

ACCOUNTING FOR TIME-DEPENDENT HEALTH STATE MEMBERSHIP IN BUDGET IMPACT ANALYSIS – CASE STUDY IN ONCOLOGY

Michal Litkiewicz, MSc; Apoorva Ambavane, MPH; Jon Tosh PhD
Evidera, London, UK

Introduction

- The Task Force by the International Society for Pharmacoeconomics and Outcomes Research (ISPOR)¹ recommends that the design for a budget impact analysis (BIA) should accurately capture the healthcare system, the size of eligible population, the current treatment mix, the uptake of a new treatment, the cost of treatments, and any changes in disease-related costs.²
- For this, the Task Force suggests using a simple cost-calculator approach because of the ease of use for budget holders.
- However, a cohort or patient-level model is suggested in instances where the budget impact of changes in an eligible population, disease progression, treatment patterns, or treatment-related outcomes cannot be captured by such a cost-calculator approach.
- Solid tumours and haematologic indications in oncology have seen the introduction of expensive treatments, including orphan medicines for rare oncological indications. The budget impact of these novel treatments is of interest to multiple stakeholders, especially budget holders and payers.³
- Our hypothesis is that the model design can potentially have a considerably impact on BIA results. However, the methodological literature for BIA is not as developed as it is for other economic evaluation topics.
- We conducted a case study to understand the impact of different model designs on BIA results and consequently, the significance for budget holders.

Objective

- This study objective is to compare BIA results using two different model designs: (1) typical simplified cost-calculator and, (2) complex cohort model approach, for a new hypothetical oncology product.
- The impact of changes in an eligible population and treatment status over time were explored using the two designs.

Methods

- Health economic models for conducting BIA were developed for a novel product for a hypothetical oncology indication using two different model designs (Figure 1).
 - Simple cost-calculator** (Figure 1): A typical simplified structure with the eligible patient cohort entering the model and commencing treatment at the start of each year. The patient cohort remained on treatment for a fixed duration (mean time to treatment discontinuation [TTD] or death).
 - Complex cohort model** (Figure 2): A complex budget impact model (BIM) with incident cohorts entering the model and commencing treatment at the start of each month. The number of patients on treatment was updated with a monthly frequency based on a TTD curve and was calculated for each cohort based on the time elapsed from treatment start.
- Two hypothetical treatments were included: Standard of Care (SoC) and New Treatment (NT). In the current scenario, all patients were treated with SoC. In the revised scenario, 80% of incident patients were treated with NT, with the remaining 20% of patients treated with SoC.
- Budget impact (BI) was calculated as the difference in total costs between the revised and current scenarios over a five-year time horizon.
- Both models shared the same input values (Table 1), including the number of patients starting treatment per year, monthly costs associated with each treatment, and treatment-specific mean TTD.
- For the complex cohort model, the number of eligible patients entered the model linearly in each monthly cycle, and the TTD curves were constructed using the exponential distribution.
- The results obtained with both model types were related to each other by expressing the BI with the simple model as a proportion of the BI with the complex model.

Figure 1. Model Design – Simple Cost Calculator

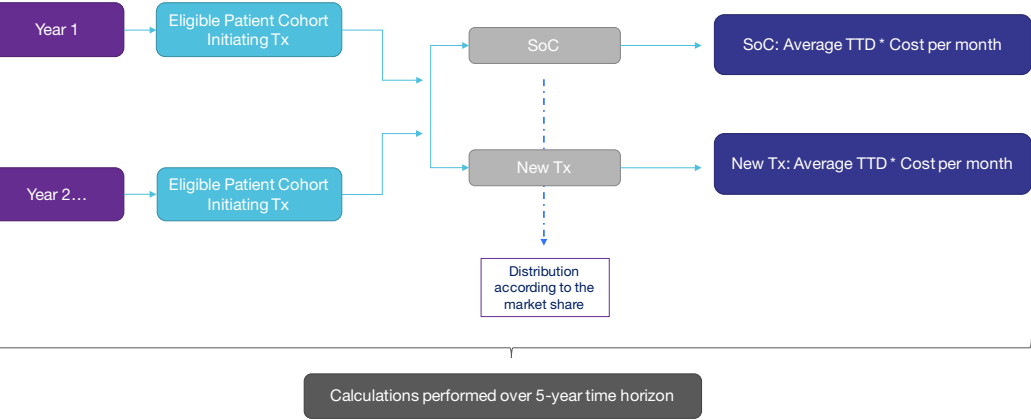


Figure 2. Model Design – Complex Cohort Model

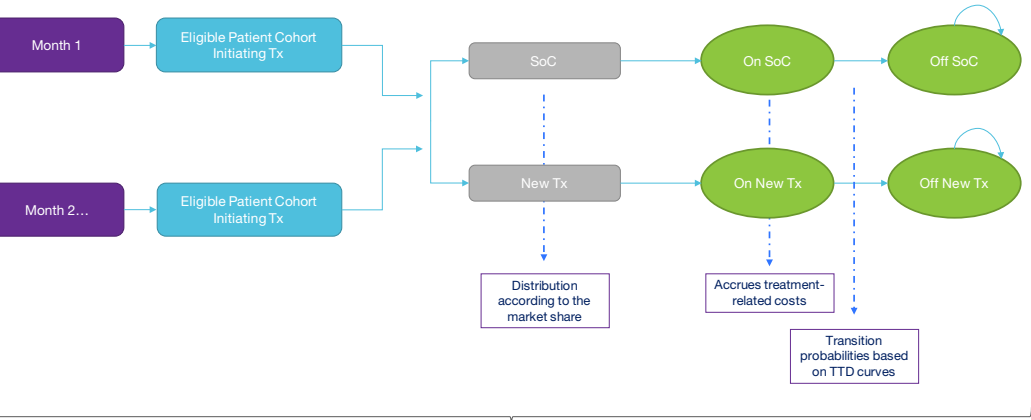


Table 1. Model Parameters

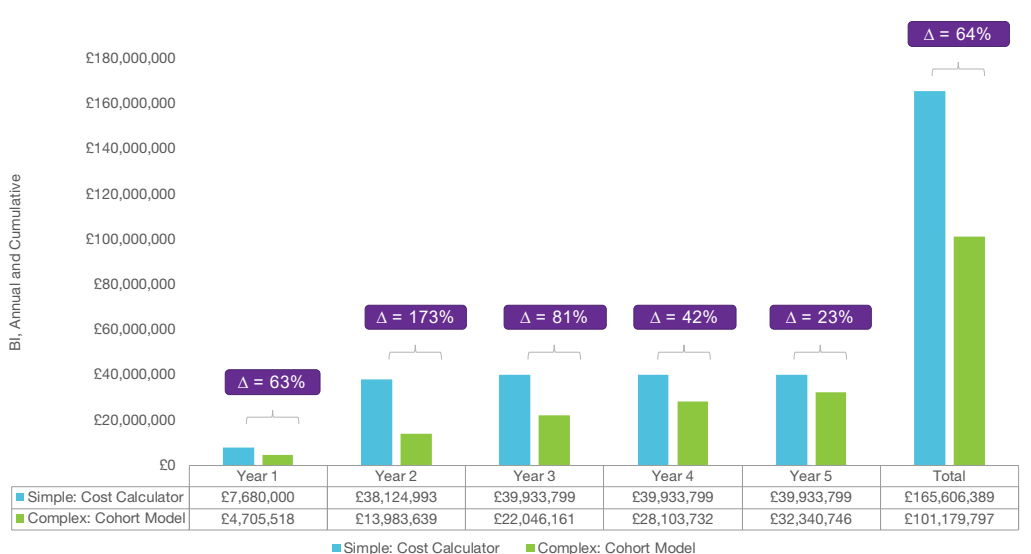
Parameter	SoC	NT
Number of patients starting treatment per year*	500	
Market share in current scenario**	100%	0%
Market share in revised scenario**	80%	20%
Mean TTD†	16 months	25 months
Monthly cost	£7,000	£8,600

* Divided by 12 to obtain the value per month for complex cohort model
** Assumed constant over time
† Assumed to follow exponential distribution for complex cohort model
Abbreviations: NT = New Treatment; SoC = standard of care; TTD = time to treatment discontinuation

Results

- The difference in BI between the two models was significant, with the largest impact observed in the initial years (Year ≤3: 63%–173% and Year >3: 23%–42%) (Figure 3). This resulted in a difference in cumulative budget impact of 64% over five years (Figure 3).

Figure 3. Comparison of Budget Impact – Simple Cost Calculator vs. Complex Cohort Model

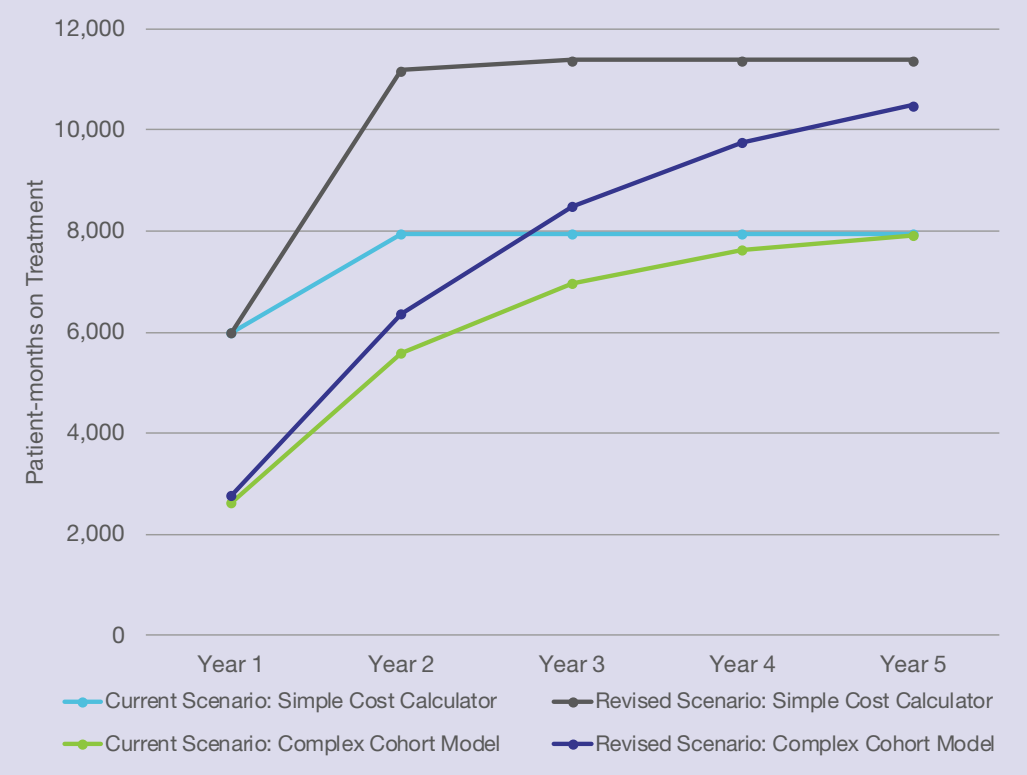


Abbreviation: BI = budget impact

Results (cont'd)

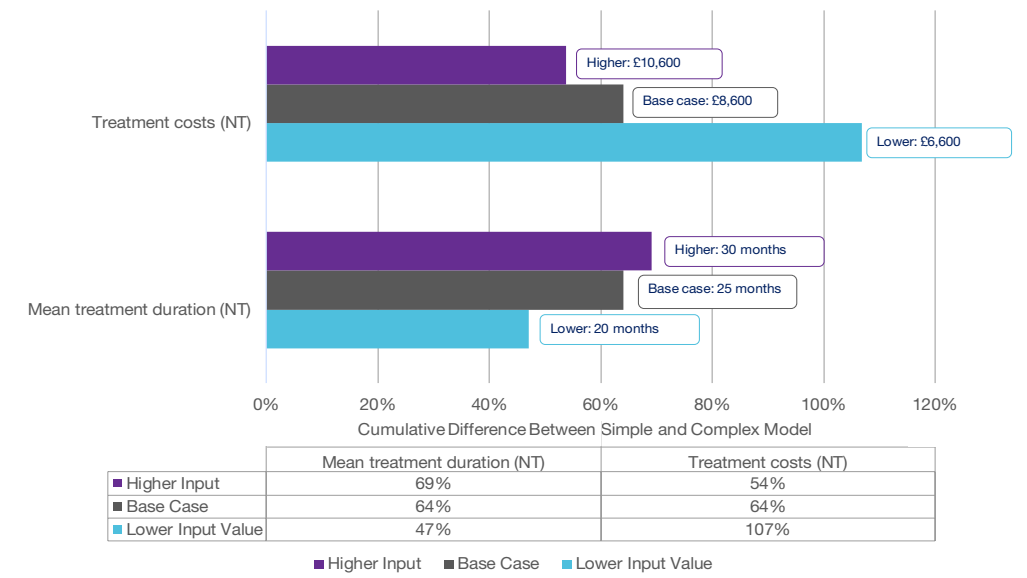
- The key difference in BI results was driven by:
 - Linear incorporation of the eligible patient cohort over a year with the complex approach when compared to the entire cohort starting at the beginning of the year
 - Equilibrium in patient numbers initiating and discontinuing treatment, as per the TTD curves, in the complex model when compared to 100% of patients being assumed to remain on treatment over the treatment duration
- The number of patient-months on treatment over a five-year time horizon was higher with the simple cost-calculator approach compared to the complex cohort model (Figure 4).

Figure 4. Comparison of Patient-Months on Treatment – Simple Cost Calculator vs. Complex Cohort Model



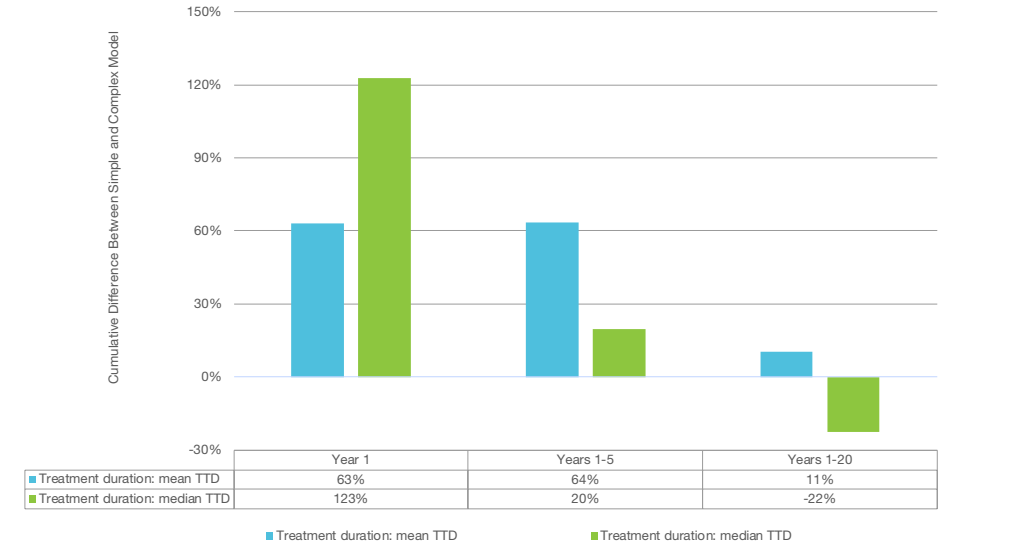
- Lower and higher input values for the number of eligible patients, and mean treatment length, market share, and cost per month for the new treatment were tested.
 - Increasing the mean cost per month for NT resulted in a higher difference in BI between the two models; however, increasing the mean treatment duration for NT resulted in a lower difference in BI.
 - Varying market share for NT and the number of eligible patients did not change the difference in BI between the two approaches.
- Extending the time horizon beyond five years showed the annual BI in both models converged to similar values after approximately 10 years (< 1% difference in year 11); however, the cumulative difference remained above 10% after 20 years. This is because the time for the results to converge is determined by the TTD and therefore how quickly the model cohort reaches a steady state. A shorter TTD means the model achieves steady state more quickly and the BI results stabilise over a shorter time horizon.
- In practice, median duration of treatment is sometimes used as a proxy for mean. Using medians of the exponential distributions instead of means decreased the five-year cumulative difference to 20% (maximum annual difference: 123% in year 1), but the long-term annual BI values no longer converged (Figure 5).

Figure 4. Scenario Analysis Results



Abbreviation: NT = New Treatment

Figure 5. Scenario Analysis Results



Abbreviation: TTD = time to treatment discontinuation

Discussion

- The analysis demonstrated that model design for a BIA may significantly impact the results, potentially by a significant magnitude.
- In oncological indications where treatment costs are high, the accurate timing of treatment discontinuation for the modelled cohort is of importance. Although not examined in this analysis, this conclusion may hold for non-oncological indications where TTD is an important determinant of BI.
- There are a number of limitations associated with this analysis, including being a hypothetical study:
 - The impact of sequential treatment use was not examined and may change the conclusions.
 - The BIA was simplified and did not include other cost components such as health-state costs or background therapies, and therefore the results were more significantly driven by treatment cost and discontinuation than they would be in a real-world analysis.
 - Finally, the analysis assumed a static uptake of the new treatment, whereas a dynamic uptake may be more realistic.
- There is a lack of literature examining model designs and their impact on budget impact. As payers and health technology assessment agencies continue to examine BI alongside cost-effectiveness, we recommend that further research be conducted to ensure best practice is followed, so that accurate BIAs are used to inform health resource decision making.

Conclusions

- In conclusion, using a simple cost-calculator approach with an annual update and mean TTD may not accurately estimate the budget impact, and for oncology indications with high cost treatments, a more complex model design is recommended.

References

- Mauskopf JA, Sullivan SD, Annemans L, et al. Principles of good practice for budget impact analysis: report of the ISPOR Task Force on good research practices--budget impact analysis. *Value Health*. 2007;10(5):336-347.
- Sullivan SD, Mauskopf JA, Augustovski F, et al. Budget impact analysis: principles of good practice: report of the ISPOR 2012 Budget Impact Analysis Good Practice II Task Force. *Value Health*. 2014;17(1):5-14.
- Schey C, Milanova T, Hutchings A. Estimating the budget impact of orphan medicines in Europe: 2010 - 2020. *Orphanet J Rare Dis*. 2011;6:62.