

The Influence of Patient Access Schemes on Appraisal Decisions by NICE in the UK

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Introduction

In the UK, the National Institute of Health and Clinical Excellence (NICE) provide recommendations on the use and reimbursement of innovative drugs in the NHS. Many of the submissions include risk sharing initiatives known as patient access schemes (PAS) that typically take the form of a discount, rebate or fixed price agreement, which aim to enhance the cost-effectiveness of the product.

PAS have become increasingly more common in submissions to NICE; a previous ISPOR abstract showed an increase in PAS inclusion in submissions from 0 in 2006 to 29% in 2009.¹ However, concerns have been raised regarding the increasing complexity and the suitability of some of these schemes compared to other cost-containment methods, as the UK shifts towards value-based pricing.

Here we aim to evaluate the influence of including PAS on appraisal decisions by NICE, and consider the impact of PAS inclusion on future submissions.

Methods

All NICE single technology appraisals and appraisals in development published on www.nice.org.uk from January 2010 to 20th April 2011 were reviewed by two individuals; terminated and suspended appraisals were excluded.

Published submissions and final appraisal determinations in which a PAS was included in the manufacturers submission were identified.

PAS were defined as the manufacturer providing a pre-defined reduction in overall cost of treatment through risk-sharing or rebate schemes.

Results

Published Appraisals

In 2010, NICE published 27 technology appraisals, of which 6 (22%) included a PAS. In the first 4 months of 2011, 9 technology appraisals were published, of which 4 (44%) included a PAS. A summary of all published appraisals from 2010 and 2011 that included a PAS is given in Table 1.

Despite the inclusion of a PAS in the submission, 3 drugs in 2010 and 2 in 2011 were given a negative appraisal. In previous years all therapies with a PAS included in the submission were given a positive recommendation.¹ Figure 1 shows the percentage of published appraisals including a PAS each year since 2007, differentiated by the status of the recommendation.

Appraisals in Development

There were 9 appraisals in development identified for which the final appraisal determination was available (subject to appeal). Of these, 5 submissions (56%) included a proposed PAS in the original or revised submission. Only 2 of these drugs received a positive appraisal.

Table 1: Summary of all published single technology appraisals containing a patient access scheme in 2010 and 2011

Year	Drug	Therapeutic Area	Status	Details of PAS
2010	Bevacizumab (TA212)	Colorectal cancer (metastatic)	Not recommended	Fixed price per cycle, free after 12 cumulative months
	Ofatumumab (TA202)	Chronic lymphoid leukaemia	Not recommended	Discounted price
	Gefitinib (TA192)	Non-small cell lung cancer (first line)	Recommended	Fixed cost and treatment < 3 months supplied for free
	Sorafenib (TA189)	Hepatocellular carcinoma (first line)	Not recommended	Rebate for every 4 th pack
	Certolizumab pegol (TA186)	Rheumatoid arthritis	Recommended	First 12 weeks of therapy provided free of charge
	Trabectedin (TA185)	Soft tissue sarcoma	Recommended	Free after 5 th treatment cycle
2011 (up to 20 th April)	Everolimus (TA219)	Renal cell carcinoma (second line)	Not recommended	Discount, commercial in confidence
	Azacitidine (TA218)	Myelodysplastic syndromes	Recommended	Discounted price
	Pazopanib (TA215)	Renal cell carcinoma (first line metastatic)	Recommended	12.5% discount, future rebate linked to outcome of trials
	Bevacizumab (TA214)	Breast cancer (in combination with taxane)	Not recommended	Cost of first 10 g is waived (not approved by the DoH)

Figure 1: Percentage of published appraisals with PAS by year

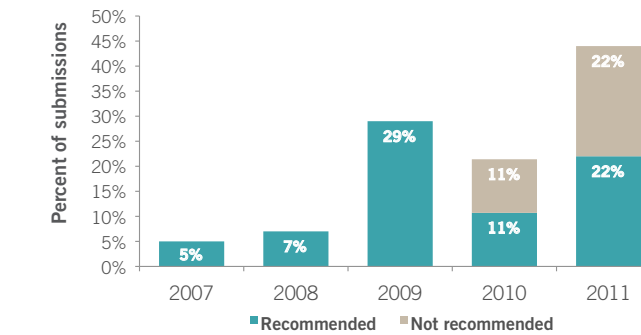
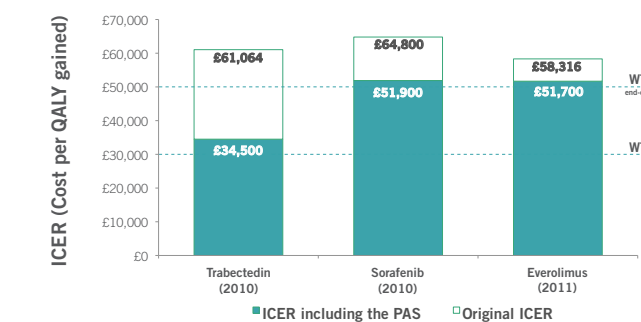


Figure 2: Change in ICER with the inclusion of a PAS



For the first time in 2010, drugs for which a PAS was included in the submission were not recommended. This trend continued in 2011, and is also reflected in the appraisals currently in development.

In many cases, despite the inclusion of a PAS in the economic model, the proposed ICER failed to fall under the assumed NICE willingness-to-pay (WTP) threshold of £30,000 per QALY. Figure 2 demonstrates how the ICERs for everolimus (TA219) and sorafenib (TA189) remained above £50,000 per QALY, even with the inclusion of a PAS. Inclusion of a PAS in the economic model for trabectedin reduced the ICER to £34,500 per QALY which when considered in light of the NICE end-of-life criteria falls below the higher assumed WTP threshold of £50,000 per QALY and therefore the drug received a positive appraisal.

For bevacizumab in breast cancer (TA214) the proposed PAS had not been approved by the Department of Health (DoH) and therefore the subsequent ICERs were not considered by the evidence review group (ERG). The excessive administrative burden of the PAS for bevacizumab in colorectal cancer (TA212) was highlighted by the ERG.

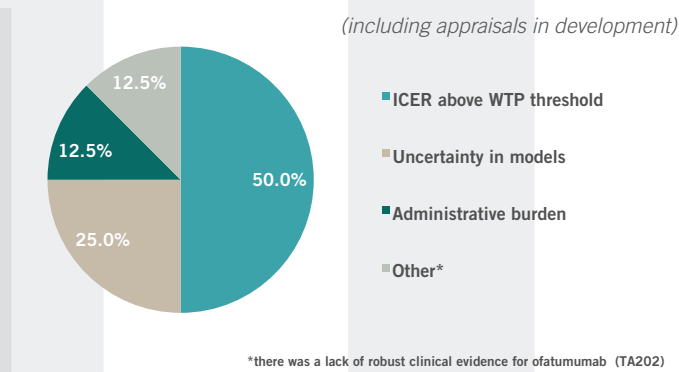
Discussion

Although the number of PAS has risen dramatically over the past 5 years, it is increasingly clear that the inclusion of these schemes is not necessarily synonymous with receiving a positive appraisal.

A PAS should be designed to enhance the cost-effectiveness of the product, and therefore increase the availability of the drug within the NHS. However, in many cases, even with the inclusion of a PAS in the economic model the ICER was still much too high to be considered cost-effective based on WTP thresholds; figure 3 shows how this was the major reason given by the ERG for 4 negative appraisals.

In the submissions for bevacizumab (TA212) and sorafenib (TA189) the ERG found a high level of uncertainty in the definition and modelling of the PAS, and therefore concluded that the resulting estimates of the ICER may not fully reflect the actual costs to the NHS. The DoH also considered the PAS for TA212 to be too complex and that the administrative burden of the scheme was underestimated by the manufacturer, which could have a negative impact on the ICER.

Figure 3: Major issue with PAS identified by the ERG in negative appraisals



The PAS proposed by GSK for pazopanib includes a 12.5% discount, but also a second rebate proposal linked to the outcome of future clinical trials comparing the drug to its rival sunitinib.² This is the first such 'value-based' scheme to be accepted by NICE, and signals a shift in focus as the UK looks to introduce value-based pricing systems from 2014.

Based on these observation, to enhance the positive influence of a PAS on future decisions, the proposed scheme must:

- Show a clear cost-effectiveness benefit compared to the original model
- Be correctly incorporated into the economic model
- Demonstrate a minimal administrative burden on the NHS

Conclusion

PAS are an increasingly important element of NICE submissions to enhance the cost-effectiveness of innovative drugs. Negative appraisal decisions made on submissions including PAS highlight how manufacturers must ensure that these schemes are easy to implement and enhance cost-effectiveness to a level deemed acceptable by NICE, in order to positively influence appraisal decisions.

References

- ¹ PHP79, Innovative pricing agreements in UK NICE submissions, ISPOR 15th Annual International Meeting 2010
- ² Hammond E. GSK agrees NHS drug rebate deal. Financial Times, UK, Dec 27th 2010.