ID de Contribution: **10** Type: **Non spécifié**

Statistical properties of minimal sufficient balance randomization approach in a stratification context, a simulation study

Introduction – Randomization is a crucial step in clinical trials and ensures balance across treatment groups. Several approaches exist (e.g. stratified permuted blocks or covariate adaptive minimization). Some of them were introduced recently such as Zhao et al Minimum Sufficient Balance (MSB) in 2015. The aim of this work is to assess the performance of MSB to grasp a better understanding of its strengths and limitations.
Methods – (i) Stratified permuted 4-blocks, (ii) Pocock & Simon's minimization (with 2 and 4 classes) and (iii) MSB were applied and compared one to another in simulated stratified and unstratified settings. The simulated scheme was based on Sepsicool-1 trial data. Data augmentation was performed using NORTA method in order to generate correlated datasets with any specified distribution. Scenarios of 5,000 datasets each were considered with variations in terms of sample sizes and correlation structures. Evaluation criteria included imbalance tests, adjusted and unadjusted statistical power, and RMSE of observed and estimated treatment effect.
Results – MSB outperformed at minimizing covariates imbalance with stratified Students-t-test and stratified Wilcoxon signed-rank test, as well as non-stratified Students-t-test (for both augmented and non-augmented scenarios). Minimization was better than all the others for non-stratified Wilcoxon signed-rank test. Since, in practice, covariate adaptive randomization procedures are rarely stratified, as the important covariates are included in the randomization, MSB remains a relevant candidate. However, no randomization procedure was clearly better than the other at reaching the true treatment effect.
Conclusion – This study showed that MSB is a valuable randomization approach in adaptive design to control for group imbalance, and to maintain a high probability in showing treatment effect.
Keywords - Randomized clinical trial, adaptive design, data simulation

Auteur principal: M. CANNAFARINA, Hugo (ENSAI)

Co-auteurs: GUENEGOU ARNOUX, Armelle (Assistance Publique-Hôpitaux de Paris URC APHP.Centre - INSERM CIC1418-EC - INSERM-INRIA HeKA); Prof. KATSAHIAN, Sandrine (Assistance Publique-Hôpitaux de Paris URC APHP.Centre - INSERM CIC1418-EC - INSERM-INRIA HeKA)

Orateur: M. CANNAFARINA, Hugo (ENSAI)

Classification de Session: Inférence causale et essais cliniques