



An evaluation of manufacturers' budget impact estimates with resource use over time in NHSScotland



© **Scottish Medicines Consortium 2008**

First published September 2008

You can copy or reproduce the information in this document for use within NHSScotland and for educational purposes. You must not make a profit using information in this document. Commercial organisations must get our written permission before reproducing this document.

www.scottishmedicines.org

Scottish Medicines Consortium Evaluation Programme

An evaluation of manufacturers' budget impact estimates with
resource use over time in NHSScotland

Executive Summary and Full Report

Prepared by the SMC Evaluation Project Team

This work was undertaken by the National Medicines Utilisation Unit, Information Services Division, NHS National Services Scotland in collaboration with the Scottish Medicines Consortium

Acknowledgements

The National Medicines Utilisation Unit (NMUU) and the Scottish Medicines Consortium (SMC) Executive are grateful to the SMC Evaluation Project Team for undertaking this work. Particular thanks go to members of the Budget Impact Steering Group, and to Scott Hill, Public Health Pharmacist, for his work on identifying experiences of NHS Boards in using SMC budget impact advice for new medicines. Valuable comments and advice were provided by members of the SMC Evaluation Reference Group and Evaluation Management Group. Thanks are also extended to the PRISMS team, ISD, for providing national prescribing data for primary care.

The NMUU and SMC Executive would like to thank individuals and organisations that have provided advice and information for this work. This includes members of Area Drug and Therapeutics Committees across NHSScotland, the SMC, the Association of the British Pharmaceutical Industry (ABPI) and public partners, who collaborated to allow this work to occur.

Contents

Executive summary	4
Full report	7
Introduction.....	7
Method of study	7
Key findings	9
Conclusion.....	11

Executive summary

Introduction

The current Scottish Medicines Consortium (SMC) submission process requires pharmaceutical manufacturers to provide SMC with an estimate of the potential budget impact of their new product for NHSScotland. Manufacturers estimate the net resource implications over the five years following a product's introduction, taking into account the product's acquisition cost and any direct effect on the use of other resources. The budget impact estimates are not part of SMC decision-making on cost-effectiveness but are intended to give NHS boards an indication of the financial implications if the product is accepted for use. This report provides an analysis of the reliability of the budget impact estimates for a sample of product submissions and reviews the experiences of NHS boards in the use of the budget impact information provided by SMC in the Detailed Advice Document (DAD).

Method

Reliability of budget impact estimates

To review the reliability of the budget impact estimates for medicines within the evaluation sample (n=207), the manufacturers' estimates were categorised as high, medium, low budget impact or potentially cost saving. This was based on the estimated budget impact for the product in NHSScotland at year 5 after its introduction (where high impact > £2 million, medium impact £0.5–£2 million and low impact <£0.5 million). A sample of submissions (n=28) was selected to compare the manufacturers' estimates with the actual expenditure within NHSScotland. Submissions selected were full submissions for a product with a single indication where usage was anticipated to be mainly in primary care. A further subset of these submissions (n=20) was then critically appraised by two health economists to determine any variables within the manufacturer's estimate that may have affected its reliability.

Experience of NHS boards' use of the budget impact information

The experience of NHS boards in the use of the budget impact information was captured through a postgraduate project undertaken in collaboration with SMC during 2007. This involved a focus group with representation from senior pharmacists and senior finance professionals (n=12) from 50% of NHS boards. The focus group explored how the budget impact information is used and adapted locally and how it contributes to local planning and decision-making. Potential areas for improvement were also considered.

Key findings

Reliability of budget impact estimates

The 207 submissions were categorised as follows: 18 (9%) were estimated to have a high impact on NHSScotland resources; 31 (15%) a medium impact; 91 (44%) a low impact and 27 (13%) were estimated

to lead to savings. In 40 (19%) of the submissions the budget impact estimate was unclear precluding categorisation.

The comparison of the budget impact estimates with actual expenditure (n=28) highlighted variation, and initial analysis identified a series of factors that may have contributed to this variation. These included: restrictions in the SMC accepted advice that were not reflected in the manufacturer's budget impact estimate, causing overestimation of the budget impact; new safety information that may have affected product uptake; unclear market penetration estimates or errors in calculation of estimates which made the manufacturer's estimate less robust; and lack of hospital utilisation information making expenditure data incomplete. From these data it was, therefore, not possible to meaningfully compare the manufacturers' estimates with the actual expenditure in NHSScotland.

The detailed critical review of a subset of these submissions (n=20) highlighted several issues with the information provided by the manufacturer. The most common issues included: in 18/20 (90%) of submissions the clinical trial drop-out rate was either not stated or not supported by the data presented in the submission; and in 17/20 (85%) of submissions no rationale was given for the product uptake rate. Less common issues included: inability to reproduce the figures in the calculation; errors noted in the calculation; and inability to validate the incidence/prevalence data. A key theme was, however, that most of the submissions had multiple issues.

Experience of NHS boards' use of the budget impact information

The four key themes that emerged from the focus group discussions were: budget impact information is an important part of the preparatory work for the managed entry of new medicines; budget impact information is often locally adapted with the involvement of clinicians; clarity on derivation of the budget impact estimates would be beneficial to NHS boards; and standard presentation format for the information on budget impact within the SMC DAD would improve the usefulness of the information to NHS boards. It was proposed that more detailed guidance for the manufacturers may improve the quality of information SMC receives from the manufacturers.

Conclusion

This investigation has demonstrated that budget impact information is valued and used by NHS boards to facilitate the local financial planning process and the effective introduction of new medicines. It also revealed, however, significant weaknesses in the quality of the budget impact information currently provided to SMC by the pharmaceutical industry. The manifold limitations in the budget impact data meant that meaningful comparisons of the estimates with actual expenditure could not be made nor the reliability of manufacturers' estimates determined.

Affordability is not a consideration for SMC in reaching decisions on the clinical and cost effectiveness of

new medicines but it is imperative that NHS boards consider the budget impact implications of those that are 'accepted for use'. Effective financial planning is critical to the managed entry of new medicines and should facilitate patient access to cost-effective new medicines at the earliest opportunity after SMC acceptance. More clarity on the methodology used to derive the budget impact estimates and more consistent approach to the estimation of budget impact would, therefore, be beneficial to NHS boards.

Budget impact analysis is a challenging field; estimates are often based on multiple assumptions and made within the context of an unpredictable clinical and financial environment. Recognising these challenges, SMC should support and facilitate joint working between NHS boards and the pharmaceutical industry to improve the budget impact estimates provided by the manufacturers and how these are communicated to NHS boards.

Full report

Introduction

The Scottish Medicines Consortium (SMC) advises NHS boards and their Area Drug and Therapeutics Committees (ADTCs) across Scotland on the clinical and cost-effectiveness of all newly licensed medicines, all new formulations of existing medicines and new indications for established products (licensed since January 2002).

As part of the current Scottish Medicines Consortium (SMC) submission process, pharmaceutical manufacturers provide SMC with an estimate of the potential budget impact of the new medicine for NHSScotland. Manufacturers estimate the net resource implications over the five years following a product's introduction, taking into account the product's acquisition cost and any direct effect on the use of other resources, eg a requirement for additional clinical tests to determine whether a patient is eligible for the treatment. The budget impact estimates are not part of SMC decision-making on cost-effectiveness but are intended to give NHS boards an indication of the financial implications if the product is accepted for use. The SMC economic assessor reviews the budget impact information provided and a brief summary is included in the SMC Detailed Advice Document (DAD).

An Audit Scotland report (A Scottish Prescription: Managing the use of medicines in hospitals. July 2005) noted that NHS board medicine budgets were still largely based on historical levels of expenditure and recommended that SMC should continue to develop its work on estimating the anticipated budget impact of new medicines.

This report provides an analysis of the reliability of the budget impact estimates for a sample of SMC product submissions and reviews the experiences of NHS boards in the use of the budget impact information provided by SMC in the DAD.

Method of study

Reliability of budget impact estimates

An agreed categorisation of budget impact was used to categorise all 207 products assessed by SMC between January 2002 and December 2005 on the basis of the predicted net budget impact for NHSScotland at year 5 provided in the manufacturer's submission. If the year 5 figure was not available, the maximum budget impact presented by the manufacturer for the product was used. Medicines were categorised as high impact > £2 million, medium impact £0.5-£2 million, low impact <£0.5 million or potentially cost saving.

A sample of submissions (n=28) was selected to compare the manufacturers' estimates with the actual expenditure within NHSScotland. Submissions selected were full submissions for a product with a single

indication that were 'accepted for use' or 'accepted for restricted use' and where usage was anticipated to be mainly in primary care. Submissions were excluded for the following reasons:

- 'Not recommended' submissions - the actual budget impact was expected to be considerably lower than estimated.
- 'Abbreviated' submission - a full submission is required for any product that could potentially result in a significant change in NHS resources.
- Submissions for products used in hospital only - utilisation data for products used in hospital are not readily available.
- Multiple licensed indications - available utilisation data do not allow the indication for the medicine to be determined thus expenditure cannot be attributed to a specific indication.
- Submissions where the manufacturer anticipated the product to be cost saving.
- Submissions where the budget impact was unclear.

A 'medicine profile' was developed for each of the 28 medicines identified. The profile included: the SMC advice; a description of the epidemiology of the indication for the medicine; NHSScotland medicines use over time, annotated with key milestones; a comparison of the actual versus estimated manufacturer's budget impact; and potential factors that may be influencing medicines use within NHSScotland.

A further subset of these submissions (n=20) was critically appraised by two health economists to determine any variables within the manufacturer's estimate that may have affected its reliability. They considered the assumptions stated in relation to the epidemiology of the condition to be treated, the product costs, the anticipated uptake of the product and other factors potentially influencing uptake. In addition, the reviewers attempted to reproduce the manufacturers' estimates from the information contained in the submissions. For each submission any discrepancies or omissions identified were noted and categorised.

Experience of NHS boards' use of the budget impact information

This was explored through a postgraduate project undertaken in collaboration with SMC during 2007. A focus group was held to gather views and experiences relating to the use of budget impact advice on new medicines provided by SMC (in the DAD as well as the more recently introduced annual forward look horizon scanning report). All NHS boards were invited to participate. The participants involved were purposively sampled based on their roles and responsibilities and were chosen to reflect the main groups involved in using the budget impact information. A total of 12 participants, representing 50% of NHS boards took part in the focus group. The key themes explored were:

- How SMC budget impact information is used locally.
- Processes used to define and adapt the information locally.
- Impact of the information on local planning and decision-making.
- How the budget impact information could be improved and how its use could be

supported to meet NHS board requirements.

Key findings

Reliability of budget impact estimates

The 207 submissions were categorised as shown in Table 1. A number of difficulties were encountered during the categorisation process including the absence of a budget impact figure for year 5. In 40 submissions (19%) the budget impact estimate provided was unclear, often because it could not be determined whether the figures presented related to the net or gross budget impact.

Table 1: Budget impact categorisation of products assessed by SMC Jan 2002 – Dec 2005 (n=207)

Category	Estimated net budget impact for NHSScotland at year 5	Number of medicines (%)
High	> £2 million	18 (9%)
Medium	£500,000 - £2 million	31 (15%)
Low	< £500,000	91 (44%)
Savings	The medicine is expected to lead to savings for the NHS	27 (13%)
Unclear	The budget impact is unclear	40 (19%)
TOTAL		207

The comparison of the budget impact estimates with actual expenditure (n=28) is shown in Table 2 (page 13). Product names have been removed so that the manufacturer cannot be identified. This analysis highlighted variation, and initial analysis identified a series of factors that may have contributed to this variation. These included: restrictions in the SMC accepted advice that were not reflected in the manufacturer's budget impact estimate, causing overestimation of the budget impact; new safety information that may have affected product uptake; unclear market penetration estimates or errors in calculation of estimates which made the manufacturer's estimate less robust; and lack of hospital utilisation information making expenditure data incomplete. From these data, it was, therefore, not possible to meaningfully compare the manufacturers' estimates with the actual expenditure in NHSScotland.

The detailed critical review of a subset of these submissions (n=20) highlighted several issues with the information provided by the manufacturer. These are categorised and quantified in Table 3 (page 15). Product names have been removed so that the manufacturer cannot be identified. Products are listed in chronological order of the SMC review date, ie product 1 is the earliest of the submissions reviewed. No detailed analysis of trends over time has been undertaken but there is no obvious pattern to indicate that the quality of budget impact information in the submissions is either improving or declining. Examples of the issues with information provided by the manufacturer included:

- The manufacturers' submissions were often incomplete or in some cases incorrect with regard to key information required to estimate budget impact.

- Estimates of patient numbers, annual treatment cost and total expenditure were not always reported.
- Information about treatment duration was often limited, eg it was often not specified whether treatment was acute or chronic.
- The dose of the product used to estimate cost was often not clear.
- The year that the stated budget impact estimate referred to was sometimes not clear.
- The source of prevalence and incidence data for the condition to be treated was often unclear and inconsistencies were noted.
- Estimated uptake of the product was often unclear or not stated.
- Market share estimations were frequently optimistic. This may be particularly true for medicines 'accepted for restricted use', since budget impact estimates provided by the manufacturer may not assume any restrictions on the product's use.
- There were arithmetical errors in some calculations.

The most common issues included: in 18/20 (90%) of submissions the clinical trial drop out rate was either not stated or not supported by the data presented in the submission; and in 17/20 (85%) of submissions no rationale was given for the product uptake rate. Less common issues included: inability to reproduce the figures in the calculation; errors noted in the calculation; and inability to validate the incidence/prevalence data. A key theme was, however, that most of the submissions had multiple issues.

Experience of NHS boards' use of the budget impact information

The key themes that emerged from the focus group were:

- A budget impact analysis is an important part of the preparatory work for the managed entry of new medicines. The information provided allows NHS boards to use the health economic and budget impact data to greater effect in their financial planning. It is expected that this information will become increasingly important in future due to constraints on resources.
- The SMC advice and the information provided in the DAD provide the foundation of the local formulary process in the majority of NHS board areas. The budget impact section is often locally adapted with the involvement of clinicians enabling the production of revised estimates to the budget impact.
- There needs to be clarity around how the budget impact estimates have been derived, for example, whether it considers the impact on the drug budget only or the overall healthcare budget. If the drug budget perspective is used there is a need for additional information on other implications, eg reduction of drug use due to replacement; increase of other drug use due to side effects; and impact on day hospital spaces etc.
- The development of guidance on budget impact analysis particular to Scotland would improve the information received from the companies and increase the usefulness of this part of the submission. A revision of how the information is requested may be appropriate.
- The section within the SMC DAD should be standardised to ensure that specific core information is

included consistently across advice issued. This should include estimated patient numbers, estimates of the rate of uptake and information on the service implications.

- Consideration should be given to linking or comparing the budget impact estimates generated through the horizon scanning programme with those in the company submission.

Discussion

This investigation validates experience within SMC to date that estimating the financial impact of new medicines is challenging and subject to considerable uncertainty. Although affordability is not a consideration for SMC in reaching a decision on a product's clinical and cost effectiveness, for many products accepted by SMC, particularly innovative new medicines, NHS boards need to consider the number of eligible patients and the associated cost of their introduction in the local population. The budget impact of a new medicine cannot be calculated using a scientific formula; many factors can have a bearing on the extent to which a new medicine will be used in practice. There are significant challenges involved in predicting, for example, the rate of uptake of innovative treatments. Anecdotally, it is perceived that the uptake rate of new medicines may vary according to the type of product and the sector it is used in, however historical data on trends in medicines use over time are lacking. In this study, manufacturers' predictions on the rate of uptake for a new product were often unsupported by current medicines utilisation data and appeared to be made in isolation. The availability of improved data on the clinical use of medicines across NHSScotland would support processes for both estimating and analysing a product's estimated budget impact. There is a need to explore general trends in medicines use, in both primary and hospital care, across the Scottish population and consider whether the findings could support improved techniques for estimating budget impact. Recognising these challenges within the NHS and pharmaceutical industry is important and necessary. However, a more robust approach to the provision of budget impact information offers the potential for more rapid access to new medicines for patients through improved financial planning. These challenges should, therefore, not be a barrier to SMC working with key stakeholders in NHS boards and the pharmaceutical industry to improve current processes and methodology for estimating the budget impact of new medicines.

Conclusion

This investigation has demonstrated that budget impact information is valued and used by NHS boards to facilitate the local financial planning process and the effective introduction of new medicines. It also revealed, however, significant weaknesses in the quality of the budget impact information currently provided to SMC by the pharmaceutical industry. The manifold limitations in the budget impact data meant that meaningful comparisons of the estimates with actual expenditure could not be made nor the reliability of manufacturers' estimates determined.

Affordability is not a consideration for SMC in reaching decisions on the clinical and cost effectiveness of new medicines but it is imperative that NHS boards consider the budget impact implications of those that

are 'accepted for use'. Effective financial planning is critical to the managed entry of new medicines and will facilitate patient access to cost-effective new medicines at the earliest opportunity after SMC acceptance. More clarity on the methodology used to derive the budget impact estimates and more consistent approach to the estimation of budget impact would, therefore, be beneficial to NHS boards.

Budget impact analysis is a challenging field; estimates are often based on multiple assumptions and made within the context of an unpredictable clinical and financial environment. Recognising these challenges, SMC should support and facilitate joint working between NHS boards and the pharmaceutical industry to improve the budget impact estimates provided by the manufacturers and improve their communication to NHS boards.

Table 2: Variance between actual expenditure and manufacturer's budget impact prediction (n=28)

Product code (R = accepted for restricted use)	Actual expenditure (£) Jan – Dec 2006*	Manufacturer's estimate (£) 2006	Variance between actual expenditure and manufacturer's estimate**(£)	Comments on manufacturer's budget impact estimates and possible influencing factors
1 [R]	£1,124,043	£1,376,760	-£252,717	Market share may be overestimated given the restrictions imposed by SMC.
2	£2,012,755 in 2003	£898,049 in 2003	£1,114,706 in 2003	Prevalence data and, therefore, number of patients eligible for treatment may be underestimated.
3	£29,278	£40,912	-£11,634	Safety concerns about this therapeutic class expected to have greatly limited product uptake.
4	£41,747	£174,577	-£132,830	Safety concerns about this therapeutic class expected to have greatly limited product uptake.
5	£597,024	£1,040,000	-£442,976	Market share may be overestimated.
6 [R]	£37,551	£7,560 - £9,450	£28,101 - £29,991	Very limited information provided by the manufacturer.
7	£1,486,952	£2,282,780	-£795,828	Manufacturer assumed displacement of another drug in therapeutic class; likely to be unrealistic.
8	£153,037	£4,072,000	-£3,918,963	Prevalence data may be overestimated. Manufacturer's estimate of patients eligible for treatment is optimistic.
9	£4,378,937	£16,298,549	-£11,919,612	Product uptake likely to have been lower than manufacturer estimated due to patent expiry of competitor product. Market share may be overestimated given range of other treatments available.
10	£440,837	£840,005	-£399,168	Market share may be overestimated.
11 [R]	£100,666	£622,346	-£521,680	Product uptake may be overestimated given the restrictions imposed by SMC.
12 [R]	£4,113,450	£22,588,608	-£18,475,158	Market share may be overestimated.
13	£206,512	£182,135	£24,377	Prevalence data may be underestimated. Estimated uptake may be optimistic. No treatment duration or dropout rate included.
14 [R]	£144,483	£940,680	-£796,197	Prevalence data may be overestimated. Market share may be overestimated given range of other treatments available.

Product code (R = accepted for restricted use)	Actual expenditure (£) Jan – Dec 2006*	Manufacturer's estimate (£) 2006	Variance between actual expenditure and manufacturer's estimate**(£)	Comments on manufacturer's budget impact estimates and possible influencing factors
15	£1,478,048	£157,206- £628,822	£1,320,842 - £849,226	Market share and treatment duration may be underestimated. Errors in calculations noted.
16	£35,219	£483,583	-£448,364	Arithmetical errors noted. Market share estimated may be optimistic.
17	£221,070	£901,891	-£680,821	No treatment duration or drop-out rate included. Product uptake estimate appears optimistic.
18 [R]	£3,460,724	£278,880	£3,181,844	Prevalence data may be underestimated. Unclear how budget impact figures calculated.
19 [R]	£66,063	£349,200	-£283,137	Prevalence of condition overestimated.
20 [R]	£118,259	£145,000	-£26,741	Errors noted in market share estimate and budget impact calculations.
21	£67,805	£128,042	-£60,237	
22 [R]	£277,665	£380,588	-£102,923	Market share may be overestimated given the restrictions imposed by SMC.
23 [R]	£1,279,972	£918,855	£361,117	Budget impact predictions complex as other new products in this therapy area expected to become available.
24 [R]	£4,746,116	£3,572,682	£1,173,434	Basis for the manufacturer's market share projections not stated.
25 [R]	£3,520,456	£1,439,538	£2,080,918	Uptake may be underestimated as manufacturer's estimate related to first line use only.
26 [R]	£269,689	£394,800	-£125,111	Product uptake may be overestimated given the restrictions imposed by SMC.
27 [R]	£162,756	£2,021,788	-£1,859,032	Product uptake may be overestimated given the restrictions imposed by SMC.
28 [R]	£12,792	£139,450	-£126,658	Product uptake may be overestimated given the restrictions imposed by SMC.

* Data source: PRISMS, ISD, Scotland from dispensed items. Excludes private prescriptions.

** Calculated as:(actual expenditure (GIC) – manufacturer's estimate)

Table 3: Discrepancies and omissions in manufacturers' budget impact estimates (n=20)

Drug	Limited information	Issues with calculations		Clinical trial drop-out rate		No rationale for uptake rate	Unable to validate incidence/prevalence	Access issues not factored into estimated uptake
		Error in figures	Figures not reproduced	Not stated	No evidence			
1		♦				♦		♦
2			♦			♦	♦	
3				♦		♦		
4				♦		♦		
5	♦				♦	♦		
6	♦			♦		♦		
7				♦		♦	♦	
8	♦			♦		♦	♦	
9				♦		♦		
10				♦		♦		
11		♦		♦		♦	♦	
12				♦		♦		
13				♦		♦		
14				♦		♦	♦	
15		♦		♦		♦		
16		♦				♦	♦	
17			♦			♦		
18			♦			♦	♦	
19				♦		♦	♦	
20		♦			♦	♦	♦	
No. with issue	3	5	3	15	3	17	9	1
% with issue	15%	25%	15%	75%	15%	85%	45%	5%

Scottish Medicines Consortium

Delta House, 50 West Nile Street, Glasgow. G1 2NP

Telephone 0141 225 6999/6989

www.scottishmedicines.org.uk