When less is not more: Trials and tribulations of achieving the required sample size in a 'real-world' cluster RCT

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Suggested talk duration (15-60 minutes)
20 minutes

Summary (max. 500 words)
Cluster randomised controlled trials (CRCT) present researchers and statisticians with a number of special challenges. As a rule, CRCT necessitate larger sample sizes than individually randomised controlled trials, due to the clustering effect, which contributes to extra variation in the outcome. This extra variation in the outcome needs to be counteracted by a larger sample size, to ensure adequate power of the study. Statistically, CRCT with a large number of small clusters provides the most efficient design in terms of sample size required for the study. However, in 'real world' settings, delivering an intervention to a large number of organisations is inefficient from the point of view of resource allocation and can considerably increase the overall cost of a study. This case study describes the challenges in recruitment and maintenance of a sample size in a three-arm CRCT of the Resident at the Centre of Care (RCC) Program. The program was designed to assist staff of residential aged care facilities (RACF) transition to the model of care that was centred on the needs and choices of residents. The trial evaluated efficacy of the program alone or with on-going clinical support, relative to care as usual. Randomisation was at the level of RACF. The primary outcome of the program was resident quality of life, measured at the level of individual residents. Secondary outcome was improved organisational climate, measured at the level of RACF. Data were proposed to be analysed with a multilevel linear regression, with individuals clustered within RACF and random intercept for RACF. Sample size calculations for the primary outcome were performed to achieve 80% power and 5% Type I error rate (2-tailed). Input parameters for the sample size calculation were based on the results of our previous studies in RACF and assumed a moderately small effect of the intervention (Cohen's d≥0.35), intra-class correlation of 0.02, an average cluster size of 20 residents per RACF, and 25% attrition at 6 months. The resultant estimate for the total sample size at baseline was 744 residents and 39 RACF (13 for each arm of the trial). The estimated budget for the study was $1.3 million AUD. To date, we were able to secure $155,000 AUD, which allowed us to recruit and randomise 9 RACFs. With the expectation of 20 residents per RACFs, our smallest detectable effect size increased to d=0.57, hence reducing our chances of detecting a

meaningful effect of the intervention. Additionally, the small number of RACFs recruited into the study means that the results of 'traditional' multilevel model for the secondary outcome (organisational climate at the level of RACF) are likely to have very low precision and power. Workable alternatives to the traditional large-sample multilevel models are urgently needed to increase feasibility of CRCT designs and optimise their implementation in 'real-world' settings.

Relevance to conference theme

Analysis of cluster randomized trials necessitates multilevel approaches to data modelling, due to clustering of units within larger groups. Traditional approaches to multilevel analysis call for large sample sizes at both the cluster and individual observation levels, which are not always achievable in 'real world' due to time and financial constraints. This case study documents some of the problems with achieving and maintaining a sample size that is necessary to reliably analyze data from a cluster-randomized controlled trial undertaken in residential aged care facilities in Australia. At the time of advent of "Big Data", our experience highlights an urgent need for workable analytical solutions to address complex research questions with "Small Data", without compromising the integrity of conclusions.

Keywords (max. 3)

Cluster randomised controlled trial; sample size; residential aged care