

SMC modifiers used in appraising new medicines

In assessing the relative clinical and cost effectiveness of new medicines, the Scottish Medicines Consortium (SMC) requires a robust clinical and economic case to be made and for the medicine to demonstrate value for money. In some specific situations SMC may exercise greater flexibility in its decision making to allow consideration of additional factors. These may allow SMC to accept either more uncertainty in the health economic case or a higher cost per Quality Adjusted Life Year (QALY). The additional factors include (but are not limited to) the following:

Where more uncertainty in the economic case may be accepted

Orphan medicines

The SMC has adopted the definition of orphan medicines provided by the European Medicines Agency (EMA) – “an orphan medicine is one licensed for treating or preventing life-threatening rare diseases affecting fewer than 5 in 10,000 people in the European Union”.

SMC requires that all submissions are comprehensive and that all sections of the product assessment form are completed. This requirement also exists for orphan drugs, for which a meaningful attempt needs to be made to present robust clinical and economic data. SMC recognises that orphan drugs may have a smaller clinical trials programme and, therefore, that less information than usual may be available for some sections (e.g. on efficacy and safety). On the other hand, other parts of the submission may require more detail, e.g. on the relevance of surrogate markers and the theoretical basis for their selection, which should then be related to quality of life data.

As with all products, the managed introduction and subsequent monitoring of orphan drugs needs to be a joint responsibility between the manufacturer and the NHS. If there is a significant lack of data on long-term outcomes with an orphan drug, this monitoring may include specific clinical audit and, where relevant, a patient register.

The assessment process for orphan drug submissions is the same as for all other drug submissions. However, recognising the limited data on efficacy, SMC will accept a greater level of uncertainty in the economic case. Additional factors, such as whether the drug: treats a life threatening disease; substantially increases life expectancy and/or quality of life; can reverse,

rather than stabilise, the condition; or bridges a gap to a “definitive” therapy, will also be considered in assessing both the level of uncertainty and cost per QALY which is acceptable.

Where a higher cost per QALY may be accepted.

SMC does not have a formal threshold cost per QALY below which cost-effectiveness would be considered shown and above which cost-effectiveness would be considered not to have been demonstrated. The cost per QALY is only part of a wider judgment of the value of a new medicine. Where the cost per QALY is relatively high, other factors also play a role in SMC’s assessment and may modify the final decision. These modifiers include (but are not limited to (see below)):

- Evidence of a substantial improvement in life expectancy (with sufficient quality of life to make the extra survival desirable). Substantial improvement in life expectancy would normally be a median gain of 3 months but the SMC assesses the particular clinical context in reaching its decision;
- Evidence of a substantial improvement in quality of life (with or without survival benefit);
- Evidence that a sub-group of patients may derive specific or extra benefit and that the medicine in question can, in practice, be targeted at this sub-group;
- Absence of other therapeutic options of proven benefit for the disease in question and provided by the NHS;
- Possible bridging to another definitive therapy (eg bone marrow transplantation or curative surgery) in a defined proportion of patients and
- Emergence of a licensed medicine as an alternative to an unlicensed product that is established in clinical practice in NHS Scotland as the only therapeutic option for a specific indication. Some possible examples include caffeine injection for the treatment of apnoea of prematurity and betaine anhydrous for the adjunctive treatment of homocystinuria.

SMC also looks at any other special issues which may have been highlighted by the manufacturer of the medicine, by clinical experts and/or by patient groups. These special issues are usually very specific to the drug or disease under consideration and are thus not readily categorised.

The modifiers are only applied for a relatively high cost per QALY when the committee is satisfied that the clinical and economic case for the medicine is robust.

June 2012

