Access to newly licensed medicines

The Royal College of Physicians of Edinburgh

The College understands that healthcare systems world-wide are unable to do everything that they possibly could in the field of patient care because of resource constraints and that all systems therefore have to make choices amongst possible ways of spending that limited resource. This may mean difficult and possibly unpopular decisions having to be made. The College believes that such decisions are best made within a formal, objective, evidence-based process.

Access to new medicines

Inevitably, new medicines are of putative real-world effectiveness at the time of launch into the marketplace and have limited safety data. Older medicines, often now available in generic versions at very modest cost, are of proven real-world effectiveness and have substantially greater evidence of safety in clinical use. While the College understands the demand to access new medicines, it would have major concerns if this threatens the use of highly effective, cheap, existing medicines. In a cash limited system it is important to take a broad perspective on the rational use of all medicines to deliver best value for patients.

The College supports the Health Technology Assessment (HTA) approach to new medicines adopted by the Scottish Medicines Consortium (SMC) (and the National Institute for Health and Clinical Excellence (NICE) in England) as an objective, structured, evidence-based method to assess and compare the relative effectiveness and cost-effectiveness of medicines (and, in NICE’s case, other healthcare interventions). The assessments are undertaken using internationally-accepted methodologies and, the College believes, are performed to high standards by both SMC and NICE.

College Fellows and Members are active participants in the SMC process, contributing on the Committee itself as well as providing expert clinical input to the SMC process. This engagement between SMC and clinicians is, the College believes, a significant strength of the Scottish process.

The College believes that the SMC outputs, which are available to view on the SMC website, show that the medicine reviews are undertaken thoroughly and expertly in a transparent process. It is vital that SMC members (and the members of the New Drugs Committee and expert clinical and economic reviewers) have the skills and experience necessary to ensure that the clinical value of new medicines is appropriately identified and then assessed alongside the additional costs of new medicines. The College notes that the quality of the SMC value assessment is not doubted by any of the petitioners.

The College notes that, as a consequence of the robust decision-making processes adopted by SMC, many new medicines in Scotland are now being offered to the NHS with a Patient Access Scheme (PAS). Most of these
schemes represent forms of discount on the list price of the medicine in question - details of the individual schemes and their magnitudes are commercially confidential but it is believed that discounts of up to 50% on the list price have been offered. The SMC process is, in these instances, improving access to new medicines by reducing the acquisition costs, allowing even more patients in Scotland to be treated for the same NHS outlay.

As noted above, concern from petitioners to the Committee is largely around the SMC’s decisions (and subsequent implementation within NHS Scotland). The College believes that, if the SMC evaluation of the relative effectiveness and cost-effectiveness of new medicines is accepted as being of high quality but the decisions regarding access in Scotland are being questioned, then it is the context and framework of SMC decision-making that must be called into question. The College is not aware that SMC has outlined any formal framework for its decisions but thinks that it has adopted a similar utilitarian approach to that adopted by the rest of the NHS in Scotland, seeking to achieve the greatest health benefits possible for the population of Scotland without wishing to discriminate positively or negatively around issues such as age, gender, disease type, rarity of disease etc.

SMC’s expertise is in the area of medicines evaluation – if the framework in which it makes its decisions is to be changed, then that is not a decision for SMC alone but for the wider NHS or potentially for wider society in Scotland. If it were considered appropriate for SMC to operate within a decision-making framework which chose to discriminate in favour of children, or cancer, or end-of-life medicines, or rare diseases (for example) – the College would ask the Committee to remember that discrimination in favour of any (or all) of these groups also implies discrimination against those not belonging to such a group, and also that Scotland could see a situation develop in which some overall level of health improvement was being sacrificed in order to favour individual patient groups.

The College believes that research into societal preferences around these difficult issues has not been examined to a great extent in Scotland and the Committee may wish to consider whether this should be explored before any change of decision-making framework is considered. Experience from elsewhere (including the NICE Citizens Council in England) should be noted.

The College thinks that the Health and Sport Committee may be well placed to initiate debate around the framework for decision-making in NHS Scotland – ultimately there may well be a role for debate and decision by the Scottish Parliament itself. The College would welcome the opportunity to be part of that debate (and indeed to facilitate such a debate if that would be helpful) but recognises that these decisions are not for healthcare professionals alone to make but for wider Scottish society.

The College believes that a considered approach to the principles underlying these difficult issues is much preferable to departing from the current process for individual medicines on a ‘case-by-case’ basis as the latter could lead to
unstructured decision-making and set precedents which could distort later decisions.

The College is aware of the issues surrounding ‘Orphan Medicines’ (medicines for orphan diseases). Such medicines receive special treatment during the European Medicines Agency (EMA) registration process and are granted longer periods of exclusivity and patent protection than ‘normal’ medicines. They are often very costly. This poses the question as to whether the use of scarce resource on very expensive medicines is appropriate when this could result in a considerable loss of overall health gain to the Scottish population.

**Individual Patient Treatment Request (IPTR)**

Concern has been expressed about the Individual Patient Treatment Request (IPTR) process within NHS Scotland. The College supports the process in principle, recognising that all patients are individuals and that SMC (and other) guidance may not always apply to the situation of an individual patient. Clinicians are well-used to individualising patient treatment and so identifying those patients to whom prevailing SMC advice should not apply is an extension of routine practice. The issue at stake is whether the individual patient in question is likely to derive substantially more benefit from a treatment than the average patient (the basis on which SMC advice is founded). If the answer is ‘Yes’ then use of a ‘not recommended’ medicine may be appropriate – if the answer is ‘No’ then the question would be why national advice should not apply in the particular case. It is right that the issue should be confined to additional clinical benefit anticipated from the medicine and that other possible reasons to specially favour an individual patient should generally not apply.

IPTR panels have a responsibility not simply to consider the special interests of the patient whose case is being made but also the interests of other patients whose care or treatment would be adversely affected if resources were used to treat the individual case. The College believes that this balance is best achieved by having the relevant specialist (± patient) make the case to a panel of non-specialists, who can consider the case and the wider ramifications.

The IPTR process will inevitably lead to different decisions being made by different panels as the particular circumstances of individual patients will vary – this is as it should be in a flexible and responsive system. The College believes it would be good practice for Health Boards to share the decisions made by IPTR panels to minimise differences in decision-making processes and criteria between different Boards, accepting, as above, that individual decisions will differ.

**Summary**

Overall, the College believes that Scotland is well served by the processes in place to control access to new medicines. Unregulated access could distort
prescribing and actually reduce overall health benefits. If there is ongoing concern that some decisions about new medicines are not what Scottish society would wish to see, then the College believes that it is the underpinning decision-making framework, rather than the technology assessment processes of SMC, which needs to be discussed, debated, researched and, perhaps, modified.

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\[1\] NICE Citizens Council: The Citizens Council provides NICE with a public perspective on overarching moral and ethical issues that NICE has to take account of when producing guidance.