Evidence-Based Decision Making in Healthcare

Designing Studies on Health Issues

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Why Do Studies?

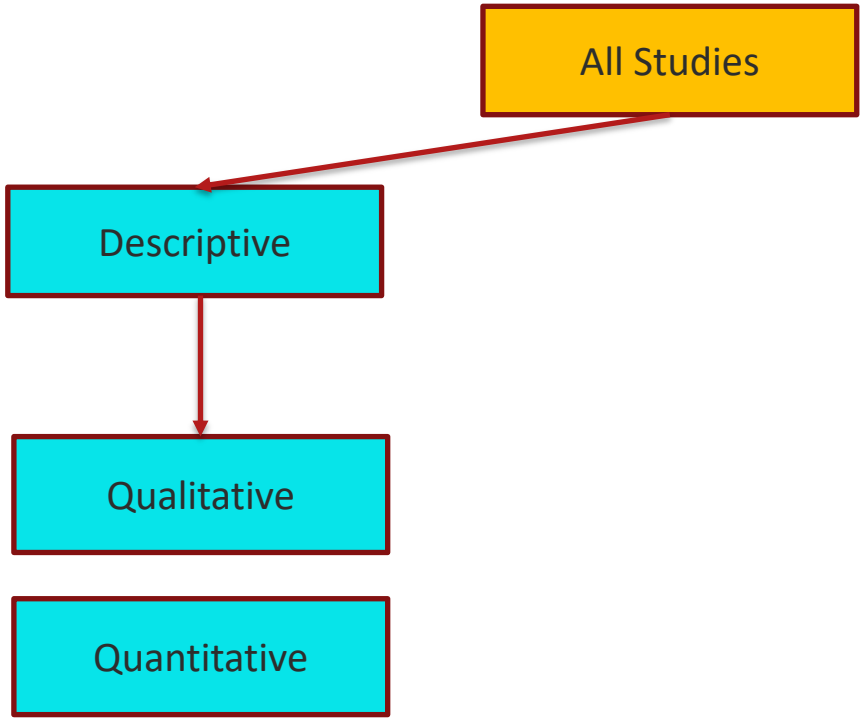
- Identify who is getting sick, e.g., age, location, race, other health conditions
- Generate ideas about why people are getting sick
- These are called “descriptive studies”

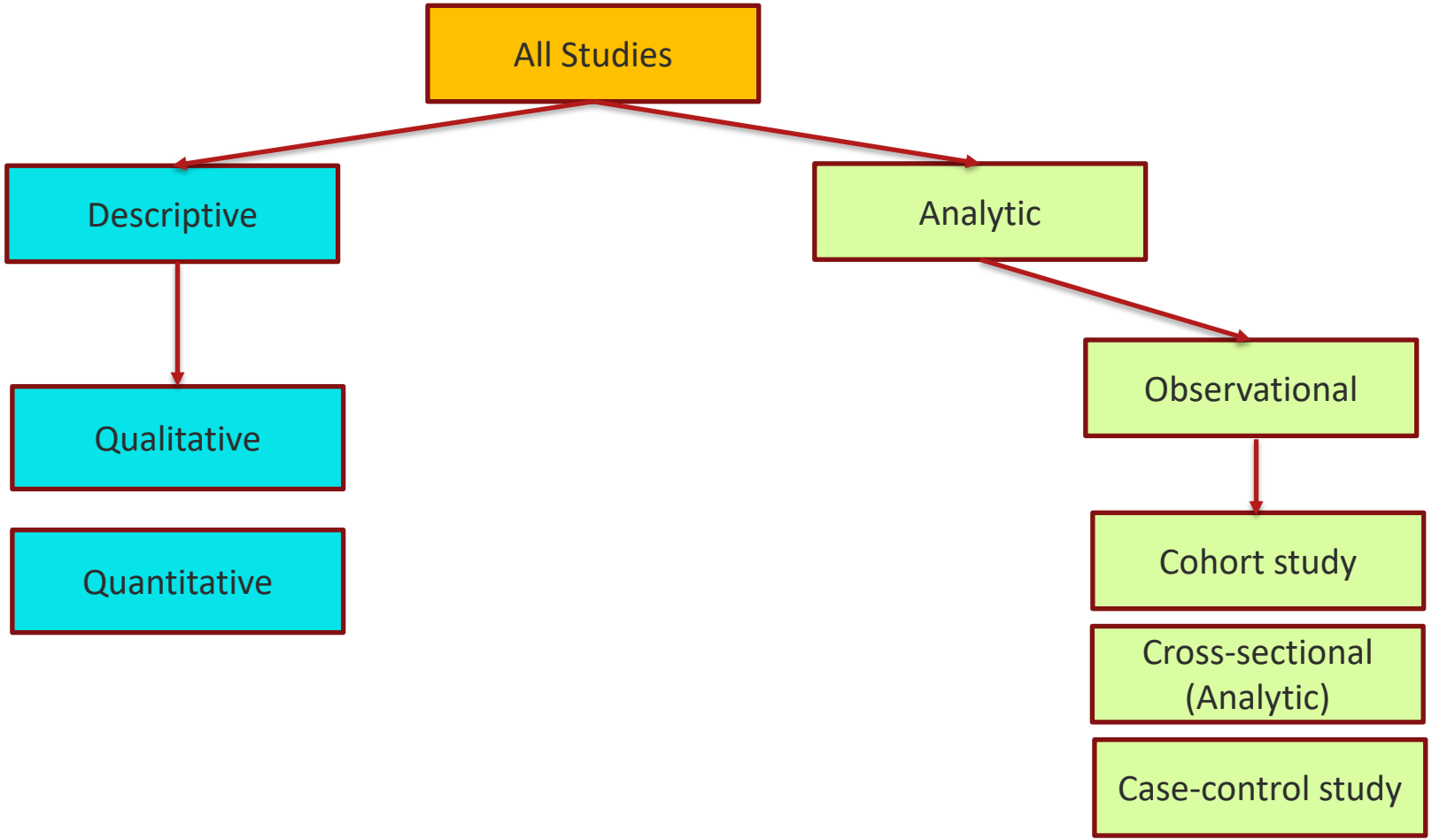
Why Do Studies?

- Prove that X causes Y
- Test whether an action prevents sickness or cures people who are sick
- These are called “analytical studies”

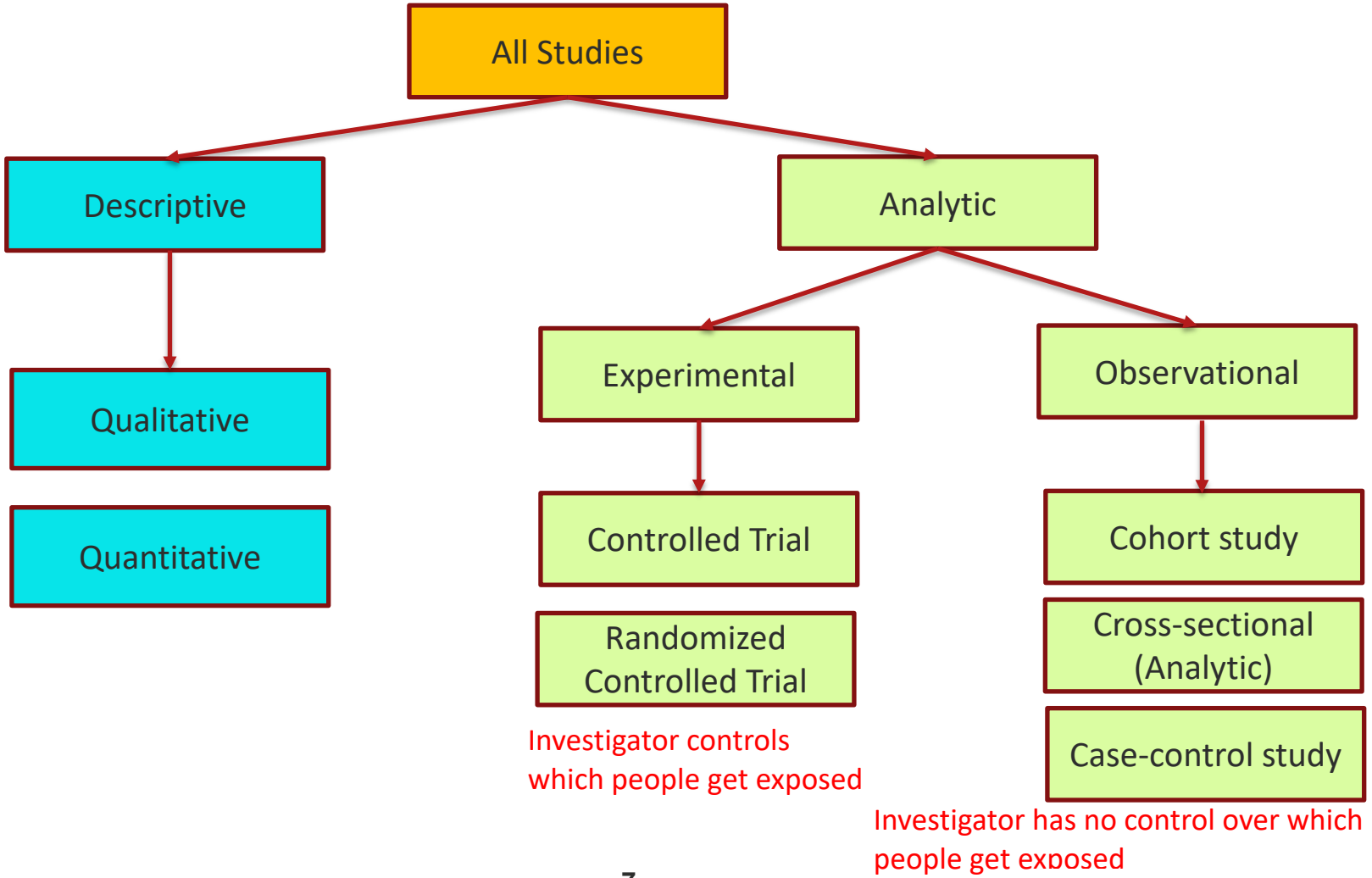
Basic Design of Studies

- Define an exposure, e.g., eating apples
- Define an outcome, e.g., having a heart attack
- Count the number of people with the exposure
- Count the number of people with the outcome
- Calculate the relationship between exposure and outcome using division and statistical methods


All Studies
Descriptive
Qualitative
Quantitative



Investigator has no control over which people get exposed


All Studies
Descriptive
Analytic
Qualitative
Quantitative
Experimental
Observational
Controlled Trial
Randomized Controlled Trial
Cohort study
Cross-sectional (Analytic)
Case-control study
Investigator controls which people get exposed
Investigator has no control over which people get exposed

Descriptive Studies

- Qualitative studies
  - Collect unstructured information from participants
  - Ask people to tell you about a problem
  - “narrative”
- Quantitative studies
  - Collect structured information from participants
  - yes/no, multiple choice
  - “survey”

Descriptive Studies

- Help you generate ideas about how an exposure and outcome may be related
- We interviewed hundreds of people
- Most of those who said they had a heart attack said they often eat candy
- Could eating candy cause heart attacks?

Analytical Studies

- Allow you to test a hypothesis
- Observational = investigator has no control over who was exposed or not
- Controlled = investigator decides which patients get exposed and which do not

Does Candy Cause Heart Attacks?

Cross-sectional

- Interview people
- Did you ever have a heart attack?
- How much candy do you eat every week?
- What % of people who had a heart attack said they eat candy every week VS % of people who have never had a heart attack that eat candy every week?

Does Candy Cause Heart Attacks?

Case-Control

- Cases: visit ER, diagnosed with heart attack
- Controls: visit ER, no heart attack
- Ask each group how much candy they eat
- Do cases (heart attack) have a greater percentage of people that eat candy than controls (no heart attack)?

Does Candy Cause Heart Attacks?

Cohort

- Enroll 1000 people in a study
- Interview them today and check in on them every three months for next five years
- What percentage of people that developed a heart attack in those 5 years also reported eating candy every week vs. those that did not have a heart attack during those 5 years?

Does Candy Cause Heart Attacks?

Controlled Trial

- Enroll 1000 people in a study
- Ask 500 people to eat candy every week
- Ask 500 people not to eat candy every week
- Interview them today and check in on them every three months for next five years
- What percentage of people developed heart attack in candy group vs. no candy group?

Does Candy Cause Heart Attacks?

Randomized Controlled Trial

- Enroll 1000 people in a study
- Randomly assign people to
  - Eat candy every week
  - Not to eat candy every week
- Interview them today and check them every 3 months for next 5 years
- What percentage of people developed heart attack in candy group vs. no candy group?

Systematic Review & Meta-Analysis

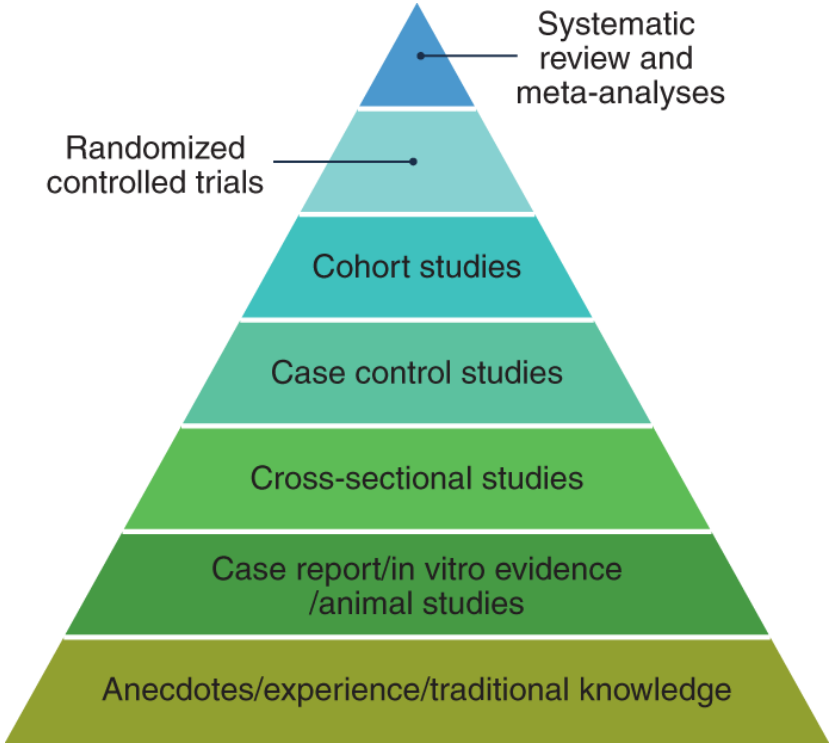
- Search databases of research to find all studies on your topic
- Review them in a standardized way
- Summarize your findings
- Meta-analysis is when you do the review above AND use special statistical methods to combine the results and report a composite result

Does Candy Cause Heart Attacks?

Meta-Analysis

- Search databases to find all studies that compared heart attacks and candy consumption
- Select those studies that are highest quality, e.g., all the randomized controlled trials
- Use statistical methods to calculate a composite estimate of whether candy causes heart attacks

Hierarchy of Evidence



https://www.nature.com/articles/s43016-021-00388-5/figures/1

Hierarchy of Evidence - Critique

- Not always ethical or practical to do an RCT
  - Emergency
  - Public health policy
  - Disease incidence is low
- RCTs have strict enrollment criteria, so do not always reflect who gets medicine in real world

Hierarchy of Evidence - Critique

- Meta-analyses considered the highest standard, but susceptible to “garbage in, garbage out”
- Observational studies are valuable
  - Represent what happens in real world
  - Can measure outcomes that only occur after a long time (decades, rather than years for RCT)
  - Can measure outcomes that occur rarely (e.g., 1 in a million)

Causal Inference

- Does X truly cause Y?
- The challenge of all health-related studies
- Criteria proposed originally in 1965 by British epidemiologist Austin Bradford Hill, who helped “prove” that cigarettes “cause” lung cancer
- Others have critiqued, but still widely used

Causal Inference

1. Strength: the magnitude of the effect
2. Consistency: the association is consistent when results are replicated with studies done in different settings using different methods
3. Specificity: doing X causes Y to happen; doing Z does not cause Y to happen

Causal Inference

4. Temporality: Exposure first, disease second
5. Dose-response: More exposure, more disease; less exposure, less disease
6. Experimental evidence: an experiment that interrupts the causal pathway prevents disease; an experiment that amplifies the causal pathway increases disease

Causal Inference

7. Biologically plausible: Association between X and Y is supported by laboratory studies and basic science

8. Coherence: Association compatible with existing theory and knowledge (similar to biologically plausible)

9. Analogy: Similar factors cause similar diseases, e.g., something similar to asbestos causes a different type of lung disease

Cause is Multiple Components

- One disease can have multiple causes
- Each cause can have multiple components
  - Some components necessary
  - Some components sufficient
  - Some components neither

Necessary, Sufficient, Neither?

- Exposure to influenza virus necessary, but not sufficient, to make you sick from the flu
- Cigarettes cause lung cancer, but cigarettes are neither necessary nor sufficient to cause lung cancer
  - Some people get lung cancer without smoking
  - Some people smoke, but never get lung cancer
- HIV is both necessary and sufficient to cause AIDS

Evidence-Based Decision Making

- This course starts with understanding how evidence is generated in health-related studies
- Next two lectures
  - Observational studies
  - Randomized controlled trials
- Subsequent lectures will discuss how to evaluate different types of studies for bias