

Submission from Karen Facey

I have been following with interest the deliberations of the Health and Sport Committee about access to newly licensed medicines, particularly orphan products. It is reassuring to see that the Committee has recognised that the processes used by the Scottish Medicines Consortium are considered robust. Most can see that they seek to abide by principles of consistency, transparency, stakeholder engagement and accountability. The SMC's standing internationally has also been recognised and it is an international perspective I would like to offer the Committee.

An international view of HTA

The work of the Scottish Medicines Consortium may be described as “health technology assessment” or HTA. HTA has been defined as *a multi-disciplinary field of policy analysis, which studies the medical, social, ethical and economic implications of development, diffusion and use of a health technology*. It is important to note that this definition goes beyond medical (clinical effectiveness) and economic (cost effectiveness) issues to consider social (patient issues) and ethical issues.

Ethical Issues

Ethical considerations are paramount in any deliberation relating to the equitable allocation of scarce resources and the consideration of individual vs collective ethics. However, I am concerned that the Committee has had little ethical input to their evidence gathering yet and so I provide a few pointers that our esteemed Scottish medical ethicists could present better than me. Indeed the Revd Kenneth Boyd was a key adviser to our first national HTA agency, the Health Technology Board for Scotland.

Access to newly licensed medicines requires difficult decisions under constrained resources. If we fund a product, that money is not available for use in another way – there is an opportunity cost. In 2002, Daniels and Sabin¹ argued that there was a need to define conditions for allocation of health care resources, providing “accountability for reasonableness”. The four conditions are

1. decisions must be publicly accessible;
2. relevance (the rationales invoked must be based on evidence, reasons, and principles that fair-minded persons would affirm);
3. mechanisms for appeals must exist;
4. regulation (public procedures must ensure the fulfilment of these three conditions).

Key here is relevance – what evidence is suitable for what product and do the same reasons and principles apply to all products? This is a particularly relevant question for orphan products.

¹ Daniels N and Sabin J. *Setting limits fairly: can we learn to share medical resources?* Oxford: Oxford University Press; 2002.

Other agencies have established processes for the special evaluation of orphan products (e.g. Ontario²) and as mentioned at Committee, the AGNSS process used in England until recently had been welcomed by many.

http://www.specialisedservices.nhs.uk/library/27/Introduction_to_AGNSS_Decision_Making_Framework.pdf

Countries in Scandinavia have led the way in terms of systematic ethical analysis of issues arising in HTA.

<http://www.htai.org/index.php?id=676>

It would be good to see some of these approaches debated and used in our own Scottish context to ensure that Scotland can show accountability for reasonableness in all its difficult decisions about resource allocation – at national SMC level and at individual health board level.

Social issues - Patients' perspectives

I noted in the debate on 29 January 2013, the Committee's excellent engagement with patients and patient organisations. It was explained how SMC has developed its approach to support patient organisations to make an evidence submission that is considered alongside the critical assessment of the clinical and cost effectiveness. As the patient representatives explained, great effort is taken to provide patient evidence but its impact is unclear.

The SMC has employed a Public Involvement Officer to support patient organisations and they lead the world in this initiative. However more could be done to ensure that the evidence submitted about patients' views is relevant and sufficiently robust to make a real impact in decision-making.

I chaired an international group of stakeholders to publish a paper on effective engagement of patients in HTA³. This highlights that only patients and their carers know what it's like to live with a condition, the challenges they face, issues with current treatments, unmet needs and experiences with the new medicine being assessed. Patient organisations can collect this information, but it is often seen as anecdotal and biased when submitted alongside more structured evidence on clinical and cost effectiveness. I would therefore suggest that Scotland needs to invest more in qualitative (social science) research to allow the structured collection and analysis of patients' perspectives so that this scientific research about patients' perspective can inform decision making. Healthcare Improvement Scotland invests in experts to assess clinical and cost effectiveness, but there is no investment in social science. We have excellent social science research units in Scotland but we do not garner their expertise often in this area.

² Winquist E, Bell C, Clarke J et al. An evaluation framework for funding drugs for rare diseases. *Value in Health*. 2012;15:982-986.

³ Facey K. Patient involvement in HTA: What added value?. *HTA and Rare Diseases - Pharm Pol and Law*. 2011;13:245-251

Best practice in the use of the social sciences to provide robust evidence about patients' perspectives in HTA can be seen from Denmark. Each of their HTAs included specific research on patient issues and a separate section about patient issues in their HTA report. A good step forward would be for every SMC Detailed Advice Document (DAD) to include a section on patient issues and for that section to draw out key parts from any evidence submission (or social science research) and explain how this has influenced the decision (or not).

In relation to decision making at board level, the minimum we should require is consistent, transparent processes that ensure appropriate patient representation at the decision-making committee. This should be followed by clear feedback on the rationale for the decision and explanation of the quality of care that the patient will receive whatever the committee decision.

Assessing (ultra) orphan products

The Committee has paid special attention to access to orphan products. The European Medicines Agency provides an orphan designation to a product treating a condition with a prevalence of 5/10,000 that is life threatening and with no other treatment options. This is a broad definition that includes not just rare diseases but orphan indications (such as last line cancer therapy). Furthermore the prevalence of the condition in Scotland can range from several thousand down to less than 10. Hence it would seem appropriate to use sub-classifications of rare diseases. This has happened implicitly in the past as can be seen by the national Risk Sharing Scheme that was established by the National Services Division for high cost, very low prevalence (volume) diseases. However, the rules for what needs to be considered by NSD and how other rare conditions are handled seem less clear.

Qualitative research is particularly pertinent to rare diseases where there may be little clinical evidence, but where patients are the real experts and only a small number of patients are required to demonstrate common themes and issues⁴.

Another important aspect for rare diseases, is the need to collect evidence of outcomes and impact after SMC approval. Although the use of registries was mentioned in the Committee discussion, this needs more scrutiny. Do we ensure that evidence is collected in a way that can be linked internationally to show evidence of long-term safety and effectiveness? Are we garnering the resources of NHS Services Information Services Division effectively? This is essential not only for rare diseases, but also for other technologies where the evidence base is sparse – such as in devices and surgery assessed by the Scottish Health Technologies Group.

⁴ Facey K, Boivin A, Gracia J *et al.* Patients' perspectives in HTA: a route to robust evidence and fair deliberation. *Int J Tech Ass in Health Care.* 2010;26:334-340

Continuing to improve Scottish HTA

Scotland is leading the world in relation to many aspects of HTA, but we should not be complacent. We should continue to seek to learn from best practice internationally and ensure that we are using our great academic resources to produce evidence for decision making that is “relevant” for all products and diseases.

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