

Bayesian Hierarchical Models of Basket Trials in the Context of Economic Evaluation

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In June 2021, we provided additional guidance on the conduct of economic evaluations for tumour agnostic products.¹ The intent of this guidance was to clarify how specific aspects within economic evaluations (e.g., target population, comparators, clinical effectiveness, cost and utilities, modelling, analysis and reporting, and uncertainty) should be considered. An area that was noted to be challenging was the availability of comparative clinical information, given the use of basket trials.

Basket trials study a primary intervention across a range of different patient subgroups (e.g., cancer types) that share the same similar features (e.g., mutation or biomarker), in which the effect of the intervention may differ. The heterogeneity among cancer types, limited sample sizes, lack of comparators, and use of surrogate end points pose challenges when considering the application to economic evaluations. Bayesian hierarchical models (BHM) have emerged as an approach to model the information from basket trials, which can be well suited to account for the heterogeneity among cancer types while borrowing information across cancer types in basket trials.

There is limited information on the use of information from BHM in economic evaluations. To address this gap, we consulted with researchers with expertise in this area to provide: an overview of BHM, and how information can inform economic evaluations and assist with the characterization of uncertainty; practical considerations when appraising the use of this evidence in an economic evaluation; and a detailed case study to illustrate how BHM can inform economic evaluations, highlighting limitations, areas for consideration, and interpretation of results.² While there is an absence of information for some populations and uncertainty regarding the effects of treatment when relying on basket trials, this approach provides transparency to leverage information across different populations and to address uncertainty.

This document addresses the evolving treatment landscape for oncology away from tumour-based approaches and toward mutation-based approaches. There is also an increased use of basket trials beyond oncology, for which these methods would apply as well. The details in this document provide areas for appraisal that we will consider when assessing economic evaluations informed by BHM, in addition to clarity around a set of methods that can be used for informing economic evaluations based on basket trials. The document also highlights areas for further research. The field of BHM is evolving, and advice in this document will need to be revisited as methods develop.

References

1. <https://www.cadth.ca/sites/default/files/pdf/mh0016-cadth-economic-guidance-for-tumor-agnostic-products-rev-june22.pdf>
2. [Bayesian Hierarchical Models \(BHM\) of Basket Trials in the Context of Economic Evaluation](#) – CADTH Journal article.