PREFERENCE-BASED ANALYSIS OF TREATMENT ACCEPTABILITY AND EFFECT IN RANDOMISED TRIALS

S.D.Walter[1]

[1]*Dept. of Clinical Epidemiology and Biostatistics, McMaster University, Hamilton, Canada*

Email: *walter@mcmaster.ca*

A new method of analysis is developed to evaluate the acceptability of treatments in a randomised trial, and their effect among compliers. A traditional *intention-to-treat* analysis avoids biases associated with the alternative *per-protocol* or *as-treated* approaches, but it provides imperfect information about the effect among patients who are committed to taking the treatment. The new *preference-based* analysis provides an estimate of treatment benefit among such patients. It also assesses the pattern of treatment acceptability and preference, information that is potentially useful in predicting the uptake of treatment after a trial.

Trial patients are characterised through the groups who would: accept either treatment if offered (*compliers*); refuse one treatment but accept the other if it is offered (two groups of *preferers*); or insist on one treatment even if it is not offered to them initially (two groups of *insisters*). We show that it is always possible to estimate the proportions of patients in these preference groups. However, constraints are required to estimate the corresponding outcome rates, and thus estimate the treatment effect in the compliers. We propose two possible sets of constraints (constant relative risk, and independent preference effects) and illustrate them by numerical examples.

Patients committed to adhering to an offered treatment, and their clinicians, will welcome a suitable estimate of the magnitude of treatment benefit they can expect.