## Short course on Response-Adaptive Methods for Clinical Trials

## October 22, 2024

## Notation sheet:

- 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  $\theta$ , 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  $Y^{(i)}$ ,  $X^{(i)}$  for covariate(s) and proportions  $\rho^{(i)}$ .
- E(.) denotes expectation
- $\mathbb{1}_C$  is an indicator function taking value 1 iff condition C holds
- 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  $\rho$
- 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  $\alpha$  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