Comparison Groups

Common Probability Models

Defining the study structure

One arm trial: Historical or matched controls
- comparison to absolute reference

Two arm trial: Placebo or Active controls
- comparison between arms

Multiple arm trial: Several treatments or doses
- global tests vs pairwise comparisons

Regression trial: Continuous dose response
- test slope parameters

Also need to consider randomization ratio across treatment arms (or distribution of dose levels)

Probability Model: Comparison Groups

No comparison group

Appropriate when an absolute criterion for treatment effect exists

Single arm clinical trial
- Cohort design

(Rarely do such absolute criteria exist. Instead, we are really invoking the use of results from previous investigations.)

Probability Model: Comparison Groups

Historical controls

Single arm clinical trial

Compare results to
- Absolute criterion derived from historical trials
- Sample from historical clinical trial (better)

Probability Model: Comparison Groups

Comment re use of single arm trials

"There are only two types of researchers:

- those with a lot of enthusiasm and no controls, and
- those with a lot of controls and no enthusiasm."

(unknown)

Probability Model: Comparison Groups

Internal controls

Each subject serves as his/her own control

- Different treatments at different times
- Different treatments for different parts of body
  - eye diseases, skin diseases

Probability Model: Comparison Groups

Concurrent control group

Two or more treatment arms

- Placebo or standard therapy
- Active treatments
  - Sometimes consider equivalence
- Multiple levels of same treatment
  - Stronger evidence sometimes obtained from dose-response
  - Identifying optimal dose