STATS 275: Statistical Consulting
Design of Studies [1]

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[1] Based on "Principles of Applied Statistics" by Cox and Donnelly

Why we design studies?

- To avoid systematic error arising from irrelevant sources that do not cancel out in the long term
- To reduce non-systematic error to a reasonable level by replication and other appropriate techniques
- To estimate realistically the extent of uncertainty in the final conclusion
- To ensure the scale of effort is appropriate; Goldilocks rule: not too limited, not too wasteful, just right!

What is involved in a study design?

- Units of analysis (e.g., patients, patient-month)
- Deciding the type of study: observational studies vs. experiments
- Avoiding systematic error using balanced design or adjustment
- Controlling random error by replication and inclusion of background variables
- Controlling the scale of effort to achieve a certain power of significance tests or a desired value for the standard error of the estimates.

Sampling a specific population

- Learning about a specific target population by studying a subset (sample)
  - Simple random sampling
  - Stratification
  - Use of control variables
  - Multi-stage and temporal sampling

Experiment

- Investigating *treatment* effect by assigning *experimental units* to different treatments and observing one or more *responses*
  - Randomization
  - Forming blocks of units
  - Replication

Observational study

- Investigators are typically passive observers (either cannot control the system or choose not to) investigating the relationship between various features of units of analysis
  - Cross-sectional: Individuals are observed at one point in time, although some background information might also be used
  - Prospective observational studies: following a group or cohort of individuals forward in time
  - Retrospective observational studies: for each identified case, one or more controls are included in the sample, and explanatory features of all units of study are determined retrospectively