Bayesian analysis of clinical trials

- Bayesian methods are being increasingly used for analysis of clinical trials.

- Incorporating prior information can increase efficiency and thus require fewer resources.

- Bayesian methods are particularly useful for adaptive trials.

- In adaptive trials, decisions about trial design (allocation of treatments or doses) change as data accrue.

- We will briefly study Bayesian versions of sample size calculations and adaptive trial design.
Sample size calculations

- “Everyone is Bayesian at the design stage.”

- Classic problem: select the sample size needed to ensure the trial has high power to detect a treatment difference.

- How to pick the required inputs?

- Bayesian approach:
Sample size calculations

- The power of the trial is:

- MCMC approximation:

  - This requires both a design prior and an analysis prior.

- Handout!
Trials for equivalence

• Sometimes the goal of a clinical trial is to demonstrate that two treatments are equivalent.
  For example:

• The hypotheses are:

  • Bayesian approach:
Interim monitoring

- Rather than collecting all $n$ observations before doing analysis, it is common to take several “looks” at the data and adapt the trial accordingly.

- The trial may be stopped if:

  - In other cases the allocation of treatments may be based on current parameter estimates.
  - A Bayesian approach uses posterior predictive probabilities to decide when to stop the trial.
  - Say we have $n_1$ observations and we are considering collecting an additional $n_2$ observations.
  - We could compute the power to reject the null if the additional $n_2$ observations are collected, and stop the trial if power is near zero or one.
  - The power can be approximated as: