Study Designs Summary

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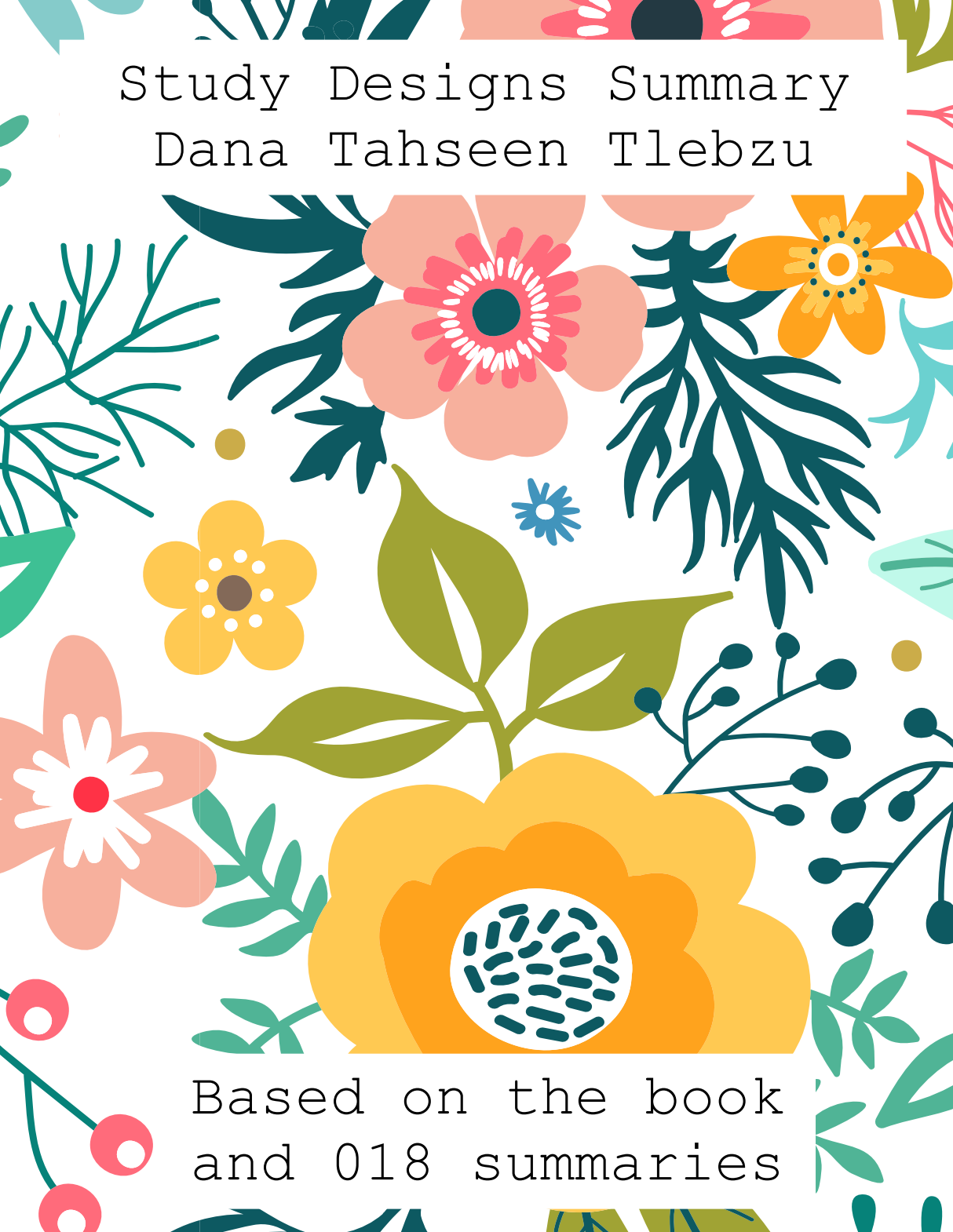


Based on the book and 018 summaries

FIGURE 8-1 Key Characteristics of a Case Series	
Objective	Describe a group of individuals with a disease.
Primary study question	What are the key characteristics of the cases included in the study?
Population	All individuals included in the study must have the same disease or disorder or have undergone the same procedure.
When to use this approach	A source of cases is available, and no comparison group is required or available.
Requirement	An appropriate source of cases is available.
First steps	1. Specify what new and important information the analysis will provide. 2. Identify a source of cases. 3. Assign a case definition. 4. Select the characteristics of the study population that will be described.
What to watch out for	A lack of generalizability
Key statistical measure	Only descriptive statistics are required.

Some case series for rare conditions may require only a handful of participants. Others may include several hundred or even several thousand individuals.

8.4 Analysis

Few numbers are required for most case series studies.

Some may report percentages (as descriptions not outcomes) **such as:**

- **Case fatality rate** (is the proportion of persons with a particular disease who die as a result of that condition)
- **Mortality rate** (is the percentage of members of a population who die of any condition during a specified time period)
- **Proportionate mortality rate** (is the proportion of deceased (dead) members of a population whose death was attributable to a particular cause)

FIGURE 9-1 Key Characteristics of Cross-Sectional Studies	
Objective	Describe the exposure and/or disease status of a population.
Primary study question	What is the prevalence of the exposure and/or disease in the population?
Population	The study participants must be representative of the source population from which they were drawn.
When to use this approach	Time is limited and/or the budget is small.
Requirement	The exposures and outcomes are relatively common, and the researchers expect to be able to recruit several hundred participants.
First steps	1. Define a source population. 2. Develop a strategy for recruiting a representative sample. 3. Decide on the methods to be used for data collection.
What to watch out for	Non-representativeness of the study population
Key statistical measure	Prevalence

Because cross-sectional studies are time- and cost-effective, they are the most popular approach used for descriptive epidemiology.

9.5 Analysis: Prevalence

We have previously discussed the prevalence which calculated at a one point in time, but sometimes we need to calculate the prevalence over a short duration of time, with all data collected within a few days, weeks, or months; therefore we call it the prevalence rate.

Prevalence Rate: the percentage of the population with a given trait at the time of the survey.

Prevalence rate ratio: ratios that compare prevalence of a characteristic in two population subgroups.

FIGURE 10-1 Key Characteristics of Case–Control Studies

Objective	**Compare exposure histories of people with a disease (cases) and people without that disease (controls).**
Primary study question	Do cases and controls have different exposure histories?
Population	Cases and controls must be similar except for their disease status.
When to use this approach	The disease is relatively uncommon, but a source of cases is available.
Requirement	A source of cases is available.
First steps	1. Identify a source of cases. 2. Assign a case definition. 3. Decide what type of control population will be appropriate for the study. 4. Decide whether cases and controls will be matched.
What to watch out for	Recall bias
Key statistical measure	Odds ratio (OR)

Now,

Odds: Compares the likelihood of having had a particular exposure to not having had it.

***Odds* ratio**: Compares the odds of exposure among cases to the odds among control The main measure of association in case-control study

If OR:

=1 (the odds of exposures for cases and controls are the same)

>1 (Cases had higher odds of exposure than controls, implying that the **exposure was risky.**)

<1 (Cases had lower odds of exposure than controls, implying that the **exposure was protective.**)

IF the C.I is entirely **lower than 1 then the odd ratio is statistically significant** ,so the exposure is **protective**.

IF the C.I is entirely **more than 1 then the odd ratio is statistically significant** ,so the exposure is **risky**.

IF the C.I overlaps **OR=1 then the odd ratio not statistically significant** in study population.

P -value>0.05 indicates no association.

P-value < 0.05 indicates statistically significant association.

FIGURE 11-1 Key Characteristics of Cohort Studies

Approach	Prospective or Retrospective Cohort	Longitudinal Cohort
Objective	Compare rates of new (incident) disease over time in people with and without a particular well-defined exposure.	Follow a representative sample of a well-defined population forward in time to look for new (incident) diseases associated with a diversity of exposures.
Primary study question	Is exposure associated with an increased incidence of disease?	Is exposure associated with an increased incidence of disease?
Population	Participants must be similar except for exposure status.	Participants must be available for follow-up months or years after enrollment.
	Because the goal is to look for incident disease, no one can have the disease of interest at the start of the study.	The study participants must be reasonably representative of the population from which they were drawn.
When to use this approach	An exposure is relatively uncommon, but a source of exposed individuals is available.	The goal is to examine multiple exposures and multiple outcomes, and time is not a concern.
Requirement	A source of individuals with the exposure is available.	The research team has adequate time and money for the study.
First steps	1. Identify a source of individuals with the exposure. 2. Decide what type of unexposed individuals will be an appropriate comparison group.	1. Select a source population. 2. Select the exposures and outcomes that will be assessed. 3. Decide how often data will be collected. 4. Develop a strategy for minimizing the burden of participation and maximizing benefits and incentives.
What to watch out for	Loss to follow-up (prospective studies) or missing records (retrospective studies)	Loss to follow-up
	Information bias in which the exposed participants are more thoroughly examined for disease than unexposed participants	Potential data management challenges if a lot of information is collected at many points in time
Key statistical measure	Incidence rate ratio (RR, also called the relative risk)	Incidence rate ratio (RR, also called the relative risk)

analysis: ↓

11.4 Analysis: Incidence Rate Ratios (RRs):

- The ***incidence rate:*** the number of new cases of disease in a population during a specified period of time divided by the total number of persons in the population who were at risk during that period.

$$I.R = \frac{\text{no. of new cases} \times 1000}{\text{population at risk}}$$

$$**RR = \frac{\text{Incidence rate among exposed}}{\text{Incidence rate among unexposed}}$$

- **incidence rate ratio (RR):**

=1 The incidence rate was the same in exposed and unexposed groups

>1 The incidence rate was higher in exposed than unexposed ,indicating that the exposure was risky

<1 The incidence rate was lower in exposed than unexposed ,indicating that the exposure was protective

Note: we can calculate **Attributable (Excess risk) as follows :

Attributable risk(AR)=Incidence in exposed-Incidence in nonexposed

Which is represented by the striped area below

Attributable Risk percent: is the proportion of incident cases among the exposed that are due to exposure ,or it is the proportion of the cases of the disease in the exposed that could have been prevented if the exposure was removed .

$$AR\% = \frac{AR}{\text{Incidence in exposed}}$$

FIGURE 12-1 Key Characteristics of Experimental Studies	
Objective	Compare outcomes in participants assigned to an intervention or control group.
Primary study question	Does the exposure cause the outcome?
Population	Similar participants are randomly assigned to an intervention or control group.
When to use this approach	Assessing causality
Requirement	The experiment is ethically justifiable.
First steps	1. Decide on the intervention and eligibility criteria. 2. Define what will constitute a favorable outcome. 3. Decide what control is an appropriate comparison for the intervention. 4. Decide whether blinding will be used to prevent participants and/or the researchers who will assess outcomes from knowing whether a participant has been assigned to the intervention or the control group. 5. Select the method for randomizing participants to an intervention or control group.
What to watch out for	Noncompliance
Key statistical measure	Efficacy

A **controlled trial** is an experiment in which some of the participants are assigned to an intervention group and some are assigned to a nonactive comparison group. A very common experimental study design used in the health sciences is a **randomized controlled trial (RCT)** in which some participants are randomly assigned to an active intervention group, the remaining participants are assigned to a control group, and all participants from both groups are followed forward in time to see who has a favorable outcome and who does not (**Figure 12-2**). RCTs and all other types of experimental study designs require careful descriptions of:

- The intervention
- The type of control that will be used and why it is appropriate
- How participants will be assigned to exposure groups
- The end point that will constitute a favorable outcome for the trial

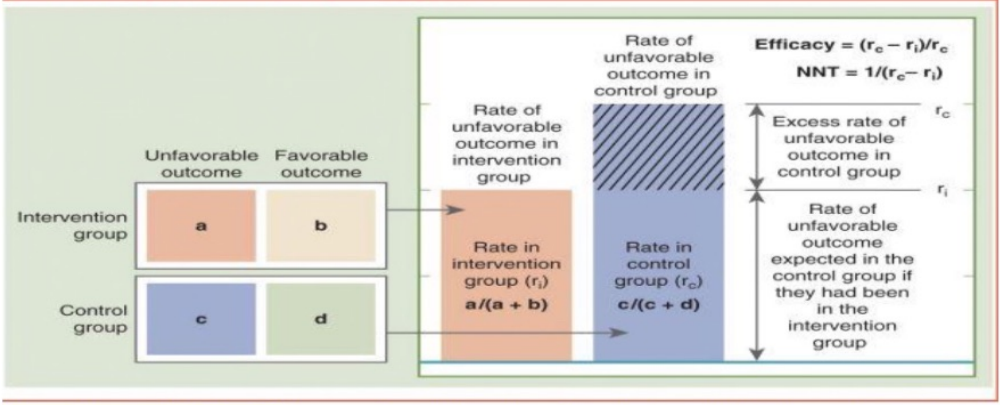
analysis: ↓

12.8 Efficacy:

Experimental studies use the statistics to quantify the impact of assigned exposure on the likelihood of having a favorable of an unfavorable outcome

Efficacy is a measure of the success of an intervention that is calculated as the proportion of individuals in the control group who experienced an unfavorable outcome but could have expected to have a favorable outcome if they had been assigned to the active group instead of the control group. A high efficacy is an indication that an intervention is successful. Efficacy typically refers to results under ideal circumstances,

FIGURE 12-9 Efficacy and Number Needed to Treat



- **The number needed to treat (NNT)** is the expected number of people who would have to receive a treatment to prevent an unfavourable outcome in one of those people (or, alternately stated, to achieve a favourable outcome in one person). A small NNT indicates a more effective intervention. If a drug intended to prevent stroke has an NNT of 5, then 5 people have to take the drug for 1 year (or some other specified time period) to prevent 1 of the 5 from having a stroke. If the drug has an NNT of 102, then 102 people have to take the drug to prevent 1 of the 102 from having a stroke.
- **The number needed to harm (NNH)** is the number of people who would need to receive a particular treatment in order to expect that one of those people would have a particular adverse outcome. A large NNH indicates a safer intervention. NNT and NNH are often used for cost-effectiveness analysis.

- **Effectiveness** is calculated with the same equation as efficacy but refers to results obtained under real-world, less-than-ideal conditions. For example, in a real-world setting, some participants might skip some doses of an

- Analysis for experimental studies typically uses either a **treatment-received approach** or **a treatment-assigned approach**.

  A treatment-received analysis of experimental data includes only the participants who were fully compliant with their assigned intervention or comparison protocol. Treatment-received analysis allows for the calculation of efficacy

  A treatment-assigned analysis (or intention-to-treat analysis) includes all participants, even if they were not fully compliant with their assigned protocol. Treatment-assigned analysis is better at measuring real-world (rather than ideal-world) effectiveness.

more related information: ↓

12.9 Screening and Diagnostic Test:

The goal of some studies of screening or diagnostic tests is to compare two assessment that are supposed to measure the same thing

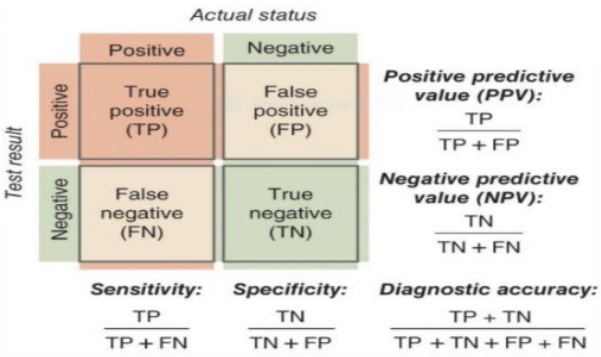
- A good test will have a value near 100% for the following **four** calculations :

1-**The sensitivity**, or true positive rate, is the proportion of people who actually have a disease (according to the reference standard) who test positive using the new test.

2-**The specificity**, or true negative rate, is the proportion of people who do not have the disease who test negative with the new test.

3-**The positive predictive value (PPV)** is the proportion of people who test positive with the new test who actually have the disease (according to the reference standard).

4-**The negative predictive value (NPV)** is the proportion of people who test negative who actually do not have the disease



For tests with a flexible cutoff point for defining positive and negative test results, there is always a trade-off between sensitivity and specificity (Figure 12-12). Increasing the sensitivity decreases the specificity. Increasing the specificity decreases the sensitivity. Consider the use of systolic blood pressure as a sign of hypertension

Three other measures are also commonly used for screening tests:

diagnostic accuracy -> is the percentage of the participants who where either turn positive or true negative.

positive likelihood ratio -› examine whether anew test is good at predicting the presence of disease.

negative likelihood ratio -› examine whether a new test is good at predicting the absence of disease.

FIGURE 14-1 Key Characteristics of Correlational Studies	
Objective	Compare average levels of exposure and disease in several populations.
Primary study question	Do populations with a higher rate of exposure have a higher rate of disease?
Population	Existing population-level data are used; there are no individual participants.
When to use this approach	The aim is to explore possible associations between an exposure and a disease using population-level data.
Requirement	The topic has not been previously explored using individual-level data.
First steps	1. Select the sources of data that will be used. 2. Decide on the variables to include in the analysis.
What to watch out for	The ecological fallacy Limited publication venues
Key statistical measure	Correlation

For most correlational studies, at least one characteristic of the populations being examined is designated as an exposure and at least one is designated as an outcome or disease. Correlational studies then examine exposure–outcome pairs. For example, a correlational study could answer questions like:

- Does the percentage of adults with multiple sclerosis tend to be higher in countries farther from the equator?
- Does the rate of asthma tend to be higher in cities with higher levels of air pollution?
- Does the prevalence of diabetes tend to be higher in provinces with a higher prevalence of obesity?

Statistical methods can be used to control for interactions among related variables.

Some correlational studies examine links between the socio-demographic characteristics of populations and health outcomes. The key exposure for this type of study might be the percentage of adults in a state who have completed at least 12 years of education, the mean household income in the state, or the median age of the state's population. Alternatively, the key exposure for a correlational study might be an environmental one, such as a city's distance from the equator, its number of rainy days in a typical year, or the city's average ultraviolet radiation index during midday in the hottest month of the year. All of these environmental measures are likely to be experienced fairly consistently across the entire population of interest. It is unlikely that part of one city would experience many more sunny days than another part of the city. A correlational study that explores an environmental exposure may be called an **ecological study**.

14.3 Correlation:

* the type of equation we use to represent the correlation depends on the level of the measurements of the variables (continuous, categorical ....)

* r^2 represent the significance

a- A positive slope → shows that higher levels of exposure are associated with higher rate of disease
b- A negative slope → snows that higher level of exposure are associated with lower rate of disease

- For a two-variable analysis, plot each population on a scatterplot with the "exposure" on the *x*-axis and the "outcome" on the *y*-axis.
- A best-fit line defines the correlation (*r*) between the two variables.
- Use linear regression to fit more complex models of

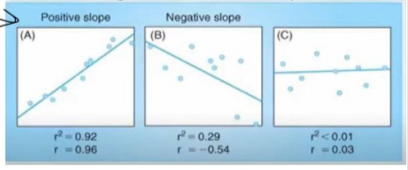


FIGURE 15-1 Key Characteristics of Reviews and Meta-analyses

Approach	Narrative Review	Systematic Review	Meta-analysis
Objective	Synthesize existing knowledge	Synthesize existing knowledge	Synthesize existing knowledge
Primary study question	What conclusions about this topic are supported by previous studies?	When all previously published studies on this topic are examined, what conclusions can be drawn?	When the results of all previously published studies on this topic are merged, what is the summary statistic?
Population	Published literature	Published literature	Published literature
When to use the approach	The goal is to describe a new perspective on a topic that can be supported by the existing literature.	The goal is to compare the findings of previous studies on a well-defined topic.	The goal is to summarize previous findings using pooled statistics.
Requirements	The researcher has excellent library access.	The researcher has excellent library access.	The researcher has excellent library access.
	The researcher has a unique perspective on the topic.	The researcher can obtain every relevant article.	The researcher has strong quantitative skills.
First steps	Decide what key message the article will convey.	1. Decide on the specific objectives of the review. 2. Select the search methods that will be used to find potentially relevant articles. 3. Select inclusion and exclusion criteria for articles.	1. Decide on the specific objectives of the review. 2. Select the search methods that will be used to find potentially relevant articles. 3. Select the inclusion and exclusion criteria for the articles. 4. Decide how to assess the quality of the studies. 5. Decide how the results of the studies will be combined into one summary statistic.
What to watch out for	Limited publication venues	Publication bias	Studies that cannot be fairly compared
Key statistical measure	No statistics are required.	No statistics are required, but reporting some results from included studies may be helpful.	Summary measures for included studies must be reported.

Conducting synthesis research is one way to become an expert in the literature on a well-defined topic. This knowledge is a good outcome in and of itself, and a tertiary analysis can also be a helpful step in preparing for future primary or secondary analyses. Well-written and comprehensive review articles often become foundational for new research in the field because they summarize what is known about an area of inquiry. Because reviews provide a concise summary of the literature, published review articles may be cited more frequently than the typical article reporting the results of a primary or secondary analysis.

There also some limitations associated with synthesis research. Not all journals publish review articles, especially reviews that the editors do not solicit, so the likelihood of publication might be lower for tertiary studies than for other study approaches. Also, reviews are sometimes regarded as exhibiting less originality than other types of scholarship. A good review requires meticulous library work followed by the careful compilation and interpretation of scientific information, yet reviews are sometimes perceived to be a less rigorous form of research than projects collecting new data or involving statistical analysis.