ESTIMATING TREATMENT EFFECT HETEROGENEITY FOR BINARY CLUSTERED DATA

E.J. Mascha[†1], J.M. Albert[2]

[1] *Cleveland Clinic Foundation, Cleveland, USA*
[2] *Case Western Reserve University,Cleveland, USA*

[†] E-mail: *maschae@ccf.org*

In a two-group parallel trial the mean causal effect is typically estimated as the difference in means or proportions for patients receiving, say, either treatment (T) or control (C). Treatment effect heterogeneity (TEH), or unit-treatment interaction, is the variability of the causal effect across individuals, and is often ignored or assume to be zero. Only Y_T or Y_C is observed for each unit in such studies, and thus the TEH is not directly estimable. Large TEH theoretically would allow more patients to be successfully treated, but small TEH is generally more desirable. We are particularly interested in estimating the 'treatment risk′ (TR) for binary outcomes, a measure of TEH which is the proportion of individuals who would succeed on C but fail on T. Previous work has shown that TR can be bounded (Albert, Gadbury and Mascha, 2005), and that the confidence interval width around it can be narrowed if the data are clustered or correlated (Mascha and Albert, 2005). Without further parameter constraints, TR is unidentifiable and can only be bounded. We show, however, that the TR can be directly estimated when the four underlying population counts comprising the joint distribution of Y_T and Y_C follow constraints consistent with the Dirichlet multinomial. We propose a test of zero treatment risk which has good size and power. Implications for medical decision-making at the policy and individual levels are discussed, and data examples given.