Access to newly licensed medicines

Supplementary Submission

Pfizer UK

I would like to extend my thanks for the opportunity to submit further points of clarification to the Health Committee on the review of Access to Newly Licensed Medicines in Scotland.

At Pfizer, we see three main areas for examination:

- The decision making framework of SMC
- Implementation of SMC advice by NHS Boards
- IPTR system

SMC Framework

The review was generated by patients who are unable to access medicines in NHS Scotland, particularly those who suffer from a rare condition and require an “Orphan Medicine.” It is critical that the system used for assessing the value of a new medicine to the NHS should fully capture health gains of all patients and wider benefits that achieve NHS objectives. Current HTA process uses the QALY to define the entire value of a medicine. This is a formulaic approach which does not include value beyond quality of life and survival benefit, and therefore fails to capture the full value that medicines can bring to society. The objectives of the NHS are wider than health gain alone. Patient experience of care is an important objective as reflected in the Better Together programme. Medicines can deliver value related to patient experience of care, such as convenience, independence, and dignity, and this is not captured within the QALY. In addition, integration of healthcare across the health and social care system can lead to benefits through the re-location and simplification of process of care. Medicines can contribute significantly to achieving these goals and enable care to be transitioned closer to the patient and encourage self care.

The SGHD and NHS commonly cite an even broader range of objectives for our health care system; these include health gain per se, but also factors such as equity, helping parents and carers, getting people back to work, etc. The SMC fails to include costs and savings accruing outside of the NHS and Social Services budgets, which further undervalues the role that medicines can bring to the economy of Scotland. Furthermore, the goal of HTA is simply to achieve the maximum number of QALYs from a given budget regardless of who benefits and who loses out. Under this system the value of a QALY is equal whether it is achieved for a serious, life threatening disease or a mild, chronic disease. No consideration is given to the national priority of the disease, the severity or level of unmet need, or whether there is justification for valuing health gain in a specific condition or patient group more highly. More failures of the HTA approach could be avoided by inclusion of such considerations, alongside the QALY, in a broader framework of value.
Fair and sustainable decisions on pharmaceutical price/reimbursement also need to account for wider industrial considerations such as the economics and risks of developing and commercialising new medicines versus the expected return on investment. For example, it may cost more and/or be higher risk to explore new areas of science involved in researching therapies for rare conditions or certain cancer sub-types. Likewise, providing more sophisticated medicines (e.g. biologics and more targeted ‘personalised’ therapies) may be more difficult. Downstream, these medicines may not necessarily be more profitable due to economies of scale, i.e. the patient numbers and thus size of market can be small. It is important to understand that medicines for rare conditions are typically more expensive than those for common conditions since the research and development costs are recouped over a smaller potential patient population. As a result, these medicines typically have relatively high prices and cost-effectiveness ratios and are therefore more likely to fail the traditional HTA cost-effectiveness threshold. Application of a standard cost-effectiveness threshold to all medicines systematically disadvantages those medicines that are developed for rare conditions since the economics of drug development in these areas are less attractive than for common conditions.

Furthermore, a recent report commissioned by Pfizer found that the SMC recommended only 49% of the orphan medicines it reviewed for use by NHS Scotland.

This is due to the reliance of the QALY based system. One of the reasons the Cancer Drugs Fund was introduced in England was to address the challenge of accessing medicines in therapy areas which historically have had significantly less positive recommendations from HTA bodies such as NICE. In addition, the establishment of AGNSS has recognised the need for an approach which includes the societal benefits of medicines when considering rare diseases.

NICE have announced they will include a wider assessment of value for Orphan medicines following the transition of the AGNSS framework into NICE. A fundamental change to the HTA QALY approach to establish a broader framework for evaluation of medicines is the only way to truly resolve these limitations and protect the future of medicines innovation and development.

We would strongly recommend that SMC creates a wider assessment of value, beyond its current modifiers in order that patients in future are not reliant on the IPTR system to access treatment to improve their health outcomes. This framework should be co created with industry partners.

As outlined at the evidence session, this could also play an important role in the linkage to Scotland’s ambition to double its economic impact in Life Sciences by 2020. The routine early adoption of innovative new medicines is a significant factor in determining where future Research and Development takes place. Scotland would increase its chances of attracting early and late-
phase clinical trials if the latest treatments, against which any new medicines
would need to be studied, were already established as the standard of care.
The perception of relatively slow adoption and restrictions on access imposed
by SMC formulary agencies is making it harder for UK affiliates to argue for
increased or even maintained investment from global headquarters in Scottish
clinical trials. This also has a concerning impact on the skill and knowledge
base of our current world leading clinicians. It is companies, not governments
or universities who make those decisions.

Implementation of SMC Advice by NHS Boards

A number of the committee recognised that from the patient’s perspective
further rounds of repetitive analysis, decision making and bureaucracy was
impossible to justify. Pfizer would support the removal of additional hurdles to
access for SMC accepted medicines.

The committee should explore the removal of further, local decision making
on access on medicines that are already SMC accepted as cost effective.
This would create a simpler process which is easier for patients to understand
and navigate. We would also like to point out that in order for a medicine to
be approved by SMC, it must demonstrate benefit over treatments currently
available and used in NHS Scotland.

IPTR System

We would draw attention to the need for a fair and transparent system which
ensures fairness across geographical and health board boundary’s. However,
it is important to stress that this route should be used in rare circumstances
and implementation of the following points will improve the IPTR system, but
will not cure the root cause of patients failing to access medicines. That can
only be done by revising the QALY based system to a wider assessment of
value by SMC.

In terms of recommendations to improve the IPTR system, we would seek
consideration of the following points:

- Additional clarity on access to medicines other than via the IPTR route; i.e.
  out-of-license use for rare diseases or off-label use (where a medicine is
  used for a purpose not included in its original license).
- Clarity on the wording of guidance on IPTRs - It needs to be clear exactly
  what situations IPTRs are for and what they are not for; and also how they
  are assessed.
- A national quality review panel, not to review individual IPTRs, but as a
  way to review how well the processes are working and to keep check on
  regional variation. This group should have a transparent membership, a
  patient representative and should publish top-level data as a means of
  driving-up standards. It should look for equitable processes and decisions
  across both approved and non-approved requests.
• An objective, transparent scoring system as a means of assessing IPTRs and their validity, to ensure uniformity and fairness across illness areas and geographically.

• The establishment of benchmarks from across Scotland of where we are with IPTRs; what is working and what is not. This would help to identify areas of good and bad practice and create a baseline.

**In summary, we would outline that**

• SMC uses an overly narrow definition of value that fails to take into account benefits that are important to patients, the NHS in Scotland and the Scottish Government.

• The evaluation approach used by SMC is based on an assumption that the goal of NHS Scotland is to maximise the health gain it gets from a given budget and does not take into account broader government priorities, including the National Outcome for improving employment opportunities, in particular removing barriers to work, support for carers and disabled people. It assumes that society values all health gain (QALYs) equally, which is inconsistent with public opinion.

• By virtue of using a single cost-effectiveness threshold, it takes no account of the economics of drug development and therefore sets an unfair test of medicines for rare conditions where the relatively small patient populations mean that companies need to set a high price in order to recoup their development costs.

• **We propose that SMC creates a wider assessment of value, beyond its current modifiers in order that patients in future are not reliant on the IPTR system to access treatment to improve their health outcomes. This framework should be co created with industry partners.**

I would be pleased to discuss any of the points raised further.

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