PHARMAC Pharmaceutical Management Agency

11 Presentation of Data and Results

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It is important that CUAs are transparent so that quality and validity can be assessed. Table 14 outlines the information to include when reporting detailed CUAs. Lower levels of analysis undertaken by PHARMAC may be less descriptive.

Table 14: Information to Include in Report for Detailed Cost-Utility Analyses

| Section | Details | Description | |
|------------------------|---|--|--|
| Context | Statement of objective and perspective of analysis. | Decision problem that prompted the analysis. | |
| | Statement of type, scope and level of analysis. | Levels of analysis include rapid, preliminary, indicative, and detailed. | |
| | Description of disease. | Symptoms | |
| | | Stage of disease | |
| | | Disease progression | |
| | | Prognosis. | |
| | Description of target population. | Age | |
| | | Gender | |
| | | Risk factors | |
| | | Prevalence | |
| Disease | | Incidence | |
| and patient population | | Ethnicity. | |
| | Description of current treatment options available. | Aim of treatment | |
| | | Indications | |
| | | Contraindications | |
| | | Dose | |
| | | Administration | |
| | | Length of treatment | |
| | | Adverse events | |
| | | Pharmaceutical Schedule listing criteria | |
| | | • ••• • • • • • • • | |

| Section | Details | Any likely amendments to treatment over time. Description | |
|------------|--|--|--|
| | | Indications | |
| | | Contraindications | |
| | Description of pharmaceutical. | Formulation | |
| | | Strength | |
| | | Dose | |
| Study drug | | Administration | |
| | | Length of treatment | |
| | | Adverse events. | |
| | Description of indication(s). | Registered and funded indication(s) | |
| | | Indication for which funding is sought (including any restrictions). | |
| | | Database searched | |
| | | Time period search undertaken | |
| | Description of literature search | Search strategy used | |
| | strategy. | Keywords | |
| | | Refinements | |
| | | Justification for excluding any citations. | |
| | Description of key clinical studies. | Design | |
| | | Study population | |
| Clinical | | Follow-up period | |
| evidence | | Intervention and comparator | |
| | | Withdrawals from treatment | |
| | | Clinical endpoints. | |
| | Critical review of clinical studies | Grade of evidence (GATE, SIGN) | |
| | | Possible sources of bias | |
| | | Methods of randomisation. | |
| | Discussion of relevance of trial results to New Zealand clinical practice. | Efficacy compared with effectiveness. | |
| | Target population. | Target population included in the analysis. | |
| | Comparator(s). | Rationale for choice of main comparator. | |

| Section | Details | Description |
|---------------------------------------|--|--|
| | | Model type |
| Model | Description of model. | Transition states |
| | | Markov states |
| | | Copy of decision tree or branch of decision tree. |
| | Time horizon and cycle length. | Justification for time horizon and cycle length. |
| | Discount rate. | Description of discount rate used for costs and benefits. |
| Outcome measures | Description of relevant outcomes and how they were measured. | Adverse events, disease progression, mortality, etc. |
| | Transformation and extrapolation. | Include information on transitional probabilities and how these were derived, including details of any extrapolation of data, synthesising data, etc. The inclusion of graphs and tables can be useful. |
| | List of parameter values. | Including confidence intervals. |
| | List of assumptions. | Assumptions regarding the structure of the model and data. |
| Health- related quality of life | Description of how HR-QoL was measured. | For example, methods for mapping to generic health state instruments, use of expert opinion, etc. |
| | Utility values used. | The health state (including a full description of the state) and corresponding utility value. |
| Costs | Description of costs. | Units of resources, unitary costs. |
| | Description of realisation of hospital costs. | Information on whether a new treatment results in real savings to DHBs, nominal savings, or additional costs. |
| | Description of data sources. | Including any strengths or weaknesses of data sources. |
| Results | | Disaggregation of costs, savings, life expectancy and quality of life gains/losses, as outlined in Chapter 9. |
| | Results derived from the model. | Discounted incremental QALYs/\$1M (point estimate and range) |
| | | Corresponding cost/QALY results (point estimate and range), placed in brackets. |

| Section | Interpretation and discussion of Details | Discussion on likely relative cost-effectiveness of Description |
|-------------------------|---|---|
| Sensitivity analysis | Results of sensitivity analysis. | Report using graphs, tables and/or elasticities. Include a full interpretation of the results. |
| | Discussion of sensitivity to modelling assumptions and data inputs. | Direction of bias and magnitude of effect. |
| Discussion | Discussion of results and other issues that should be considered under PHARMAC's Factors for Consideration. | For example, benefits to individuals and whānau other than the person treated; health need and suitability. |
| | Description of validation method and result. | For example, pharmacoeconomic review and/or clinical review. |
| Validation | Comparison with published analyses, including analyses undertaken by health technology assessment organisations. | Explanation of any differences in results. |
| Conclusions | Description of setting to which the results of analysis can be applied. | List of factors that could limit applicability in clinical practice. |
| | Description of any research in progress. | Description of how new data may alter results of analysis. |

11.1 Checklist

Table 15 is a checklist of information to include in PHARMAC base-case analyses and sensitivity analyses.

Table 15: Checklist of Information to Include in Base-Case Analyses and Sensitivity Analyses

| Section | Base-Case Analysis | Sensitivity Analysis |
|----------------------|---|--|
| Perspective | Funder (health sector) and individual, taking into account PHARMAC's Factors for Consideration. | Perspectives that include costs and health benefits to others, and costs falling outside the health sector. |
| Target population | Population most likely to receive treatment. | May consider inclusion of retrospective subgroup analyses if these data were of inadequate quality to include in base-case analysis. |
| Comparator | Current clinical practice in New Zealand. | May consider inclusion of placebo and/or most effective treatment (if different from current clinical practice). |
| Clinical outcomes | Statistically and clinically significant outcomes obtained from high-quality RCTs, systematic reviews or meta- analyses (grade of evidence of 1+ or 1++). Include impact of non-compliance if significant. | Include statistically insignificant outcomes. May consider impact of including additional sources of clinical evidence (eg unpublished trials). Test all modelling assumptions, |
| | | including any extrapolation of data. |
| HR-QoL | Base of NZ EQ-5D Tariff 2. Use GBD weights to check for consistency. | Alternative sources of utility values. |
| Pharmaceutical costs | Proposed price of pharmaceutical. | Deflate price by 2% per year as a proxy for inflation in other costs. |
| Other costs | Hospital, outpatient and patient costs. | Vary costs over likely ranges. |
| Discount rate | 3.5% | 0% and 5% |

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