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Using multicentre RCT-based individual patient-level data to populate decision analytic cost-effectiveness models for location-specific decision making: a Bayesian approach

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Objectivos (Objectives):

To develop methodology for the analysis of individual patient-level data from multicentre/multinational randomized controlled trials with the aim of estimating location-specific parameters to populate decision models for location-specific decision making.

Metodologia (Methodology):

Multilevel or hierarchical modelling is the analytical framework used to handle hierarchical cost-effectiveness data. Hierarchical modelling was developed in a Bayesian framework, that is, the estimation of the parameters was performed by Markov Chain Monte Carlo (MCMC), which were used to populate the economic decision model. Bayesian probabilistic modelling was used to evaluate the decision problem and Bayesian shrinkage estimation procedures were used to obtain location-specific cost-effectiveness estimates.

Resultados (Results):

Using data from a recently conducted economic analysis of the RITA 3 trial, location-specific cost-effectiveness measures were obtained and compared to the trial-wide results. For the analysed centres, the centre-specific cost-effectiveness planes showed higher variability in mean differential cost and mean differential QALY estimates compared to the trial wide results, with the latter having longer left tail estimate distribution. The majority of the location-specific incremental cost-effectiveness ratio results show higher cost per QALY for the intervention strategy compared to the trial wide results (approx. £41,400/QALY). With respect to centre-specific cost-effectiveness acceptability curves, the curves for the selected centres display great variability across centres in cost-effectiveness for given values of the threshold, λ. If the decision maker is willing to pay £50,000 for an additional QALY, the probability that the intervention strategy is cost-effective is, for instance, 0.34 for centre 37, compared to the 0.65 for the trial wide results.

Conclusões (Conclusions):

This work showed two important results. Firstly, it was demonstrated, through the use of one illustrative example, how a trial-based cost-effectiveness analysis may be implemented within a Bayesian framework and evaluated using Gibbs sampling MCMC methods. In particular it has provided the 'building blocks' for extending the modelling framework to allow the incorporation of more relevant evidence: (i) data may be embedded in a prior

distribution format; and (ii) data may come from different study designs (e.g. RCTs, observational studies together with expert judgement). Secondly, it was demonstrated how Bayesian hierarchical modelling could be used to estimate more appropriate cluster-specific parameters for use in decision analytic models where individual patient-level data from a multi-location trial are available. Bayesian hierarchical modelling estimates can be used to explore correctly the variability between centres/countries of the cost-effectiveness results allowing the correct quantification of uncertainty by adjusting the standard errors to reflect the estimates variability both within and between locations.