Chapter 10: Experiments and Observational Studies

We will consider two types of study designs:

- **Observational study:** a study in which researchers observe the subjects in their natural surroundings and record the variable(s) of interest, without attempting to control the subjects in any way.
- **Randomized experiment:** a study in which researchers randomly assign subjects to different treatments and then observe and compare the responses.

When examining the relationship between two variables, researchers define one to be the **explanatory variable** and the other to be the outcome or **response variable**. An **explanatory variable** is a variable that may influence or "explain" differences in a **response variable**. For example, in an observational study to determine if students who play musical instruments have higher grades than students who do not, the explanatory variable records whether or not a student plays a musical instrument, whereas the response variable records the students' grades. A randomized experiment or observational study may involve more than one variable of each type. The explanatory variables in an experiment are called **factors**.

Observational Studies

In an observational study,

- researchers **observe** differences in the explanatory variable and note whether these are related to differences in the response variable.
- researchers do **not** assign subjects to treatments.
- there is **no** manipulation of factors.

For example, in the observational study (given in the chapter 10 of the textbook) to determine if students who play musical instruments throughout high school have higher grades than students who do not, the researchers compared the academic records of some students who had chosen to take music classes with the academic records of some students who did not. The researchers did not assign students to take music classes. The students decided for themselves whether or not to take music classes. The researchers only observed the students.

Types of Observational Studies:

a) **Retrospective study:** an observational study in which subjects are asked to recall **past events** or previously recorded data about the subjects is examined.
   - data is collected and examined on events that have **already taken place**.
   - for example, the musical instrument / academic performance study is a retrospective study since the researchers selected subjects (students who had and had not taken music classes) and examined their past grades.
   - often used to study rare outcomes, such as rare diseases
   - may contain errors

b) **Prospective study:** an observational study which identifies subjects in advance and collects data as events unfold.
   - more reliable than retrospective studies

The main drawback of observational studies is that they **cannot** demonstrate a causal relationship between variables. Observational studies can help us to discover possible associations between two variables, but **association does not imply causation**. There may be a **lurking variable** which is influencing both of the variables being investigated and explains their association. For example, in an observational study involving elementary school children, we may notice a positive relationship between the number of cavities a child has and the size of the child's vocabulary. So does a child's vocabulary cause cavities? No, in this case there is a lurking variable, namely the child's age. Older children tend to have both a larger vocabulary and more cavities than younger children.

Randomized Comparative Experiments

In order to find evidence to support causal relationships, researchers use randomized experiments. Unfortunately, experiments are not always possible and observational studies have to be used instead. It may be unethical or impossible to randomly assign subjects to a specific treatment. For example, to study the relationship between smoking cigarettes and heart disease, it would be considered unethical to conduct an experiment in which people are randomly assigned to start smoking cigarettes.

The **factors** in an experiment are the explanatory variables that researchers want to manipulate (deliberately change). The specific values that researchers pick for a factor are called **levels**. For example, in an experiment to test the effect of the amount of sleep a person has had on their driving performance, the factor is amount of sleep the night before the experiment and the levels could be no sleep, 2 hours, 4 hours, 6 hours, and 8 hours. The subjects of this experiment would be randomly assigned to these five **treatments**. (If there is more than one factor, then the treatments would be the possible combinations of the levels of all of the factors.)

In an experiment,

- there should be at least one factor to manipulate and at least one response variable to measure.
- experimental units or subjects are **randomly assigned** to treatments.
- the responses are observed and compared across all treatment groups (levels).

There are four main principles of experimental design:

- **Control:** control sources of variation (other than the factors being testing) by making conditions for all of the treatment groups as similar as possible.
- **Randomize:** assign individuals randomly to different treatment groups.
  - helps to reduce bias from uncontrolled / unknown sources of variation.
- **Replicate:**
  - apply each treatment level to several subject (not just one subject).
  - repeat the entire experiment with a completely different set of subjects.
- **Block**: group similar subjects together into **blocks**. Within each block, randomly assign the subjects to the treatments.

In an experiment, bias might result if those involved in the experiment know which subjects have been assigned to each treatment. **Blinding** is the practice of keeping this information hidden from individuals involved in an experiment. The outcome of an experiment might be affected by two categories of individuals:

- individuals who might influence the results (subjects, technicians, those who administer the treatments).
- individuals who evaluate the results (researchers, physicians who examine subjects at the end of the experiment).

An experiment is said to be **single-blind** if **all** individuals in one of these categories are blinded. An experiment is said to be **double-blind** if **all** individuals in **both** of these categories are blinded.

One way to blind individuals involved in an experiment is to use a **placebo**, that is, a fake treatment which appears to be the same as the real treatment being tested. For example, in a clinical trial for a new medication, researchers could give one treatment group the drug being tested and the other a fake pill (the placebo). In this example, the second group is called a **control group**. Data collected from control groups give researchers baseline measurements to use for comparison purposes. Control groups either receive no treatment (which includes receiving a placebo) or the best alternative treatment available to the one being tested.

In an observational study or randomized experiment, the subjects have to first be selected to be in the study, which may or may not have involved random sampling. Experiments rarely use random sampling to find subjects. Once the subjects are selected, in a randomized experiment the subjects are randomly assigned to a treatment, whereas in an observational study the subjects are not assigned to a treatment. So random sampling and random assignment refer to methods used at different stages in a study design: random sampling is a method of selecting subjects for a study and random assignment is a method of assigning subjects to treatment groups after they have been selected for the study.

Whether or not a study has used random sampling or random assignment determines what kinds of **inferences** can be made from the study. We will consider two types of inferences:

- **Population Inference:** drawing conclusions or making predictions / generalizations about a population based upon information in a sample.
  - for example, using sample statistics to estimate population parameters.
  - **random sampling** is required to make population inferences.
- **Causal (cause-and effect) Inference:** the differences in the outcomes of a response variable are caused by the differences in the explanatory variable.
  - **random assignment** of subjects to treatment groups is required to make causal inferences.
  - causal inference should only be made from data from randomized experiments (which use random assignment).
  - observational studies **cannot** be used to make causal inferences (no random assignment to protect against lurking variables).

The guidelines for when to use these types of inferences are given in the following table:

		Random Sampling?	
		Yes	No
Random Assignment?	Yes	can make **both** population and causal inferences	can make causal inferences, but **not** population inferences
	No	can make population inferences, but **not** causal inferences	can **not** make either type of inference