Assessment of medicines for end of life care and very rare conditions (orphan and ultra-orphan medicines) in Scotland

Report for the Cabinet Secretary for Health and Wellbeing

20 December 2013

A rapid review undertaken by the Task and Finish Group
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On behalf of the Scottish Medicines Consortium
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<td>ADTC</td>
<td>Area Drug &amp; Therapeutic Committee</td>
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<td>Cost Consequence Analysis</td>
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<td>EMA</td>
<td>European Medicines Agency</td>
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<td>EoL</td>
<td>End of Life</td>
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<td>HST</td>
<td>Highly Specialised Technologies</td>
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<td>Health Technology Assessment</td>
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<td>Individual Patient Treatment Request</td>
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<td>New Drugs Committee</td>
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<td>NICE</td>
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<td>QALY</td>
<td>Quality-Adjusted Life-Year</td>
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<td>SMC</td>
<td>Scottish Medicines Consortium</td>
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<td>T&amp;FG</td>
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<td>VRC</td>
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Scottish Medicines Consortium

Task and Finish Group: Assessment of medicines for end of life care and very rare conditions (orphan and ultra-orphan medicines)

Final report from the Scottish Medicines Consortium

Executive summary

The Scottish Medicines Consortium (SMC) is a consortium of Area Drug and Therapeutics Committees (ADTCs), established in 2001 to advise NHS Scotland on the use of all new medicines. It has a fast, efficient, robust and independent process, based on the principles of evidence based medicine and health economics, using rigorous and widely accepted methodologies. In 2011 three Patient Interest Groups submitted petitions to the public petitions committee expressing concerns that some patients were having difficulty in being able to access effective new medicines. This prompted the Government decision to institute a review of end to end processes for access to new medicines. The Scottish Parliament’s Health and Sport Committee review into access to new medicines was published in July 2013 and the Scottish Government published its response to the Health and Sport Committee inquiry in October 2013. In addition, in October 2013 the Cabinet Secretary for Health and Wellbeing, Mr Alex Neil, directed SMC to undertake a rapid review to establish more flexible approaches in evaluating medicines for end of life care and the treatment of very rare conditions.

To carry out this review SMC established a Task and Finish Group (T&FG), with representatives from key stakeholders, including clinicians, Patient Interest Groups, the pharmaceutical industry and the SMC Patient and Public Involvement Group (PAPIG). The T&FG acknowledged that there has been variation in acceptance rates by SMC for medicines used at the end of life and medicines to treat very rare conditions. The T&FG was also mindful, however, that SMC has extremely effective and robust systems which should remain at the heart of the process. The T&FG was of the view that this reflected both the utilitarian approach taken by SMC, with the use of conventional cost-effectiveness thresholds for all medicines, and the lack of an evidence base for what people in Scotland value to guide their decisions. The aim of the T&FG was to present a proposal to Scottish Government “that would deliver substantially improved access” to these medicines for Scottish patients compared to the current system. The T&FG noted that this would be the first step in a process to determine a value-based approach for the health technology assessment of new medicines in Scotland (i.e. a Scottish Model of Value).

The T&FG agreed on the definition that SMC would use for End of Life (EoL) medicines and agreed that the brief given in relation to medicines for very rare conditions should encompass both orphan and ultra-orphan medicines (these terms are therefore used instead of medicines for ‘very rare conditions’ throughout this report). The T&FG explored a range of different methods of assessment that could be applied to these three categories of medicine and considered these options against a set of agreed key principles.

Whilst a range of approaches were considered for these categories it was concluded that a shared methodology would be appropriate for EoL and orphan medicines. The two options considered in detail were the application of a Quality Adjusted Life Year (QALY) weighting and a new approach involving Patient and Clinician Engagement (PACE) alongside existing SMC modifiers. There was consensus within the T&FG that the QALY weighting option should not be supported and that the PACE option should be recommended to Scottish Government.
For ultra-orphan medicines it was agreed that SMC should introduce a decision-making framework that is not based on the cost per QALY. The T&FG considered four different health economic techniques that could be adopted. It was agreed that the preferred approach would be for SMC to use a framework of explicit criteria for evaluating ultra-orphan medicines, without performing weighting and scoring. These criteria would include the nature of the condition, the impact of the medicine, the impact of the technology beyond direct health benefits, and value for money.

There was consensus across all stakeholder groups in support of the proposed approaches.

**Recommendations**

1. SMC should introduce new, more flexible approaches for the assessment of EoL medicines, orphan medicines and ultra-orphan medicines.

2. SMC should adopt the following methodologies, which will substantially improve access to these new medicines:

   **Medicines for EoL and orphan medicines** - If the SMC New Drugs Committee (NDC) advises that a medicine does not meet the conventional thresholds for cost-effectiveness, a PACE Meeting may be convened to allow SMC to clearly establish the views of clinicians and Patient Interest Groups on the need for the medicine, its clinical benefits, optimal place in therapy and the patient perspective. The output of the PACE Meeting will play a significant part in informing the SMC decision for the medicine, with a more powerful influence than the current modifiers.

   **Ultra-orphan medicines** - Recognising that under current SMC processes ultra-orphan medicines are unlikely to be accepted for use, SMC should introduce a decision-making framework that is not based on the cost per QALY for these medicines. A new framework of explicit criteria for evaluating these medicines, without performing weighting and scoring, will be introduced. A PACE Meeting may also be convened for these medicines.

3. SMC should work with stakeholders to introduce these new approaches as quickly as possible.

4. SMC should encourage early resubmissions for medicines that have been ‘not recommended’ for use under the current system.

5. SMC should ensure that any changes to how SMC works must be clear and understandable to the public. It was also agreed that the definitions should be applied in an enabling way, to allow flexibility.

6. SMC should work with patients and clinicians to ensure there is understanding of the new processes and to enable and maximise their contributions.

7. Scottish Government should engage with the NHS to give further consideration to mechanisms of monitoring patient outcomes after treatment with EoL, orphan and ultra-orphan medicines.

We note the Scottish Government’s intention that the SMC’s system of medicines appraisal is given time to establish itself. On this basis, the following two recommendations are made:
8. There should be an independent review of the experience with the new SMC approaches, and a decision on when this should be initiated would be taken no later than 12 months after the new approaches are introduced.

9. SMC should work with the Scottish Government to determine and enable the research required to underpin an evidence-based approach to a Scottish Model of Value.
1. Introduction

The Scottish Medicines Consortium (SMC) was established in 2001 as a consortium of Area Drug and Therapeutics Committees (ADTCs). For the last 12 years it has performed the difficult task of appraising all new medicines launched in the UK market and giving advice to the NHS in Scotland on their use. It has fulfilled a challenging role by developing a fast, efficient, robust and independent process that has responded and adapted to changing needs and new circumstances. Its procedures are based firmly on the principles of evidence based medicine and health economics, using rigorous and widely accepted methodologies. Comparisons with other Health Technology Assessment (HTA) agencies internationally have shown that SMC has one of the fastest appraisal processes in the world allowing Scottish patients early access to new medicines.

Despite SMC’s progress in adapting to changing needs in Scotland over the last decade, important concerns have been expressed recently about patients having difficulties in being able to access effective medicines, prompting the decision to institute a review of SMC processes and access arrangements. The Scottish Parliament’s Health and Sport Committee review into access to new medicines was published in July 2013 and in November 2013 the Scottish Government published its response to the Health and Sport Committee inquiry.

The Health and Sport Committee considered that existing cost-effectiveness thresholds are not always appropriate for End of Life (EoL) medicines or for medicines to treat very rare diseases. The Cabinet Secretary therefore directed SMC to apply different approaches in the evaluation of these medicines, including a rapid review of the wider aspects of value and Quality Adjusted Life Years (QALYs) in order to increase patient access to these medicines and to report the findings before Christmas 2013. It was noted that this would be the first step in a wider process to determine Scotland’s requirement to develop a value-based approach for the health technology assessment of new medicines (i.e. a Scottish Model of Value).

SMC set up a Task and Finish Group (T&FG) to undertake this rapid review and produce a report for the consideration of the SMC Committee, before submission of findings to the Scottish Government.

2. SMC Task and Finish Group

The T&FG was chaired by Professor David Webb, Christison Professor of Therapeutics and Clinical Pharmacology at the University of Edinburgh. The membership is listed at Appendix 1. The T&FG met three times (in October, November, and December 2013).

Outwith the meetings, steps were taken to ensure additional stakeholder engagement; for example, SMC held a teleconference with Patient Interest Groups to discuss implementation of the new approaches. Representatives from Breakthrough Breast Cancer, Myeloma UK, Rare Disease UK and Prostate Cancer UK took part in this event. Additionally individual members of the T&FG undertook further high level engagement work with relevant constituents e.g. the Scottish Cancer Coalition (see Appendix 2 for participating organisations) and the Association of the British Pharmaceutical Industry. The outputs of these discussions can be made available to the Scottish Government.

The T&FG considered a range of approaches SMC could take for the assessment of medicines at the end of life, orphan and ultra-orphan medicines (previously described as very rare conditions), and the clinical, operational, legal and ethical implications of these
approaches. The remit and the work undertaken to support the T&FG are shown in Appendix 3.

2.1 Key principles

The T&FG agreed that:

The proposed option must deliver substantially improved access to new medicines used at the end of life and for very rare conditions for Scottish patients, compared to the current system, reflecting the direction from Scottish Government.

Options for change were evaluated against the following general principles. That they were:

- Robust
- Transparent
- Consistent
- Equitable
- Scientifically rigorous (e.g. should not use arbitrary definitions)
- Feasible for SMC to introduce quickly
- Limit scope for gaming
- Limit scope for legal challenge
- Should not delay patient access to new medicines (i.e. should be timely).
- Should feed into the decision making process (not be ‘for the sake of it’).
- Should not destabilise SMC’s assessment of other medicines within its remit
- Be compatible with the move towards a Scottish Model of Value, as it is currently understood

3. Definitions

There is no single, widely accepted definition for rare diseases, a term that is used interchangeably with orphan diseases. There are estimated to be between 5,000 and 7,000 rare diseases, but only a relatively small proportion of these have available treatments. Collectively, rare diseases are not rare; in Scotland, 300,000 people are likely to be affected by a rare disease at some point in their lives (1 in 17 people). The Rarer Cancers Foundation suggests that between 30% and 50% of all cancer cases could be classified as ‘rarer’. Following application from the manufacturer, the European Medicines Agency (EMA) can designate orphan status to a medicine that treats a condition affecting fewer than 5 in 10,000 people, which equates to approximately 2,500 people in the Scottish population of 5 million. Ultra-orphan diseases are commonly regarded as having a prevalence of 1 in 50,000 people, which equates to around 100 people in the Scottish population of 5 million.

The brief from Scottish Government asked SMC to consider new approaches for medicines at the end of life and very rare conditions. The T&FG considered very rare conditions would encompass both orphan and ultra-orphan medicines (these terms are therefore used instead of medicines for ‘very rare conditions’ throughout this report).

3.1 End of Life medicines

The T&FG found it very challenging to define EoL medicines. The criteria currently used by NICE to define end of life were considered, as they are one of the few HTA agencies to use this categorisation. The T&FG agreed that these criteria do not adequately reflect a medicine’s benefits in terms of quality of life (as well as extension to life) and also that the requirement around 24 months of life expectancy was too specific and restrictive.
The T&FG agreed definition is:

- **EoL medicine:** “A medicine used to treat a condition at a stage that usually leads to death within 3 years with currently available treatments.”

### 3.2 Orphan medicines

The T&FG agreed that a new approach should apply to all medicines that have EMA designated orphan status (i.e. which would equate to conditions affecting fewer than 2,500 people in the Scottish population of 5 million) plus medicines that are for the treatment of an equivalent size of population (i.e. this would allow inclusion of medicines licensed for specific sub-populations with a given condition, as well as medicines to treat a rare condition in the unusual situation where the company has not requested orphan designation).

The T&FG agreed definition is:

- **Orphan medicine:** “A medicine with 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.”

### 3.3 Ultra-orphan medicines

As there is no universally accepted definition of an ultra-orphan medicine, the T&FG agreed that a new approach should apply to all medicines that treat conditions that meet the commonly accepted definition noted above (i.e. have a prevalence of 1 in 50,000 people, which equates to around 100 people in the Scottish population of 5 million).

The T&FG agreed definition is:

- **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).”

It was agreed that the language used to describe the definitions needs to be clear and understandable to the public. It was also agreed that the definitions should be applied in an enabling way, to allow flexibility.

### 4. New assessment approaches

The T&FG considered the options for assessing EoL, orphan and ultra-orphan medicine in the context of the agreed definitions. It was noted that there is considerable overlap between EoL and orphan medicines while ultra-orphan medicines were a separate, distinct category. The T&FG concluded that there would be merit in SMC adopting two different, more flexible approaches for:

- EoL and orphan medicines
- Ultra-orphan medicines

### 4.1 End of Life and orphan medicines

The T&FG agreed the principle that SMC should implement a new approach in the review of these medicines. Two options were considered in detail:
4.2 QALY weighting

Application of a QALY weighting was proposed as a simple approach that could be adopted quickly and with minimal impact on the existing SMC process. This would involve the use of a simple multiplier to improve the ICER by raising the value of the QALYs gained to bring it within the conventionally accepted threshold. Having explored this approach in some depth, however, no reliable evidence base could be found for specific weights that could be applied. Any weighting selected would be entirely arbitrary. An analysis of medicines meeting the agreed definition that had been considered and ‘not recommended’ by SMC in the last 2 years indicated that a 1.67 weighting (as implied by NICE’s EoL criteria) would change relatively few of these decisions. A multiplier of 3 would require to be applied to allow the majority of these medicines to be accepted and a multiplier of 4 would require to be applied for all medicines to be accepted.

The suitability of this approach was considered in the context of the proposed principles (see section 2.2). The benefits were transparency and consistency, and being feasible to introduce rapidly. The risks were in relation to robustness, scientific rigour and the difficulty of revising the weightings at a later stage if this was seen to be desirable as part of a move towards a Scottish Model of Value. In the absence of evidence to support the level of weighting to be applied there was consensus in the T&FG that the QALY weighting option could not be supported.

4.3 Strengthened Patient and Clinician Engagement (PACE)

The T&FG then considered a PACE option for these medicines. This would involve SMC convening a group of specialist clinicians and Patient Interest Groups to determine the need for the medicine, its clinical benefits, optimal place in therapy and the patient perspective. The current SMC process would be followed up until the New Drugs Committee (NDC) stage. If, at that stage, NDC considered the medicine was unlikely to be accepted by SMC in line with the conventional cost-effectiveness threshold, SMC would agree with the company that a PACE Meeting would be convened. Specialist clinicians and Patient Interest Groups would be asked to provide further information on the severity and level of unmet need in the condition to be treated, the perceived benefits of the medicine and its place in the treatment pathway, in particular how it might be used relative to current treatment against which it has been compared within the submission. In establishing a PACE Meeting, SMC would seek greater clarity on the potential role of the new medicine, which would help strengthen the case for the acceptance of the medicine by SMC. This approach would include criteria for treatment relative to current therapies (e.g. patients expected to benefit most) and other aspects of prescribing, such as continuation rules, and the ability to gather further evidence on patient outcomes. A key output from the PACE Meeting would be more clarity on the clinicians’ and patients’ views on the need for the medicine, e.g. on the basis of ‘value’ which is not fully captured by the QALY, such as disease severity and the level of unmet need in that population, and the impact on carers. Implicitly, this could support acceptance of medicines with higher cost per QALYs for EoL and rare medicines. As part of the T&FG’s work, the types of question that would be asked of clinicians involved in the PACE Meeting were piloted with clinicians on the T&FG in relation to a small group of SMC ‘not recommended’ cancer medicines. Feedback indicated that this approach is feasible. It is also proposed that the company should have the option to put forward a Patient Access Scheme (PAS) at the PACE stage, or to amend an existing PAS. This is a change from existing SMC process where a PAS can only be considered at time of submission and has been raised as a disadvantage of the existing process. This change would require discussion and agreement with the Patient Access Schemes Assessment Group (PASAG).

After this step the SMC Committee would then consider the medicine in line with the existing process, but with the output from the PACE Meeting playing a significant part in the decision-
making, with a more powerful influence than the current modifiers. A key strength of this approach would be allowing treatment to be targeted at populations within the marketing authorisation considered by clinicians and patients as most likely to benefit from the new medicine. The T&FG also considered that this approach would be in keeping with current developments in medicines regulation, such as adaptive licensing, as these would be a driver for HTA agencies to develop their processes to enhance input from clinicians and patients at an early stage.

The suitability of this approach was considered in the context of the proposed principles (see section 2.1). The benefits were that it would feed into the existing decision-making process, would not destabilise SMC’s assessment of other medicines within its remit, would be feasible to introduce quickly and would be compatible with the move towards a Scottish Model of Value. In addition, this approach would have the benefit of SMC advice being more fully informed by a clinician and patient perspective. The main risk was perceived to be the additional time that this step might add to the process. SMC currently appraises all new medicines earlier than any other HTA agency. Where the PACE step is invoked it would require a ‘pause’ in the SMC process of less than 3 months, which the T&FG noted to be quicker than the resubmission process following ‘not recommended’ advice. The T&FG considered the need to balance the opportunity for increased patient access to new medicines against the need for an extension to timelines. The T&FG noted the potential for smoother implementation of advice at local level due to the early close involvement of clinicians and clinical networks. It may also have the potential to complement the proposed Peer Approved Clinical System (PACS) that will replace Individual Patient Treatment Requests (IPTRs). From the industry perspective it was noted that this more qualitative approach could lack predictability for submitting pharmaceutical companies.

The detail of how the PACE step would function has not yet been fully developed. An outline of the proposed new approaches in the context of the existing SMC process is shown at Appendix 4. To mitigate the risk of extending the assessment process it was suggested that SMC would work with all stakeholders to ensure the impact on timelines is minimised and should also consider the steps it could take to plan in advance for PACE Meetings. The T&FG agreed that face-to-face meetings would be preferred in the first instance but that teleconferencing or videoconferencing could be supported. It was also agreed that for orphans and ultra-orphan medicines, where there may be very limited clinical expertise, there may be a need to involve clinicians from other parts of the UK in the process. The success of this approach would require the support of clinicians, clinical networks and Patient Interest Groups across Scotland and clear processes would need to be developed, both within SMC and within the networks, to support this enhanced involvement.

4.4 Assessment of ultra-orphan medicines

After consideration of a range of sources of evidence, including international literature on the approach taken by other HTA agencies, a report provided by the Office of Health Economics, and Professor Routledge’s report to the Scottish Government, the T&FG concluded that the rationale for using a decision-making process not based on the cost per QALY was clear for medicines that would be defined as ultra-orphans. The T&FG was supportive of a different methodology for ultra-orphan medicines to treat conditions with only around 100 or fewer eligible patients in Scotland.

The T&FG considered four different evaluation systems for medicines that would meet the ultra-orphan definition. Cost consequence analysis and multi-criteria decision analysis were investigated as potential approaches for use, as well as the NICE interim process for highly specialised technologies and an adaptation of the existing SMC modifiers. Broad definitions of these are given below alongside a diagram showing these approaches on a qualitative/quantitative continuum:
• **Multi-criteria decision analysis (MCDA)** - specified criteria, weighted in advance and scored for each medicine.

• **Cost-consequence analysis (CCA)** - a list of factors and evidence, quantifying where possible and appropriate but acknowledging the difficulty in doing this.

• **Highly Specialised Technologies (HST) interim framework** – qualitative framework with headings for discussion or to summarise evidence that is mainly opinion or anecdote. Can also incorporate quantified evidence where available, in which case it could resemble cost-consequence analysis.

• **Modifiers** - as currently used by SMC, headings for a structured discussion but with no quantified evidence.

**Figure 1: illustration of qualitative and quantitative frameworks**

The T&FG agreed that the preferred approach would be for SMC to use a framework of explicit criteria for evaluating these medicines, without performing weighting and scoring. These criteria would include the nature of the condition, the impact of the medicine, the impact of the technology beyond direct health benefits, and value for money. This approach is consistent with the interim methods being explored by NICE in England, and would therefore have 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. The T&FG suggested that it would be important to capture clinicians’ and patients’ views on ultra-orphan medicines through the PACE approach, if required. It was noted that it would be desirable to retain a cost-effectiveness ratio as part of the company submission.

It was also noted that a degree of flexibility in relation to the number of eligible patients in Scotland would be required when implementing this approach.

**5. Circumstances in which SMC may reach ‘not recommended’ advice**

There was consensus within the T&FG that SMC must be able to issue ‘not recommended’ advice for EoL/orphan and ultra-orphan medicines in some circumstances. It was agreed that this situation might arise when there was a high degree of uncertainty about the clinical benefits associated with the medicine, a high likelihood that the medicine has extremely poor cost-effectiveness, and where the submitting pharmaceutical company has not engaged effectively with the SMC process. The T&FG noted an expectation that company engagement with SMC would improve with the changes to process, as company non-
submission has become particularly challenging for Health Boards, ADTCs and clinicians, and disadvantage Scottish patients.

6. Implementation of new approach

6.1 Operationalising the new approaches

SMC is continuing to work with other stakeholders on the details of the new PACE and ultra-orphan assessment process including:

- The requirement for the company to initiate the PACE mechanism.
- The structure of the PACE Meeting and the information to be considered.
- The constitution of PACE Meetings, including the possibility of groups such as the Scottish Cancer Coalition and Rare Diseases UK to work with the SMC Patient and Public Involvement Group to nominate Patient Interest Group attendees.
- The requirement that nominated clinician members are able to give a perspective on behalf of their clinical community.

6.2 Resubmissions

A review of SMC advice over the last 3 years indicates that there are around 60 ‘not recommended’ medicines/indications that may fit the definitions of EoL/orphan or ultra-orphan and therefore be appropriate for review under the new process. There may be a high demand for the assessment of these medicines. It is hoped that pharmaceutical companies will take the opportunity to present submissions under the new process at an early stage. The T&FG noted that SMC should engage with Health Boards and ADTCs to determine which medicines should have priority on resubmission according to patient needs.

6.3 Budget impact

Although the T&FG was clear about the need to increase patient access to new medicines, there was apprehension about the opportunity costs for NHS Scotland. Conventional thresholds for cost-effectiveness are in the region of £20,000-£30,000 per QALY but the T&FG noted updated research from the University of York to develop methods for the estimation of the NICE cost effectiveness threshold on the basis of the cost of QALYs which would be displaced by new treatments. The researchers concluded that the most relevant current threshold for decision making should be in the region of £13,000 per QALY (Claxton K, 2013).

The T&FG acknowledged that SMC has an important role in allowing access to medicines at prices that reflect value for money and the changes proposed may impact on this. Concerns were raised about unintended consequences of the new approaches, for example that they might reduce the incentive for pharmaceutical companies to propose a PAS.

The T&FG considered the potential impact of the policy changes on prescribing. Importantly, this development might lead to access to these new medicines for up to an additional 1,500 patients in the first year of full operation. SMC horizon scanning intelligence on new medicines in these categories in the 2014/15 pipeline suggests that the net budget impact in year 1 may be in the order of £70M, and there may be additional costs associated with resubmissions for medicines for which SMC has previously issued ‘not recommended’ advice.

The T&FG also noted that the NICE EoL criteria and the Cancer Drugs Fund have been estimated to increase drug costs by £750M within England and Wales; this equates to an
increase of 7.5% in the total drugs budget (Raftery J, BMJ 2013). These costs are in addition to the budget impact of medicines that are accepted under existing SMC processes and there will likely be additional costs for clinical services associated with the safe introduction of these new medicines.

6.4 Review of the new arrangements

The T&FG noted the Scottish Government’s intention that the SMC’s interim system of medicines appraisal be given time to establish itself and for a Scottish Model of Value to be developed.

The T&FG recommends that there is an independent review of the experience with the new SMC approaches and that a decision on when this should be initiated would be taken no later than 12 months after the new approaches are introduced. In addition, consideration may also be given to the need for continued regular monitoring, to determine how well the new approaches accommodate the advances in new medicines.

Noting the Government’s intention that this would be the first step in a process to determine a value-based approach for the health technology assessment of new medicines in Scotland (i.e. a Scottish Model of Value), the T&FG recommends that SMC should work with the Scottish Government to determine and enable the research required to underpin an evidence-based approach to a Scottish Model of Value.

In relation to measures of success, the T&FG expects that with the introduction of two new approaches for the assessment of medicines for EoL/orphan medicines and ultra-orphan medicines the acceptance rates for these types of medicine would be expected to increase and the number of ‘not recommended’ medicines decline.

7. Conclusions and next steps

The T&FG believes that the proposals presented in this report will deliver substantially improved access to medicines for EoL, and to orphan and ultra-orphan medicines, for Scottish patients, compared to the current SMC assessment process. The T&FG recommends that SMC should work with all stakeholders to implement the new ways of working as soon as practical, subject to receiving Scottish Government approval, and SMC believes these changes could be introduced for submissions received within 2 months of that notification.

SMC Task and Finish Group
20 December 2013
References


Raftery J. Value based pricing: can it work? BMJ 2013; 347: f5941
Appendix 1

Membership

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Dr Peter Robinson, Consultant in Paediatric Metabolic Medicine, NHS Greater Glasgow & Clyde
Professor Philip Routledge, Chair of All Wales Medicines Strategy Group
Professor Colin Suckling, Chair of SMC Patient and Public Involvement Group
Professor Angela Timoney, SMC Chair

SMC support team

Ailsa Brown, Lead Health Economist, SMC
Alison Campbell, NDC Co-Vice Chair
Dr Jan Jones, Principal Pharmacist, SMC
Anne Lee, Chief Pharmacist, SMC
Rosie Murray, Manager, SMC Admin Team
Robert Sanders, Project Officer, SMC Admin Team
Dr Andrew Walker, Health Economist, Glasgow University
Appendix 2

Attendees at the Scottish Cancer Coalition meeting:

- Bowel Cancer UK
- Breakthrough Breast Cancer
- Cancer Research UK
- Cancer Support Scotland
- CLIC Sargent
- Jo’s Cervical Cancer Trust
- Macmillan Cancer Support
- Maggie’s Centres
- Marie Curie Cancer Care
- MASSCOT
- Myeloma UK
- Prostate Cancer UK
- Prostate Scotland
- Roy Castle Lung Cancer Foundation
- Scottish Cancer Foundation
Appendix 3

Remit of the Task and Finish Group

To consider factors relevant to the evaluation of medicines for end of life care and the treatment of very rare conditions and make recommendations to the Cabinet Secretary for Health and Wellbeing on how to increase access for Scottish patients.

The T&FG will work to challenging timelines, with monthly meetings during October – December 2013, to consider:

- The governing principles for the assessment of medicines at the end of life or for the treatment of very rare diseases.
- The clinical, operational, legal and ethical implications of a broader approach to the health technology assessment of medicines at the end of life or for the treatment of very rare diseases.
- The approaches taken by other Health Technology Assessment agencies (UK/EU/Aus/NZ/Canada) to assess these medicines, e.g. guiding principles; the input, if any, from wider society to underpin processes; the impact of processes adopted on access, treatment outcomes, etc.
- Options for a different approach in NHS Scotland and how they might work.

Work undertaken to support the Task and Finish Group’s outputs

The T&FG was supported by a small SMC team who undertook the background work to support the rapid review and the work up of new approaches that could be adopted for the assessment of these medicines. This work is summarised as follows:

a) Reviewing research on UK public attitudes to treatments for end of life and very rare conditions.
b) Reviewing definitions of end of life and very rare conditions and HTA approaches used for medicines in these categories internationally. This work was supplemented by a report produced by the Office of Health Economics.
c) Testing of agreed definitions of medicines for use at the end of life and for very rare conditions and how these would be applied in practice.
d) Performing a ‘lookback’ exercise on SMC decisions for cancer medicines and orphan medicines over the period November 2011 to October 2013.
e) Exploring the suitability of two analytical techniques cost-consequence analysis (CCA) and multi-criteria decision analysis (MCDA) that SMC could adopt for the assessment of medicines for very rare conditions and their ‘fit’ with the submissions that pharmaceutical companies currently make to SMC.
f) Exploring the option of introducing a QALY weight with a view to the risks and benefits of taking such an approach.
g) Exploring the feasibility of a new approach involving strengthened patient and clinician engagement (based on the conclusions of the initial work described above).
h) After the T&FG had given its support to this approach, preliminary discussions on how it would be operationalised have taken place with the key stakeholders (clinicians, Patient Interest Groups, and the pharmaceutical industry).
Appendix 4

Diagram 1 – Integration of PACE into SMC process for EoL, orphan/orphan-equivalent and ultra-orphan medicines

Diagram details:
- EOL/Orphan Submission +/− PAS
- Ultra-orphan Submission +/− PAS
- Assessment review
- New Drugs Committee
- NDC advice
- Applicant company
- not recommended
- Opportunity for PAS
- PACE Advisory Group at request of company
- Patient Interest Group Submission
- Company comments
- Scottish Medicines Consortium
- Final SMC detailed advice document
- NHS Boards
- ADTCs
- Applicant company
- Competitor company
- Advice made public

PAS = Patient Access Scheme
PACE = Patient And Clinician Engagement
* ultra-orphans assessed by new framework