16:00-17:30   Invited Session 8 (Main Room)

Combining RWD and randomized clinical trials (Chair: Nikos Demiris)

Population Average Estimates for Health Technology Assessment

Nicky Welton (Bristol University, UK):

Heath technology assessment (HTA) compares the effectiveness and cost-effectiveness of treatment options to inform national healthcare decisions. This requires estimates of an objective function (for example net-benefit) that reflects the target population and any specific subgroups that decisions are being made for. Economic models can be complex functions of multiple parameters, and there can be heterogeneity in many of the model inputs.

We show how different ways of summarising heterogeneity reflects different decision questions, and that integrating the objective function over joint covariate distributions is the appropriate summary for national decision-making. We illustrate heterogeneity in non-treatment effect parameters using a HTA of intravenous immunoglobulin (IVIG) for patients with severe sepsis, using data from the Case-Mix Programme national database from the Intensive Care National Audit and Research Centre (ICNARC) to define the target population for the decision.

For heterogeneity in multiple relative treatment effect parameters multi-level network meta-regression can be used to estimate a network meta-regression model when individual patient data is available for at least one RCT. This can be integrated over a population-specific joint covariate distribution to obtain population average estimates in a defined target population. We illustrate with synthetic data from RCTs of treatments for plaque psoriasis to obtain population average estimates in two different target populations with characteristics matching observational datasets.

Observational evidence plays a crucial role in characterising the case-mix of target populations for decision-making. Integrating the objective function, such as net-benefit, over the joint covariate distribution provides the appropriate estimate to inform national healthcare decisions.