STAT 3504

Purpose of Course

To look at the principles involved in the design and the analysis of comparative studies, both experimental and observational.

How the data is collected (design of study) is the most important part of any study as no amount of sophisticated analysis can compensate for improperly collected data. It may invalidate the entire study. Thus we will start by giving some necessary definitions, then look at what constitutes a well-planned study. Some common experimental designs will be briefly described. We will then look at some of the basic analysis methods for comparative studies (observational or experimental) and finally go back to further consideration of experimental design.

What Are Comparative Studies?

Comparative studies are basically studies to compare the means of a response variable for several different treatment populations. That is, for studying the effect on a response variable (quantitative) of 1 or more explanatory variables called, factors (qualitative or quantitative), when the interest lies in the differences between the true population means of the response variable under different conditions or treatments.

Terminology and Definitions

Study Unit
The object (person, thing, material, etc) on which the response variable measurement is made.

Experimental Study
The values of the explanatory variables (factors) are controlled (set beforehand) by the experimenter AND the treatments are RANDOMLY ASSIGNED to the experimental units.

Observational Study
Random samples are obtained without controlling the explanatory variables (factors). There is no random assignment of treatments to study units.

Factor
An explanatory variable whose effect on the response variable of interest is to be studied.

Factor Level
A particular value of the factor.

Experimental Factor
The levels of the factor are decided on (controlled) by the experimenter and assigned at random to the experimental units.

Observational or Classification Factor
The study units are observed and then classified by level of factor. There is no random assignment of factor levels to the study units.

Qualitative Factor - levels differ in kind (as in colours of packaging)

Quantitative Factor - levels differ in magnitude (as in amounts of fertilizer: 10 , 20, 30 kilos)

Single Factor Study
Study the response to the r different levels of only <u>one</u> factor.

Multifactor Study
Several factors are studied simultaneously to find their effect on the response variable. Interest lies in the effects of each of the factors on the response and on whether the factors <u>interact</u>, and if so how.

Treatments
For a single factor study the **treatments** are the levels of the factor. For a multifactor study they are **combinations** of the levels of the factors.

Mixed Study

A multifactor study in which some factors are experimental and some are observational. (See Handout Example 3 below.)

<u>Example 1</u> - on class handout

Response Y = reduction in cholesterol
Study unit = a patient with high blood cholesterol
Experimental study - as patients (units) assigned at random to the treatments
Single qualitative factor = the medical treatment
3 factor levels: A, B, C
Treatments = the factor levels A,B,C (since a single factor study)
Experimental study – as patients (study units) are assigned at random to the treatments (the 3 levels A,B.C)

<u>Example 2</u> - on class handout

Response Y = concentration of cadmium
Study unit = a water specimen
Single qualitative factor = stream
5 factor levels: the 5 different streams
Treatments = the 5 different streams (since a single factor study so treatments = factor levels)
Observational study - as can't assign the water specimens (study units) to the treatments (streams) - or vice versa.

<u>Example 3</u>

Response variable Y: not given
Study unit = trainee mechanic
2 factor study
factors: "training centre" with 3 levels - the 3 different training centres
"training program" with 2 levels – the 2 different training programs
Treatments: the 2X3 = 6 factor level combinations shown below
training centre 1 & training program 1, training centre 1 & training program 2
training centre 2 & training program 1, training centre 2 & training program 2
training centre 3 & training program 1, training centre 3 & training program 2

Mixed study - since

- one factor, "training centre" is observational as trainees were not randomly assigned to the centres (they belonged to one centre or the other). That is, the study units were not randomly assigned to the factor levels
- other factor, "training program" is experimental since at each centre the trainees were randomly assigned to the 2 programs by the experimenter. That is, the study units were randomly assigned to the factor levels.

Planning a Comparative Study

The acquisition of "good" data depends on the amount of planning that is devoted to it. The planning of observational studies and experimental studies has some common steps and of course some basic differences. The basic elements are given below.

Approach to Conducting a Well Designed Comparative Study

1. Clearly state the objectives of the study and what you want to achieve (give research hypothesis if applicable).
2. Define the response variable of interest (and how it is to be measured).
3. Specify the treatments to be compared. That is, what factors are to be studied and at what levels? Is a control treatment needed? (A control treatment is a "benchmark" treatment against which the other treatments are compared. eg a placebo treatment in drug studies.)
4. Consider what factors (variables), other than the ones we want to investigate, might affect the response variable. How should such extraneous factors be dealt with? This differs for observational and experimental studies.
5. Specify the study units. They should be representative of the population about which you want to draw conclusions. Eg. If you want to compare the effect of 4 different diet regimes on all people between 20 and 65, do not choose university students as your study units. Most students are under 30 and hence it could not be assumed that your results would apply to people over 30.
6. Consider the type of randomization (CRD, RBD, BIBD, etc) to be used for an experimental study. I.e. how to assign the treatments to the experimental units. Or, for an observational study, how random samples are to be taken .
7. Specify the model.
8. Outline the analysis to be used including hypotheses to be tested and C.I.s to be calculated. This determines how to do the calculations for step 9. It also checks that your study design is suitable for achieving the objectives.
9. Calculate how many replications (repetitions of experiment for experimental study), or number of observations on each treatment (observational study) are needed to achieve objectives – too few and study may be inconclusive- too many and you waste time & money.
10. Review above decisions and revise if necessary.

Some Important Concepts and Definitions in Experimental Design

Purpose of Experimental Design - to provide a maximum amount of information at a minimum cost i.e. statistical efficiency and economy of resources (same as for a sample survey).

Definitions

Experimental Unit - The element or *group of elements* to which a treatment is randomly assigned in one repetition of the experiment. It has an *independent chance of receiving any one of the treatments*.

eg
Consider an experiment to compare the weight gain of rats under 4 different diets (4 treatments). There are 4 cages of 3 rats each. The 4 treatments (diets) are assigned at random to the 4 cages. Here cages, not rats are the experimental units since the diets were assigned to the cages and the animals within a cage do not have and independent chance of receiving any one of the treatments (all the rats in a cage get the same diet).

Replication - Repetition of a trial of the experiment, that is, each treatment is applied independently to 2 or more experimental units.

N.B. In the above example there is no replication since each diet (trt) is applied to only 1 cage (exptl unit).

Experimental Error - Variation among experimental units treated alike. That is, it is the failure of identically treated experimental units to give the same response. It measures: i) natural variability among experimental units ii) variability in the conduct of the experiment.

Fundamental Components of an Experimental Design

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Randomization: the random assignment of treatments to experimental units (or vice versa) so that every experimental unit has an independent chance of receiving any one of the treatment. Its purpose is to prevent personal and systematic biases, both those that are unknown and those that are known but uncontrollable. That is, it helps ensure that, on average, no treatment is either favoured or hindered in the experiment. It can also be shown that randomization allows us to analyse the data as if we had independently and normally distributed error terms and hence justifies the use of F and T distributions.

> Note: Randomization may be complete as in a CRD, (completely randomized design) or restricted as in a RBD (randomized block design).

Replication - Without replication there is no measurement of experimental error. Note that there is a difference between "replication" and "repeated observations".

In the above rat example there is no measurement of experimental error since there is no replication. The measurements on the rats within a cage are repeated observations on the same experimental unit.

Local Control: the use of balancing, blocking, use of covariates, etc to reduce experimental error (thus making tests of significance more powerful and confidence interval estimates shorter).

NOTE

Experimental factors provide information about **cause and effect relationships** between these factors and the response variable since randomization tends to balance out between the treatments whatever systematic effects may be present (known or hidden), so that comparisons between treatments measure only the treatment effects. This means that randomization tends to eliminate the effects of any other, not included, variables that might affect the response.

Observational factors can only provide association relationships between the factor and the response.

E.g. Consider a study to compare the deaths in automobile accidents for those who used seat belts and those who didn't. Seat belt use (levels yes, no) has to be an observational factor as it would be completely unethical to assign no seat belt use to subjects. But this means it is only possible to conclude that lack of seat belt use is associated with a greater death rate not that it **causes** a greater death rate, since it is possible that the result was affected by an extraneous variable such as "amount of risk taking while driving". That is the larger death rate for nonusers of seat belts could be due to the fact they also took more driving risks in general.

Thus the great advantage of an experimental factor over an observational one is that it is often possible for cause and effect to be inferred if the factor is experimental but not if it is observational.

Brief Description of Some Common Experimental Designs

What is an Experimental Design?

An experimental design is simply the rule that determines the random assignment of experimental units to treatments. The simplest experimental design is the CRD and the simplest CRD is one for which there is only a single factor being considered (exptl units assigned to factor levels completely at random).

E.g. example 4 on the class examples I handout is an example of a single factor CRD – factor diet and the 50 men assigned completely at random to the 5 diets.

Completely Randomized Design (CRD)

- Randomization is unrestricted,
- Treatments are assigned to experimental units (or vice versa) completely at random: randomly select n_1 experimental units from the n_T available and assign them to treatment 1; randomly select n_2 experimental units from the remaining the n_T - n_1 and assign them to treatment 2, etc.

NOTE 1: All variation in the responses, other than that due to treatments, appears in the experimental error.

SSTO = SSTR + SSE

NOTE 2: Treatments may refer to the factor levels of a single factor experiment or to the factor level combinations of a multifactor experiment.

Randomized (Complete) Block Design (RBD)

- It is a restricted randomization design.
- The standard RBD has 1 observation on each block-treatment combination.
- If there are r treatments

1. Classify the experimental units into n **blocks** of r experimental units each in such a way that the experimental units **within** a block are as homogeneous (similar) as possible.
2. Assign the r treatments at **random** to the r experimental units **within each block**.

Here SSTO = SSTR + SSB + SSE thus SSB is removed from the experimental error.

Note: As for a CRD, treatments may refer to the levels of a single factor or to the factor level combinations of a multifactor experiment.

Example 1:
It is desired to carry out an experiment to compare the average litres/100 km for 3 different brands of gas (A,B,C). A random sample of 12 cars is available for the experiment (12 experimental units).

Using a CRD - Randomly assign 4 cars to each brand of gas and test run under the same conditions. Here, if the cars are not similar, there will be large variations in litres/100km due to the type of car (e.g. honda civic vs SUV) which will make it hard to detect any differences due to the brands of gas (i.e.the experimental error will be large).

Using a RBD - Divide the 12 cars into 4 fairly similar blocks of 3 vehicles each (based on their rated litres/100km). Randomly assign each of the 3 brands of gas to the 3 vehicles **within each block**. This reduces the experimental error by removing the differences between cars from it, thus making it easier to detect any differences between the brands of gas.

Repeated Measures Design

Repeated measures designs use the same *subject* (person, store, plant, test market, experimental animals, cities, etc) for each of the treatments under study. The treatment order is randomly assigned within each subject. Thus, the subject serves as a block and the experimental units within a block may be viewed as the different occasions when a treatment is applied to the subject. Note that here the *subjects* are the study units but NOT the experimental units. 3 different examples of repeated measures designs are given below.
Note that the key characteristic that distinguishes repeated measures designs from the other designs is that the same subject is measured repeatedly.

Examples of Repeated Measures Designs

Example a
15 test markets are to be used to study each of 2 different advertising campaigns. In each test market, the order of the 2 campaigns will be randomized, with a sufficient time lapse between the 2 campaigns that the effects of the initial campaign will not carry over into the next one. The subjects are the test markets.

Example b
200 persons who have persistent migraine headaches are each to be given 2 different drugs and a placebo, for 2 weeks each, with the order of the drugs randomized for each person. The subjects in the study are the persons with migraine headaches.

Example c
In a weight loss study, 100 overweight persons are to be given the same diet and their weights measured at the end of each week for 12 weeks to assess the weight loss over time. Here the subjects are the overweight people, who are observed repeatedly to provide information about a single treatment over time.

Example 2

In example 1 above to test differences in gas brands, the experiment could also be carried as a repeated measures design, by having each brand of gas tested by each of the 12 cars with the order of the 3 brands randomized for each car. Here the subjects are the cars.