PACE (Patient & Clinician Engagement) Overview Document

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

Introduction

The Scottish Medicines Consortium (SMC) has changed the way it evaluates end of life medicines and medicines to treat very rare conditions including the introduction of a Patient and Clinician Engagement (PACE) meeting which will give patient groups and clinicians a stronger voice in SMC decision making.

This document outlines:

- the background to why the changes happened
- Description of how the process works
- Process timelines

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.

Definitions for end of life, orphan and ultra-orphan medicines

The definitions that SMC 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 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 does the process work?

SMC now asks 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.

1) End of life and orphan medicines

A submission for an end of life or orphan medicine is made using the standard SMC submission form.

The medicine is 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 adds an additional 1-3 months onto the assessment timelines.

PACE meetings take place on the second Tuesday afternoon of each month in the SMC premises in Glasgow with up to three PACE meetings held in an afternoon.

Each PACE meeting is tailored to the medicine under consideration. The meeting is chaired by the NDC vice-chair or someone with specific experience of the SMC process and supported by SMC staff and a public partner. Representatives are sought from patient groups and clinicians from the relevant specialty (identified by Managed Clinical Networks and regional Cancer Networks). The company can also submit 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.

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

2) Ultra-orphan medicines

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

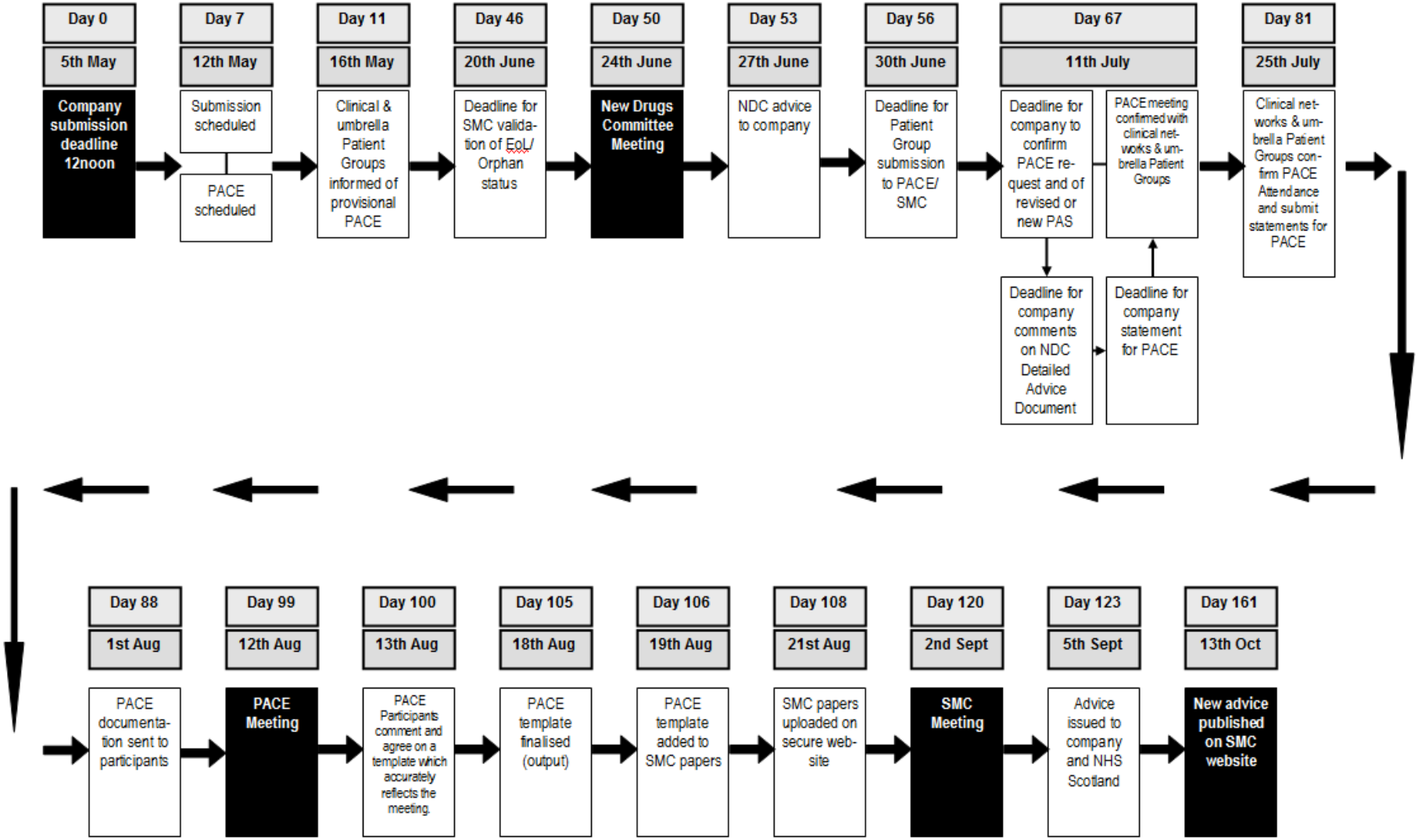
To assess ultra-orphan medicines SMC uses a framework of explicit decision-making criteria. The criteria includes: the nature of the condition, the impact of the medicine, the impact of the technology beyond direct health benefits and on specialist services, costs to the NHS and Personal Services and value for money. A cost-effectiveness ratio is still 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 happens 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 also has 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



The first decisions relating to medicines that are affected by the new processes were published in October 2014.

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