Introduction

- Cost-effectiveness models help decision-makers to ensure the most effective use of resources.
- When treatments affect mortality, such models are estimate time-to-event for outcomes such as death to estimate costs and outcomes.
- In order to obtain realistic estimates of incremental direct costs and outcomes, it is necessary to estimate the baseline survival rate.

Although parametric models may be more efficient, estimates of cost-effectiveness may be sensitive to the choice of functional form. The selection of the preferred functional form for extrapolation of survival data can be uncertain.

- Functional form selected from the set of candidate models.
- Bootstrap model averaging (BOOT) uses a weighted average of results associated structural uncertainty.
- By providing a single model estimate that incorporates uncertainty, BOOT could act as a valuable tool for any survival analysis of cost-effectiveness analysis.

However, although parametric models may be more efficient, estimates of cost-effectiveness may be sensitive to the choice of functional form. The selection of the preferred functional form for extrapolation of survival data can be uncertain.

- Functional form selected from the set of candidate models.
- Bootstrap model averaging (BOOT) uses a weighted average of results associated structural uncertainty.
- By providing a single model estimate that incorporates uncertainty, BOOT could act as a valuable tool for any survival analysis of cost-effectiveness analysis.

Results

- The preferred functional form varied across studies (Table 5).
- Different functional forms (Gompertz, gamma, Generalised gamma, log-logistic) were selected for each study.

Conclusions

- In this analysis we have focused on BIC as a measure of model fit.
- However, there are other measures and selection criteria that might be considered.
- BIC does not incorporate the clinical relevance of any extrapolation.
- Bagust and Beale (2010) highlighted that this assumption should not be considered.
- The process for conducting parametric survival analysis for cost-effectiveness analysis has been widely discussed, and recommendations for understanding the most appropriate models and incorporating structural uncertainty have been presented in the literature.

Discussion

- These results demonstrate that mean survival estimates are sensitive to the choice of functional form.
- The uncertainty in the selection of the preferred functional form is illustrated in Figure 3. The KM curves are shown for 12 bootstrapped samples.
- The bootstraps indicate that the preferred functional form varied across studies.

Limitations

- These results demonstrate that mean survival estimates are sensitive to the choice of functional form.
- The uncertainty in the selection of the preferred functional form is illustrated in Figure 3. The KM curves are shown for 12 bootstrapped samples.
- The bootstraps indicate that the preferred functional form varied across studies.

Fitting and selection of ‘preferred’ parametric survival models

- Seven parametric functional forms (Weibull, Gompertz, log-logistic, log-normal, gamma and Generalized gamma) were considered.
- For comparative studies, a joint survival model was fitted using a weighted during simulation.
- The ‘preferred’ model was defined as the one with the lowest likelihood error (log-likelihood).

Table 5: Survival predictions

<table>
<thead>
<tr>
<th>Study</th>
<th>Treatment</th>
<th>Mean</th>
<th>SE</th>
<th>RR</th>
<th>95% CI</th>
</tr>
</thead>
<tbody>
<tr>
<td>Study 1</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Study 2</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Study 3</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

References

- Lambert M, Brouwer S, Szczylik C. Survival analysis in oncology clinical trials—extrapolation with patient-level data: inconsistencies, inconsistencies, and underestimation of uncertainty that arises when a number of models are investigated, only and underestimation of uncertainty that arises when a number of models are investigated, only.
- Bagust A, Beale S. Survival analysis and extrapolation modeling of survival predictions.
- Lambert M, Brouwer S, Szczylik C. Survival analysis in oncology clinical trials—extrapolation with patient-level data: inconsistencies, inconsistencies, and underestimation of uncertainty that arises when a number of models are investigated, only.
- Lambert M, Brouwer S, Szczylik C. Survival analysis in oncology clinical trials—extrapolation with patient-level data: inconsistencies, inconsistencies, and underestimation of uncertainty that arises when a number of models are investigated, only.