Medicine for Managers

Patient access schemes in the new NHS

John Spoors

Abstract

Patient access schemes (PAS) have become an integral part of the UK pharmaceutical environment. This article investigates the historical role PAS have played in regard to health technology appraisals (HTAs), how the mechanism interacts with other features of the UK funding environment, such as the Cancer Drugs Fund (CDF), and the PAS strategies employed by pharmaceutical companies to optimise funding recommendations. It also takes a forward look towards any role PAS might have in a value-based pricing system, which is potentially facing the UK from 2014. PAS have shifted from outcome-based schemes to financially-based discounts. It is clear that as PAS have become more integral to the UK HTA environment, an acceptance of confidentiality and a requirement to prove that PAS reduce uncertainty to payers are two major developments. The article also highlights how the CDF may act as a potential disincentive for manufacturers to engage with PAS and provide the NHS with discounts to achieve cost-effectiveness. The key going forward is stability; providing a framework which allows payers to determine value, but permits pharmaceutical companies to exist in a credible pricing operating environment.

History of patient access schemes

Since the late 1950s, prices for medicines in the UK have been guided by a voluntary agreement between government and the pharmaceutical industry known as the Pharmaceutical Price Regulation Scheme, which has been revised or renewed every five or six years. The 2009 Pharmaceutical Price Regulation Scheme formally introduced the concept of PAS, to ensure drug prices, which are freely set by the manufacturer, reflect better value (Office of Health Economics, 2011). PAS, therefore, are designed to ensure patients can gain access to medicines, which due to international reference pricing, are likely to have a high cost and might not be deemed cost-effective by payers.

It is worth noting that PAS are not a new phenomenon in the UK; and actually date back to 2002, when the Department of Health and various pharmaceutical companies agreed a ‘risk-sharing scheme’ for treatments for multiple sclerosis (MS) (Department of Health, 2002). The cost-sharing initiative was agreed to ensure that MS patients could receive four disease-modifying drugs on the NHS, after the National Institute of Health and Clinical Excellence (NICE) ruled that the treatments—beta interferons and glatiramer acetate—were not cost-effective (NICE, 2002). Under the terms of the scheme, the government agreed to provide the drugs on the NHS while research was carried out to assess their long-term cost-effectiveness. The NHS would then gradually stop paying for the drugs if patients did not appear to be
benefiting. However, the scheme has not lived up to expectations and has been largely criticised for not providing value for money to the NHS.

‘For the NHS, however, the scheme can be judged only ‘a costly failure’ as suggested by the House of Commons Health Committee which has been raising concerns about the scheme for several years. The biggest losers are the other NHS patients who would otherwise have benefited from the money spent on the scheme’ (Rafferty, 2010).

The next milestone in PAS came about in 2007, with Technology Appraisal (TA) 129, bortezomib (Velcade®) monotherapy for relapsed multiple myeloma (NICE, 2007). Following a successful appeal in February 2007, the manufacturer of bortezomib, Janssen-Cilag (now Janssen) submitted a ‘risk-sharing scheme’ in which they would rebate the full cost of bortezomib for people who, after a maximum of four cycles of treatment, have less than a 50% reduction in serum M-protein (that is, less than a partial response).

The Velcade® risk-sharing scheme allowed the product to be deemed cost-effective by NICE. However, concerns have been raised around the bureaucracy in administering the scheme. Steve Williamson in his report into the uptake of PAS in the NHS observed:

‘We gathered the greatest amount of data on the bortezomib (Velcade®) VRS scheme 2002 2007 2009 2009

... The scheme has issues with tracking and ensuring all patients are stopped on bortezomib after non responders are claimed for. A key feature of the comments was the difficulty in pharmacists managing the scheme when they were not directly involved in the clinical management and testing of patient response’ (Williamson, 2009).

It is clear, therefore, that if the cost of administering a PAS diminishes the value of the initial scheme, then this model of PAS is unsustainable going forward.

In 2009, NICE ruled on four medicines for the treatment of renal cell carcinoma, sunitinib (Sutent®, Pfizer), sorafenib (Nexavar®, Bayer), temsirolimus (Torisel®, Wyeth (now Pfizer)) and bevacizumab (Avastin®, Roche). In this controversial appraisal—which was split into two parts, TA 169 (NICE, 2009a) and TA 178 (NICE, 2009b)—NICE recommended Pfizer’s Sutent® for the first-line treatment of advanced and/or metastatic renal cell carcinoma. Crucially, Pfizer submitted a PAS in which the first treatment cycle of Sutent® was free to the NHS. The key to this scheme was that it was effectively a discount, which was very simple to administer and did not constitute an excessive burden on the NHS. This was a landmark appraisal in terms of PAS, and ensured that going forward, manufacturers had to put emphasis on simple financial discounts which were easy to administer, rather than complex outcomes-based schemes as shown in Figure 1.
Confidentiality

The requirement to demonstrate value to payers is increasingly important in the current strained economic environment. It is therefore unsurprising that we have seen an increase in the number of PAS submitted, as companies strive to achieve cost-effectiveness in an era of diminishing budgets. As PAS have become integral to the HTA environment, one key development we have seen is the acceptance from NICE and the Department of Health to allow the size of the discount submitted in the PAS to remain commercial-in-confidence (CiC). Pharmaceutical companies are reluctant

New developments in PAS

Since the kidney cancer appraisals in 2009, PAS have become an integral part of the UK HTA landscape. PAS offer manufacturers the opportunity to improve their product’s cost-effectiveness, while retaining the original list price. At the time of writing, NICE has recommended 21 technology appraisals with approved PAS*, as shown in Table 1.

As you can see from the above table, PAS cover a wide range of therapy areas, and are not exclusively linked to cancer medicines. As PAS have become more commonplace, two key elements have emerged: confidentiality and reducing uncertainty.

<table>
<thead>
<tr>
<th>TA Ref</th>
<th>Treatment</th>
<th>Indication</th>
<th>Company</th>
</tr>
</thead>
<tbody>
<tr>
<td>TA129</td>
<td>Bortezomib (Velcade®)</td>
<td>Multiple myeloma</td>
<td>Janssen-Cilag</td>
</tr>
<tr>
<td>TA155</td>
<td>Ranibizumab (Lucentis®)</td>
<td>Macular degeneration (Acute wet AMD)</td>
<td>Novartis</td>
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<tr>
<td>TA162</td>
<td>Erlotinib (Tarceva®)</td>
<td>Non-small cell lung cancer</td>
<td>Roche</td>
</tr>
<tr>
<td>TA169</td>
<td>Sunitinib (Sutent®)</td>
<td>Renal cell carcinoma</td>
<td>Pfizer</td>
</tr>
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<td>TA171</td>
<td>Lenalidomide (Revlimid®)</td>
<td>Multiple myeloma</td>
<td>Celgene</td>
</tr>
<tr>
<td>TA176</td>
<td>Cetuximab (Erbitux®)</td>
<td>Metastatic colorectal cancer (first Line)</td>
<td>Merck Serono</td>
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<tr>
<td>TA179</td>
<td>Sunitinib (Sutent®)</td>
<td>Gastrointestinal stromal tumour</td>
<td>Pfizer</td>
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<td>TA180</td>
<td>Ustekinumab (Stelera®)</td>
<td>Moderate to severe psoriasis</td>
<td>J&amp;J / Janssen-Cilag</td>
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<td>Trabectedin (Yondelis®)</td>
<td>Advanced soft tissue sarcoma</td>
<td>PharmaMar</td>
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<td>Certolizumab pegol (Cimzia®)</td>
<td>Rheumatoid arthritis</td>
<td>UCB</td>
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<td>TA192</td>
<td>Gefitinib (Iressa®)</td>
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<td>AstraZeneca</td>
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<td>TA215</td>
<td>Pazopanib (Votrient®)</td>
<td>Advanced renal cell carcinoma</td>
<td>GSK</td>
</tr>
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<td>TA218</td>
<td>Azacitidine (Vidaza®)</td>
<td>Myelodysplastic syndromes, chronic myelomonocytic leukaemia and acute myeloid leukaemia</td>
<td>Celgene</td>
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<td>TA220</td>
<td>Golimumab (Simponi®)</td>
<td>Psoriatic arthritis</td>
<td>Merck Sharp &amp; Dohme</td>
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<td>TA221</td>
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<td>Chronic idiopathic (immune) thrombocytopenic purpura</td>
<td>Amgen</td>
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<td>Rheumatoid arthritis</td>
<td>Merck Sharp &amp; Dohme</td>
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<tr>
<td>TA233</td>
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<td>Ankylosing spondylitis</td>
<td>Merck Sharp &amp; Dohme</td>
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<td>High grade resectable non-metastatic osteosarcoma</td>
<td>Takeda</td>
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<td>TA238</td>
<td>Tocilizumab (RoActemra®)</td>
<td>Systemic juvenile idiopathic arthritis</td>
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<td>TA241</td>
<td>Nilotinib (Tasigna®)</td>
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<td>Novartis</td>
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<tr>
<td>TA247</td>
<td>Tocilizumab (RoActemra®)</td>
<td>Rheumatoid arthritis</td>
<td>Roche</td>
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*Please note that this does not include submissions which have included PAS, but have been rejected by NICE

Source: NICE website

Table 1: Table 1: List of technologies with approved PAS recommended by NICE for use in the NHS

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Confidentiality

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to allow competitor firms to see the size of the discount offered to HTA bodies, particularly if more than one discount is required. Therefore, permitting PAS to remain CIC, has arguably made submitting PAS a more viable concept to the pharmaceutical industry.

Reducing uncertainty

The key to any PAS is that apart from providing a value proposition to payers, it has to reduce uncertainty surrounding the product. HTA bodies, acting on behalf of payers, have to be convinced that their recommendations are not going to place an unviable burden on the healthcare system. The requirement for payers to have authoritative guidance as close to launch as possible, has meant that the evidence-base for the product is not as mature; therefore, establishing the long-term value of the product is increasingly difficult, particularly in rare diseases where the patient numbers in clinical trials may be small.

The pharmaceutical company GlaxoSmithKline (GSK) addressed this situation in an innovative way for their renal cell carcinoma product pazopanib (Votrient®) by submitting a two-part PAS in NICE TA 215 (NICE, 2011).

The innovative nature of the Votrient® scheme shows how a PAS can demonstrate value to HTA bodies and payers by reducing uncertainty. Part A of the Votrient® scheme is a straight discount which reflects current trends with regard to PAS. However, Part B of the scheme, addresses the lack of clinical data at the time of the appraisal, and provides the HTA body with a future financial ‘guarantee’ if the product does not perform as well in the head-to-head trial as expected.

Due to this PAS, Votrient® was fast-tracked* through NICE, and therefore authoritative advice was available to payers earlier than if the product had gone through the normal NICE process.

Strategy around PAS

With PAS becoming integrated within the UK HTA environment, the requirement to get strategy right for PAS has become increasingly important for pharmaceutical companies. There are effectively three opportunities to submit a PAS in the NICE process as shown in Figure 3. Crucially, manufacturers or sponsors of a technology wishing to put forward a PAS must seek approval from the Department of Health before submitting their proposal to NICE’s Patient Access Scheme Liaison Unit (PASLU).
PASLU then advises the Department of Health whether the proposed PAS is feasible within the NHS in England and Wales. It takes PASLU about 12 weeks from receiving a submission to sending final advice to the Department of Health. With set opportunities to submit PAS in the NICE process, it is vital that planning is incorporated into the HTA strategy as early as possible. There are implications for submitting PAS at different time points in the NICE process including:

- Submitting a PAS upfront shows willing from the pharmaceutical company, and the product may be available earlier to patients if the decision from NICE is positive. However, the company may have to revise the PAS and provide a further discount if the product is initially deemed not cost-effective by NICE’s appraisal committee.
- Submitting a PAS in response to an appraisal consultation document (ACD) means the pharmaceutical company can design the PAS with input from NICE’s appraisal committee. However, this will delay the appraisal and there is no guarantee the PAS will result in the product being deemed cost-effective.
- Submitting a PAS post-final appraisal determination (FAD) allows the pharmaceutical company to further tailor a scheme following a NICE appraisal. If the initial appraisal was positive, submitting a PAS can help expand a product’s indication and availability to patients while the initial positive guidance from NICE is published. However, if the decision is negative, progressing to a rapid review will delay medicine access to the patients until the pharmaceutical company can design a scheme for the product to achieve cost-effectiveness.
Cancer drugs fund and PAS
As we have seen from the previous section, there are different opportunities to submit a PAS throughout the NICE process to try and avoid a negative decision. One scenario we haven’t explored is the availability of a fund for medicines which have a negative NICE appraisal. The CDF was announced by the Department of Health in October 2010 with the aim of providing £200 million a year in funding for cancer drugs from April 2011 to the end of 2013 (Department of Health, 2010). There is an argument to be made that having a specific fund for cancer is acting as a disincentive for manufacturers to offer the NHS discounts through PAS. As Figure 4 opposite shows, if the cost to the manufacturer in implementing a PAS is more than what they estimate to be reimbursed through the CDF, then they are less likely to submit a PAS. An important point to stress is that NICE-approved medicines should be funded out of existing PCT budgets, while drugs funded through the CDF should be for medicines not recommended by NICE (Department of Health, 2012) or for those where NICE guidance is not yet currently available.

Figure 4 highlights the potential impact of short-term policies such as the CDF. While the CDF provides immediate access and short-term gains for the patient and the pharmaceutical company, given the CDF is in theory a time-limited source of funding, the long-term outcomes of such policies are still open to interpretation.

Conclusions and a look to the future
It is clear that PAS are an important and integral part of the UK HTA environment. The evolution of PAS in the UK has demonstrated the importance of ensuring that schemes submitted to the NHS are simple to administer. The essence of PAS in the new NHS are financial discounts, which may give us clues as to how any value-based assessment might work when the current Pharmaceutical Price Regulation Scheme expires at the end of 2013. If NICE is indeed at the centre of a value-based pricing system, and makes recommendations on the potential price for reimbursement, are the sort of financial discounts we are currently observing in the form of PAS a primitive version of this system?

How PAS are likely to evolve in the future remains to be seen. The key, as observed from the Votrient® example, is to design a scheme which addresses the HTA body’s concerns and reduces uncertainty surrounding the future value of the product. This may be obtained by offering a simple discount in order to achieve cost-effectiveness. However, sometimes more innovative approaches are required.

The decision on whether to offer a PAS should always rest with the pharmaceutical company—however, could more be done to encourage companies to offer a PAS? Is there any way to streamline the NICE rapid review process? Could more be done to assist the NHS with the administration of PAS to deliver efficiencies to both the NHS and pharmaceutical company? How can we improve transparency around schemes, while recognising the importance of maintaining confidentiality? The answers to these questions are complex, and go beyond the scope of this article. However, a forum on this issue involving payers, NICE, industry, administrators and patients would be a useful environment to discuss how to optimise PAS.

Finding ways of incentivising pharmaceutical companies to submit PAS would be advantageous in terms of delivering value to the NHS, however, it is worth stating that short-term policies such as the CDF potentially act as a disincentive for
manufacturers to engage with value propositions such as PAS. The CDF has largely been heralded as a success (Rarer Cancers Foundation, 2012) due to the impact it has had in improving patient access to cancer drugs. However, the long-term ramifications of this policy are not clear; and unless the Government can maintain the fund going forward, there may be damaging consequences to patients and commercially to the pharmaceutical companies in the long-term. There has been considerable frustration from the pharmaceutical industry regarding the lack of clarity from the Government on the future pricing system in the UK (PM Live, 2012). PAS remain a current solution to bridge the gap between innovation and affordability. However, the long-term viability of PAS and the CDF is surely questionable in a post-Pharmaceutical Price Regulation Scheme era. The key is stability—providing a framework which allows payers to determine value, but permits pharmaceutical companies to exist in a credible pricing operating environment.


National Institute for Health and Clinical Excellence (2009a) TA 169 Sutinib for the First-line Treatment of Advanced and/or Metastatic Renal Cell Carcinoma. NICE, London

National Institute for Health and Clinical Excellence (2009b) TA 178 Bevacizumab (First-Line), Sorafenib (First- and Second-line), Sunitinib (Second-line) and Temsirolimus (First-line) for the Treatment of Advanced and/or Metastatic Renal Cell Carcinoma. NICE, London


