Progress Update - Access to newly licensed medicines

NHS Scotland Directors of Pharmacy (DoP) and the Scottish Association of Medical Directors (SAMD)

The NHS Scotland Directors of Pharmacy (DoP) and the Scottish Association of Medical Directors (SAMD) welcome the opportunity to respond to the request from the Health and Sport Committee to follow up on its 2013 inquiry into access to new medicines and to seek an update on the effectiveness of the changes made to the Scottish Medicines Consortium’s system for approving new medicines.

The NHS Scotland Directors of Pharmacy (DoP) and the Scottish Association of Medical Directors (SAMD) are of the view that the Scottish Medicines Consortium (SMC), as a consortium of NHS Board Area Drug and Therapeutics Committees remains a valuable source of advice to NHS Boards on the clinical and cost-effective use of new medicines.

Question 1

1. To what extent have the new SMC process (implemented in April 2014) for approving medicines, current Individual Patient Treatment Requests and the new Peer Approved Clinical System (PACs) for rare conditions and end of life medicines become more transparent, less complex and delivered improved access to new medicines?

SMC processes changes are clearly described via its website and include patient, clinician and industry specific fact sheets to aid understanding of approval processes. The changes SMC has made in response to the policy recommendations and the impact of the changes made by SMC are outlined in the attached update report. The opening of SMC meetings to the public and industry provides enhanced transparency of decision making. The developments in SMC to support public involvement have also helped improve transparency and engagement.

The new SMC processes, including the Patient and Clinician Engagement (PACE) system, has improved access to 28 end of life and rare diseases medicines that previously may not have been accepted on the basis of cost effectiveness. It should be noted that the additional SMC process steps have increased the complexity of the assessment process and extended timelines for assessment.

Under the previous SMC processes it is likely that these medicines would not have been accepted for routine use and the route of access to NHS funded treatment would have been through the individual patient treatment request process (IPTR).

In the longer term the influence of the PACE process and the approval of less cost effective medicines for use in NHS Scotland may bring significant cost pressures to the NHS with the potential for greater diversion of resources.
away from other more effective and cost effective treatments including non
drug treatments. In addition the impact of the policy directive to value
medicines used at the end of life and for very rare conditions differently in
NHS Scotland may add to the cost pressures on the NHS. The willingness to
pay more for medicines used at the end of life and for very rare conditions,
may lead to unanticipated consequences in terms of the cost of medicines for
NHS Scotland.

As we enter the era of personalised medicines, potential treatment
populations will become smaller and therefore more medicines may be
classified as orphan or ultra-orphan with the option for health technology
assessment through these new, more flexible processes. This may have a
huge impact on budget with greater expenditure on very expensive medicines
with potentially marginal benefit.

The Scottish Government New Medicines Review recommended the
replacement of the existing system of IPTRs and a move to a new system
called Peer Approved Clinical System (PACS). Ministerial announcements
indicated that the national guidance would be available in spring 2014. Whilst
the CMO/CPO Letter “Access to Medicines – Transitional arrangements for
processing individual patient treatment requests”, SGHD/CMO(2013)20, 5
November 2013 and the subsequent letter: “Proposed approach to deal with
the transitional period from IPTR to PACS”, 11 December 2013 offer some
guidance there remains significant scope for interpretation and application of
interim arrangements at individual Health Board level.

If PACS is to be implemented, the DoP Group and SAMD are supportive of
the Scottish Government’s commitment to the development and provision of a
national system including a centralised patient support team to assist patients
going through the request process, training materials for clinicians, a register
of specialists to assist boards, improvements to the patient information
currently provided through Health Rights Information Scotland and the
implementation of robust auditing arrangements via Healthcare Improvement
Scotland.

A pilot to develop the PACS process which is limited to consideration of ultra-
orphan medicines not recommended by SMC has recently been launched in
NHS Greater Glasgow and Clyde. In the meantime, Boards continue to
operate under the guidance of CEL (2010) 17 “ Introduction and Availability of
Newly Licensed Medicines in the NHS in Scotland” and SGHD/CMO (2011)3:
Implementing CEL 17 (2010): Introduction and availability of newly licensed
medicines in the NHS in Scotland – Good practice guidance for NHS Board
management of individual patient treatment requests (IPTRs).

The general view across the DoP group and SAMD is that following the
introduction of more flexibility within the IPTR system, there was a rise in the
number of IPTR requests and the acceptance rates for IPTRs. Scottish
Government continues to request and receive data in relation to IPTR activity.
Comparison of these data is particularly difficult given the relatively small
numbers involved and the need for confidentiality when considering individual drugs or conditions where patient numbers can be very small (less than 5).

In relation to improving access to medicines that represent the best in therapeutic value and outcomes, the NHS Scotland Directors of Pharmacy (DoP) and the Scottish Association of Medical Directors (SAMD) would support further consideration of mechanisms to monitor patient outcomes as recommended in the SMC Task and Finish Group Report: Assessment of medicines for end of life care and very rare conditions (orphan and ultra-orphan medicines). It is recognised that there is a need for the NHS to work with partner agencies to ensure that medicines approved for use under the new process deliver the predicted benefits.

**Question 2**

**The effectiveness of any monitoring of the NHS boards Area Drug and Therapeutic Committees including the transparency of their operations and their timeliness in publishing local responses to SMC’s published advice?**

NHS Board ADTCs are required to work to the CMO timelines for local adoption / decision making with regards to new medicines approved by SMC. In February 2012 the Scottish Government issued SGHD/CMO(2012)1 “Guidance to further strengthen the safe and effective use of new medicines across the NHS in Scotland.”

One of the aims of this guidance was to standardise a timeframe for NHS Boards to consider Scottish Medicines Consortium (SMC) accepted medicines and to publish advice accordingly.

The timeframes stipulated were:

- NHS Boards are expected to reach a decision on a SMC Accepted medicine within 90 days of the issue of SMC advice to NHS Boards (this advice is confidential for the first 30 days).
- NHS Boards are expected to publish on the Board website, the formulary decision within 14 days of the decision being reached.

Additionally NHS Boards were expected to issue standard advice to reflect formulary decisions. This information is freely available to the public on Health Board internet websites. The use of these timelines, standard categorisations of decision making and internet publication have helped improve transparency of decision making. Board ADTCs have been working throughout 2015 via the ADTC collaborative, hosted by HIS, to further refine and standardise the reporting categories used by NHS Boards to provide greater transparency and improve public information and understanding in terms of decision making.

Throughout 2014/15, NHS Boards have also worked informally with the ABPI to help improve its interpretation and subsequent data capture of NHS Board decisions to ensure that the pharmaceutical manufacturers of new medicines
have an accurate picture of how new medicines are to be used in NHS Scotland.

Question 3

How the New Medicines Fund has been used and the extent to which it has improved access to new medicines for those with rare conditions?

The New Medicines Fund (NMF) represents a significant investment from Scottish Government to allow NHS Boards to implement Government policy with regard to improving access to medicines associated with end of life and medicines for the treatment of rare conditions. These medicines may not have received approval for use from the SMC in the past due to their lack of cost effectiveness. It is noted that the increase in access to new medicines is due to changes in SMC and associated Individual Patient Treatment Request (IPTR) processes and not the New Medicines Fund.

Question 4

The progress towards developing value-based assessments of new medicines and the Scottish model of value?

Progress toward developing value based assessment is challenging and it is recognised that there are other areas of care with treatments that may provide greater health gain but which are not currently afforded the level of flexibility that has been applied to end of life and rare conditions. If this policy was to be pursued it would have to be determined which patient groups and which disease states are more important and therefore more deserving than others.

The PACE process affords an opportunity for SMC to take a wider view of the value of medicines used at the end of life and for very rare conditions.

The new framework for assessment of ultra orphan medicines (medicines used to treat a condition with a prevalence of 1 in 50,000 or less (or around 100 people in Scotland)) allows SMC to use a broader decision-making framework, examining the nature of the condition, impact of the medicine, impacts beyond direct health benefits and costs to the NHS using the criteria set out above. PACE meetings are held for these medicines. Cost-consequence analysis may be provided where the submitting company judges that there are multiple relevant outcomes not readily captured within a standard health economics (Quality Adjusted Life Year (QALY) based) assessment or cost-effectiveness analysis using a single outcome measure. For these medicines, the economic analysis is a factor within the decision-making framework but will not be the predominant factor in the SMC decision.

The SMC is in a position to help determine and enable the research required to underpin an evidence-based approach to the development of a Scottish Model of Value by the Scottish Government.
Question 5

The effectiveness of the ‘pause’ mechanism in the SMC process and whether this mechanism has resulted in greater access to and improved the cost-effectiveness of new medicines

The PACE process involves a 1 to 3 month pause in the SMC assessment process and this offers pharmaceutical companies an opportunity to submit a new or revised Patient Access Scheme (PAS) aimed at improving the cost-effectiveness of the medicine. As demonstrated in our response to question 1, there has been an increase in the numbers of medicines accepted for use in NHSScotland following a PACE process.

NHS Boards should continue to receive timely advice about all new medicines. It is recognised that the assessment process is best served by pharmaceutical companies offering a competitive price from the outset.

NHS Scotland Directors of Pharmacy
Scottish Association of Medical Directors
Background

In 2013, a series of reviews were carried out into patient access to new medicines.1,2 As a consequence, SMC was asked to implement a number of recommendations to increase transparency, give patients and their representatives a greater role, and increase access to new medicines. We outline here the changes made and their impact to date.

Changes

The key changes implemented were as follows:

- All SMC meetings held in public.
- New, more flexible processes for the evaluation of medicines used at the end of life and for very rare conditions. This includes the option of a Patient and Clinician Engagement (PACE) meeting for end of life or orphan medicines.3
- A new framework for the assessment of ultra-orphan medicines. Taking a cost-consequence analysis approach, this considers the nature of the condition, impact of the new technology, value for money, impact beyond direct health benefits and on specialist services, and costs to the NHS and Personal Social Services.
- Strengthened patient and public involvement in our processes.
- Sponsor pharmaceutical company representatives participate in SMC meetings, in order to enhance industry engagement and understanding of how SMC reaches decisions.

Meetings in public

Since May 2014, all SMC meetings have been held in public. This ensures that members of the public and other stakeholders can understand how evidence is assessed and interpreted and how recommendations are made. Our intention is to hold as much of the committee discussions as possible with the public observers present. Private sessions are occasionally required to discuss information that the sponsor company considers to be commercially sensitive. Private sessions are occasionally required to discuss information that the pharmaceutical company observer

Strengthening the patient and public voice

As this is a key programme of work, we have appointed a Public Involvement Co-ordinator and a Public Involvement Officer to lead it. We have taken several steps to support public understanding of what we do and help patient groups get more involved (Figure 1). Developments have included: a refreshed process for patient group submissions on new medicines; proactive engagement, support and training for patient and carer groups; setting up a new Public Involvement Network (PIN) Advisory Group and publishing a guide to public involvement.4 We have also published a guide for the public on how SMC works.4

Patient and clinician engagement process

The aim of the PACE process is to explore the added benefits of the medicine, from both patient/carer and clinician perspectives, that may not be fully captured within the conventional clinical and economic case.3

The PACE meeting involves a round table discussion with patient representatives and healthcare professional experts focusing on how the medicine can:

- add value to the patient’s wellbeing and experience of care (for example, ability to work, impact on quality of life, symptom control)
- add value for the patient’s family and/or carers (for example, impact on family life, impact on the carer’s ability to work)

The new process also includes a second opportunity for the company to submit a patient access scheme (PAS).

The output from the PACE meeting is a consensus statement, provided to all committee members and presented at the meeting. This has a major influence on the SMC decision.

Historical data on acceptance rates show that over the period 2011-2013, 52% of orphan and cancer medicines combined were not recommended for use. An analysis of decisions on medicines considered under the PACE process between October 2014 and January 2016 shows that of the 40 medicines considered, 29 (73%) have been accepted and 11 (27%) not recommended (see Appendix).

Summary

We have worked closely with key stakeholders, including patient groups, clinicians and the pharmaceutical industry, to introduce these recommended changes. So far, experience suggests that we are meeting the desired objectives of increasing transparency and giving patients and their representatives a stronger voice in the decision-making process. Our analyses of data from the first year since the PACE process was introduced shows an increased acceptance rate for end of life and orphan medicines. We will continue to develop and improve these changes over time.

References:


www.scottishmedicines.org.uk

www.scottish.parliament.uk
### Appendix: Medicines considered through the PACE process

<table>
<thead>
<tr>
<th>Decision published</th>
<th>Accepted or accepted with restrictions</th>
<th>Not recommended</th>
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<tr>
<td>Quarter 4 2014 (October – December)</td>
<td><strong>Brentuximab vedotin</strong> (ultra orphan) <em>(Hodgkin’s lymphoma)</em>; <strong>Ipilimumab</strong> <em>(advanced melanoma)</em>; <strong>Pemetrexed</strong> <em>(lung cancer)</em>; <strong>Riociguat</strong> <em>(Chronic thromboembolic pulmonary hypertension)</em></td>
<td><strong>Trastuzumab emtansine</strong> <em>(HER-2 positive breast cancer)</em>; <strong>Pertuzumab</strong> <em>(HER-2 positive metastatic breast cancer)</em></td>
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<td>Quarter 1 2015 (January - March)</td>
<td><strong>Cetuximab</strong> <em>(metastatic colorectal cancer)</em>; <strong>Aztreonam lysine</strong> <em>(cystic fibrosis)</em>; <strong>Paclitaxel albumin</strong> <em>(pancreatic cancer)</em>; <strong>Bosutinib</strong> <em>(ultra orphan)</em> <em>(chronic myelogenous leukaemia)</em>; <strong>Dabrafenib</strong> <em>(melanoma)</em>; <strong>Ruxolitinib</strong> <em>(disease related splenomegaly / primary myeolofibrosis)</em></td>
<td><strong>Cabozantinib</strong> <em>(ultra orphan)</em> <em>(metastatic medullary thyroid carcinoma)</em></td>
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<td><strong>Ponatinib</strong> <em>(chronic myeloid leukaemia / Philadelphia chromosome positive acute leukaemia)</em>; <strong>Regorafenib</strong> <em>(ultra orphan)</em> <em>(gastrointestinal stromal tumours)</em>; <strong>Nintedanib</strong> <em>(lung cancer)</em>; <strong>Ofatumumab</strong> <em>(chronic lymphocytic leukaemia)</em>; <strong>Idelalisib</strong> <em>(ultra orphan)</em> <em>(chronic lymphocytic leukaemia)</em></td>
<td><strong>Olaparib</strong> <em>(ultra orphan)</em> <em>(ovarian cancer)</em>; <strong>Vinflunine</strong> <em>(bladder cancer)</em>; <strong>Eribulin</strong> <em>(breast cancer)</em>; <strong>Enzalutamide</strong> <em>(prostate cancer)</em>; <strong>Elosulfase alfa</strong> <em>(ultra orphan)</em> <em>(Morquio A syndrome)</em></td>
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<td><strong>Olaparib</strong> <em>(ultra orphan)</em> <em>(ovarian cancer)</em>; <strong>Vinflunine</strong> <em>(bladder cancer)</em>; <strong>Eribulin</strong> <em>(breast cancer)</em>; <strong>Enzalutamide</strong> <em>(prostate cancer)</em>; <strong>Elosulfase alfa</strong> <em>(ultra orphan)</em> <em>(Morquio A syndrome)</em></td>
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