
Article:
Woods, B.S., Sideris, E., Palmer, S. et al. (2 more authors) (2020) Partitioned survival and state transition models for healthcare decision making in oncology : where are we now? Value in Health, 23 (12). pp. 1613-1621. ISSN 1098-3015

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Appendix A

The search was conducted on January 15th 2020 in PubMed using terms relating to both partitioned survival models and terms relating to state transition modelling:

((partition[Title/Abstract] OR partitioned[Title/Abstract]) AND survival[Title/Abstract]) AND (multi[Title/Abstract] OR multistate[Title/Abstract] OR markov[Title/Abstract] OR "state transition" [Title/Abstract] OR "discrete event"[Title/Abstract] OR "patient level"[Title/Abstract])

This returned 44 results.

Abstracts were screened for three types of articles:

- Articles or reviews discussing the appropriateness of PSM
- Empirical comparisons of PSM and other modelling methods
- Articles discussing related methodological issues

Overall we identified two relevant reviews [1,2]; six relevant comparisons of methods[3-8]; and one relevant methodological article[9]. These nine studies are discussed in the main text.

The following articles met our inclusion criteria but were excluded from further discussion for the following reasons:

- Batteson et al. 2019 [10]: this study examined approaches to modelling when no OS is available. They describe use of PSM, however the model implemented actually uses PFS to predict OS so is not considered to be a PSM according to the definitions within this paper.
- Delea et al. 2014 [11]: this study compared PSM and STM. For the STM, PPS predictions were generated based on calibration of the PPS parametric survival model to match the OS predictions from the PSM model. This methodology was, by design,

intended to minimise the difference between the STM and the PSM long-term predictions and was not therefore considered to represent a methodological comparison of the two approaches.

- Gibson et al. 2019 [12]: the modelling approach in the STM was unclear, the authors report that PFS and OS analyses were used to parameterise the STM but is unclear how this was done.
- Goeree et al. 2016 [13]: the approach used in the STM was unclear and did not appear to include any conditional transition probabilities (e.g. there is no reported estimate of mortality from the progressed state).

Appendix A: Review of NICE appraisals

Table 1 summarises the appraisals reviewed. We reviewed relevant sections of the manufacturer submission, ERG report, Final Appraisal Document, committee slides and correspondence between NICE, the manufacturer and the ERG where relevant. The following data was extracted:

- Drug appraised
- Cancer
- Disease stage and treatment stage
- Whether there was reference to TSD 19
- Modelling approach used
- Health states/partitions
- Whether IPD was available for all comparators
- Justification provided for choice of approach
- For appraisals using PSM:
    - Discussion of assumptions / limitations of approach for extrapolation of OS
- For appraisals using STM:
    - Whether treatment effects and surrogacy relationships were justified
    - Whether time-dependencies in event rates was explored
    - Whether the impact of patient history on subsequent events (e.g. PPS) was discussed or modelled
    - Approach used to estimate transition probabilities
    - Internal validity of survival predictions
    - Discussion of potential biases in PPS
- For both model types
    - Assessment of external validation of OS predictions

- Discussion of credibility of extensions to survival in different health states
- Sensitivity analyses used to explore uncertainties in disease and treatment process

Table 1: Appraisals included within review

Link to appraisal documentation	Full appraisal title
https://www.nice.org.uk/guidance/ta567	Tisagenlecleucel for treating relapsed or refractory diffuse large B-cell lymphoma after 2 or more systemic therapies
https://www.nice.org.uk/guidance/ta573	Daratumumab with bortezomib and dexamethasone for previously treated multiple myeloma
https://www.nice.org.uk/guidance/ta577	Brentuximab vedotin for treating CD30-positive cutaneous T-cell lymphoma
https://www.nice.org.uk/guidance/ta578	Durvalumab for treating locally advanced unresectable non-small-cell lung cancer after platinum-based chemoradiation
https://www.nice.org.uk/guidance/ta579	Abemaciclib with fulvestrant for treating hormone receptor-positive, HER2-negative advanced breast cancer after endocrine therapy
https://www.nice.org.uk/guidance/ta580	Enzalutamide for hormone-relapsed non-metastatic prostate cancer
https://www.nice.org.uk/guidance/ta581	Nivolumab with ipilimumab for untreated advanced renal cell carcinoma
https://www.nice.org.uk/guidance/ta584	Atezolizumab in combination for treating metastatic non-squamous non-small-cell lung cancer
https://www.nice.org.uk/guidance/ta587	Lenalidomide plus dexamethasone for previously untreated multiple myeloma

https://www.nice.org.uk/guidance/ta589	Blinatumomab for treating acute lymphoblastic leukaemia in remission with minimal residual disease activity

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