Study Designs

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   - Case Control
   - Cross-sectional
   - Cohort

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   - Randomized Trail

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Study designs

Key terms
Study designs

Key terms

Exposure - predictor event
- smoking status, amount of fats ingested, new surgery tool
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*Exposure*-predictor event
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*Outcome*-response event
- weight, presence of cancer, recovery time
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-age, gender, education level
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**Bias**-a systematic difference between results of a study and true measure
- recall, psychological, selection
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**Bias**-a systematic difference between results of a study and true measure
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**Relative Risk**-ratio of the probability of the event occurring in the exposed group versus a control group
Study designs

Key terms

Prevalence-the proportion of a population found to have a condition
Study designs

Key terms

*Prevalence* - the proportion of a population found to have a condition

*Populations* - group of individuals who share a common set of characteristic
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**Prevalence**-the proportion of a population found to have a condition

**Populations**-group of individuals who share a common set of characteristic
- location: neighborhood, country, desert
Study designs

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Key terms

**Prevalence** - the proportion of a population found to have a condition

**Populations** - group of individuals who share a common set of characteristic
- location: neighborhood, country, desert
- biological factors: age, race, genetic condition
- social factors: income level, education, religion
Study designs

Three main types of study designs
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Observational- researcher does not intervene
Study designs

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- Case Report
- Case Control
- Cross-sectional
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Systematic review- uses past studies
Case Report

**Goal**: detail the aspects of the patient’s medical situation
Case Report

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Lowest level of study design
Case Report

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Consists of a single case
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Common topic of case reports:
- An unexpected association between diseases or symptoms
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Common topic of case reports:
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- Unique or rare features of a disease
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Common topic of case reports:
- An unexpected association between diseases or symptoms
- An unexpected event in the course of observing or treating a patient
- Unique or rare features of a disease
- A variation of anatomical structures
Example

Dr. Rivers treats a young and otherwise healthy patient suffering from numbness over the patient’s body.
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After an exhaustive history, Dr. Rivers believes that the numbness occurred because of a new type of sunscreen the patient used.
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Dr. Rivers treats a young and otherwise healthy patient suffering from numbness over the patient’s body.

After an exhaustive history, Dr. Rivers believes that the numbness occurred because of a new type of sunscreen the patient used.

Dr. Rivers writes up a case report describing how the numbness presented, how and why she concluded it was the sunscreen, and how she treated the patient.
Advantages
Case Report

Advantages

- Requires only one patient
- Details many different aspects of the patient’s medical situation
- Quickly inform the medical community of new trends
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Disadvantages

- Case may not be generalizable
- May emphasize the bizarre or focus on misleading elements
Case Series

**Goal**: detail a medical condition in a collection of people that is rare or otherwise unknown

Dr. Rivers examines the original patient’s friends that also used the same sunscreen. Two of the six patients also present with similar symptoms. Dr. Rivers writes a case series for the three patients that presented with the numbness.
Case Series

**Goal**: detail a medical condition in a collection of people that is rare or otherwise unknown

Expands on case report by investigating several cases
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Case Series

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Case Series

Advantages
- Requires a small number of patients
- Details many different aspects of the patient’s medical situation
- Informs the medical community of new trends quickly
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- Requires a small number of patients
- Details many different aspects of the patient’s medical situation
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Disadvantages
- Selection bias
- Unknown causality
Case Control Study

**Goal**: determine if exposure and outcome are associated.
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Compares patients who have the outcome of interest (cases) with patients who do not have the outcome (controls)
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Subject are selected by outcome status and afterward exposure status is assessed.
Case Control Study

**Goal:** determine if exposure and outcome are associated.

Compares patients who have the outcome of interest (cases) with patients who do not have the outcome (controls)

Subject are selected by outcome status and afterward exposure status is assessed

Controls must be carefully chosen to match the population the cases are from
Example

Dr. Washburne has read about Dr. Rivers’ studies and wants to find out if the numbness and the sunscreen are associated.
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He finds 20 people that have experienced numbness (cases) and 20 people that have not (controls).
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He finds 20 people that have experienced numbness (cases) and 20 people that have not (controls).

Dr. Washburne interviewed all the people to find if they used the sunscreen and finds that 16 of the people with numbness used the sunscreen while only 2 people who did not experience numbness used it.
Case Control Study

Advantages

Require only a moderate amount of time to complete
Relatively inexpensive to carry out
Useful when outcomes are rare

Disadvantages

Subject to several biases
May be difficult to find an appropriate control group
Unknown causality
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Cross-sectional Study

**Goal:** determine prevalence of outcome for entire population
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Sample is based on the entire population, not just cases and controls
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**Goal:** determine prevalence of outcome for entire population

Sample is based on the entire population, not just cases and controls

Sample taken at one specific point in time
Cross-sectional Study

**Goal**: determine prevalence of outcome for entire population

Sample is based on the entire population, not just cases and controls

Sample taken at one specific point in time

Surveys are example of cross-sectional studies
Cross-sectional Study

Example

Dr. Jayne has heard that the new sunscreen and numbness are associated and wants to know if this condition is widespread.
Cross-sectional Study

Example

Dr. Jayne has heard that the new sunscreen and numbness are associated and wants to know if this condition is widespread.

Dr. Jayne collects information on a random sample of people across the state.
Cross-sectional Study

Example

Dr. Jayne has heard that the new sunscreen and numbness are associated and wants to know if this condition is wide spread

Dr. Jayne collects information on a random sample of people across the state

He finds that the risk of numbness is low for the general state population, but high for people between the ages of 13 and 18
Cross-sectional Study

Advantages
Cross-sectional Study

**Advantages**

- Results can be applied to the population
- Relatively low cost
Cross-sectional Study

**Advantages**
- Results can be applied to the population
- Relatively low cost

**Disadvantages**
- Does not work well for rare outcomes
- Unknown causality
- Recall bias
**Goal**: determine if the exposure precedes the outcome
Cohort Study

**Goal:** determine if the exposure precedes the outcome

Also call longitudinal studies or prospective studies
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Cohort is identified before the appearance of the outcome
Cohort Study

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Also call longitudinal studies or prospective studies

Cohort is identified before the appearance of the outcome

Exposure is assessed and then participants are followed for a set amount of time
Cohort Study

Example

After seeing the findings from Dr. Jayne, Drs. Kay and Lee want to know if the sunscreen precedes the numbness
Cohort Study

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After seeing the findings from Dr. Jayne, Drs. Kay and Lee want to know if the sunscreen precedes the numbness.

Focusing on people between the ages 13 and 18, the doctors finds 500 participants and follow them for two years.
Example

After seeing the findings from Dr. Jayne, Drs. Kay and Lee want to know if the sunscreen precedes the numbness.

Focusing on people between the ages 13 and 18, the doctors find 500 participants and follow them for two years.

Over the course of the two years, 60% of the participants who use the sunscreen experienced numbness.
Cohort Study

Advantages
Cohort Study

Advantages

- Risk can be assessed
- Accurate collection of information
- Less expensive than randomized trials
Cohort Study

**Advantages**
- Risk can be assessed
- Accurate collection of information
- Less expensive than randomized trials

**Disadvantages**
- More expensive than previous studies
- Confounding caused by no randomization
- Outcome may require a long time until it occurs
Goal: determine the efficacy or effectiveness of a exposure or treatment
Randomized Trail

**Goal**: determine the efficacy or effectiveness of a exposure or treatment

Also called Randomized Control Trail or Randomized Clinical Trail
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Subjects are randomly assigned to a treatment group
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Subjects are followed for a set amount of time
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Subjects are followed for a set amount of time

Various outcomes can be investigated
5 phases of clinical trials
Randomized Trial

5 phases of clinical trials

- Phase 0-Pharmacodynamics and Pharmacokinetics (10-15 subjects)
Randomized Trial

5 phases of clinical trials

- Phase 0 - Pharmacodynamics and Pharmacokinetics (10-15 subjects)
- Phase 1 - Screening for safety (20-100 subjects)
5 phases of clinical trials

- Phase 0 - Pharmacodynamics and Pharmacokinetics (10-15 subjects)
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- Phase 2 - Establishing the efficacy of the drug (100-300 subjects)
Randomized Trial

5 phases of clinical trials

- Phase 0 - Pharmacodynamics and Pharmacokinetics (10-15 subjects)
- Phase 1 - Screening for safety (20-100 subjects)
- Phase 2 - Establishing the efficacy of the drug (100-300 subjects)
- Phase 3 - Final confirmation of safety and efficacy (300+ subjects)
5 phases of clinical trials

- Phase 0-Pharmacodynamics and Pharmacokinetics (10-15 subjects)
- Phase 1-Screening for safety (20-100 subjects)
- Phase 2-Establishing the efficacy of the drug (100-300 subjects)
- Phase 3-Final confirmation of safety and efficacy (300+ subjects)
- Phase 4-Postmarketing surveillance (number of subjects varies)
Example

Dr. Simon is part of a Phase 4 trial to determine how much the sunscreen is effecting the risk of numbness.
Randomized Trial

Example

Dr. Simon is part of a Phase 4 trial to determine how much the sunscreen is effecting the risk of numbness

Focusing on subjects between the ages of 13-18, Dr. Simon follows 200 subjects who are using the sunscreen over the course of a year
Example

Dr. Simon is part of a Phase 4 trial to determine how much the sunscreen is effecting the risk of numbness.

Focusing on subjects between the ages of 13-18, Dr. Simon follows 200 subjects who are using the sunscreen over the course of a year.

Initial results appear inconclusive with only 50% of the subjects experiencing numbness.
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Dr. Simon is part of a Phase 4 trial to determine how much the sunscreen is effecting the risk of numbness.

Focusing on subjects between the ages of 13-18, Dr. Simon follows 200 subjects who are using the sunscreen over the course of a year.

Initial results appear inconclusive with only 50% of the subjects experiencing numbness.

After adjusting for confounding variables, it appears that 90% of the subjects on a particular acne medication experience numbness.
Randomized Trial

Advantages

Strong cause and effect association
Can adjust for confounding
Easier to remove biases than observational studies

Disadvantages

Usually the most expensive study design
Volunteer bias
Randomized Trial

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**Systematic Review**

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Can employ meta-analysis
- contrasting and combining results from different studies
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Highest level of analysis

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Based on both published and unpublished results
Example

Dr. Reynolds reviews all the literature on the connection between sunscreen and numbness
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He finds that 87% of the patients that experienced numbness were on the same acne medication.
Systematic Review

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Based on the finding from the RCT, he goes back to get data from previous studies.

He finds that 87% of the patients that experienced numbness were on the same acne medication.

Dr. Reynolds makes a recommendation to the FDA to inform them about the interaction between the two items.
Systematic Review

Advantages
Systematic Review

Advantages

- Strongest form of medical evidence
- Less time and cost than conducting a new RCT or cohort study
- More reliable and accurate than individual studies
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Disadvantages

- Difficult to determine if studies could be combined
- Requires using results from other researchers
The lowest level of study design is what type of study?
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- case study
Questions

The lowest level of study design is what type of study?
- case study

Which type of study assess outcome first?

- case-control study

What type of study follows subjects for a set amount of time?
- cohort and randomized trial

What type involves no new subject?
- systematic review

Which studies are not affected by recall bias?
- cohort and randomized trial
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- randomized trial
References

