A SIMULATION-BASED COMPARISON OF METHODS FOR ANALYSING BINARY OUTCOMES FROM CLUSTER RANDOMISED TRIALS

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We used simulation to compare bias, standard error, root mean square error and confidence interval coverage of several methods for estimating the intervention effect log odds ratio for cluster randomised trials with binary outcomes. The methods considered were: marginal logistic regression models using Generalised Estimating Equations with information sandwich estimates of standard error (GEE/R), random effects logistic regression models using maximum likelihood with adaptive quadrature (RELR), cluster-level *t*-test (CL/T), weighted cluster-level *t*-test (CL/W) and random effects cluster-level linear regression (CL/RE). The methods were compared across trials simulated with different numbers of clusters per trial arm, numbers of subjects per cluster, intraclass correlation coefficients (ICCs), and intervention versus control arm proportions. Two thousand datasets of clustered binary responses were generated for each combination of design parameter values. GEE/R generally had acceptable properties, including close to nominal levels of confidence interval coverage, when appropriate adjustments were made for data with relatively few clusters. CL/T and a modified version of CL/RE for studies with small numbers of clusters had good properties for trials where the number of subjects per cluster was sufficiently large and the ICC was sufficiently small. The properties of RELR were poor when the ICC and the number of subjects per cluster were both too large.