Patented Medicine Prices Review Board
Conseil d'examen du prix des médicaments brevetés

Budget Impact Analysis Guidelines: Needs Assessment

npduis

Analytical Study Series

National Prescription Drug Utilization Information System

Canada

Ce document est également disponible en français sous le titre
"Lignes directrices portant sur l'incidence du prix d'un médicament sur les budgets des régimes d'assurance-médicaments : Vérification du besoin"

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The PMPRB recognizes the extensive work effort that was put into this report by the PMPRB research team of the Policy and Economic Analysis Branch. The PMPRB also recognizes the staff from the Compliance and Enforcement Branch of the PMPRB for its input.

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Highlights

- The Patented Medicine Prices Review Board (PMPRB) has undertaken the project: *Budget Impact Analysis Guidelines* as a National Prescription Drug Utilization Information System (NPDUIS) project.
- In December 2004, the F/P/T Pharmaceutical Issues Committee (PIC) endorsed Phase One of the project, which is to assess the need for developing national budget impact analysis (BIA) guidelines. Phase Two of this project is to develop guidelines.
- This report is based on: a survey of all the NPDUIS Steering Committee members; an analysis of 35 BIAs made available to the PMPRB by jurisdictions; and, a review of current literature.
- A BIA looks at the financial impact on a drug plan's budget of the possible listing of a drug onto its formulary. Public drug plans routinely use BIAs for decision-making purposes. The Common Drug Review (CDR) uses BIAs when Priority Review based on cost savings is requested.
- Drug plan managers often find the BIAs submitted to them by manufacturers to be unsatisfactory. The survey and the analysis of 35 BIAs indicate the main issues to be: lack of transparency; inaccurate or mis-applied assumptions; generalized analysis – non-specific or inaccurate to jurisdiction and/or plan; inappropriate choice of comparators; and overall quality.
- The literature review indicates that there are limited guidelines on the conduct of BIAs in OECD countries although there is a plethora of economic evaluation guidelines. In Canada, some provinces offer templates for manufacturers to follow.
- The use of BIAs and economic evaluations are complementary. An economic evaluation addresses the issue of "cost-effectiveness", whereas a BIA addresses the issue of "affordability". Both are necessary to make informed decisions about the possible listing of a drug on a formulary.
- The main advantage of having BIA guidelines is to establish a set of principles or best practices in designing and implementing budget impact analysis, hence increasing the reliability and usefulness of the BIA report.
- The findings in the report confirm the need for BIA guidelines.

1 Introduction

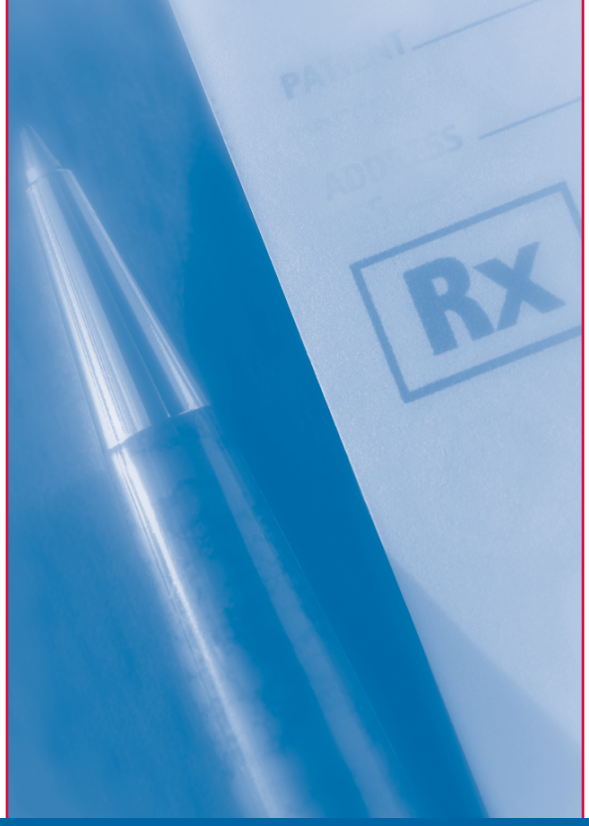
In September 2001, Federal/Provincial/Territorial Ministers of Health announced a multi-faceted approach to better pharmaceuticals management. Among other things, they agreed to establish a National Prescription Drug Utilization Information System (NPDUIS) to *"provide critical analyses of price, utilization and cost trends so that Canada´s health system has more comprehensive, accurate information on how prescription drugs are being used, and sources of cost increases."*

The NPDUIS is established as a partnership between the Canadian Institute for Health Information (CIHI) and the PMPRB. For the PMPRB, the NPDUIS represents a natural evolution of the work that was previously conducted under a Memorandum of Understanding between the Minister of Health and the PMPRB.

The impact on budget planning from new or about to be launched drug products was identified as one of the possible analytical studies in the business case developed jointly by CIHI and the PMPRB in 2001. Later, the NPDUIS Steering Committee and the Pharmaceuticals Issues Committee (PIC) endorsed the project to develop guidelines for conducting budget impact analysis provided that the need is established through this report.

This report is based on: a survey of all the NPDUIS Steering Committee members; an analysis of 35 BIAs made available to the PMPRB by jurisdictions; and, a review of current literature.

Of the 12 participants in the survey, 10 were provincial drug plan managers and the remaining two represented CIHI and Health Canada. The survey was undertaken to assess the overall effectiveness and utility of current budget impact analyses. The survey report and questionnaires are presented in Appendix 1.a and 1.b, respectively.



Background

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Drug expenditure is one of the fastest growing components of total spending on health care despite the fact that drug prices have remained relatively stable over the last decade.[1] The recently published report *National Health Expenditure Trends 1975–2004* by CIHI, shows that in 2004, 47.3% of the estimated $18.0 billion spent on prescribed drugs in Canada was financed by the public sector; this is an increase from 42.5% in 1998.

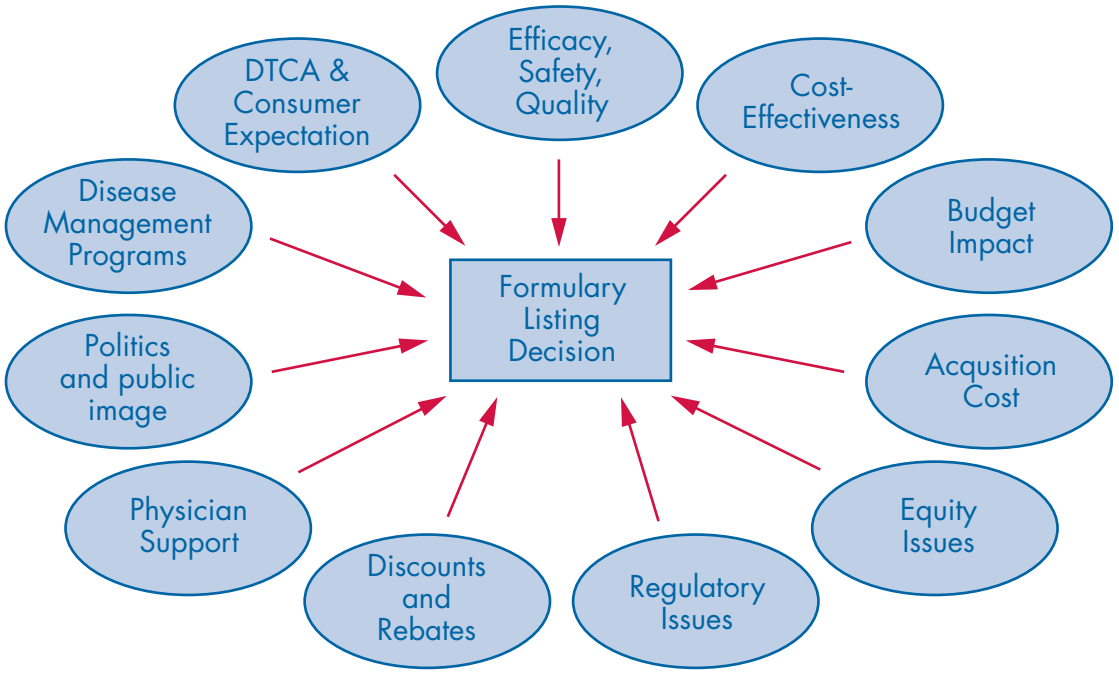
Economic consequences of listing a drug onto the formularies are becoming more and more important with rising drug costs. This fact is evident in the number of initiatives undertaken in recent years towards pharmaceuticals management. Priority setting for the most efficient allocation of scarce resources is a challenge that drug program decision-makers face on a day to day basis. The challenge is magnified by the pressures that decision-makers face from both economic and non-economic sources. Figure 1 lists some of the factors that influence formulary listing decisions.

Australia was the first country to require pharmaceutical companies to produce economic data in support of their applications to be listed onto the formulary.[2] Drug programs in many OECD countries including Canada require pharmaceutical manufacturers to provide both economic evaluations and budget impact studies for new drugs. Most countries that use economic evaluations have developed, or are in the process of developing guidelines. Although budget impact analyses are used extensively there are no standard practices in preparing these reports.

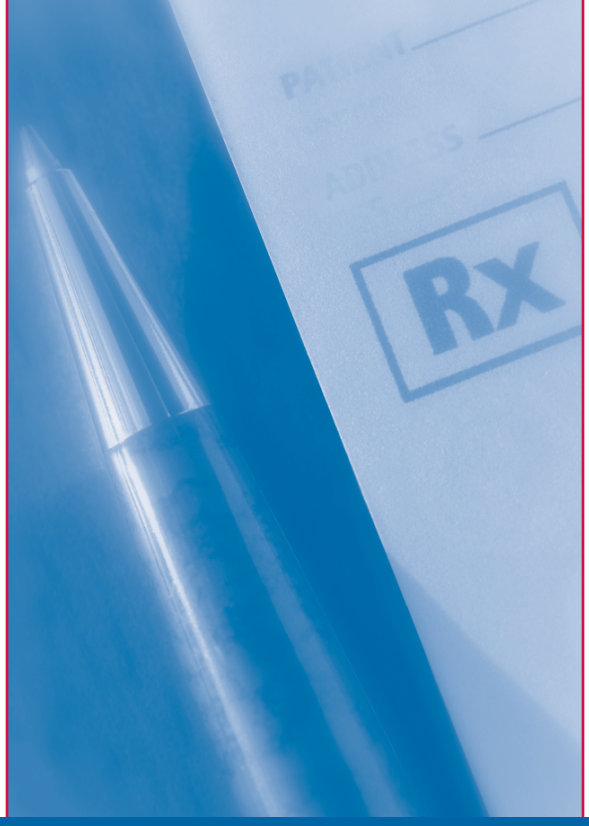
1. Canadian Institute for Health Information (CIHI).
2. Survey of Pharmacoeconomic Assessment Activity in Eleven Countries: Michael Dickson, Jeremy Hurst and Stephane Jacobzone; OECD Health Working Papers No 4; May 2003.

Figure 1

Factors influencing formulary listing decisions



Source: Adapted from *A Format for Formulary Submissions, Version 2.0*, Academy of Managed Care Pharmacy (AMCP), USA, 2002



What is a BIA?

3

A BIA looks at the financial impact on a drug plan budget of the possible listing of a drug onto its formulary. The data needs for such analysis are extensive, and includes (but not limited to) incidence and prevalence data, the extent to which existing treatments will be replaced, the length of treatment, the price of the drug, expected market share and the total cost. Budget impact analyses and economic evaluations may share many of the same data requirements but are quite distinct from one another in their scope and reporting of results (Trueman, Drummond and Hutton, 2001).

3.1 Why Do We Need BIAs?

A BIA addresses the issue of affordability. Properly constructed BIAs can facilitate making reimbursement decisions, help define the specific role of a product and assist in creating benchmarks against which health systems can measure future product performance (Fry, Avey and Sullivan, 2003).

In response to rising drug costs and their pressure on health care budgets provincial governments in Canada and other OECD countries have introduced many cost containment policies throughout the last decade. For example, in Canada, Ontario introduced price freezes for all drugs listed on its formulary in 1994; British Columbia introduced reference based pricing for specific therapeutic categories in 1995. Health care authorities are more and more interested in getting "value for money" given the financial constraints in which they have to operate. Thus in most OECD countries drug manufacturers in addition to providing scientific data proving efficacy, safety and quality, have to produce economic data as well. They have to demonstrate that the new product would not only be cost-effective but would be affordable as well. Cost-effectiveness and affordability are widely labeled in literature as the "fourth and fifth hurdle" to market.

3.2 Use of BIAs

All F/P/T drug plans require manufacturers to submit BIAs as part of their submission package for listing purposes. The Common Drug Review (CDR) requires manufacturers to submit BIAs in accordance with jurisdictional requirements. Financial analysis is particularly important in the CDR process when manufacturers ask for Priority Review based on cost savings.[3] In some jurisdictions financial analyses are considered by Expert Advisory Committees (EAC) in their deliberations.

In the survey, most drug plan managers responded that they use BIAs to determine affordability after the EAC and/or the CDR has made its recommendation based on clinical efficacy and cost-effectiveness. BIAs are also used as a baseline against which the actual utilization of the drug is assessed. In some jurisdictions, BIAs are used as the basis for developing written agreements with manufacturers.

3.3 Difference between Economic Evaluations and BIAs

3.3.1 "Cost-effectiveness" vs. "Affordability"

Both affordability and cost-effectiveness are the objectives for drug plan decision-makers, since they have to stay within their annual budgets and use the money efficiently at the same time. The demonstration of cost-effectiveness or "value for money" of a new drug is required for being able to prioritize interventions to be funded and maximize efficiency.

An economic evaluation (using techniques like cost-effectiveness analysis or cost-utility analysis) is an incremental analysis concerned with the relationship between cost and consequence, whereas, BIAs reflect impact on total costs. An economic evaluation may assist drug plan managers to efficiently allocate available funds, but it does not provide information to help them stay within the limited budgets in the years following the drug's introduction. It is possible that a highly cost-effective new drug is unaffordable. This issue is exemplified by the concerns over the provision of "statins" in the UK (Trueman, Drummond and Hutton, 2001). In spite of their favourable clinical effectiveness and cost-effectiveness, the prescribing of statins was found well below the advised level. It was suggested that this may have been associated with the cost of providing such treatment. It was estimated that 8.2% of the population aged 35 to 69 years old would benefit from statin treatment, and the adherence to the advised level would cost a single health authority in the UK approximately 20% of its annual drug budget.

3.3.2 Perspective: Societal vs. Drug Plan Budget

A full economic evaluation adopts a societal perspective in which all the costs and savings of a health intervention are captured.[4] In reality, the decision-maker of a particular drug plan rarely takes into account the costs and savings falling out of his or her own program. If "silo budgeting" is the norm, i.e., where each health care component is managed separately and has its own budget, it is less likely that the plan manager will take into account cost savings elsewhere in making reimbursement decisions.

3. Submission Guidelines for Manufacturers, Common Drug Review; July 2004
4. Many published economic evaluation guidelines recommend that analysis be conducted from both societal and from the health care funding authority perspective. *International Society for Pharmacoeconomics and Outcomes Research*; http://www.ispor.org/PEguidelines/index.asp

3.3.3 Time Horizon

The time horizon of the health-care decision-maker is often much shorter than what a reasonable economic evaluation would call for. To determine the value of a new treatment, the economic evaluation has to take into account its long-run costs and savings, i.e., sustainability. However, most drug plans have to operate within an annual budget. For a plan manager it is generally difficult to invest a significant proportion of his or her current budget on a drug when the prospective benefits from the investment cannot be achieved in the short run, unless the drug replaces a significantly more expensive course of treatment. Therefore, while the introduction of a highly cost-effective new drug may be socially optimal in the long-run, the plan manager still needs to know the short-term impact on the plan budget.

3.4 Complementarity of Economic Evaluations and BIAs

In comparing BIAs relative to economic evaluations in decision-making, the BIA is generally seen as complementary to an economic evaluation. While an economic evaluation answers questions as to whether a drug is cost-effective taking into consideration a drug's overall contributions to the health care system, a BIA answers the question of whether a drug is affordable. Both are necessary in evidence-based decision-making.

In the Canadian context, the Canadian Expert Drug Advisory Committee (CEDAC) considers pharmacoeconomic analysis along with scientific evidence to recommend whether or not to list a drug for reimbursement purposes. F/P/T drug plans reserve the right to make listing decisions based on, among other things, affordability. In order to get a reliable assessment of affordability the decision-maker needs a reliable BIA.

The survey indicated that in jurisdictions where budgetary constraints are a high priority, the BIA weighs more than the economic evaluation. In other words, even drugs with demonstrated cost-effectiveness may be deemed unaffordable and therefore not be reimbursed. Others manage to address the expense associated with cost-effective drugs through other mechanisms, such as use "limited use" criteria.

4 Existing Guidance

Existing guidance in terms of guidelines or templates is limited. In some countries economic evaluation guidelines or submission guidelines allude to the need for financial impact analysis. In others, such as below, there is further guidance around the issue.

4.1 BIA Guidelines

The first detailed guidance on conducting BIAs for the pharmaceutical industry came from the Australian Pharmaceutical Benefits Advisory Committee (PBAC). The *2002 Guidelines for the Pharmaceutical Industry on Preparation of Submissions to the Pharmaceutical Benefits Advisory Committee (PBAC)* gives detailed guidance on how a BIA should be conducted and reported. The guideline specifies the time horizon, the approach to be taken to estimate population affected, and considerations that should be included in calculating the net financial impact on the budget of the Australian Pharmaceutical Benefits Scheme (PBS).

In England and Wales, guidance is provided by the National Institute of Clinical Excellence (NICE) for technology appraisal. The NICE recommends that manufacturers should provide a budget impact analysis, which should be conducted from the perspective of the National Health Service (NHS) and the Personal Social Service (PSS); and that it should be based on patient groups being treated; for new technologies the analysis should include estimates of the changing budget impact over a three to five year time horizon due to varying diffusion rates and an estimate of impact once diffusion has reached a "steady state". The NICE guidance also specifies that where new technology is expected to replace an existing treatment, the net budget savings should be calculated.

The Polish Society for Medical Decision Making, the professional association for physicians, initiated consultations on a set of guidelines focused on financial analysis. The Polish guidelines were adapted from the Australian guidelines and partly from the guidelines issued by the NICE (Orlewska and Mierzejewski, 2004). In addition, the Polish guidelines bring attention to some general but desirable attributes, like target population, probability of redeploying resources, the need for reporting results both in natural and monetary units and the need for interactive models.

The Academy of Managed Care Pharmacy (AMCP) in the United States in its publication *Format for Formulary Submissions, 2002* requires manufacturers to ensure transparency in their analytical methods. The AMCP lists twelve elements that should be present in an acceptable economic and budget impact analysis. In its guidelines, the AMCP encourages manufacturers to consult with health-system staff in the early stages of model development to ensure the incorporation of appropriate comparator products and end points.

In 2005 the International Society for Pharmacoeconomics & Outcomes Research (ISPOR) formed a task force to develop "a coherent set of methodological guidelines" for those developing or reviewing budget impact analyses.[5] The task force was formed in recognition of the fact that a comprehensive economic assessment of a new health care intervention at the time of launch requires both cost-effectiveness analysis and budget impact analysis.

4.2 BIA Templates for Manufacturers in Canada

Templates are used as structured information requirements. These are suggested by drug plans to guide manufacturers in preparing the budget impact reports for reimbursement purposes. Currently there are no standard templates for data or reporting formats in Canada.

All new chemical entities are reviewed by the CDR.[6] The submission requirements as per CDR's *Submission Guidelines for Manufacturers, July 2004*, include reporting on the budget impact of the drug on participating jurisdictions, prepared in accordance with jurisdictional needs. Saskatchewan does not ask for BIAs but requires manufacturers to provide "expected market share information to allow for an accurate projection of the impact of a new product"; British Columbia and the Atlantic provinces require manufacturers to submit BIAs but do not provide any templates. Alberta, Manitoba and Ontario provide manufacturers comprehensive templates. However, there are differences in the type of information and the level of detail that is required. A comparison of the templates designed by the Ontario Drug Benefit program, Alberta Blue Cross and Manitoba Health[7] is provided in Appendix 2.

5. http://www.ispor.org/workpaper.budget_impact.asp.
6. Quebec is not participating in the CDR at this time.
7. It should be noted that the templates of Alberta and Manitoba are the same.

5 Need for BIA Guidelines

"…Indeed, if decision-makers and reimbursement authorities wish to use budget impact analysis as a criterion for the introduction of new medicines, then guidance is a necessity as this is arguably the most uncertain aspect of their data needs… Without it, such decisions run the risk of degenerating into ill informed negotiations based on evidence of a variable quality."

(Trueman, Drummond and Hutton, 2001)

The emphasis on the requirement for submitting economic information to decision-makers was followed by the production of pharmacoeconomic guidelines in many OECD countries. The ISPOR Web site lists 28 guidelines from 23 countries (21 pharmacoeconomic guidelines, 6 submission guidelines for formulary listing, 1 for journal publication). The data needs for BIAs and the cost side of economic evaluations are similar; both are subject to a high degree of uncertainty around most of the key inputs and share similar challenges. However, the scope and reporting requirements of BIAs are different enough from economic evaluations to warrant specific guidelines tailored for budget impact analysis and not be treated as a variant of pharmacoeconomic guidelines.

5.1 Gaps in Current BIA Reports

An analysis of 35 BIAs submitted to provinces by drug manufacturers was made available to the PMPRB. The PMPRB analyzed the BIAs against attributes that are considered in literature as desirable in a BIA report to ensure high quality. A detailed analysis is provided in Appendix 3. The main issues raised were: lack of transparency; lack of adequate justifications for assumptions used; and, inconsistencies in price and utilization data within and across BIAs. These issues were also raised by the survey respondents. Accordingly, the common gaps identified are:

Lack of transparency
All respondents pointed to the lack of transparency in manufacturers' submissions. Specific areas lacking clarity include how assumptions are derived, methodology, incidence and prevalence information and data sources.

Inaccurate or mis-applied assumptions
Respondents indicated that input assumptions in modeling the budgetary impact of new drugs are frequently unrealistic and underestimated. Assumptions regarding key variables, for example disease incidence and prevalence, patient switching, length of treatment are frequently inaccurate or misleading.

Generalized analysis - not specific or accurate to jurisdiction and/or plan
All respondents described BIAs that were not specific to the jurisdiction or the drug plan to be less useful. Manufacturers extrapolate BIAs to various jurisdictions, and in reality, this is not a straight line extrapolation. Prevalence, population, drug plan structure and drug plan population served are all variable across regions and drug plans. As well, budget impact varies with geographic locations: overall treatment costs and access to some specialized treatments or practitioners are different in remote communities.

Inappropriate choice of comparators
Some manufacturers' submissions compare the submission drug to non-standard treatments and sometimes to placebo. An accurate analysis would assess the drug against current accepted and reimbursed treatments and dosing regimens.

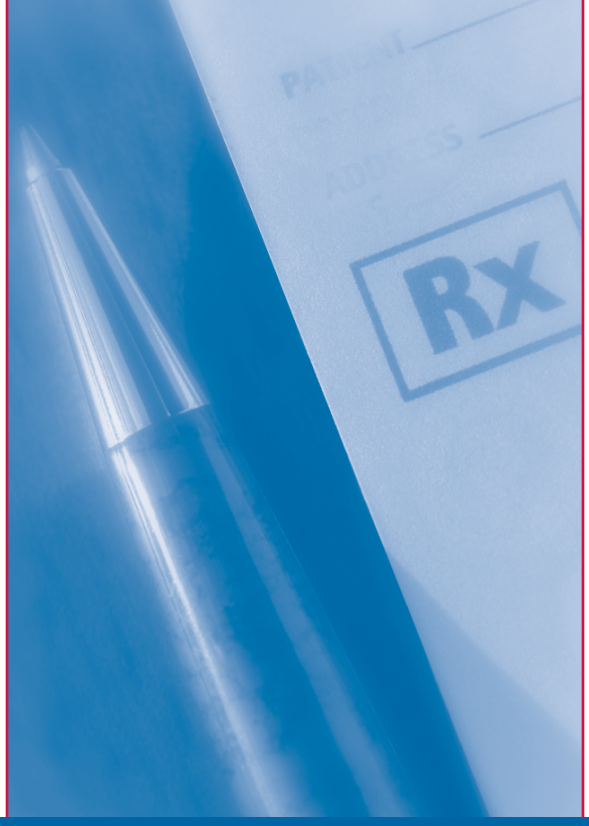
Quality
Concerns were raised about the accuracy of claims made in BIA reports regarding the cost implications of listing a drug. Evaluation following formulary listings has shown that there is often a significant variance between actual impact on the drug budget and that predicted by the manufacturers in their BIA forecast. As a result, there are concerns about the overall quality of the BIA reports provided to drug plans by manufacturers.

5.2 NPUDIS Steering Committee on Need for BIA Guidelines

The survey showed that all NPDUIS Steering Committee members support the need for national guidelines. They indicated that national guidelines will be more useful if they can be adapted by each jurisdiction to reflect such things as disease prevalence, demographics, geography and drug plan design. They also felt that national BIA guidelines, if used by manufacturers, would result in better quality BIAs and save them time in reviewing such reports. To ensure the use of BIA guidelines by manufacturers the need for manufacturer buy-in and commitment was stressed (see Appendix 1.a for details).

Steering Committee members identified the following criteria to be kept in mind when developing guidelines:

- make them user friendly, interactive and simple;
- make them a tool that industry will use;
- avoid asking for data that cannot be validated, will not be reliable or will not be used (e.g. esoteric or nice-to-have data);
- ensure that the guidelines are rigorous;
- make them understandable to decision-makers;
- make them available in e-format, CD-ROM, perhaps on central Web site in interactive format; and
- ensure specificity for jurisdictions



Conclusion

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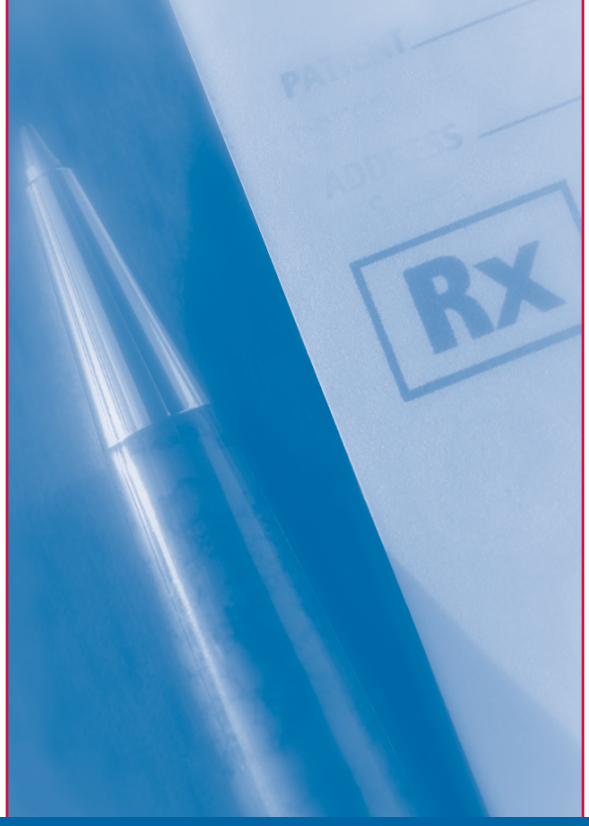
The findings in this report confirm that there is a need to develop BIA guidelines. Furthermore, through the survey, the NPDUIS Steering Committee members have identified the requirements of a BIA that would prove to be more useful.

The most obvious reason for establishing guidelines is that if followed, they would facilitate the production of more reliable and useful BIA reports. There is no single methodology that can be applied to every new health technology or drug but the guidelines can establish a set of principles or best practices that will give decision-makers some degree of comfort in using the BIAs submitted to them by manufacturers. On the other hand, manufacturers will benefit from having a clear and consistent set of directions concerning information requirements and their reporting formats.

7 Next Steps

An external expert will be engaged to design BIA guidelines. An advisory group will be formed to oversee the project. This group will consist of members from the NPDUIS Steering Committee and CCOHTA. The guidelines will be peer reviewed.



Further Work

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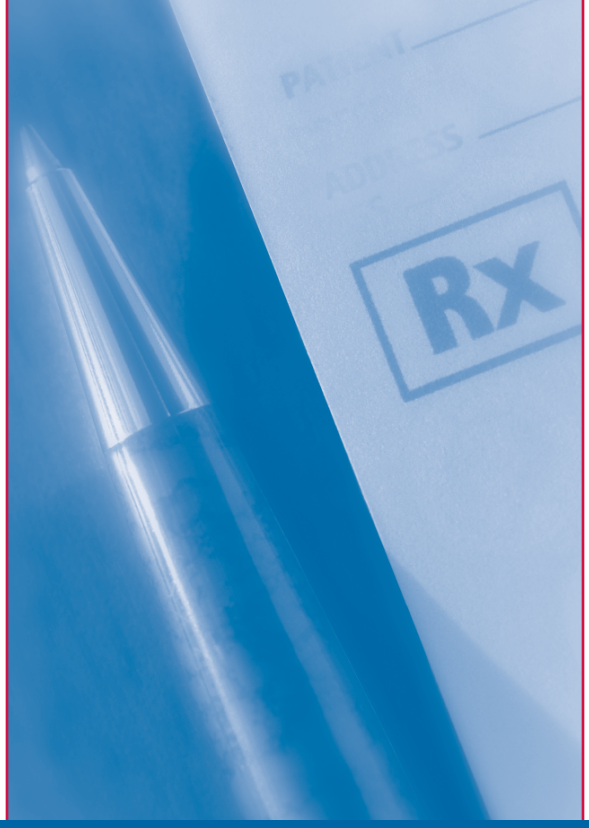
Implementation of Guidelines

Having guidelines in place will not guarantee their uptake. We have seen that although some provinces have templates, they are not always followed. Further collaborative work needs to be done to ensure that both economic evaluation guidelines developed by CCOHTA and BIA guidelines are adopted by manufacturers.

Private Sector Drug Plans

Several survey respondents suggested doing a similar survey with private sector drug plan managers, and commented on the benefits of seeing a BIA for the entire population, with analysis of both public and private budget impact.

Appendix 1.a: Survey Report



Following is the survey report prepared by Panacea Canada Inc. The consultant was responsible for preparing and conducting the survey of Steering Committee members to elicit their views on budget impact analysis reports and the need for guidelines.

Executive Summary

This project was undertaken to assist the Patented Medicine Prices Review Board (PMPRB) in developing national guidelines for Budget Impact Analyses (BIA). It is anticipated that these guidelines would be used by drug plan managers, manufacturers, and other stakeholders to estimate the financial impact of introducing new drug products onto drug formularies. The project comprised two phases, a detailed background survey that assessed specific content for a BIA guideline and a telephone interview that explored BIA utility more generally. Of the twelve survey respondents, ten were drug plan managers and the remaining two represented the Canadian Institute for Health Information (CIHI) and Health Canada's Pharmaceutical Policy Unit.

Use of Budget Impact Analyses

All drug plan managers who participated in this survey use BIAs. Most use them to estimate potential cost impact of a drug on their drug plan. In general, respondents review and validate manufacturer-submitted BIAs; if a manufacturer-submitted BIA is not available, some drug plan managers will generate a BIA internally. Availability and quality of manufacturer-submitted BIAs appears to vary across jurisdictions and across manufacturers.

In commenting on the usefulness of BIAs in various scenarios, respondents agreed that more information is better for decision-making, but only if it is reliable and can be validated. Usefulness also depends on the specificity of information provided in terms of prevalence in their jurisdiction and relevance to the population covered by their plan. All drug plan managers stated that BIAs are useful in determining potential impact of a new intervention on their drug budget, few use the information to assess the impact on the overall health system budget. While affordability is an issue in many jurisdictions, some are not working with a hard-capped budget, and so the BIA's role in determining affordability in these jurisdictions is less important.

While a BIA is potentially useful in predicting impact on health services resources, payers of these additional health services resource costs vary depending on the design of the drug plan and the variable existence of private party payers across jurisdictions. Most drug plans are funded in silos, and so will not accrue savings, benefits or costs beyond the impact on the drug budget.

The survey examined drug categories in which a BIA is applied. While some respondents commented that a BIA should be used in all submissions, others differentiated on the basis of potential impact: if there is no anticipated impact on the drug plan budget, there is no need for a BIA. Specific drug categories in which a BIA may be helpful include new chemical entities, single source products, formulation changes, new indications, line extensions and resubmitted products. In rare cases, BIAs are used for multiple source products.

In terms of the relative influence of a BIA, in some jurisdictions the influence is very significant: even positive recommendations from the CDR and the local expert advisory committee will not result in a listing if the drug has an impact on expenditures. BIAs have significant influence in a number of situations, including those in which: data accurately reflect the specific drug plan's population; the drug cost is high and the budget increase will be significant; there is targeted clinical evidence requiring limited use criteria; and where clinical/relative efficacy is "as good as" the comparator(s) but the BIA shows greater budget impact.

Some respondents commented that clinical value, safety and efficacy have more influence than a BIA in the listing decision. Others cited the influence of stakeholder pressure that sometimes outweighs a BIA in decision-making. Skepticism about BIA assumptions can prevent the analysis from influencing decision-making.

The survey explored the merits of collaborating with manufacturers in the development of BIAs. In general, respondents agreed that there would be a theoretical advantage, however, most commented that they would not have the time or resources to do this. Several respondents suggested that this collaboration might occur at a national level.

In comparing weighting of BIAs relative to economic evaluations in decision-making, the BIA is generally seen as complementary to an economic evaluation. Several respondents commented on the usefulness of CDR economic evaluations in their decision-making. In those jurisdictions where budgetary constraints are a high priority, the BIA weighs more than the economic analysis. In other words, even drugs with demonstrated cost-effectiveness may be deemed unaffordable. Others manage to address the expense associated with cost-effective drugs through restrictions on use, and will find ways to pay for them. In the minority of jurisdictions, cost-effectiveness weighs higher than BIAs in decision-making. Cost-effectiveness analyses have not historically been "trusted" because the projected savings and benefits are rarely if ever actualized.

Many respondents described the need to find a balance between cost-effectiveness and budget containment. Once again, the CDR was cited as being very helpful in evaluating cost-effectiveness and clinical benefit. In most jurisdictions, when budget impact is significant, the cost-effectiveness argument has to be very solid to justify additional spending. Sometimes, there is no tradeoff: even for very cost-effective drugs, if there is no additional budget, the drugs are not added. Many respondents commented that there is very low tolerance to predicted benefits that are vague and can't be validated or quantified. Several commented that the listing decision can be affected by stakeholder pressure, regardless of budget impact or cost-effectiveness.

Respondents included the following as criteria for a useful BIA guideline: make it user friendly, interactive and simple; ensure that it's a tool that industry will use; exclude data that can't be validated, won't be reliable or won't be used; ensure that the guideline is rigorous; make it understandable to decision-makers; and make it available in electronic formats. Respondents affirmed the importance of specificity: a national guideline will be more useful if it can be adapted by each jurisdiction to reflect prevalence, demographics, geography, drug plan design etc.

A number of respondents commented on the desirability of centralized submission and review of BIAs, in parallel to the CDR process. The process would need to address inter-plan differences and the need for specificity described above. One suggestion was to have CDR review the high-level analysis, including comparators and assumptions. The role of NPDUIS databases in this approach was highlighted.

Most respondents commented that a national BIA guideline, if used by manufacturers, would save time and result in better analyses. Reduced duplication of effort across jurisdictions is welcome.

The need for manufacturer buy-in and commitment was stressed. Experience in one jurisdiction that has a template for BIAs has shown that manufacturers see the template as a broad guideline, and few if any have actually used it.

Some plan managers supported the value of sharing BIA information across jurisdictions. Other respondents agreed that it would be interesting to see BIAs from other jurisdictions, but were doubtful about the real utility of this information. Differences among plans would make it difficult to draw comparisons.

Several respondents suggested doing a similar survey with private sector drug plan managers, and commented on the benefits of seeing a BIA for the entire population, with analysis of both public and private budget impact.

Desirable BIA components

Respondents rated the importance of various components in BIAs. Their ratings and commentaries are summarized in this report in the following categories:

- Perspective
- Data sources, data reliability and data relevance
- Indications affected
- Populations affected
- Rate of adoption
- Time horizon
- Complementary demands
- Substitution of existing treatments
- Probability of re-deploying resources
- Transparency
- Uncertainty and sensitivity analyses
- Results reporting

In most cases there was clear consensus on those components that drug plan managers would prefer to see in BIAs. Details are included in the body of the report.

Participants

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Introduction

PMPRB intends to develop national guidelines for Budget Impact Analyses (BIA). It is anticipated that these guidelines would be used by drug plan managers, manufacturers, and other stakeholders to estimate the financial impact of introducing new drug products onto drug formularies.

The NPDUIS Steering Committee contends that the main advantage of having special guidelines is to establish principles of best practice in designing and implementing financial analysis, hence increasing the reliability and utility of the analysis.

As part of this project, a survey of public drug plan managers has been undertaken to assess the overall effectiveness and utility of current Budget Impact Analyses. This report summarizes the results of the survey. Of the twelve survey respondents, ten were drug plan managers and the remaining two represented CIHI and Health Canada's Pharmaceutical Policy Unit.

The survey comprised two phases: a detailed background survey that assessed specific content for a BIA guideline and a telephone interview that explored more generally BIA utility. The survey and interview guide are appended. All twelve respondents participated in both phases of this project.

General Use of Budget Impact Analyses

All drug plan managers who participated in this survey use BIAs. Most use it to estimate potential cost impact of listing a drug in their plan. In some cases an Expert Advisory Committee (EAC) uses it in their deliberations, in others, the drug plan manager uses it to determine affordability after the EAC has made its recommendation based on clinical efficacy and cost-effectiveness. Several respondents described a process in which they use a BIA after receiving a positive recommendation from the Common Drug Review (CDR) process.

One respondent requires a BIA with every submission. In all cases, respondents review and validate manufacturer-submitted BIAs. If a manufacturer-submitted BIA is not available, some drug plan managers will generate one internally. Availability and quality of manufacturer-submitted BIAs appear to vary across jurisdictions and across manufacturers.

Several respondents commented that they use the BIA as a baseline against which the actual utilization of the drug is assessed. In some jurisdictions, BIAs are also used as the basis for developing written agreements with manufacturers.

Although template BIAs are available in some jurisdictions, manufacturers generally don't use them.

Specific Use of Budget Impact Analyses

Survey participants answered a series of questions intended to clarify when and how BIAs are used. Detailed commentary on how BIAs are used and in what scenarios follow in the sections below.

Scenarios in which Budget Impact Analyses are used

In assessing the usefulness of BIAs in various scenarios, survey respondents made the comments below. Regardless of the scenario, more information is better for decision-making, but only if it is reliable and can be validated. Usefulness also depends on the specificity of information provided in terms of prevalence in their jurisdiction and relevance to the population covered by their plan.

Impact of new intervention on formulary and/or health system budget

All drug plan managers stated that BIAs are useful in determining potential impact of a new intervention on their formulary budget. Four stated that they only consider the impact on the drug budget, and not on the health system budget.

Many respondents stated that they note the projected health system impacts beyond the drug budget, but have limited influence beyond the drug expenditure. System savings may be used as discussion points in EAC deliberations.

Affordability and budget management

Nine respondents find a BIA useful in addressing affordability. One noted that a BIA is useful here only if it's accurate. Two respondents commented that a BIA is useful in increasing predictability of expenditure.

Some jurisdictions are not specifically aiming toward a certain budget, and so affordability is less important.

Impact of a new intervention on health services resources

Seven respondents find a BIA useful in predicting impact on health services resources. Payers of these additional health services resource costs vary depending on the design of the drug plan and the variable existence of private party payers across jurisdictions. Most respondents commented that they are funded in silos, and so will not accrue savings, benefits or costs beyond the impact on the drug budget.

Raising issues about other drugs

One respondent commented that a BIA sometimes raises issues/flags about other drugs that are helpful for the drug plan manager. For example, a BIA can flag comparator drugs in which dosing regimens or indications fall outside criteria for use.

Other ways in which a BIA can be useful

Several other areas were identified by respondents, including:

- creating ownership by the manufacturer to provide more accurate and fulsome information;
- monitoring utilization after drug is added to the benefits list; and
- assisting the manager of the NIHB Plan in harmonizing with provincial drug plans.

Drug categories and the BIA

Respondents were asked to identify drug categories in which a BIA is applied. Several respondents commented that a BIA should be used in all submissions. Some differentiated on the basis of potential impact: if there is no anticipated impact on the drug plan budget, there is no need for a BIA. Otherwise a BIA is desired for drugs anticipated to have either positive or negative budget impacts.

Commentary on specific drug categories follows below.

New Chemical Entities

All respondents agreed on the importance of a BIA in this category, and many identified it as the highest priority for BIAs. The analysis is especially useful here because it's more difficult to evaluate impact in the absence of an existing market, and there are no previous data to look at or rely on.

Single source products

Most respondents use BIAs for single source product submissions; some were unclear on the distinction between single source products and new chemical entities.

Formulation changes

Drug plan managers use BIAs to assess submissions for formulation changes, especially those that are likely to have a significant impact, for example a sublingual tablet reformulated as a transdermal patch.

New indications

Most respondents use BIAs in submissions for new indications. It was suggested that it would be helpful to include all possible indications for a product, not just those targeted for reimbursement.

Line extensions

Most respondents use BIAs for line extensions, which they identified as new dosages in existing formulations.

Resubmitted products

BIAs are helpful for re-submissions, defined as submissions made to address concerns raised during the original submission, containing new evidence or factors for consideration.

Multi source products

Two respondents indicated that in some cases, a BIA is used for multiple source products. An example given was Apo-omeprazole.

BIA influence on decision-making

Respondents commented on scenarios in which a BIA would influence their decision-making. In general, any submission that will have an impact on the drug plan budget should be accompanied by a BIA. Even when the drug will have a neutral impact, some respondents indicated that they consider a BIA necessary in order to make an informed decision.

When incremental cost-effectiveness of the drug is significant

Most of the drug plan managers indicated that even if a drug is substantially more cost-effective, a BIA is still needed in order to make an informed decision. In some jurisdictions, incremental cost-effectiveness may not outweigh budget impact in decision-making.

When new money is required
Most drug plan managers require a BIA when new money is required to reimburse the drug.

Size of patient population
Population size is not necessarily a determinant in a BIA's influence on decision-making. The population covered by the drug plan is more relevant, and a drug indicated in a small population can have significant impact if it's expensive, especially in smaller jurisdictions.

Importance of affordability in decision
Affordability is not an issue in all jurisdictions; some respondents stated that they don't have hard-capped budgets. However for most respondents, affordability is an important factor in decision-making.

Large impact on service delivery (e.g. impact on physician visits, diagnostic test costs)
For most respondents, this was cited as the least likely scenario in which a BIA influences decision-making. Most drug plan managers will note this information, but it doesn't ultimately affect the listing decision.

Some respondents commented that this information is frequently unreliable.

Weighting of a BIA in reimbursement decisions

Respondents were asked to describe, qualitatively, the weighting that a BIA has in a reimbursement decision.

Significant influence
In some jurisdictions, the influence is very significant: even a positive recommendation from CDR and the EAC will not result in a listing if the drug costs money. If it saves money, it moves forward.

Respondents indicated that a BIA has significant influence when:

- data accurately reflects the specific drug plan's population;
- the drug cost is high and the budget increase will be significant;
- there is targeted clinical evidence requiring limited use criteria; and
- clinical/relative efficacy is "as good as" but the BIA shows greater budget impact than comparators.

Competing influences
Clinical value needs to be demonstrated before a drug will be considered for listing; Some respondents indicated that the BIA is usually the consideration after safety and clinical effectiveness.

Another competing influence is that of lobby pressure, that can outweigh a BIA in decision-making.

Somewhat of an influence

If the impact of the drug will be neutral or cost saving, for some respondents the BIA is not an important factor in decision-making. If the budgetary impact is minor, and the drug has other advantages, the BIA is examined closely. In some jurisdictions, skepticism about BIA assumptions prevents the analysis from influencing decision-making. It's seen as just a good starting point. In written agreements, forecasting numbers may change compared to initial BIA, which reflects on how realistic BIAs are, and increases skepticism.

Depends on scenario

One respondent indicated that the weight of a BIA in decision-making depends on the type of drug: for breakthrough drugs, it's very important; for me-too drugs, it's less important; and for line extensions it's minimally important.

Collaboration with the manufacturer in the development of BIAs

In general, respondents agreed that there would be a theoretical advantage to collaborating with manufacturers. The possibility exists to develop a BIA that is more useful, reliable and valid. It would be helpful to learn from the manufacturer what assumptions they make. In most cases, drug plan managers receive the BIA from the manufacturer and validate it for use in their province: frequently there is significant re-work to be done. Collaboration might also be helpful in understanding how the manufacturer is marketing the drug and what they're doing to influence prescribing. Finally, the BIA may be used to gain buy-in for later agreements.

In spite of the possible advantages, however, most respondents commented that they would not have the time or resources to do this. Several respondents suggested that this collaboration might occur at a national level. The model and assumptions could be developed centrally, and leave the specifics in terms of prevalence and drug plan parameters to the drug plan managers.

One respondent wondered whether manufacturers would be willing to collaborate. Another commented that in the current scenario, manufacturers have to address multiple jurisdictions and perhaps they would prefer a centralized approach. Concern was raised about the optics of this collaboration, and the need for a level playing field in which all manufacturers have the same opportunity to collaborate in the same way.

One respondent stated that collaboration already exists in her jurisdiction, mainly at the outset of the process through provision of province-specific information, comparators, considerations, cost escalators, and assumptions. It appears to be helpful to provide contextual information about the plan or province to the manufacturer.

One respondent commented that the inclusion of a manufacturer contact name and coordinates would be very helpful.

The BIA as a planning tool

In most jurisdictions the BIA is used to forecast budgets into a 3 – 5 year horizon. It's use as a planning tool beyond forecasting is limited.

It is also used to monitor the listing decision. BIAs help to assess the degree of inappropriate use against the baseline and to evaluate whether limited-use criteria are working. Effective follow through was described as resource intensive by many drug plan managers.

Some respondents stated that they had limited confidence in the BIA's accuracy, validity and reliability and would hesitate to use it as a planning tool.

BIAs and risk-sharing agreements

Most of respondents do not have risk-sharing agreements in their jurisdictions, but commented that if they did, BIAs would be very useful as a baseline for the agreement. The BIA would establish the magnitude of the risk for the province, and might be more accurate, reliable and valid if the manufacturer knew it would be the baseline for a risk-sharing agreement.

Of those respondents who had experience in risk-sharing agreements, all agreed that the BIA is very important as a baseline against which to measure utilization. It has been their experience that a risk-sharing agreement results in sharper forecasting by the manufacturer. Frequently the original BIA is revised for the risk-sharing agreement, resulting in limited confidence in the initial analysis.

BIAs and price level negotiation, price/volume agreements

None of the jurisdictions negotiate price levels or price/volume agreements, but respondents noted that if they did, a BIA would be important to establishing the agreement. Several respondents noted that an agreement would provide incentive for manufacturers to develop more accurate, reliable and valid BIAs.

Impact of economic data on drug plan decision-making

In most jurisdictions, the BIA is seen as complementary to an economic evaluation. Respondents used different terminology in discussing economic evaluations, including pharmacoeconomics analyses, health economic analyses, and cost-effectiveness, pointing to the need for clear definitions in a BIA Guideline.

Several respondents commented on the usefulness of CDR recommendations, including the economic evaluations, in their decision-making.

In some jurisdictions, decision-making examines clinical benefit first, followed by cost-effectiveness and finally the BIA. If a clinically-effective drug is more cost-effective than its comparators, the jurisdictions will find ways to pay for it.

In some jurisdictions, economic data are used by the expert advisory committee, but not used internally in the drug plan.

Several respondents commented that economic analyses had not been historically well done, and thus had not swayed listing decisions. Where a rigorous, peer-reviewed analysis is available, the cost-effectiveness studies weigh higher.

Relative importance of budget compared to incremental cost-effectiveness in decision-making

In several jurisdictions, where budgetary constraints are a high priority, the BIA weighs more than the economic analysis. In other words, even for drugs that are very cost-effective there still might not be money to afford them. Others manage to cover the expense associated with some cost-effective drugs through limited use criteria, and usually find a way to pay for them. In the minority of jurisdictions, cost-effectiveness weighs higher than BIAs in decision-making.

Several respondents indicated that the listing decision is a clinical decision first and foremost. Clinical benefits are weighed first, before cost-effectiveness and budget impact.

Cost-effectiveness analyses have not historically been "trusted" because the projected savings and benefits are rarely if ever actualized.

Magnitude of Trade-off between Cost-effectiveness and Budget Containment

Many respondents described the need to find a balance between cost-effectiveness and budget containment. Once again, the CDR was cited as being very helpful here in demonstrating cost-effectiveness and clinical benefit. In most jurisdictions, when budget impact is significant, the cost-effectiveness argument has to be very solid to justify additional spending.

In many jurisdictions, every effort is made to add clinically effective drugs to the formulary, and additions are managed on a case by case basis. Drug Plan managers might offset the addition of a cost-effective drug by removing other drugs, and limiting use of the new drug. Budget impacts are considered, but in these jurisdictions, it is unlikely that access will be denied.

In other jurisdictions, there is no trade-off. Even for very cost-effective drugs, if there is no money to pay, the drugs are not added. One respondent stated that in her province, they haven't listed a new drug in 3.5 years if it costs money, even those with demonstrated cost-effectiveness.

Respondents indicated that when validity of an economic evaluation is questionable, with limited proof that benefits will actually occur, the cost-effectiveness argument is lost. An increase in the budget is a known quantity. There is very low tolerance to list based on predicted benefits that are vague and can't be validated or quantified.

Several respondents commented that the listing decision can be affected by stakeholder pressure, regardless of budget impact or cost-effectiveness.

Thoughts on a national BIA guideline

All respondents supported the usefulness of a national guideline. Following are some additional comments and suggestions.

Create a useful guideline

Respondents included the following as criteria for a useful guideline:

- make it user friendly, interactive and simple;
- make it a tool that industry will use;
- avoid asking for data that can't be validated won't be reliable or won't be used (e.g. esoteric or nice-to-have data);
- ensure that the guideline is rigorous;
- make it understandable to decision-makers; and
- make it available in e-format, CD-ROM, perhaps on central Web site in interactive format.

Need for specificity

Most respondents described the importance of specificity. A national guideline will be more useful if it can be adapted by each jurisdiction to reflect prevalence, demographics, geography, drug plan design etc.

Centralized submission and review

A number of respondents commented on the desirability of centralized submission and review of BIAs, in parallel to the CDR process. The process would need to address inter-plan differences and the need for specificity described above. One suggestion was to have CDR review the high-level analysis, including comparators and assumptions. The role of NPDUIS databases in this approach was highlighted. One respondent commented that tying a BIA guideline to economic guidelines would help payers and the industry. Several respondents commented on the lack of availability of the necessary pharmacoeconomic skill set; this expertise is limited and so centralization wherever possible would be helpful.

Time and labour savings

Most respondents commented that a national BIA guideline, if used by manufacturers, would save time and result in better analyses. Reduced duplication of effort across jurisdictions is welcome.

Manufacturer participation

Many respondents questioned how manufacturers might be influenced to use the guideline. Currently there is a range in quality of submissions. What might be the incentives and penalties? Would a drug plan manager refuse those that are poorly done?

Experience in one jurisdiction that has a template for BIAs has shown that manufacturers see the template as a broad guideline, and few if any have actually used it. The need for manufacturer buy-in and commitment was stressed.

Share BIA information across jurisdictions

Some plan managers supported the value of sharing BIA information across jurisdictions. One commented that it would be great to have all the BIAs on one spreadsheet by province and territory, with assumptions clearly stated.

Other respondents agreed that it would be interesting to see BIAs from other jurisdictions, but were doubtful about the real utility of this information. Differences among plans would make it difficult to draw comparisons.

Other Comments and Advice

Private Sector Drug Plans

Several respondents suggested doing a similar survey with private sector drug plan managers, and commented on the benefits of seeing a BIA for the entire population, with analysis of both public and private budget impact.

Specific components that drug plan managers would prefer to see in BIAs

Respondents were asked to rank a series of BIA components on a 5 point scale, where 1 was not at all important, 3 was important and 5 was very important. Detailed data are found in the appendix; numbering in the table below corresponds to numbering in the appended detail. The following is a summary of components that were ranked as very important, and those ranked as less important or not important. Respondent comments are also included.

Very Important (ranked at 4 or 5 by more than 50% or respondents)	Less important or not important (ranked at 3 or lower by at least 50% of respondents)	Respondent Comments
1.0 Perspective		
1.1 The BIA should clearly state that it is prepared from the drug plan payer's perspective (100%) 1.2 The BIA should clearly state costs and savings consistent with the drug plan payer's perspective (92%) 1.3 Relevance of health care costs, savings and benefits should be justified from the drug plan payer's perspective (92%) 1.4 Results should be presented in aggregate and disaggregate form (75%) 1.5 Results should address the impact on the health care budget for drugs. (65%)	1.6 Results should address the impact on the overall health care budget (50%)	• Items 1.1 – 1.3 are most important • 1.1: This is often the issue with regards to usefulness of these reports. Manufacturers make a number of assumptions related to the payer which may be inaccurate or are built on a model that is based on market sales information which is then made to "fit" the payer's model • 1.4: Aggregate/disaggregate: not clear what is meant by this – is this groupings by age or groupings by different drug programme? • 1.6 Our Drug plan will only be able to consider the impact on drug budget since other related costs are not its responsibility • 1.6: Information is needed on the separation between plans covered in our jurisdiction: Health versus other ministries, but the impact on Health is useful • 1.6: Overall Health care budget: those would be interesting but these savings are not often realized
2.0 Data Sources, Data Reliability and Data Relevance		
2.1 Clear identification of source(s) of epidemiological data: Incidence (82%) 2.2 Clear identification of source(s) of epidemiological data: Prevalence (91%) 2.3 Clear identification of source(s) for current patterns of care and resource use (90%) 2.4 Clear identification of source(s) for Anticipated Resource use (73%) 2.5 Clear identification of source(s) for Anticipated Resource costs (64%) 2.6 Prevalence and Incidence specified for national, jurisdictional and cultural groups (64%) 2.7 Assumptions clearly stated in absence of referenced data (83%) 2.8 Relevance of all data justified (83%) 2.9 Relevance of all assumptions justified (92%)	2.10 Ability to vary assumptions in an interactive model (59%)	• Need to be clear about what we mean by relevance, and by resource use • The source identification is relevant only when it relates to the patient population we cover under our program. Otherwise, it provides context only and doesn't directly relate to the patient population impacted by the decisions to list. • Recommendation from Common Drug Review – very important • Our drug plan is only responsible for the cost of drug and cost of pharmacy resources. Costs of other resources are outside our mandate • 2.10: Interactive model would be valuable only if quality data are received in the BIA

Very Important (ranked at 4 or 5 by more than 50% or respondents)	Less important or not important (ranked at 3 or lower by at least 50% of respondents)	Respondent Comments
3.0 Indications Affected		
3.1 Clearly delineated indications for use (83%) 3.2 Projection of inappropriate use (e.g. wrong population, wrong dose, use not supported by literature) (67%) 3.3 Projected leakage out of indication (67%)		• 3.2: If drug plan manager restricts to certain usage, this doesn't matter • How is leakage different from inappropriate use? • The indications should link to the reimbursement status that the manufacturer is seeking • Although important, I don't think it's likely that a manufacturer is going to accurately report projections of leakage and inappropriate use. • Would also like to see experience in other jurisdictions – uptake, inappropriate use etc.
4.0 Populations Affected		
4.1 Clear description of patient groups in which drug is indicated (92%) 4.2 Sub population analysis: description of sub-populations most likely to benefit (67%) 4.3 Projection of use in inappropriate population (e.g. wrong age group, indication not supported by literature in this population) (59%) 4.4 Projected leakage out of population (64%)		• All competing drugs in a class should be well defined to determine the market and its characteristics. Determining patient groups per drugs should come after • Clear determination of patient groups should be based on rules for a province plan such as limited coverage based on second line therapy etc. • The indications should link to the drug plan population • Although important, I don't think it likely that a manufacturer is going to accurately report projections of leakage and inappropriate use. • Populations affected more useful than indications; the former will provide information on expenditure in my jurisdiction
5.0 Rate of Adoption		
5.1 Estimation of different rates of diffusion over 3-5 year period (84%)	5.2 Projected impact of induced demands (manufacturer promotion, direct to consumer advertising, information from the internet, other sources) (58%) 5.3 Impact of new technology on health seeking behaviour (65%)	• Items 5.2 and 5.3 would be most difficult to evaluate • 5.2: Note that DTCA is not legal in Canada, but cross border media may influence Canadian behaviour • I have never seen modeling this detailed. Close to impossible to predict growth rates like that • I don't think the latter two points could be accurately quantified and therefore, may not be of much use
6.0 Time Horizon		
6.1 Predicted financial implication over at least 2 years after date of listing (58%) 6.2 Predicted financial implication over at least 3-5 years after date of listing (82%) 6.3 Annual financial implication until drug is predicted to reach peak or stable market share (60%)		• Most products tend to have their growth peak within the first two years

Very Important (ranked at 4 or 5 by more than 50% or respondents)	Less important or not important (ranked at 3 or lower by at least 50% of respondents)	Respondent Comments
7.0 Complementary Demands		
7.1 Estimated use and cost of other drugs used concomitantly (84%)	7.2 Cost of preconditions for effective introduction of drug (e.g. training. Technology. Diagnostic facilities) (83%) 7.3 Estimated elasticity of input supply (re-training, re-tooling) (89%)	• 7.2 Few drugs have such an impact. Companies are picking up some of these costs (e.g. Remicade infusion centres) • The cost would be relevant if it is expected that it would be borne by the publicly-funded health care system
8.0 Substitution of existing treatments		
8.1 Identification of therapy/therapies likely to be replaced by new intervention (100%) 8.2 Description and costing of patient switching scenarios (100%) 8.3 Estimated substitution rates (90%)		
9.0 Probability of re-deploying resources		
	9.1 Predicted probability of redeploying labour savings to other areas of care (100%) 9.2 Predicted probability of redeploying capital savings to other areas of care (100%) 9.3 Projection of how savings will be realized over time (89%)	• Analysis should always revolve around referent group saving to Pharmacare Budget. Savings in other health areas shouldn't be compared unless they are substantial and well proven. This is unlikely for costing at the hospital level
10.0 Transparency		
10.1 Clear understanding of all input assumptions (92%) 10.2 Clear understanding of all assumed relationships among variables and resulting outcomes (e.g. disease states and hospitalizations) (86%) 10.4 Inclusion of multiple scenarios in the model (57%) 10.5 Decision-maker access to the model (54%) 10.6 Description of the relationship between intermediate and final therapeutic endpoints (i.e. how is the projected long term impact for new agents derived?) (59%)	10.3 Make predictive model as interactive as possible (58%)	• 10.1: Since the model you build is as good as its assumptions, it is critical to present them clearly • 10.3: Interactive model would be useful but depends on the quality of information in the BIA • 10.4: inclusion of too many scenarios is not helpful. Prefer one or two, with the optimal/most appropriate use scenario as most important • 10.5: Decision-maker access to the model is usually not beneficial unless the creator gives a walk through. Otherwise things can be misinterpreted
11.0 Uncertainty and sensitivity analysis		
11.1 Inclusion of sensitivity analyses for parameters where there is uncertainty or lack of agreement (75%)		• From an economics perspective, sensitivity analyses are a standard requirement of any serious cost benefit analysis… would want to see these in a BIA as well
12.0 Results Reporting		
12.1 Costs presented in aggregate and disaggregate form (58) 12.3 Cost/Claim (55%) 12.4 Total treatment costs (67%)	12.2 Report results in natural and monetary units (natural = hospital beds, nursing time, lab tests, diagnostic equipment) (66%)	• 12.1: Disaggregate form is only important if the methodology is explained in clear detail. Often BIAs do not include all of the assumptions used in the analysis. Therefore, reviewing the disaggregate information loses its value • 12.2: Our drug plan is not responsible for these other costs

Survey Results

(n = 12)

Section One: Perspective

Respondents ranked their agreement with the following statements (1 = strongly disagree 3 = agree 5 = strongly agree)

BIA Component	1	2	3	4	5	n
1.1 The BIA should clearly state that it is prepared from the drug plan payer's perspective				2 *17%*	10 *83%*	12 *100%*
1.2 The BIA should clearly state costs and savings consistent with the drug plan payer's perspective			1 *8%*		11 *92%*	12 *100%*
1.3 Relevance of health care costs, savings and benefits should be justified from the drug plan payer's perspective			1 *8%*	1 *8%*	10 *84%*	12 *100%*
1.4 Results should be presented in aggregate and disaggregate form		1 *8%*	2 *17%*	5 *42%*	4 *33%*	12 *100%*
1.5 Results should address the impact on the overall health care budget	1 *10%*		4 *40%*	3 *30%*	2 *20%*	10 *100%*
1.6 Results should address the impact on the health care budget for drugs	1 *8%*		3 *25%*	2 *17%*	6 *50%*	12 *100%*

Comments:

- Items 1 – 3 are most important
- The BIA should state that it is based on the payer's perspective but this is often the issue with regards to usefulness of these reports. Manufacturers make a number of assumptions related to the payer which may be inaccurate or are built on model that is based on market sales information which is then made to "fit" the payer's model
- NIHB will only be able to consider the impact on drug budget since other related costs are not its responsibility
- Aggregate/disaggregate: not clear what is meant by this – is this groupings by age or groupings by different drug programme
- Information is needed on the separation between plans covered in our jurisdiction: Health versus other ministries, but the impact on Health is useful
- Overall Health care budget: those would be interesting but these savings are not often realized

Section Two: Data Sources, Data Reliability and Data Relevance

Respondents ranked the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

BIA Component	1	2	3	4	5	n
2.1 Clear identification of source(s) of epidemiological data: Incidence			2 *18%*		9 *82%*	11 *100%*
2.2 Clear identification of source(s) of epidemiological data: Prevalence			1 *9%*		10 *91%*	11 *100%*
2.3 Clear identification of source(s) for current patterns of care and resource use			1 *10%*	1 *10%*	8 *80%*	10 *100%*
2.4 Clear identification of source(s) for Anticipated Resource use		1 *9%*	2 *18%*	3 *27%*	5 *46%*	11 *100%*
2.5 Clear identification of source(s) for Anticipated Resource costs		1 *9%*	3 *27%*	2 *18%*	5 *46%*	11 *100%*
2.6 Prevalence and Incidence specified for national, jurisdictional and cultural groups		2 *18%*	2 *18%*	1 *9%*	6 *55%*	11 *100%*
2.7 Assumptions clearly stated in absence of referenced data			2 *17%*	1 *8%*	9 *75%*	12 *100%*
2.8 Relevance of all data justified			2 *17%*	2 *17%*	8 *66%*	12 *100%*
2.9 Relevance of all assumptions justified			1 *8%*	1 *8%*	10 *84%*	12 *100%*
2.10 Ability to vary assumptions in an interactive model		2 *17%*	5 *42%*	4 *33%*	1 *8%*	12 *100%*

Comments:

- Need to be clear about what we mean by relevance, and by resource use
- The source identification is relevant only when it relates to the patient population we cover under our program. Otherwise, it provides context only and doesn't directly relate to the patient population impacted by the decisions to list.
- Recommendation from Common Drug Review
- We are only responsible for the cost of drug and cost of pharmacy resources. Costs of other resources are outside our mandate
- Interactive model would be valuable only if quality data are received in the BIA

Section Three: Indications Affected

Respondents ranked the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

BIA Component	1	2	3	4	5	n
3.1 Clearly delineated indications for use			2 *17%*	3 *25%*	7 *58%*	12 *100%*
3.2 Projection of inappropriate use (e.g. wrong population, wrong dose, use not supported by literature)		1 *8%*	3 *25%*	2 *17%*	6 *50%*	12 *100%*
3.3 Projected leakage out of indication		1 *8%*	3 *25%*	3 *25%*	5 *42%*	12 *100%*

Comments:

- 3.2: If drug plan manager restricts to certain usage, this doesn't matter
- How is leakage different from inappropriate use?
- The indications should link to the reimbursement status that the manufacturer is seeking
- Although important, I don't think it's likely that a manufacturer is going to accurately report projections of leakage and inappropriate use
- Would also like to see experience in other jurisdictions – uptake, inappropriate use etc.

Section Four: Populations Affected

Respondents ranked the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

BIA Component	1	2	3	4	5	n
4.1 Clear description of patient groups in which drug is indicated			1 *8%*	1 *8%*	10 *84%*	12 *100%*
4.2 Sub population analysis: description of sub-populations most likely to benefit			4 *33%*	2 *17%*	6 *50%*	12 *100%*
4.3 Projection of use in inappropriate population (e.g. wrong age group, indication not supported by literature in this population)		1 *8%*	4 *33%*	2 *17%*	5 *42%*	12 *100%*
4.4 Projected leakage out of population			4 *36%*	3.5 *32%*	3.5 *32%*	11 *100%*

Comments:

- All competing drugs in a class should be well defined to determine the market and its characteristics. Determining patient groups per drugs should come after
- Clear determination of patient groups should also be based on rules for a province plan such as limited coverage based on second line therapy etc.
- The indications should link to the drug plan population
- Although important, I don't think it likely that a manufacturer is going to accurately report projections of leakage and inappropriate use
- Populations affected more useful than indications (Section Three); the former will provide information on expenditure in my jurisdiction

Section Five: Rate of Adoption

Respondents ranked the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

BIA Component	1	2	3	4	5	n
5.1 Estimation of different rates of diffusion over 3-5 year period	1 8%	1 8%		3 25%	7 59%	12 100%
5.2 Projected impact of induced demands (manufacturer promotion, direct to consumer advertising, information from the internet, other sources)	1 8%	3 25%	3 25%	4 34%	1 8%	12 100%
5.3 Impact of new technology on health seeking behaviour	1 8%	3 25%	5 42%	2 17%	1 8%	12 100%

Comments:

- Items 2 and 3 would be most difficult to evaluate
- Note that DTCA is not legal in Canada, but cross border media may influence Canadian behaviour
- I have never seen modeling this detailed. Close to impossible to predict growth rates like that
- I don't think the latter two points could be accurately quantified and therefore, may not be of much use

Section Six: Time horizon

Respondents ranked the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

BIA Component	1	2	3	4	5	n
6.1 Predicted financial implication over at least 2 years after date of listing		1 8%	4 33%	4 33%	3 25%	12 100%
6.2 Predicted financial implication over at least 3-5 years after date of listing		1 9%	1 9%	5 45%	4 37%	11 100%
6.3 Annual financial implication until drug is predicted to reach peak or stable market share		2 20%	2 20%	3 30%	3 30%	10 100%

Comments:

- Most products tend to have their growth peak within the first two years

Section Seven: Complementary Demands

Respondents ranked the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

BIA Component	1	2	3	4	5	n
7.1 Estimated use and cost of other drugs used concomitantly		1 8%	1 8%	1 8%	9 76%	12 100%
7.2 Cost of preconditions for effective introduction of drug (e.g. training, technology, diagnostic facilities)	1 8%	5.5 46%	3.5 29%		2 17%	12 100%
7.3 Estimated elasticity of input supply (re-training, re-tooling)	1 11%	4.5 50%	2.5 28%	1 11%		9 100%

Comments:

- Few drugs have such an impact. Companies are picking up some of these costs (e.g. Remicade infusion centres)
- The costs would be relevant if it is expected that it would be borne by the health care system

Section Eight: Substitution of existing treatments

Respondents ranked the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

BIA Component	1	2	3	4	5	n
8.1 Identification of therapy/therapies likely to be replaced by new intervention				2 *17%*	10 *83%*	12 *100%*
8.2 Description and costing of patient switching scenarios				2 *17%*	10 *83%*	12 *100%*
8.3 Estimated substitution rates			1 *10%*		9 *90%*	10 *100%*

Comments:

Section Nine: Probability of re-deploying resources

Respondents ranked the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

BIA Component	1	2	3	4	5	n
9.1 Predicted probability of redeploying labour savings to other areas of care	3.5 *32%*	4.5 *41%*	3 *27%*			11 *100%*
9.2 Predicted probability of redeploying capital savings to other areas of care	3 *28%*	4 *36%*	4 *36%*			11 *100%*
9.3 Projection of how savings will be realized over time		3 *34%*	5 *55%*	1 *11%*		9 *100%*

Comments:

- Analysis should always revolve around referent group saving to Pharmacare Budget. Savings in other health areas shouldn't be compared unless they are substantial and well proven. This is unlikely for costing at the hospital level

Section Ten: Transparency

Respondents ranked the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

BIA Component	1	2	3	4	5	n
10.1 Clear understanding of all input assumptions			1 8%	2 17%	9 75%	12 100%
10.2 Clear understanding of all assumed relationships among variables and resulting outcomes (e.g. disease states and hospitalizations)			1.5 14%	1.5 14%	8 72%	11 100%
10.3 Make predictive model as interactive as possible		2 17%	5 41%	2 17%	3 25%	12 100%
10.4 Inclusion of multiple scenarios in model		1 8%	3 25%	3 25%	5 42%	12 100%
10.5 Decision-maker access to the model	1 10%	2 18%	2 18%	3 27%	3 27%	11 100%
10.6 Description of relationship between intermediate and final therapeutic endpoints, i.e. how is the projected long term impact for new agents derived?		1 9%	3.5 32%	2.5 23%	4 36%	11 100%

Comments:

- Inclusion of too many scenarios is not helpful. Prefer one or two, with the optimal/most appropriate use scenario as most important
- Since the model you build is as good as its assumptions, it is critical to present them clearly
- Decision-maker access to the model is usually not beneficial unless the creator gives a walk through. Otherwise things can be misinterpreted
- Interactive model would be useful but depends on the quality of information in the BIA

Section Eleven: Uncertainty and Sensitivity analyses

Respondents ranked the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

BIA Component	1	2	3	4	5	n
11.1 Inclusion of sensitivity analyses for parameters where there is uncertainty or lack of agreement			3 25%	4 33%	5 42%	12 100%

Comments:

- From an economics perspective, sensitivity analyses are a standard requirement of any serious cost benefit analysis… would want to see these in a BIA as well

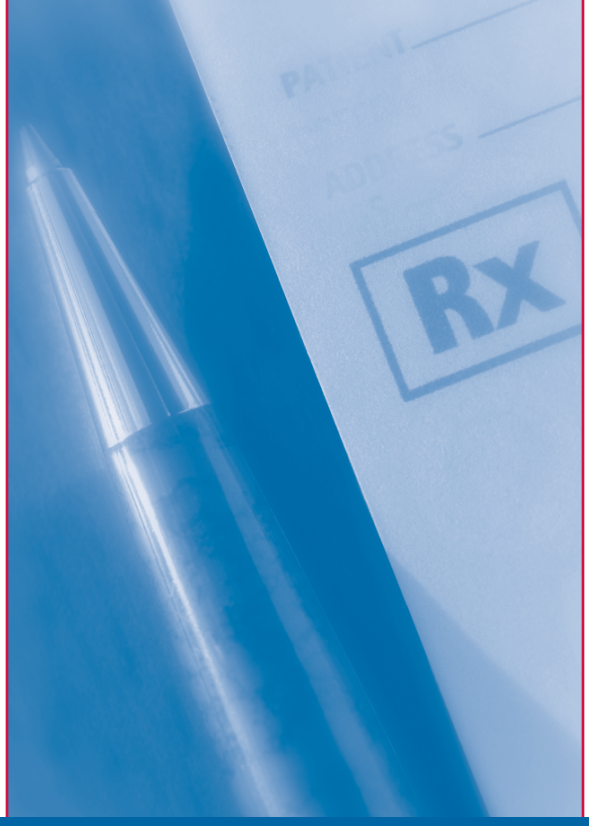
Section Twelve: Results Reporting

Respondents ranked the importance of the following data in a BIA (1 = not at all important 3 = important 5 = very important)

BIA Component	1	2	3	4	5	n
12.1 Costs presented in aggregate and disaggregate form		1 *9%*	4 *33%*	3 *25%*	4 *33%*	12 *100%*
12.2 Report results in natural and monetary units (natural = hospital beds, nursing time, lab tests, diagnostic equipment)		3.5 *29%*	4.5 *37%*	2 *17%*	2 *17%*	12 *100%*
12.3 Cost/Claim	1 *9%*	1 *9%*	3 *27%*	2 *19%*	4 *36%*	11 *100%*
12.4 Total treatment costs			4 *33%*		8 *67%*	12 *100%*

Comments:

- Disaggregate form only important if the methodology is explained in clear detail. Often BIAs do not include all of the assumptions used in the analysis. Therefore, reviewing the disaggregate information loses its value
- We are not responsible for these other costs



Appendix 1.b: Survey Questionnaire

Telephone Interview Questions

"Cost-Effective Drugs Can Still be Costly"
(A. Laupacis)

Introduction

The questions below are intended to complement the survey on the importance of various components of Budget Impact Analyses (BIA). Drug plan managers will receive both the survey and this question guide in advance of the telephone interview. Ideally, they will have completed the survey prior to the interview.

Definitions

For the purposes of this survey, we define a Budget Impact Analysis as the macro-consequences of the possible inclusion of a drug in a provincial drug budget (Orlewska and Mierzejewski, 2004). It addresses the issue of affordability and staying within budget constraints, and tries to predict the impact of a new intervention on health services resources (Brown and Boucher, 2002)

Economic evaluations (EEs) assess the overall cost-effectiveness of a drug from a societal perspective. A full economic analysis compares two or more treatment alternatives in terms of both costs and consequences. It comprises two key elements: (a) a comparison between alternative choices and (b) analysis of both costs and consequences (Brown, 2001, Drummond, 1997).

Interview Questions

1. Do you use BIAs?
    a. If yes, in what ways do you use them?
    b. If no, what prevents you from using a BIA?

2. In which of the following ways do you think a BIA is most useful (please identify all that apply)?
    a. Impact of new intervention on formulary or health system budget
    b. Addresses issue of affordability and staying within budget
    c. Tries to predict impact of a new intervention on health services resources
    d. Other – please specify

3. For which drugs should a BIA apply?
    a. NCEs
    b. Single source products
    c. Line extensions
    d. Resubmitted products
    e. Other – please specify

4. Does a BIA influence your decision-making in any of the following scenarios?
    a. When incremental cost-effectiveness of the drug is significant
    b. When new money is required
    c. Large size of patient population
    d. Importance of affordability in decision
    e. Large impact on service delivery (e.g. impact on physician visits, diagnostic test costs)
    f. Other – please specify

5. How much weight does a BIA carry in making reimbursement decisions?

6. Please comment on whether you believe it would be useful to collaborate with the manufacturer in the development of a BIA?

7. What, in your opinion, are the most common weaknesses of the BIAs that you receive from manufacturers?

8. Is a BIA helpful as a planning tool? For example, in the following:
    a. Assessment of the likelihood of attaining theoretical benefits
    b. Assessment of the need for additional resources (e.g. complementary costs: drug that requires special training to administer, new diagnostic tests)

9. Are BIAs used in developing risk-sharing agreements in your province/territory?

10. Are BIAs used to negotiate price levels or price/volume agreements in your province/territory?

11. Does economic data have an impact on decision-making in your drug plan?

12. How important is budgetary impact compared to incremental cost-effectiveness ratios (economic evaluations) in decision-making?

(Note: BIAs and economic evaluations (EEs) are described as complementary to one another. EEs generate cost-effectiveness data and BIAs generate affordability data. The BIA assists in determining whether a plan can afford a drug, even if it has been declared cost-effective through an EE. Does the plan manager agree with this complementarity? Canada has guidelines for EEs but not for BIAs.)

13. What is your estimate of the magnitude of the trade-off between budget containment and cost-effectiveness?

14. In what ways would a national BIA guideline be useful to you? What would prevent you from supporting the use of the guideline?

15. What additional comments do you have as a result of this conversation and/or the survey?

PMPRB Budget Impact Analysis Project

Final Survey

Introduction

This questionnaire is intended to elicit your opinion on the importance of various components of a Budget Impact Analysis (BIA). These components were identified in a literature review. Your answers to this survey will assist in developing a BIA Guideline that reflects the needs of Canadian Drug Plan Managers. Telephone interviews will clarify and augment the information collected in this survey.

Please answer the questions in the sections below from your perspective as drug plan manager for your province of territory.

Please contact Karen Graham of Panacea Canada Inc. with any questions.
(v. 705-835-3788, e.kgraham@panaceacoaching.com.)

Survey Sections:

1. Perspective
2. Data sources, data reliability and data relevance
3. Indications affected
4. Populations affected
5. Rate of adoption
6. Time horizon
7. Complementary Demands
8. Substitution of Existing Treatments
9. Probability of re-deploying resources
10. Transparency
11. Uncertainty and sensitivity analyses
12. Results Reporting

Section One: Perspective

Please rank your agreement with the following statements (1 = strongly disagree 3 = agree 5 = strongly agree)

• The BIA should clearly state that it is prepared from the drug plan payer's perspective	1	2	3	4	5
• The BIA should clearly state costs and savings consistent with the drug plan payer's perspective	1	2	3	4	5
• Relevance of health care costs, savings and benefits should be justified from the drug plan payer's perspective	1	2	3	4	5
• Results should be presented in aggregate and disaggregate form	1	2	3	4	5
• Results should address the impact on the overall health care budget	1	2	3	4	5
• Results should address the impact on the health care budget for drugs	1	2	3	4	5
Please list and rank other components in this category that would be helpful for you:					
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5

Comments:

Section Two: Data Sources, Data Reliability and Data Relevance

Please rank the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

• Clear identification of source(s) of epidemiological data: Incidence	1	2	3	4	5
• Clear identification of source(s) of epidemiological data: Prevalence	1	2	3	4	5
• Clear identification of source(s) for current patterns of care and resource use	1	2	3	4	5
• Clear identification of source(s) for Anticipated Resource use	1	2	3	4	5
• Clear identification of source(s) for Anticipated Resource costs	1	2	3	4	5
• Clear identification of source(s) for other data: Please list and rank other data:	1	2	3	4	5
–	1	2	3	4	5
–	1	2	3	4	5
–	1	2	3	4	5
–	1	2	3	4	5
• Prevalence and Incidence specified for national, jurisdictional and cultural groups	1	2	3	4	5
• Assumptions clearly stated in absence of referenced data	1	2	3	4	5
• Relevance of all data justified	1	2	3	4	5
• Relevance of all assumptions justified	1	2	3	4	5
• Ability to vary assumptions in an interactive model	1	2	3	4	5
Please list and rank other components in this category that would be helpful for you :					
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5

Comments:

Section Three: Indications Affected

Please rank the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

• Clearly delineated indications for use	1	2	3	4	5
• Projection of inappropriate use (e.g. wrong population, wrong dose, use not supported by literature)	1	2	3	4	5
• Projected leakage out of indication	1	2	3	4	5
Please list and rank other components in this category that would be helpful for you :					
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5

Comments:

Section Four: Populations Affected

Please rank the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

• Clear description of patient groups in which drug is indicated	1	2	3	4	5
• Sub population analysis: description of sub-populations most likely to benefit	1	2	3	4	5
• Projection of use in inappropriate population (e.g. wrong age group, indication not supported by literature in this population)	1	2	3	4	5
• Projected leakage out of population	1	2	3	4	5
Please list and rank other components in this category that would be helpful for you :					
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5

Comments:

Section Five: Rate of Adoption

Please rank the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

• Estimation of different rates of diffusion over 3-5 year period	1	2	3	4	5
• Projected impact of induced demands (manufacturer promotion, direct to consumer advertising, information from the internet, other sources)	1	2	3	4	5
• Impact of new technology on health seeking behaviour	1	2	3	4	5
Please list and rank other components in this category that would be helpful for you :					
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5

Comments:

Section Six: Time horizon

Please rank the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

• Predicted financial implication over at least 2 years after date of listing	1	2	3	4	5
• Predicted financial implication over at least 3-5 years after date of listing (status quo)	1	2	3	4	5
• Annual financial implication until drug is predicted to reach peak or stable market share	1	2	3	4	5
Please list and rank other components in this category that would be helpful for you :					
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5

Comments:

Section Seven: Complementary Demands

Please rank the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

• Estimated use and cost of other drugs used concomitantly	1	2	3	4	5
• Cost of preconditions for effective introduction of drug (e.g. training, technology, diagnostic facilities)	1	2	3	4	5
• Estimated elasticity of input supply (re-training, re-tooling)	1	2	3	4	5
Please list and rank other components in this category that would be helpful for you :					
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5

Comments:

Section Eight: Substitution of existing treatments

Please rank the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

• Identification of therapy/therapies likely to be replaced by new intervention					
• Description and costing of patient switching scenarios	1	2	3	4	5
• Estimated substitution rates	1	2	3	4	5
Please list and rank other components in this category that would be helpful for you :					
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5

Comments:

Section Nine: Probability of re-deploying resources

Please rank the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

• Predicted probability of redeploying labour savings to other areas of care	1	2	3	4	5
• Predicted probability of redeploying capital savings to other areas of care	1	2	3	4	5
• Projection of how savings will be realized over time	1	2	3	4	5
Please list and rank other components in this category that would be helpful for you :					
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5

Comments:

Section Ten: Transparency

Please rank the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

• Clear understanding of all input assumptions	1	2	3	4	5
• Clear understanding of all assumed relationships among variables and resulting outcomes (e.g. disease states and hospitalizations)	1	2	3	4	5
• Make predictive model as interactive as possible	1	2	3	4	5
• Inclusion of multiple scenarios in model	1	2	3	4	5
• Decision-maker access to the model	1	2	3	4	5
• Description of relationship between intermediate and final therapeutic endpoints, i.e. how is the projected long term impact for new agents derived?	1	2	3	4	5
Please list and rank other components in this category that would be helpful for you :					
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5

Comments:

Section Eleven: Uncertainty and Sensitivity analyses

Please rank the importance of the following items in a BIA (1 = not at all important 3 = important 5 = very important)

• Inclusion of sensitivity analyses for parameters where there is uncertainty or lack of agreement	1	2	3	4	5
Please list and rank other components in this category that would be helpful for you :					
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5

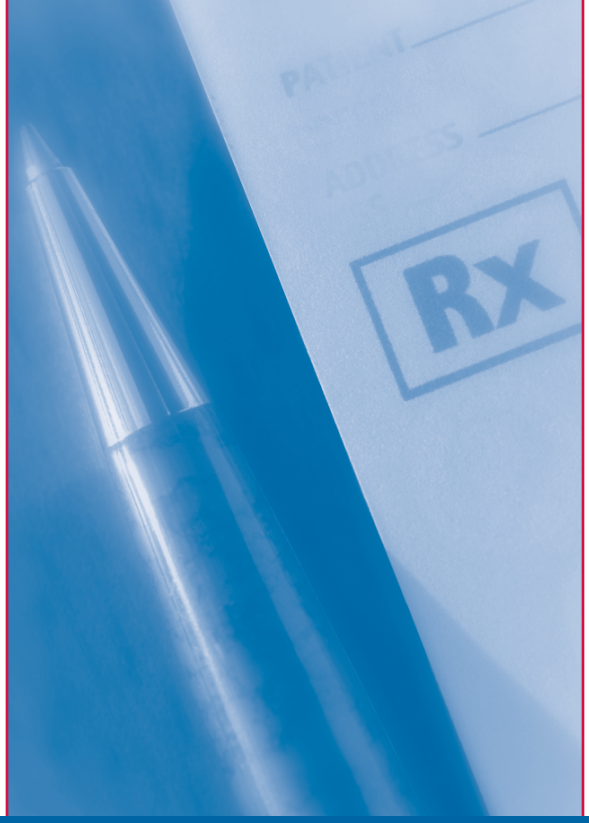
Comments:

Section Twelve: Results Reporting

Please rank the importance of the following data in a BIA (1 = not at all important 3 = important 5 = very important)

• Costs presented in aggregate and disaggregate form	1	2	3	4	5
• Report results in natural and monetary units (natural = hospital beds, nursing time, lab tests, diagnostic equipment)	1	2	3	4	5
• Cost/Claim	1	2	3	4	5
• Total treatment costs	1	2	3	4	5
Please list and rank other components in this category that would be helpful for you :					
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5
• Other:	1	2	3	4	5

Comments:



Appendix 2: Comparison of Templates

Comparative Analysis of Published Templates

Ontario Drug Benefit (ODB)	Alberta Blue Cross and Manitoba Health
Drug/Indication/Dosage information	
This information is not clearly required in the BIA report	This information has to be provided in the first two sections of the BIA
Drug Cost	
No specific requirements on what drug cost should be used in the BIA, i.e. mark-ups, dispensing fee, co-payments, and claim duration etc.	All of them are clearly specified.
Prevalence of Disease	
Both templates have requirements on the information of prevalence of disease, i.e. the number of patients with the disease for which the medication is intended for the total population and for the population covered by the program.	
There are no detailed requirements on how to estimate the prevalence.	There are two requirements: • Data should be Alberta specific. If an extrapolation of national data or data from other provinces was used, it must have been justified. • Prevalence data may not be available in some instances, and it may be extrapolated from claims data. In this case, justification must be provided and necessary assumptions must be clarified.
Market Share	
All templates clearly state that market shares for years one, two and three must be reported.	
Require information on projected market share of both the submitted product and comparator products, and the growth rate of the market shares. The projected market shares are required to be reported as the number of patients and claims.	Only requires information on the projected market share of the submitted product, but it requires clear indication of where and what proportion proposed market is coming from, which means own market growth and substitution from comparator drugs have to be considered. Projected market shares are required to be reported as number of patients and percentage of the total market.

Continued on page 48

Continued from page 47

Ontario Drug Benefit (ODB)	Alberta Blue Cross and Manitoba Health
Budget Impact	
The net impact is required to be calculated as: A) ODB expenditure on submitted product 1. Cost per claim 2. ODB claim per year 3. Total expenditure B) ODB expenditure on comparator products 1. Cost per claim 2. ODB claim per year 3. Total expenditure Net impact= A-B	There is one section that requires the result of total direct drug costs of the submitted drug, and another section requires the result of incremental drug costs (savings). It does not specify how to calculate the required results and only emphasizes the results should be based on the above information (prevalence of disease, projected market shares and drug cost); and all the assumptions and references should be clarified.
Sensitivity analysis	
Requires the result of confidence interval (H/M/L).	One-way and/or muti-way sensitivity analyses are required, and the methods used to calculate the sensitivity analyses, all the assumptions, references must be included.

Appendix 3: Analysis of BIAs submitted to provincial drug plans

Appendix 3 is a summary of approaches taken in the conduct of the BIAs provided to the PMPRB by jurisdictions. We received 35 BIAs: Four from Nova Scotia; five from NIHB; ten from Saskatchewan; seven from New Brunswick, five from Newfoundland and Labrador; one from Manitoba, and three from British Columbia.[8]

The sample BIAs that we received were summarized against the following attributes:

Time Horizon:
The time horizon for a BIA should ideally be until the proposed drug is predicted to have achieved a peak or stable market share. Most jurisdictions ask manufacturers to forecast impact for three years.

Among 35 BIAs, 30 estimated the annual budget impact for the subsequent 3 years after the new drug is listed on the provincial formulary. Three studies set the time horizon at 4 years. Two studies set the time horizon at 5 years.

Population Affected:
Population affected for purposes of a BIA is usually defined based on approved indications and population that are eligible for reimbursement through the program for which BIA is prepared. For decision-making purposes it is also useful to know about potential off-label use of the new drug.

Total number of patients could be estimated based on the number of beneficiaries of a drug plan, prevalence and incidence rates of a disease and pertinent literature.

We observed two methods of estimating population affected. Eleven BIAs were population based and the remaining BIAs were claim based. The claim based studies used the historical trend of claims made to drug plans to forecast the numbers of claims in the future. The changes of the size of affected population were implicitly reflected by the number of claims. Most BIAs clearly identified population affected.

8. Drug and manufacturer names have not been identified in order to maintain confidentiality of the BIAs shared with the PMPRB by jurisdictions.

Data Source:
The data used can be divided into 5 categories: prescription-level data, claim-level data, market data, prevalence and incidence rate data, and population data. The choice of data depended on the estimation method used. None of the BIAs used clinical trial data. Data on the influence of new therapy on mortality, progression of disease, side effects and so on are expected to come from clinical trial data.

Data sources included provincial drug plans, Statistics Canada, manufacturer's internal databases, and existing medical literature.

Cost per prescription/claim/patient:
All BIAs reported results in monetary units. The assumptions on costs per claim/prescription/patient are varied across studies and even drugs in a single study. There are inconsistencies in considerations of wholesale/pharmacy mark-ups, co-payments and dispensing fees. Choices across BIAs included ex-factory price, ex-factory price plus wholesale/pharmacy mark-up, retail price, list price, price adjusted by co-payment, etc. In some of the BIAs the cost per claim of comparator drugs and the proposed drug was not comparable. For example, in one BIA, cost per claim for the comparator drugs included mark-ups, co-payments, and dispensing-fees; for the proposed drug, it is not clear whether cost per claim is ex-factory price or whether it includes wholesale/pharmacy mark-ups.

Market share (of total) and sources of business (from comparators):
The estimations of market share and sources of business for the new drugs can be divided into 5 categories:

a) Estimation based on the experience of the same product that was launched at an earlier date in other provinces or United States.
b) Estimation based on the historical trend of a similar product.
c) Arbitrary assumptions.
d) Expert, physician opinions.
e) Estimation provided by some other organizations but no details given.

Market growth: Market growth in this context refers to the increase of the total annual sale of the drugs for this indication. Only one BIA assumed that there is no market growth in the study period. All the other BIAs considered market growth. Among them, two BIAs took into account further market expansion led by the new drug while the others assumed that there is no such market expansion.

Methods:
While there are considerable differences in details, the estimation methods of budget impact can be generally divided into 2 categories: Population-based studies and claim-based studies. Which method was adopted mainly depended on data availability.

Population-based method:

1) Forecast the population that is eligible to the relevant drug plan in each year of the study period.
2) Total number of claims in each year = Population x Prevalence rate x Percentage of patients who seek treatment x Percentage of patients who are eligible to the drugs in this category x number of claims per patient
3) Total cost **without** the new drug = Total number of claims x cost per claim
4) Total cost **with** the new drug = Total number of claims x market share of the new drug x cost per claim for the new drug + Number of claims for other drug x cost per claim for other drug
5) Budget impact = Total cost **with** the new drug – Total cost **without** the new drug

Claim-based method:

1) Forecast the number of claims in the future based on the historical trend of the number of claims. In two BIAs linear regression was employed. One BIA used a discrete choice model. All the other BIAs didn't give details on the projection methods.
2) Total cost **without** the new drug = Total number of claims x cost per claim
3) Total cost **with** the new drug = Total number of claims x market share of the new drug x cost per claim for the new drug + Number of claims for other drugs x cost per claim for other drugs
4) Budget impact = Total cost **with** the new drug – Total cost **without** the new drug

None of the BIAs adequately justified input assumptions.

Sensitivity Analysis:

Sensitivity analysis is used to test the impact of uncertain factors on final results. Literature identifies two major sources of uncertainty: uncertainty about the true numerical values of the parameters used as inputs and model uncertainty: model structure uncertainty and modeling process uncertainty. It is believed that given the level of uncertainty involved in most input parameters, conducting a sensitivity analysis is one of the most crucial steps in a BIA. Nine BIAs did not conduct sensitivity analyses; four BIAs conducted multi-way sensitivity analysis and the rest conducted one-way sensitivity analyses. The variables most commonly examined included affected population size, market shares, market growth, probability of efficacy, and price variations. None of the BIAs conducted sensitivity analysis on model structure or functional forms used.

Potential Impact of expected future events:

Two BIAs considered expected future events. One of the BIA, considered the possible introduction of a generic version of one of the comparator drugs; price of the generic version was used to reflect the true future budget impact. The other BIA explored off-label use in a sensitivity analysis.

Population Sub-groups Analysis:

Population sub-groups are often identified to determine groups that would benefit the most from a new therapy and therefore allow the decision-maker to prioritize or "ration" availability of treatment accordingly. Two BIAs considered population sub-groups.

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