Bayesian modelling of resource use and cost data in trial-based cost-effectiveness analysis

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Background: Trial-based cost-effectiveness analysis (CEA) requires the collection of detailed information regarding the quantity of healthcare resources used during the study period by each patient in the study. Each item of resource use is subsequently multiplied by item-specific unit costs or prices in order to quantify the economic impact associated with the use of every resource used in the process of care. The ultimate objective is to estimation of a total cost for each individual enrolled in the trial. This paper argues that standard approaches to cost modelling in CEA, which model individual-level total costs only, might be sub-optimal and that alternative analytical strategies could be usefully employed.

Objective: To explore the pro and cons of adopting multivariate statistical modelling strategies to analyse resource use and cost data in trial-based CEA, with the secondary objective of assessing the additional benefits in terms of research opportunities deriving from the adoption of more sophisticated modelling strategies.

Methods: Using data from a recent large trial-based CEA, this paper compares Bayesian multivariate modelling strategies for (a) resource use and (b) cost components against a more traditional (c) univariate modelling approach for patients' total costs.

Results: Multivariate statistical models of resource use and cost components provide an advantage compared to their univariate counterparts. First, resource use data is often a mix of continuous (e.g. length of stay in theatre), count (e.g. number of GP visits), and categorical variables, which are better represented using distributions that describe the characteristics of the data more appropriately. In some circumstances Normal approximation is a viable alternative, and it could be a good staring point in the analysis. Modelling cost components also provides a good strategy when data are sparse or to avoid the complexity of modelling a large number of resource use items. Secondly, the univariate analysis of total costs suppresses important information. Multivariate analysis of resource use data and cost components could shed light on differences in the mix of resources used in different centres and countries. Furthermore, by modelling the relationship between resource use categories the analyst could efficiently obtain more robust estimates of the parameters of interests. Finally, explicit modelling of resource use data or cost components is a preferable approach when the researcher needs to deal with missing and incomplete data in the data set.

Conclusions: Multivariate modelling is a promising analytical strategy for the analysis of resource use and cost data in clinical trials. This method enables can be extended to the use of multi-centre and multi-national trials data to help explore the between location variability in the cost results. Furthermore, the proposed approach can be used within the more general multivariate framework for the analysis of cost-effectiveness data to accommodate patient and centre and or country specific variables.