Access to newly licensed medicines

Novartis Pharmaceuticals

Novartis develops innovative medicines for a wide range of diseases, including:

- Age-related macular degeneration
- Multiple sclerosis
- Chronic Obstructive Pulmonary Disease (COPD)
- Cystic fibrosis
- Chronic myeloid leukaemia
- Renal cell carcinoma
- Thalassaemia and sickle cell disease
- Subependymal giant cell astrocytoma (a type of benign brain tumour)
- Myelofibrosis

Some of these medicines are SMC approved, others are not. Our interest in IPTRs stems from the fact that we are keen to ensure that the system for accessing medicines which have not been recommended by the SMC or are in the process of being appraised is fair, robust and transparent.

Our submission covers the following points:

1. A brief outline of our understanding of the purpose of the IPTR process
2. Problems with the IPTR process and potential solutions
3. A more radical approach: A national IPTR system with ring-fenced funding
4. The role of the Scottish Medicines Consortium (SMC) and suggestions for improvements to current processes
5. The impact of the current lack of access to new cancer medicines on trial placement decisions, clinician retention and recruitment and patient benefits

Executive Summary

Novartis welcomes the Health and Sport Committee inquiry into general issues regarding the approval process for newly licensed medicines and the IPTR process. This is taking place against the background of much debate about access to cancer medicines in Scotland.\(^2,3\)

The IPTR process

We believe that it is important to provide further input into the assumption that the IPTR process constitutes an adequate route for patients to access medicines that have not been approved by the SMC. Internal Novartis sales data and anecdotal evidence from discussions with clinicians have shown that patients struggle to obtain funding for such medicines through IPTRs due to the strict criteria applied to prove exceptionality. In the area of cancer medicines this creates inequity between Scotland and England as English patients are able to access funding for cancer drugs through the Cancer Drugs Fund (CDF).
While we are keen to emphasise that the IPTR process is not an adequate route to funding for drugs that are not SMC approved we believe that there are further steps that could be taken to improve the process itself:

- National decision-making with ring-fenced funding
- Greater transparency
- Less bureaucracy
- Removal of the exceptionality requirement for patients with orphan and/or ultra-orphan diseases
- Implementation and audit of peer support
- Standardisation of IPTR forms across all Health Boards

**SMC**

Novartis is fully supportive of the SMC but would like to highlight the need to review its processes for appraising ultra-orphan drugs since the current system hinders patient access and raises questions as to whether it constitutes an efficient use of resources.

**Impact of lack of access to cancer medicines on trial placement decisions, clinician retention and recruitment and patient benefit**

Novartis’s ability to place trials of innovative cancer medicines in Scotland is being impacted negatively by the lack of access to drugs that have become standard of care in England and other parts of the world. We have had discussions with clinicians suggesting that the inability to participate in the trial of new drugs and subsequent lack of access to successful drugs for use in a clinical setting is of significant concern. In the long run this situation may have a negative impact on Scotland’s ability to attract and retain clinician talent, especially if the CDF in England continues beyond 2014. More importantly, the lack of patient access to trials puts Scottish patients at a distinct disadvantage as they are unable to enter trials that may lead to successful treatment. A further point is that this risks undermining the Scottish Government’s commitment to boost innovation in Scotland as announced by the Scottish Health Secretary in her *Statement of Intent for Innovation in Health* in June 2012.

**1. The purpose of the IPTR process**

This is a key question since it lies at the heart of the debate about access to medicines in Scotland. There seems to be a perception that provided a clinician recommends a particular medicine for use in a patient, IPTRs can be used to allow patients to access that medicine even if it is not routinely funded on NHS Scotland. The reality is, however, that IPTRs are not an easy route to obtain funding. Instead, for drugs that are not recommended by the SMC, clinicians have to demonstrate that their patient is clinically exceptional, i.e. different to the overall patient population or likely to benefit more from a treatment than the average patient with the same disease.

To provide an example from our portfolio: everolimus is licensed as a second-line treatment for patients with advanced renal cell carcinoma (aRCC). Out of the 879 patients in Scotland who are estimated to develop renal cell carcinoma each year, 116 are estimated to be eligible for treatment with everolimus (based on 2009 figures). This is a conservative estimate as the incidence of kidney cancer is...
increasing. However, the SMC did not approve everolimus in this indication and as a result only six Scottish patients have accessed the treatment since it was licensed in 2006. By contrast, in England more than 700 patients have been treated with everolimus for aRCC since the introduction of the CDF. The reason for this low level of access is that most patients fit the characteristics of the patient population included in the relevant trials and are therefore rejected. And those who fall outside these characteristics are denied funding because there is no evidence that the treatment will work for them.

A further example from our portfolio is fingolimod, a disease modifying therapy in highly active relapsing multiple sclerosis which was ‘not recommended’ by the SMC on 12th March 2012. Since the drug was launched clinicians have submitted approximately 10 IPTRs in order to prescribe fingolimod for their patients across several Health Boards, with only 3 being approved to date. This again is a disease area where most patients fit the characteristics of the patient population included in the relevant trials, making access through the IPTR process difficult.

This all supports our argument that the IPTR process constitutes a major barrier to funding that is not easily overcome even if the process itself worked perfectly.

2. Problems with the IPTR process and potential solutions

Lack of transparency

Historically, the Scottish Government has not collected data on IPTR submissions and approvals per Local Health Board. This means that there is little transparency in the system and no robust evidence to determine to what extent approval levels vary from Health Board to Health Board and why.

Recommendation: A system to be put in place which collects data centrally on a regular basis to identify the number of requests submitted per Health Board, the number of approvals, the type of treatment approved and associated costs. Patient confidentiality issues may dictate that not all of this data can be made public. However, general conclusions should be published on a regular basis.

Uncertainty about the right level of requests v approvals

Taken at face value the high approval rates reported by the Scottish Government suggest that IPTRs are an effective way of accessing treatment. However, clinicians may be deterred from submitting requests, not only because of the difficulty of proving exceptionality but also because:

- Some Health Boards appear to exert pressure on clinicians not to submit any requests
- The bureaucracy and length of time it takes to obtain a decision (this is particularly relevant in relation to patients with metastatic cancer where the patient’s disease is usually so advanced that there is no time to wait for the protracted IPTR process to run its course)
- They are concerned about raising expectations that may not be met
- Health Boards are able to deny funding to proven exceptional patients on grounds of affordability
- Clinicians struggle to understand how to demonstrate exceptionality
Recommendation 1: The Health and Sport Committee may want to call on the Government to conduct a survey of clinicians to establish the impact of these barriers on submission rates. This would help validate work undertaken by the ABPI in Scotland.

Recommendation 2: The Health and Sports Committee should explore these issues with the Royal College of Physicians during their evidence session.

Exceptionality and orphan/ultra-orphan diseases

Patients with orphan and ultra-orphan diseases face particular hurdles when trying to access funding for treatment via an IPTR. The smaller the overall patient population, the greater the challenges in defining what constitutes an exceptional patient. Patients with very rare diseases are therefore at a disadvantage over patients with common diseases, particularly if the clinician treating them is not a specialist in their disease, and will therefore find it more difficult to define an ‘exceptional’ patient. In many cases it may not be possible for individual Health Boards to draw on the expertise of Scottish specialists let alone specialists from their own area. This problem is exacerbated by the fact that patients with rare diseases tend to have considerably fewer treatment options.

Examples of rare diseases from our portfolio where, due precisely to their low prevalence, there is limited clinical expertise across the UK include Cushing’s disease, acute myeloid leukaemia and aggressive systemic mastocytosis. These are disease areas where Novartis foresees potential concerns around the availability of appropriate clinical expertise on IPTR panels unless the problem of exceptionality in the IPTR process is properly addressed.

Recommendation 1: The Health and Sport Committee should consider calling for the removal of the exceptionality requirement from requests for ultra-orphan medicines where the SMC’s acceptability criteria are difficult to achieve. Alternatively, funding for these patients could come automatically from the National Service Division without requiring patients to go through the IPTR process.

Extending this approach to all patients with orphan diseases as suggested by Rare Disease UK could substantially increase access to medicines in Scotland. However, this extension may be difficult to justify since the methodological problems affecting SMC appraisals of ultra-orphan drugs do not apply to orphan drugs to the same degree and a decision to remove exceptionality from orphan drugs would require a substantial increase in the funding currently provided for IPTRs.

Recommendation 2: When exploring the cost implications of removing the need to prove exceptionality from patients with ultra-orphan and orphan diseases the Health and Sport Committee may want to explore the costs of running IPTR panels in each of the Scottish Health Boards and what savings could be made by establishing a national IPTR panel rather than funding individual IPTR panels in each Local Health Board.

Peer support proposed in CEL17

The last Chief Executive Letter clarifying IPTR processes (CEL17) suggested the provision of peer support to enable clinicians to submit high quality requests with the
support of other clinical experts. The Scottish Government has in the past stated its commitment to monitoring NHS board progress in implementing CEL17 guidance.\textsuperscript{16}

However, recent responses to a number of Parliamentary Questions tabled by Jackie Baillie MSP suggest that the Government does not know whether the concept of peer support has been implemented and what the results may have been (these questions can be found in full in the reference).\textsuperscript{17}

**Recommendation:** The Health and Sport Committee should explore with the Royal College of Physicians whether a peer support process has been put in place and whether there is any evidence that it is being used.

**Standardisation of IPTR forms across all Health Boards**

Discussions with stakeholders in Scotland have highlighted a concern regarding variations in IPTR forms in terms of length and information requirements.\textsuperscript{18} To improve the chances of equitable decisions there should be a universally agreed form, and ideally one that can be filled in reasonably quickly. We are also concerned that the time some clinicians are spending completing forms could be better spent focussing on patient care.

**Recommendation:** The Health and Sport Committee should explore with stakeholders the possibility of a standardised, universally agreed IPTR form. The Committee may wish to question Health Boards directly on any potential barriers to adopting a universal IPTR form.

3. **A more radical approach - A national IPTR panel with ring-fenced funding**

A national IPTR panel to deal with all IPTR requests would be the most straightforward way to ensure consistency in the application of the IPTR process. This would require some thought about the membership of the panel, the role of clinical experts and the involvement of individual patients as well as the funding of approved IPTRs. One option would be to establish a ring-fenced budget (potentially pooling the budgets from Health Boards currently spent on IPTRs) to fund treatment for patients who have been shown to be exceptional.

**Recommendation:** The Health and Sport Committee should examine the option of a national IPTR panel and approval process with ring-fenced funding to cover the cost of approved requests.

4. **The role of the SMC**

In Novartis’s view, the current SMC process is thorough and fit for purpose in the vast majority of cases. Although the SMC wish to review such treatments, it is unclear whether their standard decision-making criteria are suitable, particularly when the evidence base is limited as a result of the very small patient population affected. Further, it is not clear whether undertaking a full review of treatments for very rare diseases is a good use of NHS Scotland’s resources; it is also unlikely to be commercially viable for companies to undertake the work required for a full submission when it cannot meet the SMC’s necessarily stringent requirements. By definition, the number of patients affected is small but their need is invariably great.
Recommendation: When taking evidence from the SMC the Health and Sport Committee should elicit their views on potential alternative ways to appraise medicines for ultra-orphan indication, such as abbreviated submissions. This may both improve patient access to treatment and make the best use of available resources.

5. Impact of lack of access to cancer medicines on trial placement decisions, clinician retention and recruitment and patient benefit

Novartis’s ability to place trials of innovative cancer medicines in Scotland is being impacted negatively by the lack of access to drugs that have become standard of care in England and other parts of the world. We have had discussions with clinicians suggesting that the inability to participate in the trial of new drugs and subsequent lack of access to successful drugs for use in a clinical setting is of significant concern. In the long run this situation may have a negative impact on Scotland’s ability to attract and retain clinician talent, especially if the CDF in England continues beyond 2014. More importantly, the lack of patient access to trials puts Scottish patients at a distinct disadvantage as they are unable to enter trials that may lead to successful treatment.

References

1 The views expressed in this document reflect our own discussions with stakeholders, some of whom have spoken to us in confidence and are therefore reported as anecdotal evidence.
4 Novartis briefing, Barriers to conducting clinical trials in Scotland (available on file)
6 Novartis internal calculation of patient population eligible to receive treatment with Afinitor for advanced Renal Cell Carcinoma (aRCC) (available on file)
8 Novartis internal sales figures (available on file)
9 See reference 1 – this point is based on anecdotal evidence / confidential discussions
11 Novartis internal sales figures (available on file)
12 See reference 1 – this point is based on anecdotal evidence / confidential discussions
13 Further information on cushing’s disease can be found at: http://www.patient.co.uk/doctor/cushings-syndrome - accessed on 20th August 2012
14 Further information on acute myeloid leukaemia can be found at: http://www.nhs.uk/conditions/Leukaemia-acute/Pages/Introduction.aspx - accessed on 20th August 2012
15 Further information on aggressive systemic mastocytosis can be found at http://www.orpha.net/consor/cgi-bin/OC_Exp.php?Expert=98850&lng=EN – accessed on 20th August 2012
See reference 1 – this point is based on anecdotal evidence / confidential discussions

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03 September 2012