HTA31: Health Economic Modelling Approaches in Cell and Gene Therapies

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Background & Objective

BACKGROUND

A limited number of cell and gene therapy products have been through Health Technology Appraisal (HTA) assessments and therefore limited data exists on the common approaches to economic model structure and design. Understanding common themes, may inform manufacturers approach to HTA.

OBJECTIVE

To explore possible heterogeneity between approaches utilised in building cost effectiveness models for cell and gene therapies. Also, to seek common approaches and discuss likely strategies for cell and gene therapy modelling approaches.

Methods

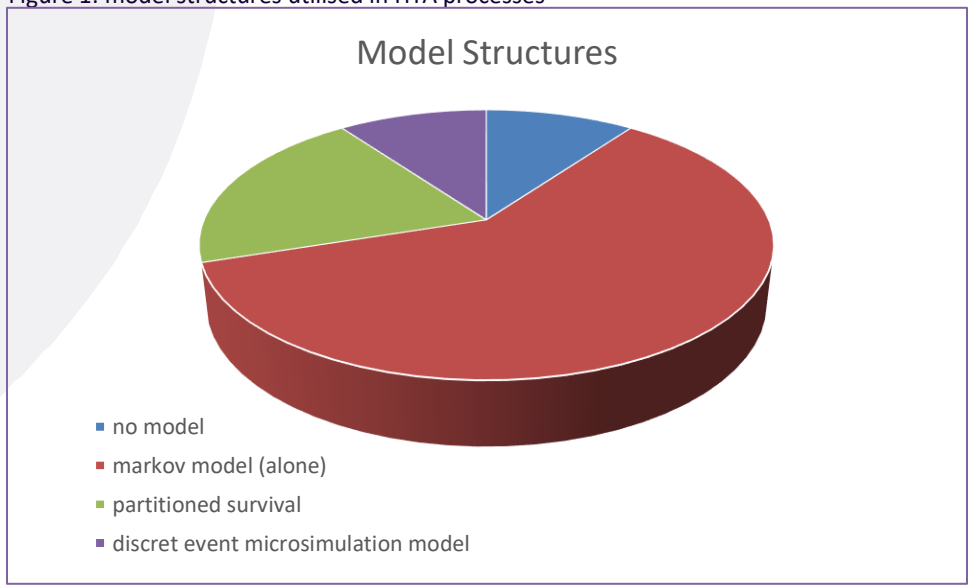
Cell and gene therapy products were identified, and a targeted literature review was carried out to identify publications and Health Technology Appraisals (HTAs) relating to these products.

The structures of cost-effectiveness models were examined to look for common features.

Results

Of the 10 identified products that had marketing authorisation granted in the EU, 9 had cost-effectiveness models built for them. One product had multiple model structures: in this case analysis focused on the model structure used for the product's HTA assessment. 56% of the models used a Markov Structure (alone), with transition states ranging from 3 states to 6 states; 22% of products used partitioned survival models; 11% used a discrete event microsimulation model; and 11% used a 2-stage model (i.e., a decision tree for 12 months and a Markov transition state modelling thereafter).

Figure 1: model structures utilised in HTA processes



Conclusion

Manufacturers and health economists are clearly attempting to use familiar modelling structures despite the obvious paucity of data and issues arising from using short-term data to model long-term disease benefits. These strategies appear to be quite effective in securing successful reimbursement. It seems sensible to assume that, where possible, a Markov model approach is the obvious starting point to demonstrate the cost effectiveness of cell and gene therapies. A less common approach is to use a discrete event microsimulation or a decision tree for the early phase of treatment and transition to a Markov model thereafter, which can allow for the modelling of more long-term effects.

Discussion

Model structures are ordinarily driven by data availability and therefore it may be worthwhile exploring the data available for each cell or gene therapy.

It must also be considered of course, that model structures may be more suitable to a therapeutic area. For example, partitioned survival modelling is used to follow a theoretical cohort through time as they move between a set of exhaustive and mutually exclusive heath states, most often used in oncology.

In short, when considering a model structure data and therapy area are likely to play an important role in consideration of approach.

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