**Economic evaluation of Cluster RCTs: comparison of methods when applied to highly imbalanced cluster size**

More cluster RCTS are now being conducted, where randomisation takes place in groups, rather than an individual person basis. However, the majority of economic evaluations conducted alongside cluster RCTS to date have not used appropriate methods to take account of both clustering and correlation between costs and outcomes. Given there are a number of theoretically appropriate methods to analyse such data, it is important to question if choice of method matters and if so, to understand when it is appropriate to use the different methods available.

In this paper, we will compare various statistical techniques dealing with clustered samples. We use data from the **O**ccupational **T**herapy in **C**are **H**omes (**OTCH**) cluster randomised trial. This intervention was conducted on residents of UK care homes, assessing benefits of occupational therapy (OT) to improve stroke outcomes, compared with usual care (no routine OT intervention). 1040 participants were recruited from 228 care homes (clusters): 472 allocated to the control arm (114 care homes) and 568 to the intervention arm (114 care homes). The number of participants in each cluster ranged from 1 (n=30) to 23 (n=1), with only 11 homes having more than 10 participants (mean number of participants per home = 4.56). Resource use data and EQ-5D scores were collected at baseline, 3, 6 and 12 months post-randomisation, thus 1 year costs were estimated from an NHS perspective.

We will compare a number of theoretically appropriate methods of analysis for this economic evaluation conducted alongside a cluster RCT, in line with methods proposed by **Gomes**[[1]](#footnote-1). These methods include:

1. Seemingly unrelated regression (SUR)
2. Generalized estimating equations (GEE)
3. Non-parametric two-stage bootstrap
4. Multilevel modelling.

Whilst **Gomes** used simulated data, we will attempt to fit **OTCH** trial data, which recruited a much larger number of care homes (n=228) with a much wider variation in cluster size (range 1-23) than had been modelled in previous analysis. The focus of our discussion will look at how well each of the clustering models performs when the assumption of homogeneous cluster size does not hold.

1. Gomes, Manuel, et al. "Developing appropriate methods for cost-effectiveness analysis of cluster randomized trials." *Medical Decision Making* 32.2 (2012): 350-361. [↑](#footnote-ref-1)