DESIGNS OF EPIDEMIOLOGIC STUDIES

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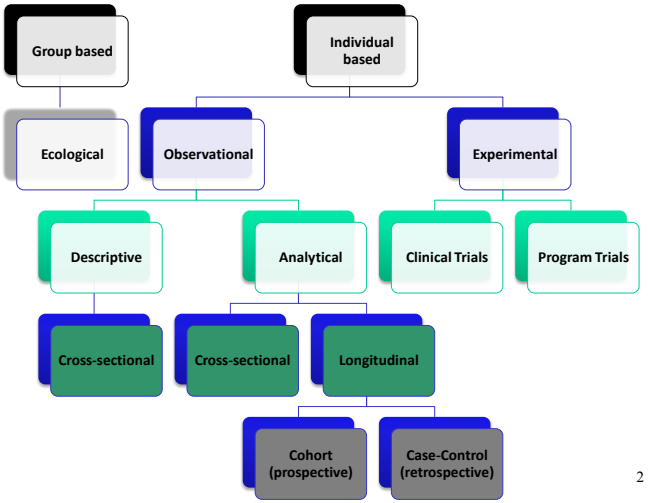
Study Designs



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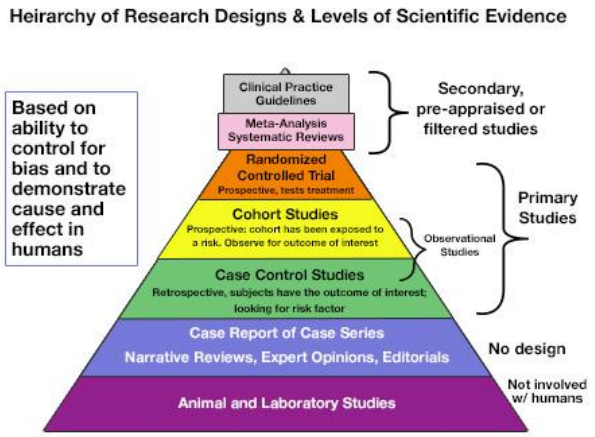

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2012 Forrest & Miller.

The major study designs differ in several respects:

- Unit of observation
- Manipulation of exposure
- Randomization
- Direction of investigation
- Timing of data collection
- Data collection methods

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- **Unit of observation**
- Manipulation of exposure
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Unit of observation

Group based (ecological)

Individual based

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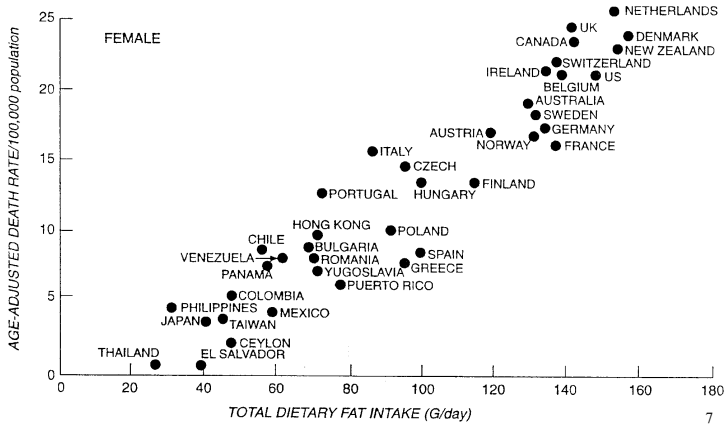
ECOLOGICAL STUDY



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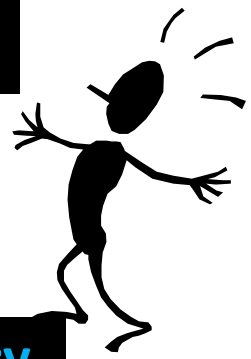
BEWARE!!!

of

Ecological Fallacy



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Ecological Fallacy

"Mistaken assumption that a statistical association observed between two group-level variables is equal to the association between the corresponding variables at the individual level"
(Gail & Benichou, 2001)

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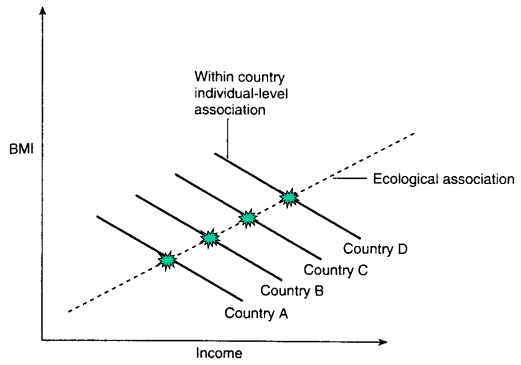
Hypothetical associations of income with BMI within and between countries



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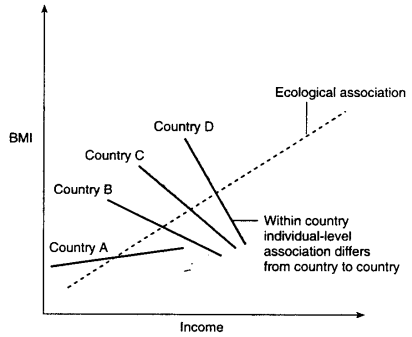
Hypothetical associations of income with BMI within and between countries



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Individual-based Studies

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The major study designs differ in several respects:

- Unit of observation
- **Manipulation of exposure**
- Randomization
- Direction of investigation
- Timing of data collection
- Data collection methods

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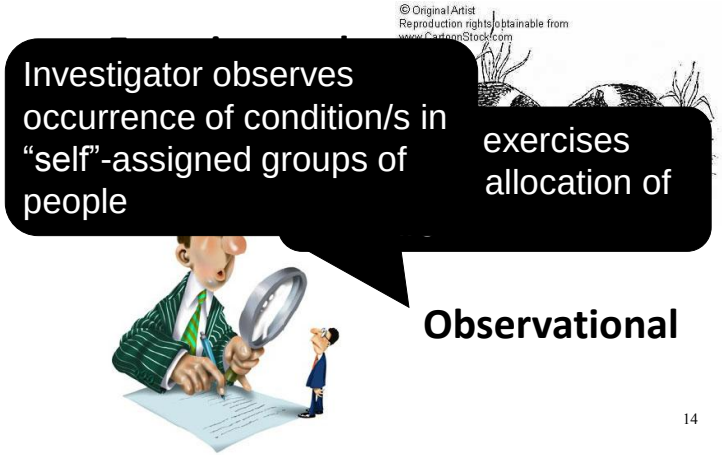
Manipulation of exposure



Observational

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Observational Studies

- Investigator observes occurrence of condition/s in "self-assigned" groups of people; exposure not assigned
- Carried out in more natural settings - "natural experiments"
- Often most practical and feasible
- Less control over study situation; results more susceptible to distorting influence

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Experimental Studies

- Investigator exercises control over allocation of exposure
- More powerful than observational studies for testing etiological hypotheses
- For ethical reasons the possibilities of conducting experiments in humans is limited

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The major study designs differ in several respects:

- Unit of observation
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- **Randomization**
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- Timing of data collection
- Data collection methods

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- Is exposure of interest controlled by investigator?
- In controlling the exposure, are study participants randomly assigned to different exposure conditions?

Exposure control?	Randomization?	Study type

18

- Is exposure of interest controlled by investigator?
- In controlling the exposure, are study participants randomly assigned to different exposure conditions?

Exposure control?	Randomization?	Study type
N	N	Observational

19

- Is exposure of interest controlled by investigator?
- In controlling the exposure, are study participants randomly assigned to different exposure conditions?

Exposure control?	Randomization?	Study type
N	N	Observational
Y	Y	Experimental

20

- Is exposure of interest controlled by investigator?
- In controlling the exposure, are study participants randomly assigned to different exposure conditions?

Exposure control?	Randomization?	Study type
N	N	Observational
Y	Y	Experimental
Y	N	Quasi-experimental

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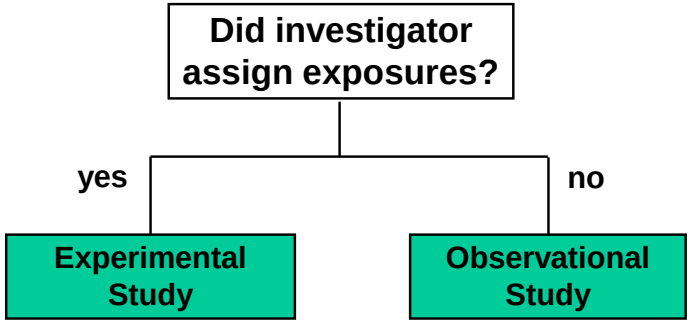

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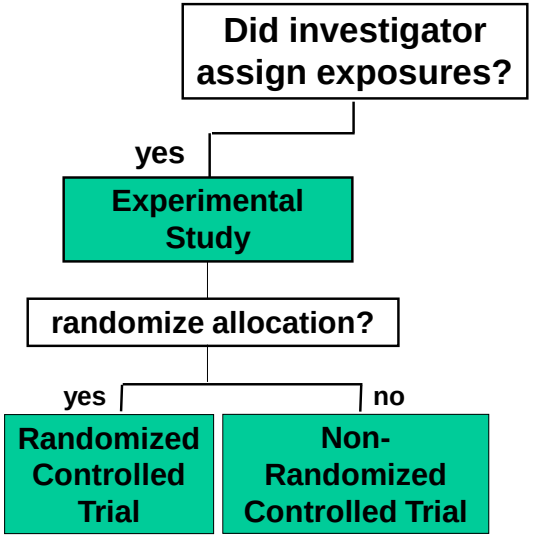
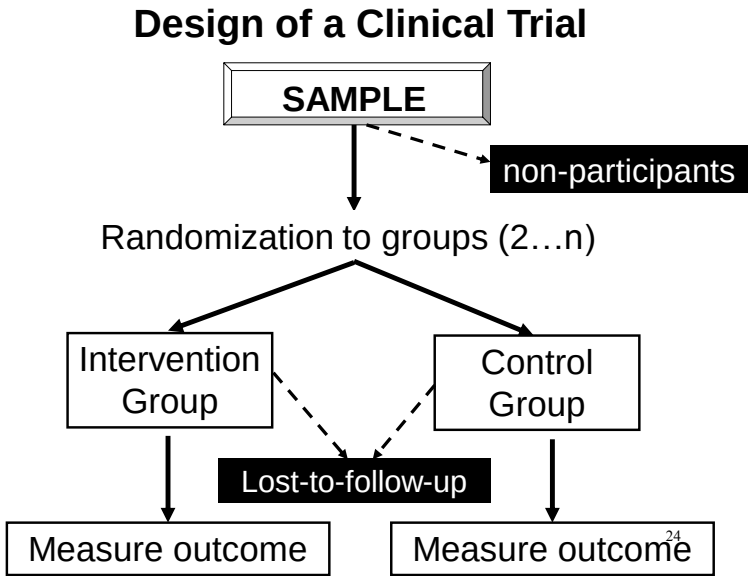
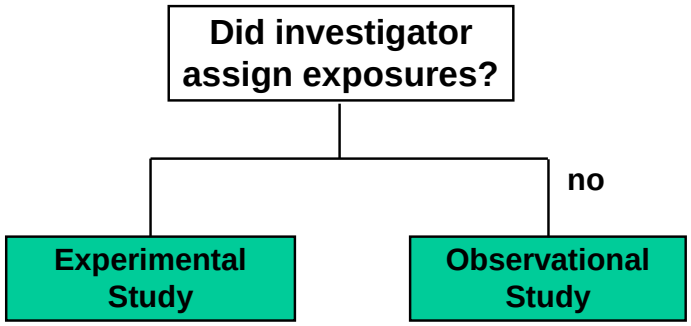

Did investigator assign exposures?
yes
Experimental Study
randomize allocation?
yes
no
Randomized Controlled Trial
Non-Randomized Controlled Trial


Design of a Clinical Trial
SAMPLE
non-participants
Randomization to groups (2…n)
Intervention Group
Control Group
Lost-to-follow-up
Measure outcome
Measure outcome


Did investigator assign exposures?
no
Experimental Study
Observational Study

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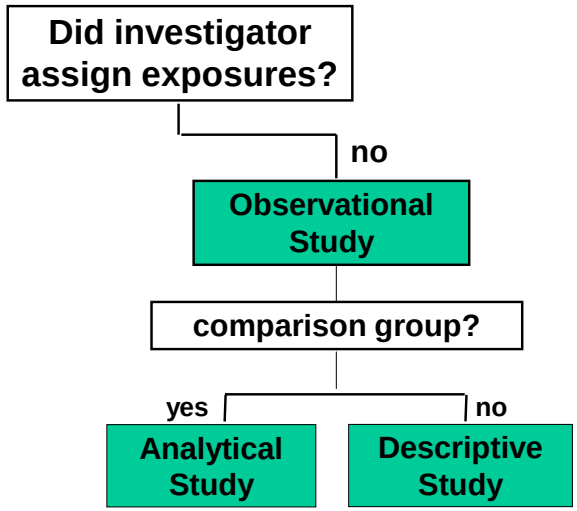
Did investigator assign exposures?
no
Observational Study
comparison group?
yes
no
Analytical Study
Descriptive Study

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Descriptive Study

- Sets out to describe a situation

  ex. Distribution of depression in a population in relation to sex, age and other characteristics

Analytical Study

- Sets out to test hypotheses or detect associations

  ex. Identify factors that explain higher rates of depression among women

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Descriptive Study

- Often no a-priori hypothesis

Analytical Study

- Must have clear and measurable hypothesis
- At least 1 dependent (outcome) variable and 1 independent ("exposure") variable

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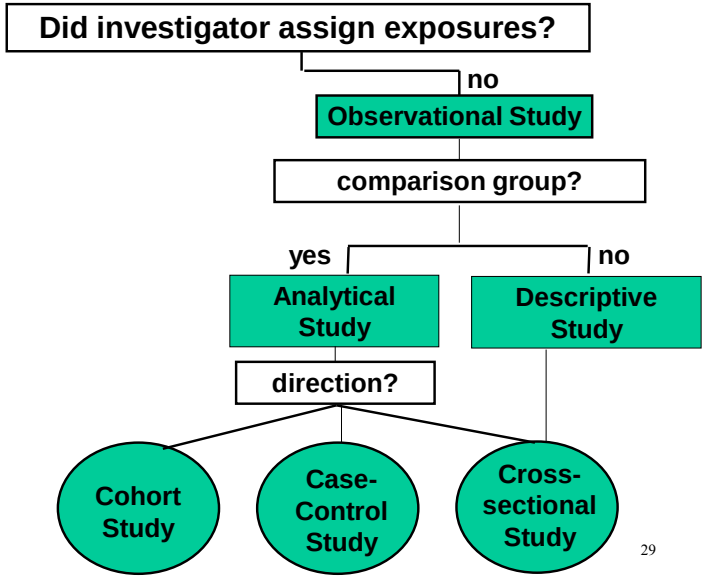

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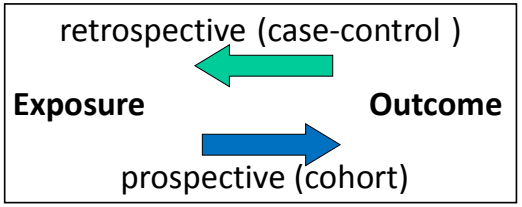
Directionality of investigation



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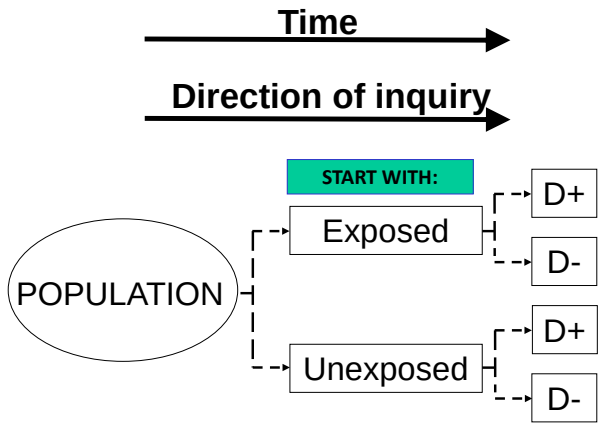
Design of a PROSPECTIVE Study



Prospective Study

- Step 1 in a prospective study design: identify relevant group/s of people and collect information about their **exposure** history
- Step 2: Follow these people over time and measure incidence of **outcome/s** of interest

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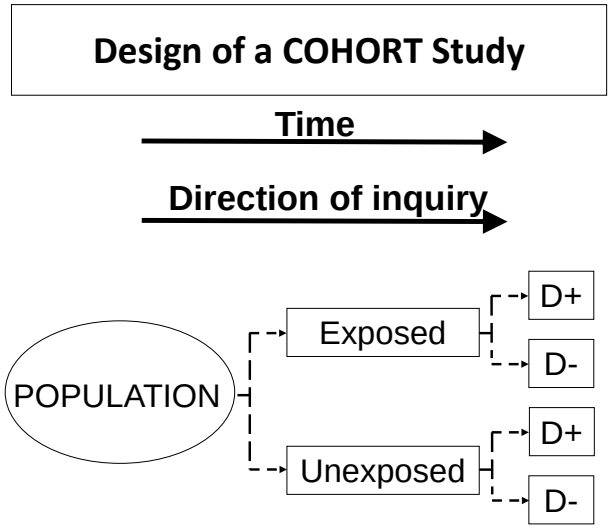
Design of a COHORT Study
Time
Direction of inquiry
POPULATION
Exposed
Unexposed
D+
D-
D+
D-

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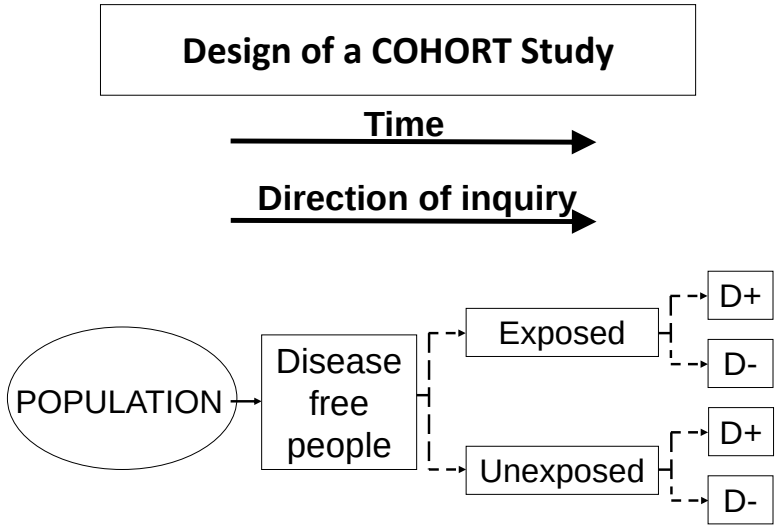
Design of a COHORT Study
Time
Direction of inquiry
POPULATION
Disease free people
Exposed
Unexposed
D+
D-
D+
D-

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2*2
if only life was so simple…

		Outcome Status	
		present	absent
Exposure Status	exposed		
	not exposed		

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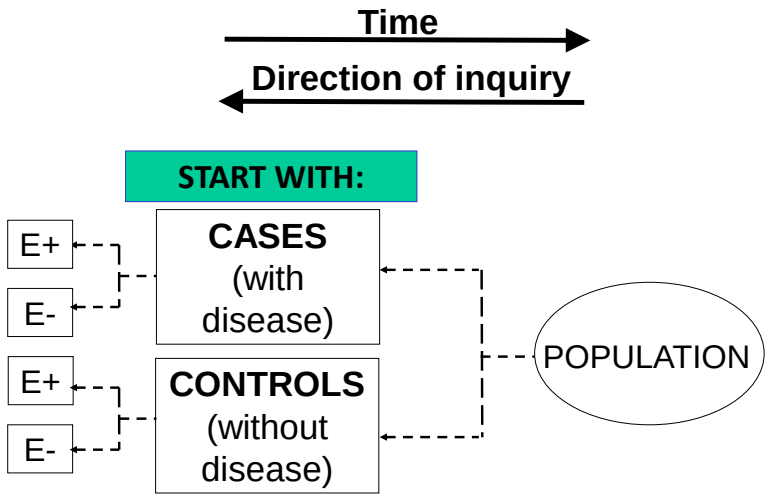
Design of a RETROSPECTIVE Study



Retrospective design

		Outcome Status		
		present	**absent**	**Total**
Exposure Status	**exposed**			
	not exposed			
	Total			

Strengths of case-control studies

- Well suited to study etiology of rare outcomes, e.g. cancer, congenital malformations.
- Can easily study multiple exposures
- Efficient if long delay between exposure and outcome
- Require fewer individuals (i.e. relatively inexpensive)

Strengths of case-control studies

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- Require fewer individuals

Strengths of cohort studies

- Well suited to study rare exposures
- Can easily study multiple outcomes
- Provides direct measure of risk of outcome among exposed and unexposed persons
- Not dependent on recall of past exposures
- Begins with “healthy” persons thereby preventing 'selective survival' bias

Limitations of case-control studies

- Inefficient for rare exposures
- Not well suited to study multiple outcomes
- Time sequence of exposure and outcome can be unclear
- Does not provide data on absolute risk
- Relies on information about past exposures that may be prone to bias

Limitations of case-control studies

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Limitations of cohort studies

- Inefficient for rare outcomes
- Not well suited to study multiple exposures
- Assessment of exposure status may influence participant's behavior
- Definitions of exposures and outcomes may change over time
- Difficult if long delay between exposure and outcome
- Lost-to-follow-up bias

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Limitations of cohort studies

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- Assessment of exposure status may influence participant's behavior
- Definitions of exposures and outcomes may change over time
- Difficult if long delay between exposure and outcome
- Lost-to-follow-up problem

Main limitation of the prospective design is time & cost

Especially when studying chronic diseases that may only become apparent years after the exposure/s of interest or may require years of exposure to "cause" the outcome

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Historical Prospective Design

- **Alternative strategy to "concurrent" prospective design to reduce time/costs**
- **Requires identifying a defined cohort from some time <u>in the past</u>**
- **Follow-up period is the time since determination of exposure status until present (or future)**
- **Incidence/risk measures can be estimated in the same manner as in a concurrent prospective study**

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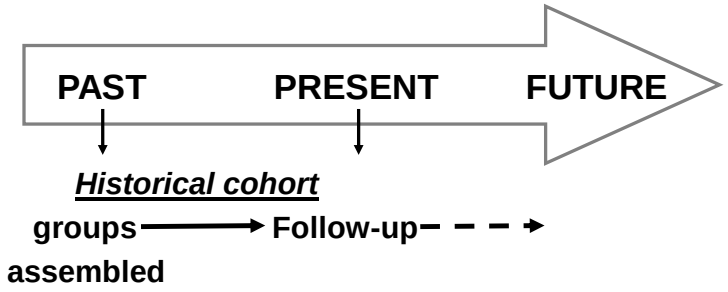
Historical and concurrent cohort studies



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Historical Prospective Design

Particularly useful when the exposure under investigation is "unique" in some way. e.g., occurred only in the past, occurred in specific groups of people

Often applied to the study of acute environmental exposures.

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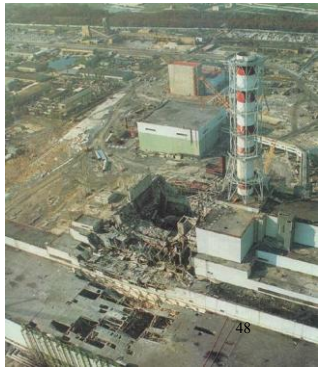
Historical Prospective Design

Example: thyroid cancer risk among people exposed to the 1986 Chernobyl nuclear-reactor accident



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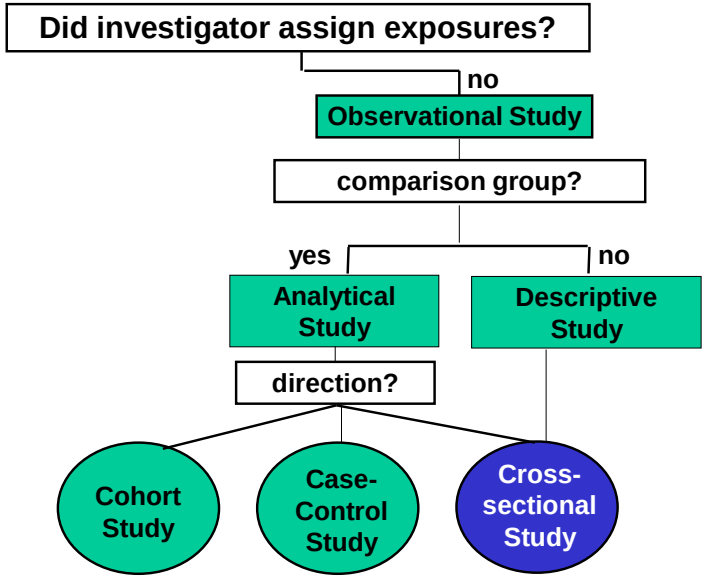
Historical Prospective Design

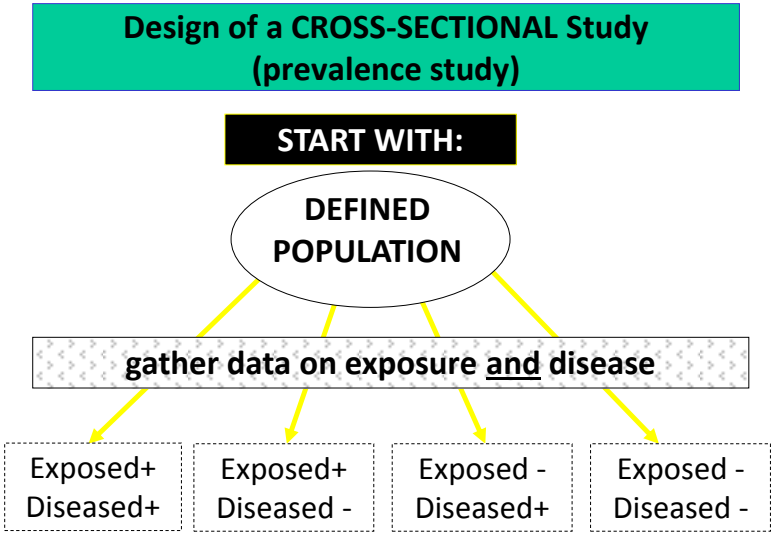
Possible sources of information about exposure status:

- Industrial worker records
- Military records
- Insurance companies or health care provider companies
- Registries of persons receiving specific medical treatment

Historical Prospective Design

- Sometimes difficult to obtain comprehensive list of persons who experienced the outcome (less problematic in a mortality study)
- Often difficult to obtain information about other exposures in the cohort


Did investigator assign exposures?
no
Observational Study
comparison group?
yes
no
Analytical Study
Descriptive Study
direction?
Cohort Study
Case-Control Study
Cross-sectional Study


Design of a CROSS-SECTIONAL Study (prevalence study)
START WITH:
DEFINED POPULATION
gather data on exposure and disease
Exposed+ Diseased+
Exposed+ Diseased -
Exposed - Diseased+
Exposed - Diseased -

Cross-sectional design

		Outcome Status		
		present	absent	Total
Exposure Status	exposed			
	not exposed			
	Total			

Cross-sectional design

		Outcome Status		
		present	absent	Total
Exposure Status	exposed			
	not exposed			
	Total			n

Cross-sectional design

		Outcome Status		
		present	absent	Total
Exposure Status	exposed	?	?	
	not exposed	?	?	
	Total			n

Cross-sectional (prevalence) study

- Usually involves a representative (random) sample of a dynamic population
- Examines exposures and outcomes simultaneously
- Based on prevalence data

Cross Sectional Study

Strengths:

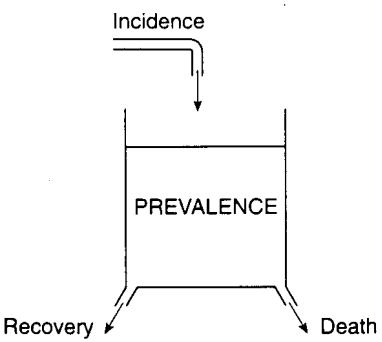
- Relatively inexpensive and quick
- Usually carried out in a single time point
- Efficient for describing target population characteristics
- Efficient for studying common chronic diseases
- Useful for generating new etiological hypotheses
- Useful for evaluation of health services

Cross Sectional Study

Limitations:

- Not efficient for studying rare outcomes or those of short-duration
- Cannot provide direct estimates of risk
- Difficult to interpret temporality between exposure and outcome
- Not useful for determining causal effects
- Includes prevalent and incident cases; identifies a mix of risk factors (incidence) and prognostic factors (duration)

Relationship between prevalence and incidence



Evaluation Studies

- Appraise the value of health care
- 2 types:
  - Program reviews
  - Program trials

Program Review

- Evaluates a specific program provided to a specific population, community, group of patients
- Concerned with the "welfare" of that population and directed at guiding decisions regarding the program under evaluation
- Similar to a physician's review of treatment given to an individual patient: concern for the patient rather than testing the effect of treatment

Program Review (cont.)

- Usually descriptive; no control group
- Assumes the program activities are beneficial and evaluates whether activities are conducted as planned
- Information on outcomes can be included under the assumption that changes are a result of the program

Program Trial

- Evaluates a type of service provided to a population, community, group of patients
- Concerned with the program's "value" and generalizibility of program's effectiveness to other populations
- Similar to a clinical trial of a new drug, findings must be relevant not only to the participants of the trial

Program Trial (cont.)

- Focus on "Outcomes"
- Analytical investigation requiring a control population in order to account for outside influences
- Program planning and implementation must allow for this from the outset