Mathematics 120: Lecture 2

Design of Experiments

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Designing an experiment

- Suppose a new treatment (such as a new drug, a new surgery procedure, or a new manufacturing process) is thought to be an improvement on the treatment currently in use.
- An investigator may conduct an experiment to compare the treatments.
- To do so, she would first find a group of subjects.
- The subjects are divided into a *treatment group* and a *control group*.
- The control group receives the old treatment, or perhaps no treatment.
- Note: if there is an old treatment, there might be three groups in the experiment: a group which receives the new treatment, a group which receives the old treatment, and a group which receives no treatment.
- At the end of the experiment, the investigator compares the differences in success rates between the groups.

Randomized controlled experiments

- The best experiments (for statistical purposes) are
  - Controlled - The subjects are divided into treatment and control groups.
  - Randomized - The subjects are assigned to groups by some random mechanism.
  - Double-blind - Neither the subjects, nor those who evaluate the responses, know which group they are in.
- Moreover, any group which does not receive a treatment should receive a *placebo*, that is, a treatment that looks like the actual treatment, but is known to have no effect (for example, a sugar pill).

Randomized controlled experiments (cont'd)

- Importance of randomization:
  - Subjects will differ in their susceptibility to the effects under investigation.
  - If, say, one particular subject is more susceptible than another, randomization ensures that that subject is equally likely to be in any of the groups.
  - Consequence: it is possible to use mathematics to compute the probability that any observed difference in response to the treatment amongst the groups can be attributed just to randomness.
  - That is: it may be the case that the randomization technique put more susceptible cases into the treatment group than into the control group, but the probability that this happens can be calculated.

Examples

- Salk vaccine trial
- Portacaval shunt
- Coronary bypass surgery

Observational studies

- In an *observational study*, the investigator has no control over the groups and merely observes the effects of the various treatments (or lack of treatment).
- Example: Studies of the effects of smoking on health.
- Observational studies may establish an association between a treatment and an outcome.
  - But association does not necessarily mean causation.
  - In particular, there may be a *confounding* factor, something which is associated with both the treatment and the effect being studied.
- Example
  - R. A. Fisher suspected that the association between smoking and lung cancer was in fact confounded by some genetic factor.
  - Subsequent studies of twins have shown there not be a genetic confounding variable.
- Example: Non-randomized surveys are very susceptible to confounding.

Examples

- Clofibrate trial
- Pellagra
- Ultrasound
- Sex bias in graduate school admission