Chapter 2: Study Designs

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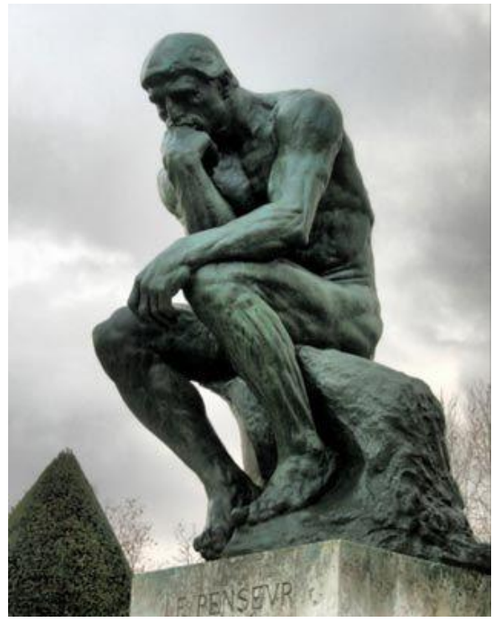
Studies

When increasing knowledge with the use of data, we need to make sure that when we collect data, that it will be capable of answering our scientific question of interest.

Study Design is the methodology that is used to collect the information to address the research question.

If I spend all the time and money to collect this data,
will it be able to answer my research question?

Will it cover the range of values that I am interested in.



Observational Studies vs. Randomized Studies

Observational Studies: Studies based on observations collected on subjects.

Example: Collect a data on cancer patients and non-cancer patients (Control). Determine the risk factors that are significantly different between the two groups.

Observational Studies vs. Randomized Studies

Randomized (Experimental) Studies: Studies in which a sample of subjects are selected, and then an intervention is applied to a random subsample.

Example: A sample of 100 subjects are taken. A random subsample of 50 subjects are assigned to a particular treatment, and the remaining 50 subjects are assigned to a placebo. Test if two groups are significantly different in terms of an outcome of interest.

2.1 Vocabulary

Bias – A systematic error that introduces uncertainty in estimates of effect or association.

Example: Every time I weigh myself on my home scale, I'm 5 lbs lighter than at the Doctors office.

Blind/double blind – The state whereby a participant is unaware of his or her treatment status. A study is said to be double blind when both the participant and outcome assessor are unaware of the treatment status.
Example: The researcher knows if I'm getting a med but I don't.
Example: Neither the researcher nor I know if I'm getting the med.

2.1 Vocabulary

Clinical Trial – A specific type of study involving human participants and randomization to the comparison groups.

Example: When the COVID vaccine was being tested, a random diverse group of human participants were recruited and assigned to receive and not receive it.

Cohort – A group of participants who usually share some common characteristics and who are monitored or followed over time.

Example: The initial group of COVID participants.

2.1 Vocabulary

Concurrent – At the same time; optimally comparison treatments are evaluated concurrently or in parallel.

Example: Two groups being observed in a study are at the same time in parallel and not one after the other.

Confounding – Complex relationship among variables that can distort relationships between the potential risk factors and the outcome.

Example: Studying how much coffee people drink but don’t account for the daily temperature.

2.1 Vocabulary

Cross Sectional – At a single point in time.

Example: Six weeks into drug study, I'm going to take the cross section of data and analyze it for progress.

Incidence (of disease) – The number of new cases (of disease) over a period of time.

Example: The flu incidence is 1 per 1000 in December.

2.1 Vocabulary

Per Protocol – An analytic strategy whereby only participants that adhered to the study protocol are analyzed.

Example: In my study the control group was supposed to not drink alcohol, but at check in we did blood draws and we can see which ones drank alcohol. We eliminated them from analysis.

Placebo – An inert substance designed to look, feel, and taste like the active or experimental treatment.

Example: Your studying weight loss drug and so you give one group the drug and another a sugar pill.

2.1 Vocabulary

Prevalence – The proportion of individuals with the condition at a single point in time.

Example: The prevalence of left-handed people is 1/6th in the US.

Prognostic factor – A characteristic that is strongly associated with an outcome such that it could be used to reasonably predict whether a person is likely to develop a disease or not.

Example: BMI is a prognostic factor for heart disease.

2.1 Vocabulary

Prospective – A study in which information is collected looking forward in time.

Example: A study gives a vaccine and observes how many people become infected.

Protocol – A step-by-step plan for a study that details every aspect of the study design and data collection plan.

Example: A clinical trial has a strict plan of how many participants are assigned to each group and exactly when and how much treatment they receive.

2.1 Vocabulary

Quasi-experimental design – A design in which subjects are not randomly assigned to treatments.

Example: A researcher conducts a study of patients at one hospital versus another. Subjects were not put in one pool and assigned to each group.

Randomization – A process by which participants are assigned to receive different treatments.

Example: A researcher has 100 participants and randomly assigns half to receive the treatment and half to receive the placebo.

2.1 Vocabulary

Retrospective – A study in which information is collected looking backward in time.

Example: A researcher at a hospital looks back in time in a database of patients that developed a disease.

Stratification – A process whereby participants are partitioned or separated into mutually exclusive or non-overlapping groups.

Example: A researcher partitions their study participants into age groups.

2.2 Observational Study

A **case report** is a detailed report of the specific features of a particular participant or case.

Example: A patient came into the hospital having very unique symptoms of a disease. The doctor after treating the patient, writes up the history, treatment, and outcome of this specific patient for a medical journal.

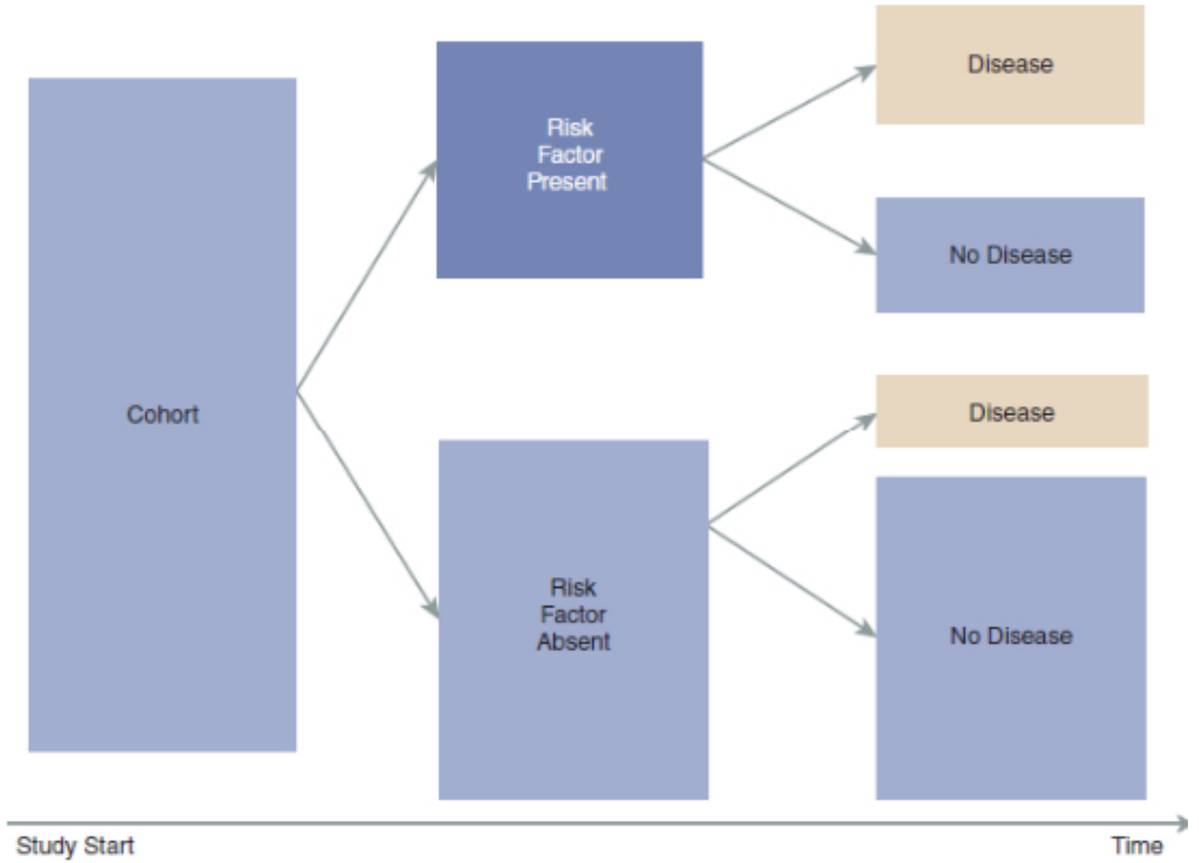
2.2 Observational Study Designs

A **cross-sectional survey** is a study conducted at a single point in time.

Example: The Centers for Disease Control (CDC) wants to determine the prevalence of tuberculosis in February. They conduct a survey at this point in time.

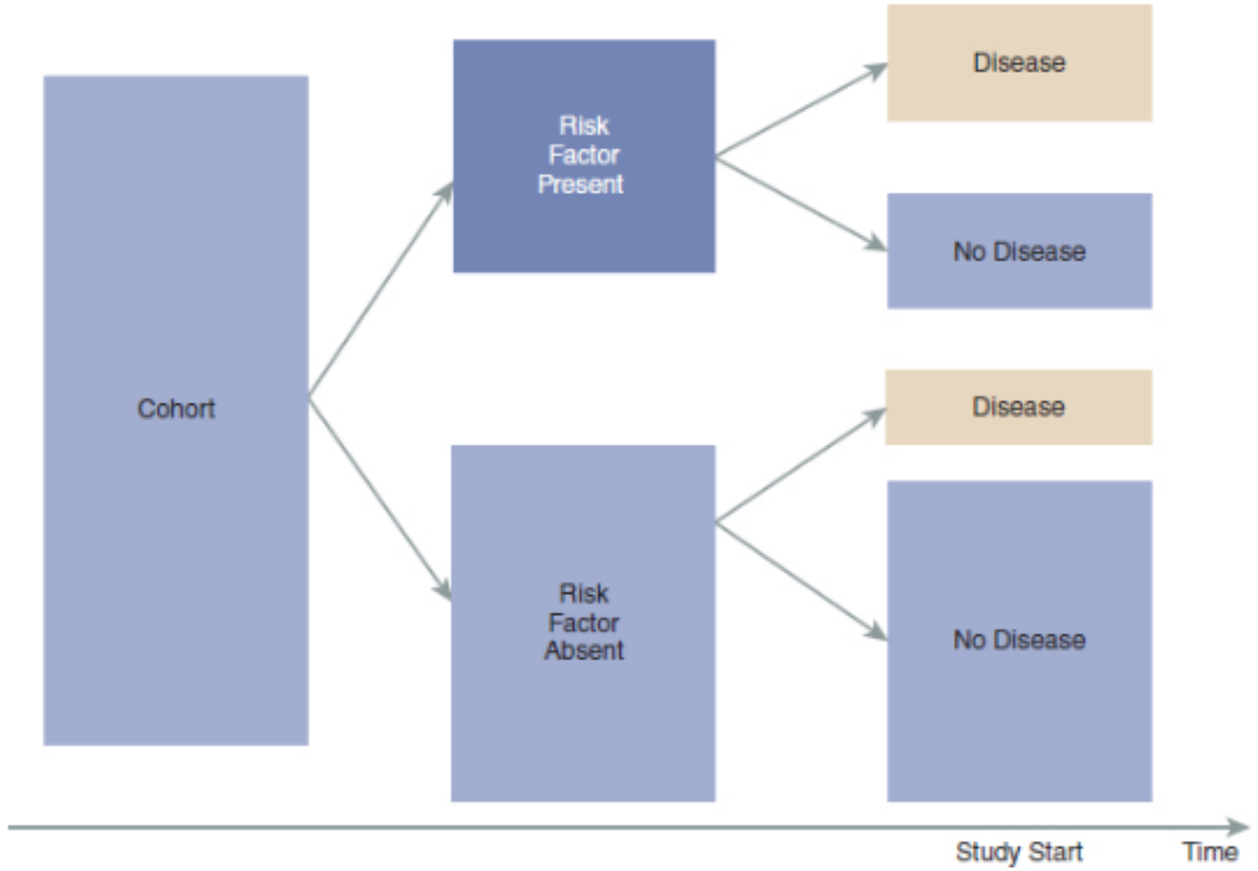
2.2 Observational Study Designs

A **cohort study** involves a group of individuals who usually meet a set of inclusion criteria at the start of the study.



2.2 Observational Study Designs

In a **retrospective cohort study**, the exposure or risk factor status of the participants is determined retrospectively, or looking back in time.

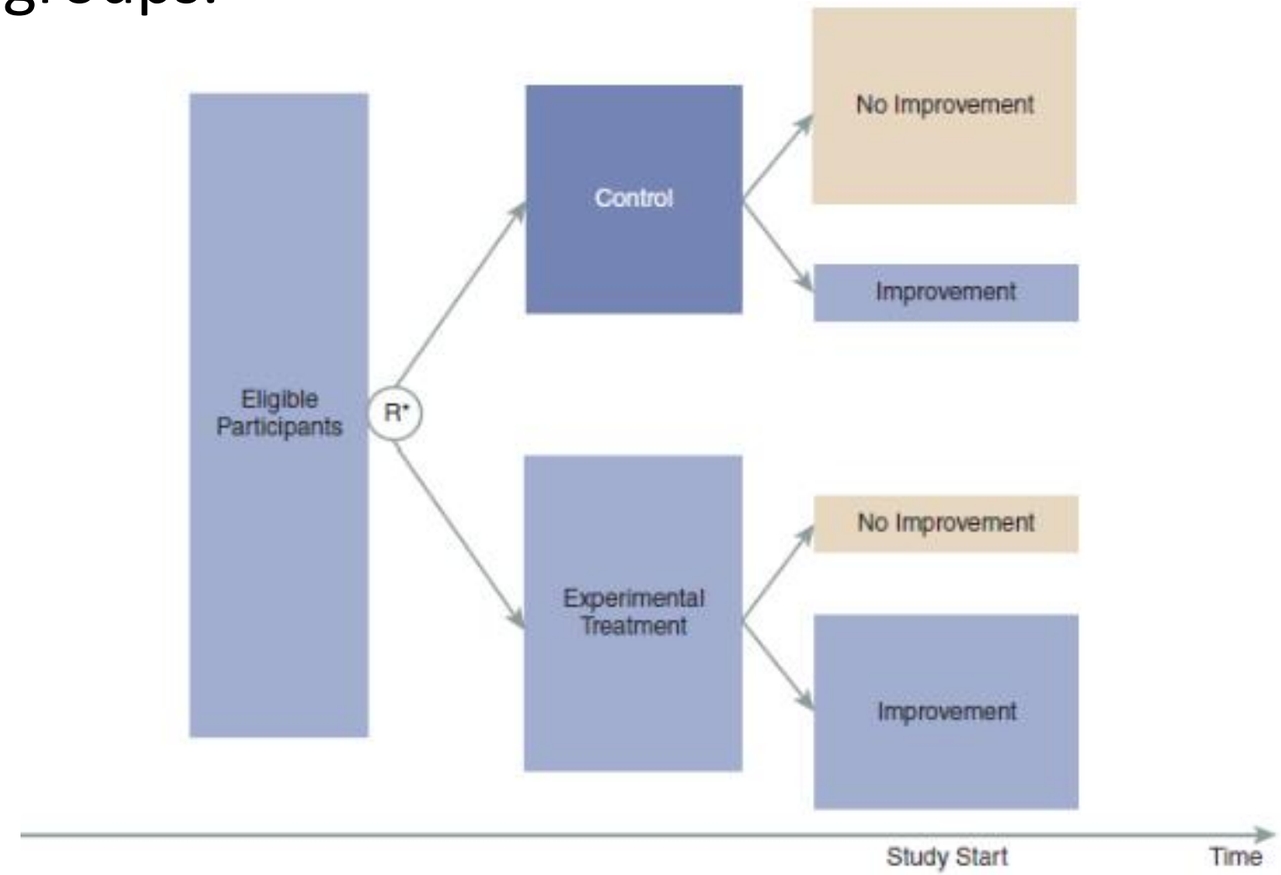


2.3 Observational Study Designs

The **case-control study** is a study often used in epidemiological research where again the question of interest is whether there is an association between a particular risk factor or exposure and an outcome.

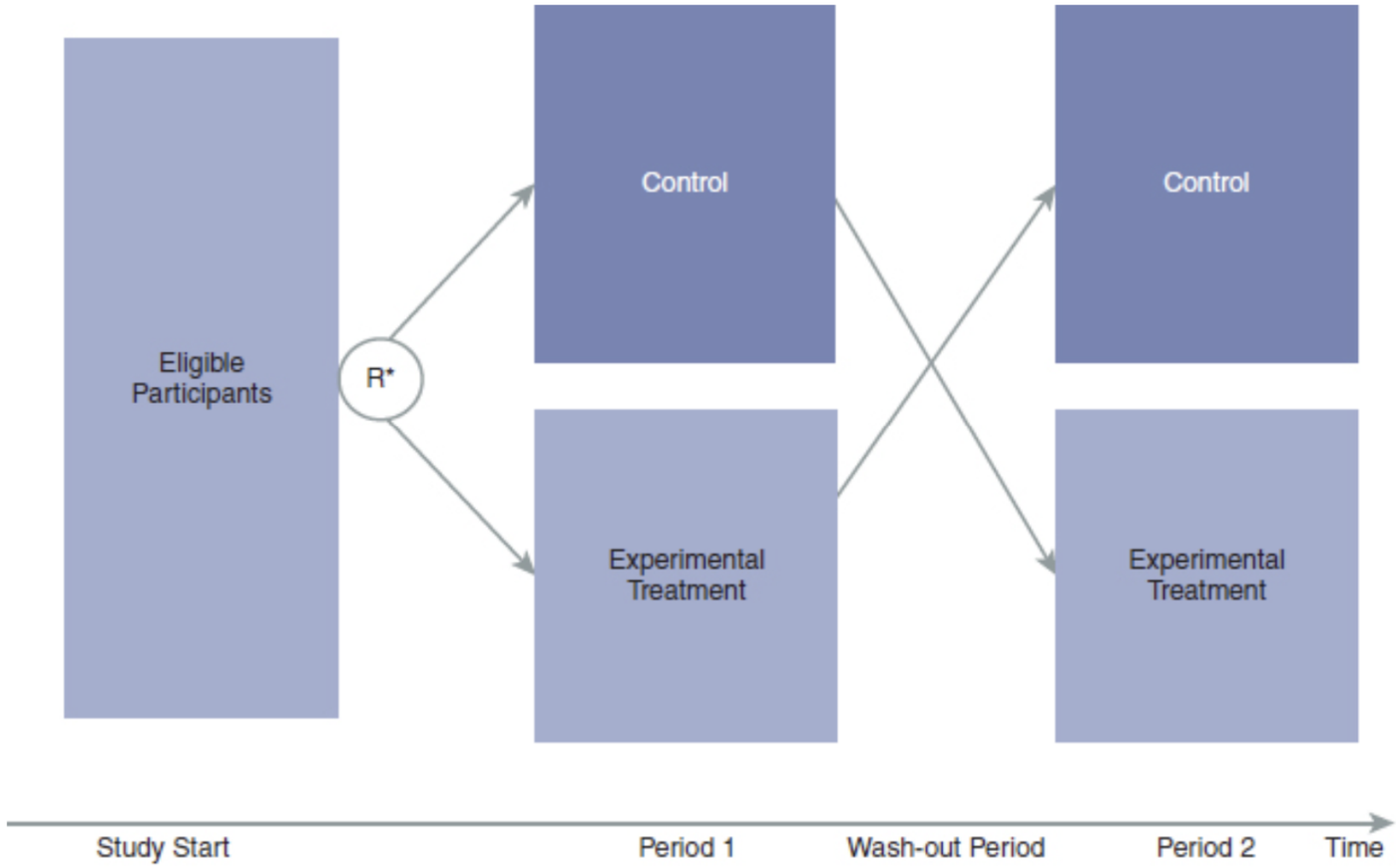
2.3 Randomized Study Designs

The **randomized controlled trial** is a design with a key and distinguishing feature – the randomization of participants to one of several comparison treatments or groups.



2.3 Randomized Study Designs

The **cross-over trial** is a clinical trial where each participant is assigned to two or more treatments sequentially.



Questions?

Homework 2

Read Chapter 2.

Problems # 12, 13, 14, 16