

# **INTERNATIONAL SOCIETY FOR PHARMACOECONOMICS AND OUTCOMES RESEARCH (ISPOR) METHODS GUIDANCE**

## **A REPORT OF THE ISPOR TASK FORCE II ON BUDGET IMPACT MODELING**

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# Topics

- Introduction to the Task Force
- Task Force Report
  - Outline of Report
  - Key Recommendations
- Reviews and Publication

# Background

- Evidence requirements from payers include mandates for budget impact analyses (BIA)
- With an aging population and a sluggish economy, concern about the budget impact of new drugs is increasing
- Since the first ISPOR BIA Task Force report many publications have appeared reporting the results of BIA using a variety of approaches
- The second ISPOR BIA Task Force has produced an updated methods guidance on the conduct and reporting of budget impact analyses.

# 2007 Budget Impact Analysis Paper

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VALUE IN HEALTH

## 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

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**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

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

# Task Force II Mission

- Develop an updated, coherent set of methodological guidelines\* for those developing or reviewing budget impact analyses (BIA)
- Develop an updated template for presenting the results of budget impact analyses (BIA) that is useful for decision makers

\* Not an instruction manual

# Task Force Members

- Jo Mauskopf, RTI-HS, USA and Sean D. Sullivan, U. Washington, USA (Co-Chairs)
- Maria Swift – ISPOR Staff
- Federico Augustovski, IECS, Argentina
- Jaime Caro, McGill U., Canada
- Karen Lee, CADTH, Canada
- Mark Minchin, NICE, UK
- Ewa Orlewska, Center for Pharmacoeconomics, Poland
- Pete Penna, Formulary Resources, US
- Jose-Manuel Rodriguez Barrios, Stryker, Spain
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# Report Outline

- Abstract
- Task Force Process
- Introduction
  - Purpose
  - Intended Use
  - Context
- Recommendations for Analytic Framework
- Recommendations for Inputs and Data Sources
- Recommendations for Reporting Format
- Recommendations for Budget Impact Computer Program
- Concluding Statement

# Purpose of BIA

- Budget Impact Analysis (BIA): an essential part of a comprehensive economic assessment of a health care technology increasingly required, along with cost-effectiveness analysis (CEA), prior to formulary approval or reimbursement.
- Purpose: To estimate the expected changes in a health care system's expenditures after adoption of a new intervention.

# Intended Audience

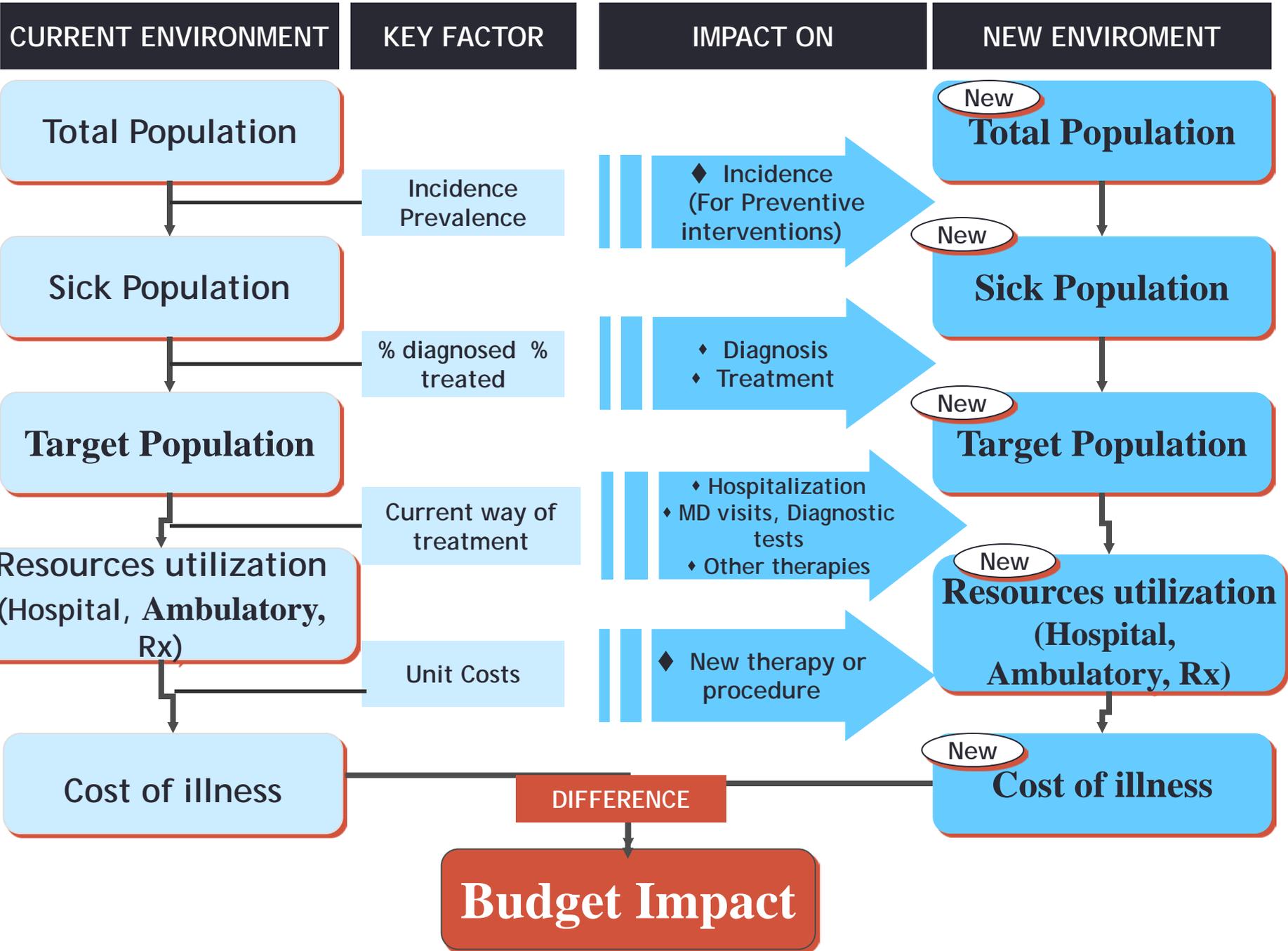
- Health care decision makers who are responsible for local, regional or national budgets
- Research analysts who perform these studies for health care decision makers
- Others:
  - Patient advocacy groups
  - Health care professionals
  - Drug and other technology manufacturers
  - Those developing guidelines for their settings

# Context

- HTA agencies start to request budget impact analyses (National Institute for Health and Care Excellence (NICE), Academy of Managed Care Pharmacy (AMCP), Pharmacy Benefits Advisory Committee (PBAC) (1990's to present))
- BIA perspective (1998)
  - Prevalence (population)
  - Decision maker (no reference case)
- ISPOR Task Force I (2007)
- Increasing number of publications (2007-2013)
- Country-specific guidelines (e.g. Australia, Canada, Poland, Thailand )
- ISPOR Task Force II (2012-2013)

# BIA – Analytic Framework

- A BIA is a means of synthesizing available knowledge at the time of a coverage or formulary listing decision to estimate the likely financial consequences of that decision to the health care system or health care plan
- A BIA provides a valid computing framework that allows users to apply their own input values and view financial estimates pertinent to their setting



# Elements of the Analytic Framework

- Features of the health care system
- Perspective
- Use and cost of new and existing interventions
  - Eligible population
  - Current interventions
  - Uptake of new intervention and market effects
  - Off-label use
  - Cost of current or new intervention mix
- Impact on other costs
  - Condition-related costs
  - Indirect costs
- Time horizon
- Time dependencies and discounting
- Choice of computing framework
- Uncertainty and scenario analysis
- Validation

# BIA - Recommendations for Analytic Framework I

- Features of the health care system to include those that impact the budget
  - E.g. access restrictions, co-payments, practice patterns
- Perspective of the analysis is that of the budget holder
  - May include health care, social service, other costs
  - Flexible design to allow presentation of perspectives of interest
- Population
  - All patients eligible for the new intervention during time horizon of interest
  - Note: the new intervention may change the size of the eligible population and its distribution by disease severity

# BIA - Recommendations for Analytic Framework II

- ***Current interventions***
  - Mix of all currently used for the eligible population including, if appropriate off-label interventions
- ***Uptake of new intervention and effects on current intervention mix***
  - Consider whether replacement of a current intervention, added to a current intervention, or used where previously no intervention
  - Allow user flexibility to enter different assumptions about changes in the intervention mix after introduction of the new intervention
- ***Off-label use of the new intervention***
  - Not included unless specifically requested by the budget holder
- ***Cost of current or new intervention mix***
  - Determined by multiplying the budget holder prices by the proportion of the eligible population using each intervention

# BIA - Recommendations for Analytic Framework III

- ***Condition-related costs***

- Condition related costs should be included if credible data are available and they impact the budget during the relevant time horizon
- Results should be presented both with and without the impacts on condition-related costs

- ***Indirect costs***

- Indirect costs should not be routinely included in a BIA

- ***Time horizon***

- That of relevance to the budget holder between 1 and 5 years

- ***Time dependencies and discounting***

- Include the impacts of inflation and changes in prices (e.g. patent expirations) during the analysis time horizon
- Discounting the budget estimates to a net present value is not relevant for most budget holders

# BIA – Recommendations for Analytic Framework IV

- ***Choice of computing framework***
  - A simple cost calculator using spreadsheet software is the recommended approach (e.g. NICE costing templates)
  - Where changes in eligible population size, disease severity mix or intervention patterns cannot be credibly captured using the cost-calculator approach, a condition-specific cohort or individual simulation model may be used but adapted to account for those entering and leaving the eligible population over time
- ***Uncertainty or scenario analyses***
  - Present one-way sensitivity analyses
  - Present scenario analyses of relevance to the budget holder based on plausible alternative assumptions and/or input values
- ***Validation***
  - Face validity and program verification should always be completed
  - For additional credibility, initial year budget impact estimates can be compared to current observed costs in the health system

# BIA – Recommendations for Inputs and Data Sources I

- ***Size and characteristics of the eligible population***
  - Current size of eligible population
    - Preferred approach from the budget holder's population directly
    - Alternative sources include national or regional data on incidence or prevalence adjusted by the characteristics of the population of interest and by proportion diagnosed and treated
  - Current characteristics of the eligible population
    - Preferred approach from the budget holder's population directly
    - Alternative source for proportion at each level of disease severity from published studies of disease incidence, prevalence or progression
  - Change in size and characteristics of the eligible population
    - Efficacy data from clinical trials or cross sectional data from registries
  - Eligible population for a chronic condition or vaccination program
    - Consider two population cohorts eligible for the new intervention: newly eligible and catch-up subgroup (became eligible before new intervention was available)
    - Size of catch-up subgroup should be obtained from the budget holder or from studies of disease incidence, prevalence or progression

# BIA – Recommendations for Inputs and Data Sources II

- ***Current and new intervention mix***
  - Current intervention mix
    - Preferred approach from the budget holder's population directly
    - Alternative sources include registries, claims databases, local surveys, market research
    - Should allow for current mix to change over the BIA time horizon based on past changes, market research or expert opinion
  - New intervention mix
    - Three approaches to estimate uptake of the new intervention:
      - Data from another jurisdiction where the intervention has been introduced
      - Producers estimates
      - Previous experience of uptake of a similar intervention in budget holders population
    - To determine the impact on use of the current interventions, market research, producer estimates or expert opinion should be used

# BIA – Recommendations for Inputs and Data Sources III

- ***Cost of current and new intervention mix***
  - Cost of current intervention mix
    - Use budget holders acquisition costs after adjustment for any discounts, rebates etc where available
    - If not available use published wholesale acquisition costs, list prices or formulary costs
    - Costs associated with administration and monitoring, where relevant, should also be included
      - resource use estimates from the budget holder or product labels
      - unit costs applied to resource use estimates
    - Costs of managing side effects or complications should also be included
      - rates of adverse events taken from product labels or clinical publications
      - Costs for treatment of adverse events from published sources or derived applying unit costs to resource use estimates derived in consultation with treating physicians
  - Cost of new intervention mix
    - Dosing, administration, monitoring, side effects costs for the new intervention should be derived using the product label and clinical trial data supplemented by clinical expert opinion

# BIA – Recommendations for Inputs and Data Sources IV

- ***Use and cost of other condition-related services***
  - Changes in health outcomes
    - Data from health outcomes studies (e.g. clinical trials) should be used but adapted to a population perspective
  - Changes in use of health care services
    - Local data are preferred
    - Alternative sources are consultation with treating physicians to derive treatment algorithms for different health states supplemented by observational data
  - Unit costs of health care services
    - Opportunity costs for the budget holder are the preferred source
    - An alternative source are cost accounting estimates

# BIA – Recommendations for Inputs and Data Sources V

- ***Ranges and alternative values for uncertainty and scenario analyses***
  - Ranges for Uncertainty analyses
    - Preferred source is the budget holder
    - Alternative sources include ranges from published studies; arbitrary ranges (e.g.  $\pm 20\%$  or  $50\%$ ) are not recommended
  - Scenario analyses
    - Budget-holder specific information should be used to create relevant scenarios based on health plan or system population age and gender distribution and condition incidence and prevalence, as well as health plan or system treatment patterns and drug and other health care service costs
    - If adherence or persistence is included in a scenario analysis:
      - assumptions about its impact on intervention costs should be based on database or prospective studies applicable to the budget holder
      - assumptions about its impact on condition-related costs should be based on published studies, pharmacokinetic or pharmacodynamic data or expert opinion

# BIA – Recommendations for Reporting Format

- ***Use a consistent and standard format***
  - *Report should follow the main headings of the framework, inputs and data sources sections*
- ***Model Framework, Assumptions, and Inputs***
  - Should be presented in sufficient detail to allow for replication by a researcher
- ***Output***
  - Budgetary implications represented by monetary flow (total or per capita) over the analysis time horizon.
  - Components of budget impact to be displayed.
- ***Graphical and tabular display***
  - Present figure of the model structure
  - Present table of model assumptions
  - Present table with all input values
  - Present table with disaggregated outputs
  - Present schematic representation of uncertainty/scenarios
- ***Reporting combined cost-effectiveness and budget impact analyses.***
  - Provide full description of both models following published reporting guidelines

# BIA – Recommendations for Computer Model

- ***Development and use of computer programs for reporting the results of BIA***
  - Create simple spreadsheet program
  - Default input parameter values included with text description of each parameter and reference to data source and any calculations used to derive the input parameter value from the data source
  - User able to change all input parameter values
  - User able to restore defaults
  - Results presented in tabular and graphical format
  - Results presented at different levels of aggregation
  - User able to change the scope of the analysis including the time horizon and the cost components included

# Reviewers and ISPOR Staff

- Primary Review Group:
  - Lieven Annemans, PhD
  - C. Daniel Mullins, PhD
  - Bong-Min Yang, PhD
- Reviewed by ISPOR Membership:
  - 84 Reviewers
- ISPOR Staff: Maria Swift

# 2014 Budget Impact Analysis Paper

## Budget Impact Analysis - Principles of Good Practice: Report of the ISPOR 2012 Budget Impact Analysis Good Practice II Task Force

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