Treatment Appraisals in Scotland

What is the SMC and their role in medicine assessment?

The Scottish Medicines Consortium (SMC) provides advice to NHS Scotland about the value for patients of newly licensed medicines. Medicines is a broad term that includes treatments and gene therapies, such as Zolgensma. Before a medicine can be prescribed routinely in Scotland, it has to be accepted for use by the SMC. As the NHS has limited resources, the SMC works to make sure that those medicines which represent good value for money are accepted for routine use as quickly as possible so that they can benefit patients. The SMC is made up of lead clinicians, pharmacists, and health economists together with representatives of health boards, the pharmaceutical regulatory body and members of the public.

It is important to note that when the SMC approves a treatment, this only covers Scotland. England have its own governing body, the National Institute for Health and Care Excellence (NICE) where Northern Ireland and Wales tend to follow NICE's advice.

How do SMC processes work?

When a pharmaceutical company has completed its clinical trials and data collection, it will submit an application for SMC appraisal. Once this application is submitted, the SMC Committee will consider a range of factors when deciding whether to accept a new medicine under review, such as the clinical and health economic evidence provided by the manufacturer as well as evidence submitted by patient groups to decide whether the medicine provides value for money.

The SMC can make the following decisions on a medicine: accepted, accepted with a restriction(s) (for example, the medicine can only be accepted in a particular group of patients with the condition), accepted on an interim basis, or not recommended. For rare disease treatments, the most likely outcome is an interim acceptance which allows patients to access the treatment for a certain time whilst additional evidence is collected to address any outstanding uncertainties. Interim acceptance is similar to NICE's managed access arrangement in England.

What types of SMC appraisals are used to assess rare disease treatments?

All treatments will follow the general review process (explained below). However, for some very rare conditions, treatments may be reviewed under the ultra-orphan pathway. The ultra-orphan pathway is a form of managed access agreement for very rare conditions affecting fewer than 1 in 50,000 people - around 100 people or fewer in Scotland. If a treatment under this pathway is considered clinically effective by the SMC, then it will be made available on the NHS for at least three years while information on its effectiveness is gathered. After which, the SMC will then review the additional evidence and make a final decision on its routine use in NHS Scotland.

What are the different stages of an SMC appraisal?

Regardless of whether a treatment falls under the ultra-orphan pathway, the following steps are followed to evaluate a treatment:

1) Preliminary review

Once the manufacturers have submitted an application, the New Drugs Committee (NDC) will review and discuss the scientific evidence of the medicine. If the outcome of the NDC discussion on a medicine is 'Not Recommended' and the medicine is for a rare condition or end of life, the manufacturing

company can request the medicine is discussed at a PACE (Patient and Clinician Engagement) meeting before it goes to the SMC Committee.

2) PACE meeting

The PACE meeting gives patient groups and clinicians a strong voice in SMC decision making. The aim of the meeting is to describe the added benefits of the medicine, from both patient and clinician perspectives, which may not be fully captured within the conventional clinical and economic assessment process. There is an opportunity for submitting patient groups to participate in PACE meetings and, together with clinical experts, the meeting establishes the added value a new medicine will bring to the lives of those with the condition and that of their families/carers. The output of the PACE meeting is called the PACE Statement which is included in the papers for SMC Committee to consider. A summary of the PACE meeting is presented at the SMC Committee, together with summaries of the NDC discussion and the patient group submission(s).

3) SMC Committee meeting

The application is then reviewed by the SMC Committee who will review the medicine's scientific evidence provided by the company. In addition to analysing a new medicine's scientific evidence provided by the pharmaceutical company, understanding the experiences of patients, their families and carers is a key element of the SMC decision making process. It is important for SMC members to fully understand how a new medicine impacts the quality of life of patients and families/carers. This enables SMC to make a fully informed decision on whether or not to recommend a new medicine. That's why it's very important that, as part of the assessment process, the SMC are able to capture the impact of conditions and the potential benefit new medicines may have on patients and families/carers through a patient group submission.

The meetings are held in public and are attended by pharmaceutical company representatives, patient group representatives and members of the public. Approximately 6 medicines are discussed at each Committee meeting. Submitting patient group representatives can contribute to the discussion about the medicine for which they have provided a submission, to answer questions from committee members or to clarify any points on their submission for Committee members.

The Committee votes on whether to accept or not recommend a medicine for NHS use in Scotland at the meeting based on the evidence presented. The final decision of the SMC is made public approximately 4 weeks later. If the medicine is accepted, this allows time for the pharmaceutical company to be informed and for the ADTCs (Area Drug and Therapeutic Committees) to be informed - they work with individual NHS Scotland health boards on their medicine formulary. If the medicine receives a 'not recommended' decision, the pharmaceutical company may then decide to resubmit the medicine to SMC.

A treatment may be rejected if it isn't deemed cost-effective which means that a treatment was deemed too expensive for very little to no benefit to the patient. As the health service has limited money to spend, difficult choices must be made about what treatments can be made routinely available. However, that's why it is important to have special pathways for rare disease in place (as these treatments do tend to be more expensive) to ensure the benefit is appropriately captured to properly demonstrate cost effectiveness which will enable the SMC to approve a treatment.