A COMPARISON OF METHODS FOR ASSESSING TREATMENT EFFECT HETEROGENEITY

Graham Dunn[1], Richard Emsley[1], Andrew Pickles[1]

[1]Health Methodology Research Group, The University of Manchester, UK

When calculating the treatment effect of a subject in a study, an assumption is often made that the treatment effect is constant across subjects. In fact, this is not required for valid causal inference and furthermore it is unlikely to hold with the possibility of substantial variation present amongst the subjects' effects. Where this heterogeneity depends on observed variables, instrumental variable (IV) methods will still provide unbiased estimates of treatment effects. However, if there is unmeasured confounding and the confounder predicts the decision to receive treatment, IV methods can give biased estimates of the treatment effect, and the degree of bias is partly determined by the strength of the instrument. This is known in the econometrics literature as essential heterogeneity.

Three different methodological solutions to this problem are highlighted from the literature. We compare these methods using data from a randomised trial which investigates the effects of two interventions on early episode schizophrenia (SoCRATES) and through Monte Carlo simulation.

Summarising the approaches, Heckman et al (2006) propose using local instrumental variables to estimate a parameter denoted as the marginal treatment effect, which can be weighted to give alternative effect estimates such as the average treatment effect. Wooldridge (2007) applies a control function in estimating the parameters of the correlated random coefficient model and also introduces additional assumptions which give valid effect estimates from the standard IV approach. Dunn and Bentall (2007) describe how a structural mean model equates to IVs estimated through two stage least squares (2SLS), and allows for essential heterogeneity whilst measuring treatment pathways through potential mediators, which can be measured with error.

As well as comparing these three approaches, the use of instrumental variables estimated by generalised method of moments and maximum likelihood is illustrated. We also show how missing data can complicate the analysis, and be accounted for in each of the methods.