Access to newly licensed medicines

James Whale Fund for Kidney Cancer

This response is submitted on behalf of the James Whale Fund for Kidney Cancer (JWF), the UK’s leading specialist kidney cancer charity. The JWF seeks to help reduce the harm caused by kidney cancer by increasing knowledge and awareness, providing patient information and by supporting research into the causes, prevention and treatment of the disease. The JWF has recently established a new branch of the charity in Scotland to support Scottish patients in the fight against kidney cancer.

Executive Summary

Kidney cancer is a rare cancer which is notoriously difficult to detect and diagnose. Effective treatment is therefore often the only option available to patients. The difficulties involved in accessing certain types of treatment for kidney cancer in Scotland is an area of great concern to the JWF and the patients on whose behalf we campaign, which is why we welcome the opportunity to provide this evidence to the Health and Sport Committee.

The JWF has always remained close to the kidney cancer patients we serve and our evidence reflects the views of the patient community as much as possible. We hope that this submission is informative and constructive, and we would be happy to discuss it with you at any time. Contact details can be found at the end of this document.

1. Early Detection / Diagnosis of Kidney Cancer

Like many rare cancers, kidney cancer is notoriously difficult to detect.\(^1\) It is essential that this is borne in mind when investigating access to treatments. Whilst research and investment into early detection and diagnosis is hugely important (and the JWF welcomes this wholeheartedly), the only option available to many kidney cancer patients is effective treatment in order to slow the progression of the disease. It is therefore vital that we have mechanisms in place (i.e. the Scottish Medicines Consortium (SMC) and Individual Patient Treatment Requests (IPTRs)) that are fair, workable, transparent, carefully monitored and have the necessary funding behind them.

2. Scottish Medicines Consortium (SMC)

We recognise the difficult job the SMC sometimes has in appraising new medicines. However, we are concerned that the SMC’s processes are currently inadequate for assessing treatments for rare cancers like kidney cancer, which by definition affect smaller cohorts of patients. Fewer patients involved in fewer trials means there may be less information available with which to make robust appraisals of new treatments. It is therefore concerning that new treatments for rare cancers are evaluated in the same way and using the same health economics as treatments for more common cancers. We would urge the Committee to investigate this issue with the SMC.
3. Individual Patient Treatment Requests (IPTRs)

3.1 Inequality and inequity

The Committee will be well aware of the feelings of inequality felt by many cancer patients in Scotland who see their counterparts south of the border gaining access to treatments not recommended by the SMC through the CDF. We can confirm that this is a view held by many of the kidney cancer patients with whom we discuss these matters.

 Whilst we understand that a CDF in Scotland might not be the solution (and that the CDF in England has its own problems), it is essential that kidney cancer patients undergoing great deals of anxiety and distress feel that, at the very least, there is sufficient Health Board funding available for approved IPTRs and that the IPTR process is fair across the whole of Scotland.

 We are concerned about reported inconsistencies across different Health Boards in a) numbers of IPTRs being submitted by clinicians and b) approval rates of IPTRs. This might be due to the difficulties involved in proving exceptionality (more information below), issues of funding for approved IPTRs at individual Health Board level, or inconsistencies in procedures and expertise on individual IPTR panels across Scotland. Whatever the reasons, this inconsistency needs to be investigated in detail by the Committee. One potential solution is for the Scottish Government to consider a more centrally governed process for the assessment and funding of IPTRs.

3.2 Transparency

A large part of ensuring that the IPTR process is fair and provides a consistent route to access across Scotland is to carefully monitor its effectiveness. Despite regularly updated CEL17 guidance, we are concerned that the Government is not doing this. When questioned on certain aspects of the IPTR process the Government’s default line seems to be that the required information is ‘not held centrally.’ This lack of transparency – and therefore accountability – is not acceptable, and we would urge the Committee to consider options for more robust oversight and monitoring of the IPTR process.

A related point is the transparency of the process in the patient’s eye. Patients need to feel empowered when going through the difficult process of seeking treatment, and ensuring that clinicians properly explain the processes involved and that there is clear, easily-accessible information available on the IPTR process is absolutely essential. The Committee may wish to explore this issue with the Royal College of Physicians.

3.3 Timeliness

One concern expressed by certain patients is the length of time that IPTRs can take to process. It is not just the emotional anxiety of waiting for the results of an application that is the issue; it is also the deterioration in a patient’s condition that this can create. Coupled with the perceived opacity of the process, this is an incredibly distressing situation for patients and their families. The Committee should investigate ways to streamline the IPTR process.
3.4 Exceptionality

It is our understanding that the IPTR process only results in access to drugs if it is possible to prove that a patient could benefit from a drug *more than the average patient*. Fundamentally, we find it hard to justify having to prove exceptionality at all when the clinical benefit of a treatment is not in question.

On a more technical point, we are concerned that it is so much more difficult to define exceptionality when dealing with smaller patient populations and that this might be having an effect on the numbers of IPTRs being submitted by clinicians, as well as the number being approved by individual IPTR panels. We would urge the Committee to thoroughly investigate the concept of exceptionality and whether it is appropriate for all types of cancer.

4. Value-Based Pricing

Looking to the future, it is of growing importance that the Scottish Government is engaging effectively with the UK Government over future pricing arrangements for newly licensed medicines in Scotland. We know that the Scottish Government has had assurances that it will be involved in the design of any scheme, but it is important that more detail is provided swiftly and at regular intervals. Although we understand that the Committee’s immediate concerns are the current processes for appraising newly licenced medicines and the IPTR process, it might wish to consider ways in which patient groups can be kept regularly informed of these important discussions.

James Whale Fund, Understanding Kidney Cancer Information Leaflet, 

Examples can be found here: 
http://www.scottish.parliament.uk/S4_ChamberDesk/WA20120711.pdf
http://www.scottish.parliament.uk/S4_ChamberDesk/WA20120514.pdf

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