Appendix 1 – PHARMAC Guidelines for Reviewing CUAs

< previous (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/11-presentation-of-data-and-results/) | next > (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/appendix-2-discounting/)

The following guidelines are referred to by PHARMAC when reviewing in-house CUAs, or CUAs provided by pharmaceutical suppliers.

Model Input/ Assumption	Questions
Type of analysis	What type of analysis was undertaken (eg CUA, CEA, CMA, CBA)? Was this appropriate?
Target population	Was the analysis based on the correct target population (ie the target population most likely to receive treatment)?
Time horizon and cycle length	Were the time horizon and cycle length appropriate and justified in terms of the underlying disease and the effect of interventions?
Comparator	Have the appropriate comparator(s) been used in the analysis? Is this the treatment that most prescribers would replace in NZ clinical practice, and the treatment prescribed to the largest number of patients (if this differs from the treatment most prescribers would replace)?
Treatment regimen (including dose)	Does the report describe all relevant treatment paths? Is the correct pharmaceutical dose used?
Efficacy	Is the model based on the best-quality data available? Were the sources of data used in the model clearly stated? Is there any evidence to suggest selective use of data?
Health states and model structure	Is justification of the choice of health states within the model provided? Have any important health states been omitted from the model? Is the model transparent? Does the model appear to be unnecessarily complicated or simplified too much?
	Does the analysis outline the assumptions relating to the structure of the model? Are the

Model Input/ Assumption	Questions
	assumptions reasonable and justified?
	Have all relevant statistically significant clinical events been included in the base-case analysis?
Key assumptions and inputs	Did the analysis extrapolate data to the longer term, or extrapolate intermediate clinical endpoints to final outcomes? If so, was this appropriate, justified, and modelled using the correct methodology? Was this tested in the sensitivity analysis?
	Have data from different sources been combined? If so, are the data compatible and combined using appropriate methodology?
	Is there a clear and reasonable justification of how data have been incorporated into the model (ie the methodology used in the calculation of probability values)?
	Have the probability values been calculated accurately given cycle length?
	Has a half-cycle correction been included? If not, what justification is given?
Quality of life	How was quality of life measured? Was this method justified?
	If subjective values were used, were these validated and tested in the sensitivity analysis?
	Were the estimated utility values reasonable?
	Were utility values adjusted for cycle length?
	Were utility values discounted?
Pharmaceutical cost	Were pharmaceutical costs calculated correctly?
	Were there any rebates that have not been included?
	Is a generic pharmaceutical likely to become available in the near future?
	What dose was used in the cost calculations and where was this information sourced? (Note that the dose should be based on the dose used in the key clinical trials unless there is evidence of efficacy for different doses in clinical practice.)
	Are there likely to be dose adjustments over time?
	If relevant, was the correct bodyweight used in the calculation of pharmaceutical cost?
	Were dispensing fees included?
Non-pharmaceutical cost	How is the pharmaceutical administered? Have all costs associated with administration been taken into account?
	Have hospital costs been calculated correctly using NZ DRG cost weights? Were these volume-adjusted?
	Are you aware of any costs that appear to be inaccurate?
	Have any important and relevant costs been excluded?
	Were costs discounted?
Discount rate	Was the correct discount rate used?
Results	Was the cost per QALY reported as a range as well as a point estimate?
	Were there any important factors that have been excluded from the analysis that could have an impact on the results?
	In your opinion, are the conclusions of the analysis justified?

Model Input/ Assumption	Questions
	Were all key inputs and assumptions varied in the sensitivity analysis?
Sensitivity analysis	Were the range and choice of variables used in the sensitivity analysis justified?
	Were the results of the sensitivity analysis interpreted correctly?
Report	Did the report list any factors that could limit the applicability of the results (eg differences in patient population)?
	How could the analysis be improved? Describe the overall quality of the report.

< previous (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/11-presentation-of-data-and-results/) | next > (https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/economic-analysis/pfpa/appendix-2-discounting/)

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