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Use of evidence in cost-effectiveness decision modeling: Taxonomy for current practice and methods

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Objetivos (Objectives):

The evidence base informing economic evaluation models is hardly ever derived from a single source. Evidence used to characterise disease natural history, treatment relative effectiveness, resource use and utility parameters, may derive from observational studies, clinical trials, secondary analyses and/or expert opinion. To reflect the needs of a particular decision problem it is the researcher's decision which evidence and analysis method best estimates the required parameters. This paper proposes a nomenclature and provides guidance on how distinct sources of evidence could be used to inform economic modelling.

Metodologia (Methodology):

A systematic review of the literature was conducted to identify recent economic evaluation studies from bibliographic databases and other sources. Both applied and methodological articles were reviewed and an examination of the methods employed to synthesize evidence from distinct sources to derive cost-effectiveness estimates was performed.

Resultados (Results):

Using the results of the systematic review, the proposed nomenclature considers the following dimensions: type of model parameter (relative effectiveness, resource use, utilities, natural history or a combination of the previous), sources of evidence (single or multiple sources), type or format(s) of this information (individual or aggregate-level data or a combination of both) and whether the estimation is concerned with a single or multiple parameter frameworks. For the different taxonomy scenarios, possible synthesis methods are described and analysed, ranging from the common statistical techniques that fall within the meta-analysis family (e.g. when several studies report summary results on the same parameter of interest and one wants to combine these into a single quantitative estimate) to more complex methods as the Bayesian hierarchical related regression framework that enables the combination of individual and aggregate-level data to inform a single parameter. Although some of the synthesis options herein discussed are already well described and established in the literature, other options need further research before being used to address decision making within the health care framework.

Conclusões (Conclusions):

The matrix of possible derived scenarios characterizes the key structural ways and assumptions for using the evidence in cost-effectiveness modelling. This work allowed the (i) categorisation of evidence sources to inform model structure and parameter estimates; (ii) assessment of methods used to synthesize or transform evidence from different study designs; and (iii) drawing guidance on these analysis methods.