Access to newly licensed medicines

Myeloma UK

Myeloma UK firmly believes that all patients should have the right to expect fair and equitable access to the treatments their clinicians deem to be the most appropriate for them.

Over recent months, there has been much discussion and criticism over the role of the SMC and IPTR processes as well as speculation over whether the current system of how new medicines are approved for use on NHS Scotland is fit for purpose.

Myeloma UK welcomes the initiation of a comprehensive and evidence-based public debate on the complex but critical issues of access to newly licensed medicines. We hope this will help the Scottish Government to determine both the true nature and the extent of the issues and to work to find appropriate, fair and sustainable solutions. To this end, we hope that you find our written evidence useful.

1. General comments

The SMC and the IPTR processes are currently, rightly or wrongly, implicitly linked. As things stand, the IPTR process relies on the SMC to make decisions about whether a new treatment should be made available. It is based on the assumption that only a small subset of, what are referred to as ‘exceptional’ patients, will need access to a treatment in the absence of a positive SMC decision.

For a range of reasons the SMC do occasionally make negative decisions on newly licensed medicines that have been shown to be highly effective but fall short of the rigorous clinical and cost effectiveness assessment conducted by the SMC. This invariably causes a huge amount of frustration and anger among the medical and patient communities as well as the wider general public.

In such cases, IPTR’s are then often used as a surrogate decision-maker. This is despite them never having been designed to carry out such an important role.

It is therefore incumbent on the Scottish Government to:

- put in place a specific fit for purpose system to cater for this group of newly licensed medicines
- attempt to identify and understand the underlying reasons for negative SMC decisions and seek to address these underlying issues. This should in turn reduce the need for, or at least the role of, a surrogate decision-making system
It is also necessary to accept that despite the best efforts of all involved, it is entirely acceptable to say no to a newly licensed medicine if it truly has not demonstrated clinical and cost effectiveness and does not address serious unmet clinical need.

2. Scottish Medicines Consortium

Myeloma UK has an excellent working relationship with the Scottish Medicines Consortium (SMC) and is highly confident that it makes thorough assessments of all newly licensed medicines.

The modifiers that the SMC use in their decision-making are applied more pragmatically than the comparable but not identical end-of-life criteria used by the National Institute for Health and Clinical Excellence (NICE). Where the SMC say ‘no’ to new medicines this is only after a thorough and detailed analysis of the evidence, price and relevance to NHS Scotland clinical practice.

We have also traditionally encountered fewer problems with local access to myeloma treatments (particularly through the IPTR process in Scotland than in England (particularly Velcade®, Revlimid® and Mozobil®), something which can in part be attributed to the smaller time delay between licensing and the SMC appraisal of new treatments (approximately three months).

Due to the nature of the remit of the SMC, it is inevitable that in some circumstances they will have to issue (sometimes controversial) negative recommendations on newly licensed medicines. It is the job of the pharmaceutical industry, the SMC, patient groups, clinicians and the Scottish Government to work together to ensure that these situations are as infrequent as possible.

Industry submissions

Where drugs do not get through the SMC appraisal process, a major reason is often poor or uncertain clinical trial data, combined with unjustifiably high prices. This means that even where there is an unmet need for a treatment, the SMC simply cannot justify the expense for a treatment that has inadequate evidence of clinical effectiveness. It is also not within the remit of the SMC to negotiate with industry over price.

The Scottish Government and the wider health sector should ensure that increasing pressure is placed on the pharmaceutical industry to reduce the uncertainty surrounding their treatments. Industry should be encouraged to improve the design and relevance of their clinical trials in order to facilitate the collection of data that is necessarily robust to withstand the scrutiny of the SMC.

The problem of uncertainty around a treatment can stem back to the initial trial design in the Phase III European licensing studies. The comparators used within these trials can make it difficult for the SMC to establish how the
treatment will fit into NHS clinical practice and in some circumstances patient cross-over occurs which makes it difficult to establish the full clinical benefit that patients may gain from the treatment. In addition to this, the quality of life data gained from these studies is often below the quality that is required by the SMC to assist their decision-making.

To this end, Myeloma UK is adamant that we need joined-up thinking from early phase clinical trial design and licensing trial design through to pricing, approval and implementation – the SMC process should not be looked at in isolation. We need to find ways for stakeholders across these issues to work together to ensure that the system works as efficiently as possible and that most importantly patients get access to the treatments that they need. It should be acknowledged that some of the solutions needed to address the issues of access to medicines fall beyond the remit of the Scottish Government on its own and requires collaborative thinking and working at both the UK and European levels.

When there is uncertainty around a treatment, or where there is good evidence about a treatment but the price is just too high, ‘modifiers’ and ‘patient access schemes’ are the tools available to the SMC to help get the treatment ‘across the line’. However, it should be noted by the Health and Sport Committee that these should be viewed as temporary, ‘stop-gap’ measures that do not address the root causes of why treatments are unable to be approved by the SMC.

**Patient group submissions**

It is important that patient groups provide robust and well-evidenced submissions to the SMC to assist in their decision-making and that these submissions are objective and balanced.

From our experience, the SMC takes patient submissions into full account when formulating their decisions as they are able to provide information about the impact a treatment will have on a patient’s quality of life that clinical data cannot.

We have therefore welcomed the appointment of the Patient Liaison Officer within the SMC who provides patient groups with detailed advice on completing submissions for appraisals to make these the best they can be. This has been a big improvement in assisting patient groups in understanding the SMC processes and in equipping them with the skills to work more closely with them on their decision-making.

We have also directly informed the SMC that it would be beneficial for them to include an explanation of how they have taken into account patient group submissions in their decision-making when they publish their final advice on new treatments so that patient groups understand how their submissions been used and also for the SMC to include examples of best practice patient group submissions for reference on their website.
SMC and orphan diseases

Myeloma UK welcomes the debate stimulated by the Public Petitions Committee consideration of PE1398 on improving access to orphan medicines in Scotland.

We agree that orphan diseases often have more difficulty getting through the SMC, due to the smaller patient population available to take part in the clinical trials. There is always going to be a level of uncertainty around the data gathered from the clinical trials, and due to the small population, the price is always going to be much higher.

The SMC do have modifiers available to them relating to treatments for orphan diseases. This gives the SMC a degree of flexibility within the process to approve these treatments, where there is greater uncertainty around the product.

However, we think that there is also room for industry to work on better trial design for orphan disease products and in turn make more strategic use of the smaller patient population - stemming from early phase clinical trials through to licensing. Reducing the uncertainty around products for orphan diseases should assist the SMC with more robust decision-making on these medicines.

Moving forward, there is a need to further investigate how treatments for orphan diseases are brought to market and the reasons why treatments for orphan diseases are turned down by the SMC. Only then can we fully understand, make judgements and reach solutions on this issue.

Value-based pricing

Value-based pricing (VBP) is the most important topic that needs to be considered by the Health and Sport Committee, as it will impact dramatically on the role of the SMC and on access to medicines for patients in Scotland.

There is currently a large degree of ambiguity over how the new proposed new system will operate across the UK and in particular there is a lack of clarity over the impact it will have in the devolved regions. The pricing of newly licensed medicines is a matter reserved for the UK Government, but value is a devolved responsibility. Under the PPRS it is currently the role of the SMC to assign a value to a treatment in the context of the Scottish population and NHS. However, as the VBP system will assign a ‘value’ to a treatment during the pricing process, there are obvious implications for the SMC and to devolution more widely.

The next six months are fundamental for the development of VBP, as the negotiations are beginning between the Association of British Pharmaceutical Industry (ABPI) and the Department of Health (DH) in England. During this time, the Scottish Government should urgently engage with the DH in England to discuss and seek clarification on how Scotland and the SMC fit into the new
system. This is an important (and perhaps the only) opportunity for the Scottish Government to ensure that VBP works in the interest of patients in Scotland.

3. Individual Patient Treatment Requests (IPTRs)

It should be noted by the Health and Sport Committee that the IPTR process was not designed to make decisions over high-cost medicines and questions can be raised about whether it is a process that is fit-for-purpose. Whilst this is the case, at Myeloma UK we are keen to make the process the best it can be for patients across disease areas.

Clarification

In recent months there has been a lot of controversy over the IPTR process, in particular as it is being advertised as a way for patients to gain access medicines that have been turned down by the SMC.

It is crucial that the Health and Sport Committee and the Scottish Government are clear that the IPTR process is not designed to pick-up funding for the treatments that have received a ‘no’ from the SMC. It is instead designed for a small subset of patients who need access to treatments outside of routine clinical practice on the NHS. The increased expectation of the role of the IPTR process has caused obvious disappointment and anger amongst patients who have applied for funding and have had these requests turned down. We therefore believe that the Scottish Government needs to clarify the intended purpose of IPTRs in order to limit the confusion around the process.

Recent policy developments

Myeloma UK very much supported the previous inquiry of the Public Petitions Committee (PE1108) into access to cancer treatments in Scotland and has been pleased with the Government response to the issues raised in the Petition and the subsequent reforms that have been made since its closure in 2011.

The CEL 17 and its subsequent update document have gone a long way towards improving the decision-making and transparency around the IPTR process. It is absolutely vital that patients feel that their IPTR requests are being dealt with in a transparent and uniform way and that the decision made by their health board would be likely be echoed by other health boards in their particular circumstances.

We have heard that the IPTR process has been improved by mandating who should be on the panel, as this helps to standardise the decision-making processes. Having the correct level of expertise involved on IPTR decision-making panels is particularly important for rare diseases such as myeloma. However, this may have an impact on the timeliness of decisions and should be monitored.
Myeloma UK is pleased that the rationale for IPTR decisions is being collected within Health Boards for information and best practice sharing purposes. The Scottish Government also need to ensure that the IPTR processes across Health Boards in Scotland are continually monitored and information on the implementation of the CEL guidance should be routinely collected and published centrally. This is important to enable the Government to identify problems within the system when they arise and to ensure patients are continually subject to fair and transparent decision-making.

Finally, the IPTR process should also be thought of in the context of VBP, given that changes to drug pricing may impact on this process after 2014. Getting clarification (as suggested above) from the UK Government on how VBP will operate in Scotland post-2014 will be useful in determining how the IPTR will fit into this.

4. Conclusions

Myeloma UK hopes that the Health and Sport Committee finds the above comments useful to inform their inquiry.

Over recent months, there has been a lot of controversy and criticism over access to newly licensed medicines across Scotland. However, the real extent of the problem has not yet been determined and without this, solutions cannot be agreed. We hope this inquiry will be the first step towards deciphering exactly where in the system of drug licensing and approval the problems lie and that stakeholders can begin to work together to find a solution. It should also be acknowledged that this perhaps cannot be achieved at a Scotland level alone.

Myeloma UK
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