Care Excellence (NICE) and the EMA. For orphan and ultra-orphan medicines the definitions will apply to the full population of the licensed indication.

How will the new process work?

We will ask pharmaceutical companies to state in their submission to SMC whether the medicine is in one of these categories and to provide supporting evidence and rationale.

End of life and orphan medicines

A submission for an end of life or orphan medicine will be made using the same submission form as before.

The medicine will be evaluated by the New Drugs Committee (NDC) in the usual way. If the advice for the medicine is ‘not recommended’ following NDC, the pharmaceutical company can choose to request that SMC convenes a PACE meeting. This process will add an additional 1-3 months onto the assessment timelines, but should avoid many of the resubmissions that would previously have been made.

PACE meetings will generally take place on the second Tuesday afternoon of each month in the SMC premises in Glasgow. It is proposed that all referrals to PACE will take place on the next available date and that up to two PACE meetings could be held in an afternoon.

Each PACE Group will be tailored to the medicine under consideration. The meeting will be chaired by a member of NDC/SMC or someone with specific experience of the SMC process and supported by SMC staff and, where possible, a public partner from the Patient and Public Interest Group (PAPIG). Representatives will be sought from relevant PIGs and clinicians from the relevant specialty (identified by relevant Managed Clinical Networks). The company will also be able to make a brief statement for consideration by the PACE group.

Capturing a medicine’s benefits

The aim of the PACE group is to describe the added benefits of the medicine, from both patient and clinician perspectives, that may not be fully captured within the conventional clinical and economic assessment process.

These may include, but are not limited to:

Clinical Issues:
For example: unmet need, severity of the condition, specific patient groups that may benefit more from use of the medicine, place in the patient pathway, service/infrastructure changes/benefits as a result of using the medicine.

May 2014
**Added value of the medicine for the patient:**
For example: impact on quality of life such as the ability to work or continue in education/function, symptoms such as fatigue, pain, psychological distress, also factors such as convenience of the treatment, whether it allows self-care or the ability to maintain independence and dignity, out of pocket expenses.

**Added value of the medicine for the patient’s family/carers:**
For example: time for accompanied visits for treatment, requirement for assisting the patient with personal care and support, out of pocket expenses, impact on family life, and impact on the carer’s ability to work.

A PACE template will be completed during the meeting and the content agreed by group members. This will be included in the SMC meeting papers for the medicine alongside the NDC detailed advice document, company comments, PIG submission(s) and any new or revised Patient Access Scheme (PAS) submission. The output from the PACE group will be a major factor in the SMC decision.

**Ultra-orphan medicines**

A submission for an ultra-orphan medicine used in extremely rare conditions will be assessed in a different way to the current process, although the submission will move through the New Drugs Committee and the Scottish Medicines Consortium in the same way as before.

To assess ultra-orphan medicines SMC will use a framework of explicit decision-making criteria. The criteria will include: the nature of the condition, the impact of the medicine, the impact of the technology beyond direct health benefits and on specialist services, costs to the NHS and Personal Services and value for money. This approach is consistent with the interim methods being explored by NICE in England, and therefore has the potential to address the issues raised by patient groups in relation to ensuring equitable access to medicines for rare diseases for residents of Scotland and those in England and Wales. A cost-effectiveness ratio will still be requested as part of the company submission to assess value for money but there may be circumstances in which the choice of economic appraisal methodology has to be more flexible given the available data and nature of the condition.

It is important to capture clinicians’ and patients’ views on ultra-orphan medicines through the PACE approach, if required, and this would happen in the same way as described under end of life/orphan medicines.

**Patient Access Schemes**

If the NDC’s advice for an end of life, orphan or ultra-orphan medicine is ‘not recommended’, the company will also have the option to offer a new or revised Patient Access Scheme aimed at making their product better value for the NHS in Scotland.
Figure 1: Sample PACE timeline

Day 0
5th May
Company submission deadline 12noon

Day 7
12th May
Submission scheduled

Day 11
16th May
Clinical & umbrella PIGs informed of provisional PACE

Day 46
20th June
Deadline for SMC validation of Eol/Orphan status

Day 50
24th June
New Drugs Committee Meeting

Day 53
27th June
NDC advice to company

Day 56
30th June
Deadline for patient interest group submission to PACE/SMC

Day 67
11th July
Deadline for company to confirm PACE request and of revised or new PAS

Day 81
25th July
Clinical networks & umbrella PIGs confirm PACE Attendance and submit statements for PACE

Day 88
1st Aug
PACE documentation sent to participants

Day 99
12th Aug
PACE Meeting

Day 100
13th Aug
PACE request for additional company information if required

Day 105
18th Aug
PACE template finalised (output)

Day 106
19th Aug
PACE template added to SMC papers

Day 108
21st Aug
SMC papers on website

Day 120
2nd Sept
SMC Meeting

Day 123
5th Sept
Advice issued to company and NHS Scotland

Day 181
13th Oct
New advice published on SMC website
We have agreed to have the new process in place for medicines submitted from the May submission date. The timescales in Figure 1 (above) reflect how medicines will move through the new process. The first decisions relating to medicines that are affected by the new processes would be published in October 2014.

Figure 2: Integration of PACE within Scottish Medicine Consortium’s submission process