Principles of Good Practice for Budget Impact Analysis: Report of the ISPOR Task Force on Good Research Practices— Budget Impact Analysis

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ABSTRACT

Objectives: There is growing recognition that a comprehensive economic assessment of a new health-care intervention at the time of launch requires both a cost-effectiveness analysis (CEA) and a budget impact analysis (BIA). National regulatory agencies such as the National Institute for Health and Clinical Excellence in England and Wales and the Pharmaceutical Benefits Advisory Committee in Australia, as well as managed care organizations in the United States, now require that companies submit estimates of both the cost-effectiveness and the likely impact of the new health-care interventions on national, regional, or local health plan budgets. Although standard methods for performing and presenting the results of CEAs are well accepted, the same progress has not been made for BIAs. The objective of this report is to present guidance on methodologies for those undertaking such analyses or for those reviewing the results of such analyses.

Methods: The Task Force was appointed with the advice and consent of the Board of Directors of ISPOR. Members were experienced developers or users of budget impact models, worked in academia, industry, and as advisors to governments, and came from several countries in North America, Oceana, Asia, and Europe. The Task Force met to develop core assumptions and an outline before preparing a draft report. They solicited comments on the outline and two drafts from a core group of external reviewers and more broadly from the membership of ISPOR at two ISPOR meetings and via the ISPOR web site.

Results: The Task Force recommends that the budget impact of a new health technology should consider the perspective of the specific health-care decision-maker. As such, the BIA should be performed using data that reflect, for a specific health condition, the size and characteristics of the population, the current and new treatment mix, the efficacy and safety of the new and current treatments, and the resource use and costs for the treatments and symptoms as would apply to the population of interest. The Task Force recommends that budget impact analyses be generated as a series of scenario analyses in the same manner that sensitivity analyses would be provided for CEAs. In particular, the input values for the calculation and the specific cost outcomes presented (a scenario) should be specific to a particular decision-maker's population and information needs. Sensitivity analysis should also be in the form of alternative scenarios chosen from the perspective of the decision-maker. The primary data sources for estimating the budget impact should be published clinical trial estimates and comparator studies for efficacy and safety of current and new technologies as well as, where possible, the decision-maker's own population for the other parameter estimates. Suggested default data sources also are recommended. These include the use of published data, well-recognized local or national statistical information and in special circumstances, expert opinion. Finally, the Task Force recommends that the analyst use the simplest design that will generate credible and transparent estimates. If a health condition model is needed for the BIA, it should reflect health outcomes and their related costs in the total affected population for each year after the new intervention is introduced into clinical practice. The model should be consistent with that used for the CEA with regard to clinical and economic assumptions.

Conclusions: The BIA is important, along with the CEA, as part of a comprehensive economic evaluation of a new health technology. We propose a framework for creating budget impact models, guidance about the acquisition and use of data to make budget projections and a common reporting format that will promote standardization and transparency. Adherence to these proposed good research practice principles would not necessarily supersede jurisdiction-specific budget impact guidelines, but may support and enhance local

recommendations or serve as a starting point for payers wishing to promulgate methodology guidelines.

Introduction

Definition and Intended Use

Budget impact analysis (BIA) is an essential part of a comprehensive economic assessment of a health-care technology and is increasingly required, along with cost-effectiveness analysis (CEA), before formulary approval or reimbursement. The purpose of a BIA is to estimate the financial consequences of adoption and diffusion of a new health-care intervention within a specific health-care setting or system context given inevitable resource constraints. In particular, a BIA predicts how a change in the mix of drugs and other therapies used to treat a particular health condition will impact the trajectory of spending on that condition (see Fig. 1). It can be used for budget planning, forecasting and for computing the impact of health technology changes on premiums in health insurance schemes.

Users of BIA include those who manage and plan for health-care budgets such as administrators of national or regional health-care programs, administrators of private insurance plans, administrators of health-care delivery organizations, and employers who pay for employee health benefits. Each has a need for clearly presented information on the financial impact of alternative health-care interventions, yet each has different and specific evidentiary requirements for data, methods, and reporting.

Budget impact analysis should be viewed as complementary to CEA, not as a variant or replacement.

Whereas, CEA evaluates the costs and outcomes of alternative technologies over a specified time horizon to estimate their economic efficiency, BIA addresses the financial stream of consequences related to the uptake and diffusion of technologies to assess their affordability. Admittedly, both CEA and BIA share many of the same data elements and methodological requirements, but there are important differences in how these data and methods are incorporated into the models because of their different intended use. There may be circumstances where the CEA indicates an efficient technology while the BIA results indicate that it may not be affordable. In such instances, there is, unfortunately, no current scientific guidance on how to resolve this dilemma.

History of BIA

Mauskopf et al. published an analytic framework for budget impact modeling in 1998 [1]. Others have struggled with the need to include budget impact as part of comprehensive economic evaluation [2–6]. Since the 1990s, several regions in the world including Australia, North America (Canada, United States), Europe (England and Wales, Belgium, France, Hungary, Italy, Poland) and the Middle East (Israel), have included a request for BIA alongside the CEA, when submitting evidence to support national or local formulary approval or reimbursement. Other countries have typically performed their own BI analysis (The Netherlands) rather than requesting the BIA from
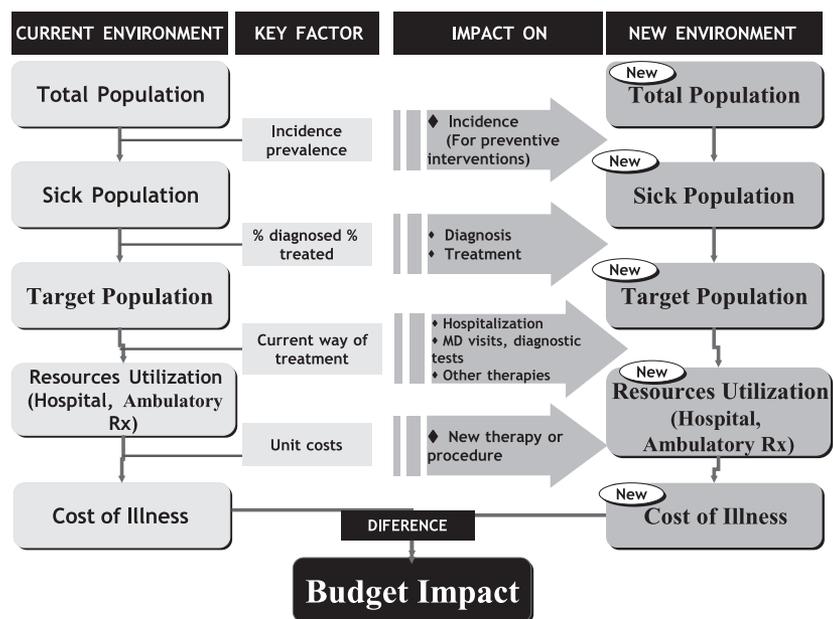

Figure I Budget impact schematic. Adapted from Brosa et al. [39].

the manufacturer, although voluntary submission is permitted. Country-specific guidelines for constructing BIAs are also available [7–16]. These guidelines are variable in terms of defining what constitutes a BIA and most of them provide only limited details on the important factors in a BIA. An exception are the Polish guidelines [15], which provide precise recommendations on perspective, time horizon, reliability of data sources, reporting of results, rates of adoption of new therapies, probability of redeploying resources, inclusion of off-label use, and sensitivity analysis.

Despite the increased demand for BIA, a recent literature review indicates that the number of studies published in peer-reviewed journals is limited [17]. Some of these publications present cost studies that focus on the annual, 2- to 3-year or lifetime costs for a specific cohort of people or a representative individual being started on competing treatments [18–22]. A more limited number of published studies attempt to estimate explicitly the financial and health-care service impact of a new technology for a well-defined national or health plan population [23–36]. There is ongoing debate as to whether BIAs should be publicly available for review and, if so, what parts should be published and/or made available for review upon request.

Task Force Process

The cochairs of the ISPOR Task Force on Good Research Practices—Budget Impact Analysis, Josephine A. Mauskopf and Sean D. Sullivan, were appointed in 2005 by the ISPOR Board of Directors. The members of the Task Force were invited by the cochairs to participate, with advice and consent from the ISPOR Board of Directors. Individuals were chosen who were experienced as developers or users of budgetary impact models, who were recognized as scientific leaders in the field, who worked in academia, industry, and as advisors to governments, and who came from several countries. This document reflects the authors' own experiences developing budget impact models and select publications, but is not intended as a comprehensive review of the literature.

A reference group of ISPOR members from whom comments would be sought also was identified. The Task Force held its first meeting at the ISPOR 10th Annual International Meeting in Washington DC in 2005 and held open Forums at the ISPOR 8th Annual European Congress in Florence in 2005 and at the ISPOR 11th Annual International Meeting in Philadelphia in 2006.

The Task Force reviewed other ISPOR guidance documents that were developed to inform good scientific conduct [37,38] and National Guidelines for BIAs [7–16]. The Task Force held teleconferences and used electronic mail to exchange outlines and ideas during the subsequent months. Sections of the report were prepared by Task Force members and a draft of the complete report was then prepared by the cochairs, and circulated to the Task Force members for review. A face-to-face meeting of the Task Force was held to discuss the draft and make revisions. This draft report was then sent to a group of primary reviewers chosen to represent a broad range of perspectives. The reviewers are identified in the Acknowledgments section of the report. Following this review, a new draft was prepared by the Task Force members and made accessible for broader review by all ISPOR members. This final report reflects the input from all of these sources of comment.

Purposes of the Document

The purposes of this document are: 1) to develop a coherent set of guidelines for those developing or reviewing budget impact analyses; and 2) to develop a format for presenting the results of budget impact analyses that is useful for decision-makers.

The intended audience is research analysts who perform budget impact analyses for health-care decision-makers as well as health-care decision-makers who are responsible for local or national budgets. Others who may find this document useful include members of the press, patient advocacy groups, health-care professionals, drug and other technology manufacturers, and those developing guidelines for their settings.

The panel recognizes that the methods for performing and reporting budget impact analyses continue to develop. This report highlights areas of consensus as well as areas where continued methodological development is needed. The guidance is divided into three main sections: 1) analytic framework; 2) inputs and data sources; and 3) reporting format.

Recommendations for Analytic Framework

For BIA, a description of the health condition, its treatment and outcomes, is the essential component of the analytic framework. The purpose of a BIA is not to produce exact estimates of the budget consequences of an intervention, but to provide a valid computing framework (a "model") that allows users to understand the relation between the characteristics of their setting and the possible budget consequences of a new health technology (or a change in usage of current health technologies). The BIA is a means of synthesizing the available knowledge at a particular point in time for a particular decision-maker to provide a range of predictions specific to that decision-maker's information needs based on realistic estimates of the input parameter values. Thus, the outcomes of the BIA should reflect scenarios that consist of a set of specific assumptions and data inputs of interest to the decision-maker rather than a scientifically chosen "base" or "reference" case based on assumptions and inputs intended to be generally applicable.

This section presents the Task Force recommendations for the key elements of the analytic framework for BIA. It addresses the overall design, the perspective, the scenarios to be compared, the population, time horizon, costing, sensitivity analysis, discounting, and validation.

Design

Proper design of the analytic framework is a crucial step in BIA. The design must take into account the current understanding of the nature of the health condition and the evidence regarding the current and new technologies. There are several dimensions that must be considered: acuteness of the health condition, whether it is self-limiting, and the type of intervention (preventive, curative, palliative, one-time, ongoing, periodic). These dimensions will affect the degree to which time-dependence is important in the design, how the size of the population is estimated, the unit of analysis (episode vs. patient, for example), how the intervention uptake is addressed, and the choice of computational framework.

These guidelines cannot address the details of design of the analytic framework, but rather highlight the key aspects to consider. It is important that whatever choices are made, they be clear, justified, and with a view to the simplest design that will meet the needs of the analysis.

Whether or not a health condition model is needed depends on the type of health condition and interventions at issue. For a chronic health condition, where time dependency tends to be a major concern, a health condition model is likely to be needed. The model should be constructed so that it is consistent both with a coherent theory of the natural history of the health condition and with available evidence regarding causal linkages between variables. Techniques currently used, such as Markov models, might be appropriate, but newer techniques such as discrete event simulation, agent-based simulation, and differential equations models may be considered if they are likely to be accepted by the decision-maker. It is important that researchers be alert to advances in modeling methods as well as to methodology requirements of payers rather than commit them to a given technique exclusively. For acute, self-limiting health conditions where the episode is the unit of analysis, simpler techniques using deterministic calculations may be used.

All of these methods are supported by a variety of software which is continually evolving. The software chosen and the resulting model should be accessible to the users in the sense that it should allow them to review all the model calculation formulae and to change the assumptions and other inputs interactively; indeed, even the design of the model may result from collaboration with the intended users.

Perspective. Budget impact analyses are primarily intended to inform health-care decision-makers, especially those who are responsible for national, regional, or local health-care budgets. Therefore, the recommended perspective is that of the budget holder. Thus, unlike a CEA, where the recommended perspective is that of society, which includes all cost implications of an intervention, a BIA needs to be flexible enough to generate estimates that include various combinations of health care, social service and other costs, depending on the audience.

The drawing of budget boundaries is a highly local exercise. In particular, some budgets may have a very narrow focus. For example, in one location the pharmacy budget holder will only be concerned with the expenses for drugs but in another, this may be subsumed within a total hospital budget. Thus, the perspective of a given budget holder may cover very different elements according to location. Whereas it is mandatory for the analyst to address the needs of the selected budget holders, it is also desirable for the analytic framework to be able to encompass broader (or even narrower) budgetary envelopes. In this way, the analysis will not only be able to show the decision-maker what they need to see, but also can extend beyond that to provide a more comprehensive view of the fuller economic implications of the intervention.

Scenarios to be compared. Budget impact analyses generally compare scenarios defined by a set of interventions rather than specific individual technologies. The reference scenario should be the current mix of interventions for the chosen population and subgroups. The current mix may include no intervention as well as interventions that might or might not be replaced by the new intervention. It may also include off-label use. Introduction of a new technology sets in motion various marketplace dynamics, including product substitution and possibly market expansion. These need to be modeled explicitly with realistic and justifiable assumptions before the comparisons among scenarios can be made. Thus, the analysis should consider how the current mix of interventions is likely to change when the new intervention is made available. For example, the new intervention might be added to all existing interventions or it might replace all of the current interventions or only those in a particular drug class. These constitute the new scenarios.

The BIA should be transparent regarding the assumptions made about the current mix of interventions and the changes expected as the new intervention is added to the mix. The budget impact model should be designed to allow alternative assumptions regarding the scenarios to be compared.

Population. The population to be included in a BIA should be all patients who might be given the new

intervention in the time horizon of interest. Specifying who is included in this population is not straightforward. It depends, of course, on the approved indication, but it also reflects local intended restrictions on use (and reimbursement), possible beyond-restriction use, induced demand (i.e., the proportion of previously untreated patients who now seek treatment because of improved outcomes, greater convenience, or fewer side effects), and the extent to which practitioners adopt the technology or change patterns of use of existing ones. The budget impact model must be designed to allow for examination of the effect of alternative assumptions about the nature and size of the treated population as well changes in its nature and size over time. The Task Force did not recommend inclusion of off-label use of the new technology in these scenarios since generally accepted methods for doing this are not yet available.

Typically, these populations are open in the sense that individuals enter or leave the population depending on whether they currently meet the analyst's criteria for inclusion (e.g., by developing the indication, meeting the intended restrictions, no longer having symptoms, etc.). This is in contrast with CEA where populations are closed (i.e., a cohort of patients is defined at the start and all remain members throughout the analysis). For example, if one of the criteria defining the population is a moderate severity of illness, then patients with mild disease are not part of the population but may enter when the disease progresses; similarly, patients who are initially in the population with moderate disease may exit as the illness advances to a severe stage.

Subgroups. The analytic framework should allow for subgroups of the population to be considered so that budget impact information can be made specific to these segments. Such aspects as disease severity or stage, comorbidities, age, sex, and other characteristics that might affect access to the new intervention, or its impact on the budget, might be taken into account. This may also inform decisions regarding use of the new technology as a "first line" intervention or reserving for use in patients failing other alternatives. The choice of subgroups must be founded on available clinical and other evidence from epidemiological studies, local knowledge, and so on.

Time horizon. Budget impact analyses should be presented for the time horizons of most relevance to the budget holder. They should accord with the budgeting process of the health system of interest, which is usually annual. The framework should allow, however, for calculating shorter and longer time horizons to provide more complete information of the budgetary consequences. A particularly useful extension of the time horizon for a chronic health condition is to reflect the impact that might be expected when a steady state would be achieved if no further treatment changes are assumed. This will vary with the condition and with the impact of the new intervention, but will generally be longer than the current budget period because of costs and benefits that accrue over time. Although time horizons that go beyond a few years are subject to considerable assumptions, they may in exceptional cases be required to cover the main implications of the health condition (e.g., some vaccinations). In any case, results should be available disaggregated over time in periods appropriate to the budget holder (e.g., quarterly, annual, etc.). Hence, to be most useful, the output must be the period by period level of expenses and savings rather than a single "net present value."

Costing

The steps in costing are identifying the resource use that may change, estimating the amount of change, and valuation of these changes. In a BIA, identification must be done according to the perspective and interest of the budget holder (see above). Moreover, the resource use considered should be that which is relevant to the health condition and intervention of interest over the chosen time horizon. The Task Force members did not reach agreement on whether or not future costs should be included for other health conditions that might be incurred when the new intervention results in additional survival. On this point, the Task Force proposes that the analyst should use her/his best judgment, given payer requirements and perspectives, when including or excluding future unrelated costs.

In general, the resource use profile should reflect the actual usage and the way the budget holder values these resources. Thus, the valuation of these resources refers to the expenditures expected to accrue (in the short-run variable costs only and in the long-run both fixed and variable costs) rather than the opportunity costs per se. It is the transaction prices that are relevant, including any rebates or other modifiers that may apply. For example, in some countries, readmissions within a certain period will not generate another payment and in other jurisdictions, the physician's fee depends on the number of times the patient is seen within a period.

In some cases, the intervention alters resource use and, thus, the capacity of the system, but this may have no direct monetary consequence for the budget holder because the system will not adjust financially within the time horizon (e.g., personnel may not be redeployed or let go). It may still be desirable to describe this impact on health services because it has implications for planning health system organization.

The impact on productivity and other items outside the health-care system costs should not routinely be included in a BIA as these are not generally relevant to

the budget holder. One exception may be when budget impact analyses are intended to inform the decision-making of private health insurers or employers. Such organizations may have a vested interest in maintaining a healthy and productive workforce and, thus, they may be able to offset productivity gains against increased health-care costs. Another exception may be health-care systems relying on tax payments where lost production due to morbidity could have important implications for the payment of health.

Sensitivity Analysis

There is considerable uncertainty in a BIA. Therefore, a single "best estimate" is not a sufficient outcome. Instead, the analyst should compute a range of results that reflect the plausible range of circumstances the budget holder will face. Indeed, it might be argued that the analytic framework itself is the most important product of a BIA rather than any particular set of results. It is useful to consider both a most optimistic and most pessimistic scenario. Having said this, the ranges to be presented must be based on realistic scenarios regarding the inputs and assumptions—a task that should be done collaboratively with the decision-makers because they are best placed to make many of the key assumptions and to supply data for the ranges of input parameter values.

Various forms of sensitivity analysis (univariate, multivariate, probabilistic, etc.) may be carried out. Their usefulness depends on the amount and quality of available data and the needs of the decision-maker. For example, there is little point to an extensive probabilistic sensitivity analysis when little is known about the degree of variability and the extent of correlation among parameters.

Discounting

As the BIA presents financial streams over time, it is not necessary to discount the costs. The computational framework should be constructed so that the decision-maker can readily discount these results according to local practice back to a decision time point if they wish to do so.

Validation

Like all models, those used for BIA must be valid enough to provide useful information to the decision-maker. The steps to be followed in validation are conceptually identical to those already identified in the ISPOR Modeling Studies Task Force Report and are therefore not repeated here [37].

Recommendations for Inputs and Data Sources

There are six key elements requiring inputs for the modeling framework of a BIA:

- Size and characteristics of affected population;
- Current intervention mix without the new intervention;
- Costs of current intervention mix;
- New intervention mix with the new intervention;
- Cost of the new intervention mix; and
- Use and cost of other health condition- and treatment-related health-care services.

These six elements can be combined to calculate the budget impact of changing the treatment mix. The Task Force recommends possible data sources for deriving the inputs for each of these elements. Apart from efficacy and safety which are assumed to be generalizable aspects of the interventions, the inputs are local. In many jurisdictions, the required data may not exist or may be difficult to obtain. Nevertheless, analyses should be as evidence-based as possible, with expert opinion only used where alternative sources of data are not readily available. If expert opinion is used, care should be taken to frame the questions and choose the experts in ways that generate reliable and generalizable information. For example, the experts should be asked for responses to questions that they know the answer to (e.g., how often do you schedule follow-up visits for a certain type of patient). No matter what the data source, the BIA should include measures of the range of possible input parameter values.

Size and Characteristics of the Population

The estimated sizes of the population and of the relevant subgroups over time are critical for a determination of the budget impact. The ideal way to obtain this estimate would be from the epidemiological data in the decision-maker's own population before and after the introduction of the new technology. As these data are not usually readily available even for the current technologies, various alternative methods can be used to provide default estimates for a budget impact model.

One approach is to employ epidemiological data from nationally representative populations, adapted to the age, sex, and racial mix of the decision-maker's overall population. This generally involves the application of successively more restrictive inclusion criteria to the decision-maker's overall population. This process requires rates such as the prevalence of the condition, the proportion of patients with a particular severity or usage pattern, and other relevant features for the health condition and technologies being examined. In addition, change in prevalence over the time horizon of the model because of new incident cases and people leaving the population through death or other changes in disease progression must be applied over time to ensure that the size of the population continues to reflect the prevalence with the current and new technologies. This approach is relevant when people are the unit of analysis. For some conditions,

however, it is an episode of illness that is the unit of analysis (e.g., a migraine attack), and then it is the frequency of episodes in the population that must be estimated with the current and new technologies.

Another approach is to obtain directly from providers their estimates of the number of people in their setting who would be part of the relevant population based on their current and anticipated new treatment patterns and aggregating this up to the budget holder's level.

Regardless of the method used, it is important for BIA to estimate not only the starting size of the population (or number of episodes) but also the way these are likely to evolve over time with and without the new technology. Hence, for the typically used open population, estimates of the inflows and outflows must be made.

Given the difficulties in obtaining data to provide accurate estimates of the population size, analysts should consider multiple sources including national statistics, published and unpublished epidemiological data in the relevant, or similar, settings; registries; naturalistic studies carried out for other purposes; claims data; and even expert opinion. The calculations used to derive the population estimate should be presented in disaggregated format so that a decision-maker could adjust the calculations to reflect their population.

Current Technology Mix

For each population subgroup, it is necessary to identify the interventions used currently and estimate the proportion of patients using them, or proportion of episodes in which they are used. Technologies may include no active treatment as well as drugs, devices, surgical or other modes of treatment. Some people may receive more than one type of treatment which should be recorded separately in the current technology mix table. Table 1 gives an example of what these input parameters might look like. Although labeled "current," this technology mix may also evolve over time even in the absence of the new technology and this must be taken into account in budget impact calculations.

Once again, the best data source for the current technology mix for the different population subgroups

is the decision-maker's own database. If these data are not available, then published information on current treatment patterns, such as the results of primary or secondary data studies or medical text books or review articles, can be used. In addition to these data sources, market research data or expert opinion on current and evolving treatment patterns may be used.

Cost of Current Intervention Mix

The cost of the current technology mix involves multiplying the decision-maker's valuation of the technology by the number of people who receive each one in each population subgroup. These costs should include the acquisition of the product, administration or implantation or other procedure costs as well as any monitoring over the relevant time horizon. Costs of managing any side effects should also be included in the cost of current technology mix as a separate line item.

The BIA should address the impact of compliance and persistence with therapy on the cost of treatments. This must take into account whether the payer bears the cost anyway (e.g., even if poorly compliant, the patient still picks up the prescription). The assumptions regarding compliance rates and persistence with treatment should be based on the best available evidence, which may come from database studies or specific date collection or expert opinion. The relative compliance and persistence on therapy should be reported at various time intervals. If patients do not fill all the recommended prescriptions, then the cost of treatment should be reduced. In addition, the cost to the decision-maker should take into account drug discounts and patient deductibles and copays.

New Technology Mix

The new technology mix depends on the rate of uptake of a new technology as well as the extent to which a new technology replaces current technologies or is added to them. The rate of uptake is likely to change over time as physicians and patients become familiar with a new technology. There are several ways to estimate the new technology mix. One way is to use the producer's estimates of market share over the first few years after launch if these data are made available. An assumption must then be made as to whether the new intervention will be given in addition to current technologies or whether it will substitute for some or all of the current technologies. For example, a new technology might reduce the use of a subset of the currently used technologies equi-proportionately (e.g., all drugs in a particular class) or it might be added to all of the current technologies. The assumptions should be transparent and the model structured so that the budget impact of alternative assumptions about the new technology mix can be calculated. Another way to estimate the new technology mix is to incorporate

Table 1 Current technology mix

Drug name	Percentage	Number
Drug A (combination of drugs B and C)	20.0	5,810
Drugs B and C in separate doses	6.1	1,772
Drug B	10.2	2,963
Drug C	7.5	2,179
Drug D	13.7	3,980
Drugs C and D in separate doses	21.0	6,101
No therapy	21.5	6,246
Total	100	29,050

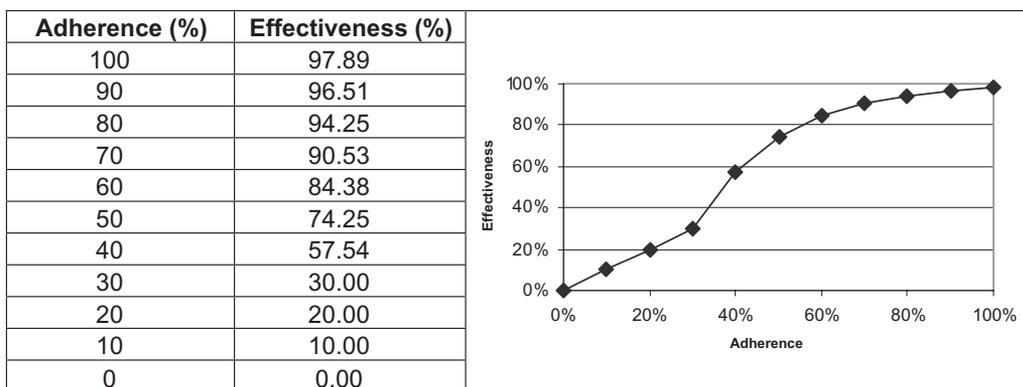| Adherence (%)	Effectiveness (%)
100	97.89
90	96.51
80	94.25
70	90.53
60	84.38
50	74.25
40	57.54
30	30.00
20	20.00
10	10.00
0	0.00

Figure 2 Adherence and effectiveness. Notes: The relationship between effectiveness and adherence may be estimated based on observed data or expert opinion or pharmacokinetic and pharmacodynamic data. The relationship in this figure is based on expert opinion:
Effectiveness Relative to Trial Data = Adherence rate (AR) if AR $\leq$ 30%.
Effectiveness Relative to Trial Data = $1 - \exp[-5 \times (AR - 0.2287)]$ if AR > 30%.

directly in the analytic framework usage rules that account explicitly for the new treatment pathways available, thus explicitly modeling how people switch to the new drug. For example, they may only switch when they have failed on current therapy. Other ways of estimating the new technology mix involve extrapolating previous experience on product diffusion with the same technology in other settings or with similar interventions in the budget holder's setting.

Cost of New Technology Mix

Costing of the new technology mix follows the same process as for the current mix except that for technologies not yet on the market, the price may have to be assumed if it is not yet set. In this case, we recommend that the assumed technology cost be transparent and justified. In addition, any uncertainty in the price should be readily able to be incorporated into alternative scenarios for the sensitivity analyses.

Use and Cost of Other Condition-Related Health-Care Services

Although the health outcomes associated with different technologies are not generally estimated explicitly as part of a BIA, we recommend that they be estimated and added to the BIA through changes in the cost of treating the health condition of interest. Thus, alternative technology mixes are likely to result in changes in the symptoms, duration, or disease progression rates associated with the health condition and, thus, in changes in the use of all other condition-related health-care services. These changes will have an impact on the health plan budget.

In order to compute these changes in health outcomes and the associated changes in costs over the time horizon of the BIA, we recommend that estimation techniques similar to those described in the ISPOR Modeling Studies Task Force Report and the Cost-Effectiveness Analysis alongside Clinical Trials Task Force Report be used but simplified where possible and adapted so that the estimates of the health outcomes are generated from a population perspective and presented for each year that is included in the BIA [37,38]. For an acute or episodic illness, this adaptation is straightforward. For a chronic or progressive illness, this adaptation may require an extension of the cost-effectiveness health condition model to account for the open population and time-dependencies required for a BIA.

The BIA must be transparent about the assumptions made about the impact of noncompliance or reduced compliance on effectiveness and about safety issues associated with underutilization or overutilization of treatment and must allow them to be changed. If there are no published data on the relationship between compliance and health outcomes, then either pharmacokinetic or pharmacodynamic data or expert opinion are possible alternative data sources. Figure 2 presents a hypothetical example of the relationship between adherence and effectiveness that was generated using expert opinion.

Recommendations for Reporting Format

This section presents a recommended reporting format for BIAs. The format presented below should be understood as the preferred ISPOR structure for the reporting of any study regarding BIA. In view of the decision-maker-specific scenario basis that we have recommended to be adopted for BIA, this format gives only general directions for reporting.

Report Introduction

The introduction of the report of a BIA study should contain all the necessary relevant epidemiological, clinical, and economic information.

Epidemiology and treatment. The introduction of a BIA study should present relevant aspects of the prevalence and incidence of the particular disease as well as information on age, sex, and risk factors.

Clinical impact. The clinical information should consist of a brief description of the pathology, including underlying pathophysiological mechanisms, and of the prognosis, disease progression, and existing treatment options, all of which are relevant to the design of the BIA study.

Economic impact. The economic impact information should include any previous related studies on the condition of interest and associated therapies, for example, previous BIA studies in the condition of interest for another technology, cost-of-care studies, and cost-effectiveness studies.

Technology

This section should contain a detailed description of the characteristics of the new technology compared with the current technologies: indication, onset of action, efficacy, side effects, serious adverse events, intermediate outcomes, and adherence. A summary of the clinical trials is given, including information on the design, study population, follow-up period, and clinical outcomes.

Objectives

The objective of the BIA should be clearly stated. This will be tied to the perspective(s).

Study Design and Methods

The report should specify the design of the BIA, which will usually involve a modeling study. The following characteristics of the model should be described.

Patient population. This paragraph should clearly specify the study population. The report should identify and justify differences between the clinical trial populations and the BIA population.

Technology mix. The chosen technology mix with and without the new technology should be discussed and justified. The choice of the technology mix is primarily based on the local treatment patterns and clinical guidelines and this choice should be justified.

Time horizon. The time horizon(s) for the study should be presented and its choice justified. The choice for the study period should be appropriate to the budget holder.

Perspective and target audience. This paragraph should clearly identify the perspective(s) from which the study is performed, the costing that is accomplished and the target audience (i.e., for which decision-making body the study is intended). Ideally, the model should be flexible enough to model the perspective of the budget holder and those of other stakeholders with whom the budget holder must interact. This requires disaggregation into the various cost components and categories of interest to these parties. In all cases, the perspective should be clearly stated and transparent to the budget holder.

Model description. This section should contain a complete description of the structure of the BIA model, including a figure of the model. The description should allow the reader to identify outcomes for all treated patients during the study period, including patients with treatment failure.

Input data. The parameter values assumed for all the clinical data items and all the cost data items for all the scenarios modeled should be presented in the report. The level of detail should be such that the reader could duplicate all the calculations in the model.

Data sources. The sources of model inputs should be described in detail. The strengths, weaknesses, and possible sources of bias, that may be inherent in the data sources used in the analysis, should be described. Selection criteria for studies and databases should be discussed and an indication is given of the direction and magnitude of potential bias in the data sources which were used.

Data collection. The methods and processes for primary data collection (e.g., for a Delphi panel) and data abstraction (e.g., for a database) should be described and explained. The data collection forms which were used in the study should be included in the appendix of the report (e.g., the questionnaire for the Delphi panel, or the abstraction protocol for the database).

Analyses. A description of the methods used to perform budget total and incremental analyses should be provided. The choice of all of the scenarios presented in the results should be documented and justified.

Results

Both total and incremental budget impact should be presented for each year of the time horizon. Both annual resource use and annual costs should be presented. The estimates of resource use should be listed in a table (if possible classified by technology application, technology side effects, and condition related) which shows the change in use for each year of the time horizon. Another table should show the aggregated and disaggregated (e.g., pharmacy, physician

visit, outpatient tests, inpatient care, and home care) costs over time after applying costing information to the resource use. In general, budget impact estimates should be presented as a range of values, based on alternative possible scenarios rather than a single point estimate.

Annual health outcomes for each year of the time horizon do not need to be reported, but may be presented if these results are of interest to the decision-makers. For example, the health outcomes might be of interest to the decision-makers when a large budget impact is accompanied by large health benefits.

The results of the scenarios (sets of assumptions and inputs and outcomes) analyzed should be described. These scenarios may consist of optimistic, pessimistic, and most likely input values determined from the sensitivity analysis of the key variables from the perspective of the decision-maker. We recommend that the results of all sensitivity analyses be presented as a Tornado diagram.

Inclusion of Graphics

Graphical snapshots of the model's structure and data can be useful in summarizing for the user, who may wish to copy them for inclusion in their own internal reporting. Use of the following tools is recommended:

Figure of the model. A graphical representation of the model structure makes it easier for the budget holder to understand what is represented by the outputs. Simple flow diagrams are recommended to be included with the model description.

Table of assumptions. Listing the major assumptions in tabular form can improve the transparency of the model, particularly to the relatively inexperienced user and should be included with the model description.

Tables of inputs and outputs. Similarly, collecting the model inputs and their data sources and outputs in tables provides a useful snapshot for the user and should be included with the text on input data and data sources.

Schematic representation of sensitivity analysis. Analysts should be encouraged to use diagrams (such as Tornado diagrams which show graphically the impact on the budget impact of feasible ranges of each input parameter) as a simple way of capturing the key drivers of the model and presenting them to the user and should be included along with the text on the results of the scenario analyses.

Appendices and References

The enclosure of relevant appendices to reports is encouraged. The appendices may cover the intermediate results (e.g., of individual Delphi panel rounds),

study audit reports and the names and addresses of participating experts and investigators.

Budget Impact Computer Model

Because budget impact models need to be flexible enough to provide budget impact estimates for different health-care decision-makers, it is critical that the software used to perform the model calculations is designed with both default input parameter values based on credible national or local values and with the capability for the user to enter values that represent their own particular situation. The model should be programed so that the user can restore the original default parameters easily.

The model should be programed as easy-to-use spreadsheets. For example, all input parameters would be presented on one input worksheet and outputs displayed in one or more worksheets in a logical manner that summarizes the findings for the user. Graphical output is often useful in the model. Introductory worksheets should be included to describe the structure, assumptions, and use of the model. All sources and assumptions associated with input parameters should be displayed with the parameters themselves and full references should be included on a reference worksheet. The model calculations should be accessible to the user and clearly and comprehensively presented.

In many cases, the budget holder will be interested in modeling from more than one perspective. In such cases, model developers are encouraged to design the user interface so that the user can toggle between the different perspectives easily.

The user should be able to change easily any of the input parameters. Color coding the input cells is a useful way of doing this. Changing the inputs allows the user to test various input scenarios. It may be useful to provide sample scenarios.

Finally, we recommend that the model be programed so that the user can readily perform sensitivity analyses of relevance to their population.

Concluding Statement

Budget impact analysis is important, along with CEA, as part of a comprehensive economic evaluation of a new health technology. Some published examples of budget impact analyses are described in the review by Mauskopf et al. [17]. We propose here a framework for creating budget impact models, guidance about the acquisition and use of data to make budgetary projections and a common reporting format that will promote standardization and transparency. Adherence to these proposed good research practice principles would not necessarily supersede jurisdiction-specific budget impact guidelines, but may support and enhance local recommendations or serve as a starting

point for payers wishing to promulgate methodology guidelines.

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