Short course on Response-Adaptive Methods for Clinical Trials

February 26, 2024

Notation sheet:

- \bullet Index for patient is i
- Index for arm is k
- Number of patients on arm k as a random variable (RV) is N_k
- Fixed number of patients in a trial n
- Maximal number of patients in a trial n_{max}
- Notation for the potential outcome is Y_{ki}
- The parameter of interest of Y_{ki} is binary: p_k
- The estimator \hat{p}_k is the MLE of p_k .
- The treatment effect of interest is θ , when Y_{ki} is binary: $\theta = p_k p_0$
- The variable a_{ki} is a binary indicator and equal to 1 if patient i is assigned to treatment k and 0 otherwise.
- The vector a_k indicates whether each patient i in n patients got assigned to treatment k
- The vector a_i indicates whether each treatment k got assigned to patient i or not
- The vector $a_k^{(i)}$ lists the binary indicators or treatment assignments up to patient i. Similar Notation is used for outcomes $Y^{(i)}$ and proportions $\rho^{(i)}$.
- E(.) denotes expectation
- $\mathbb{1}_C$ is an indicator function taking value 1 iff condition C holds
- \bullet Notation << and >> means much smaller or much larger than
- Notation for the limiting assignment proportion in a multi-armed trial $\rho = (\rho_0, \rho_1..., \rho_K)$.

- But notation for the assignment proportion to the experimental arm for two armed trials in Lectures 2 and 3 is ρ
- Notation for the randomisation probability in a multi-armed trial $p^* = (p_0^*, p_1^*..., p_K^*)$.
- Statistical power and type I error rate of a statistical test T(.) is denoted as $1-\beta$ and α respectively
- BRAR denotes the original untuned Thompson sampling algorithm.
- (Lecture 3) Notation for the lower stopping boundary for a group sequential trial is l
- (Lecture 3) Notation for the upper stopping boundary for a group sequential trial is uNotice that notation l,u in Lecture 3 not necessarily in (0,1) as in Lecture 1
- \bullet Notation x_k in Lecture 4 denotes number of successes observed in treatment arm k