

# Focused Research Questions and Study Designs

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## ❖ Part 1

- Types of research questions
- The PICO / PEO structure

## ❖ Part 2

- Overview of Research Designs
- Experimental design: RCT
- Types of Observational designs
  - ✓ cohort, case-control, cross-sectional

# The Structure of Research

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## The "hourglass"



The diagram is an hourglass shape with a red top bar and a green base. The top bulb is wider than the bottom bulb. The text is centered within the hourglass shape.

Start broad.  
Narrow down, focus in.  
Operationalize.  
**OBSERVE**  
Analyze data.  
Draw conclusions.  
Generalize to target population.

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## PART 1

# Elements of a focused research question

*“In good science, questions come first. Science is just a tool for answering those questions.”*

*--John Bargh*

# Three examples

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- In **hospital EDs** how is **overcrowding** defined? **Descriptive**
- In a **patient with a chronic disease** seen in the ED, what is the relationship between **literacy** and **medication adherence**?  
between medication adherence and return ED visits? **Analytic**
- What are the **effects** of **biphasic defibrillation waveforms** compared to **monophasic** for resuscitating **patients experiencing OHCA**? **Analytic**

# The PICO/PEO structure

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- ❖ All studies have
  - a defined **population** from which subjects are studied
  - **Outcomes** that are measured
- ❖ For analytic or experimental studies
  - **Interventions** or
  - **Exposures** that are applied to different groups

# Focusing the question

	Patient or Problem	Intervention /Exposure	Comparison (if necessary)	Outcomes
Ex 1	Hospital ED settings			Measures of overcrowding
Ex 2	patients with asthma	secondary school completers	Non-completers	Improper use of MDI (correctly performing <3 of 6 steps in inhaler usage)
Ex 3	Adults experiencing OHCA of presumed cardiac origin with VF/VT as presenting rhythm	Biphasic waveform defibrillation pulse	Monophasic	Primary: Overall rate of ROSC Secondary: survival to hospital discharge

# Stating the hypothesis

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- ❖ Biphasic waveform defibrillation pulse increases the overall rate of ROSC by **20%** compared to monophasic in adults experiencing OHCA of presumed cardiac origin.
- ❖ What is the LWBS rate in the SGH A&E in the last quarter?



# Rules and principles

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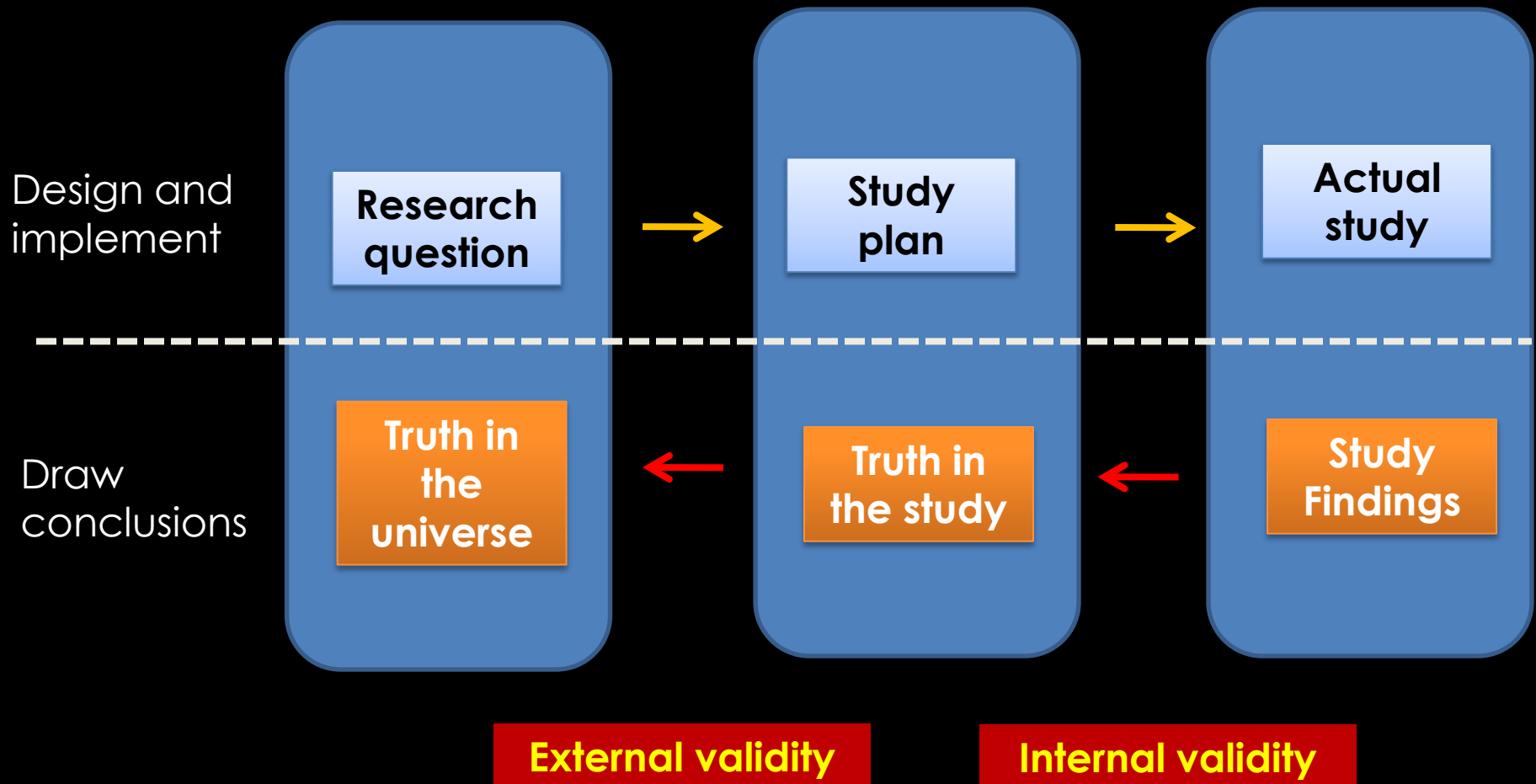
- ❖ Keep the research question focused
- ❖ State the problem clearly and completely
- ❖ If there are multiple questions, subcategorize as primary and secondary questions

Part 2

# Study Designs

***“100% of all disasters are failures of design, not analysis.”***  
-- Ron Marks, Toronto, August 16, 1994

# External and internal validity



# Random and Systematic Errors

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## ❖ **Random errors**

- Sources: sampling and measurement

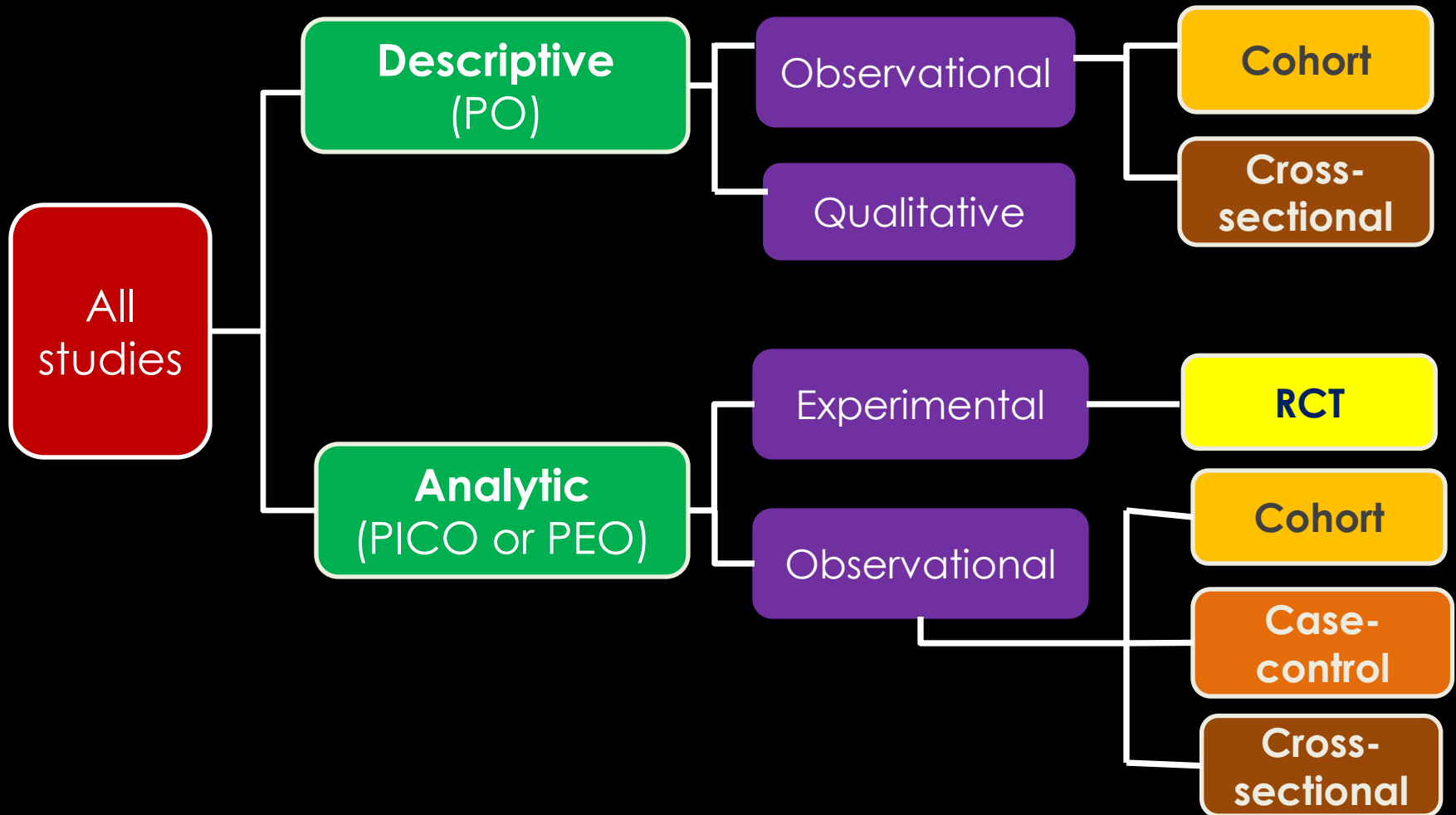
## ❖ **Bias**

- Sources: selection, confounding, measurement

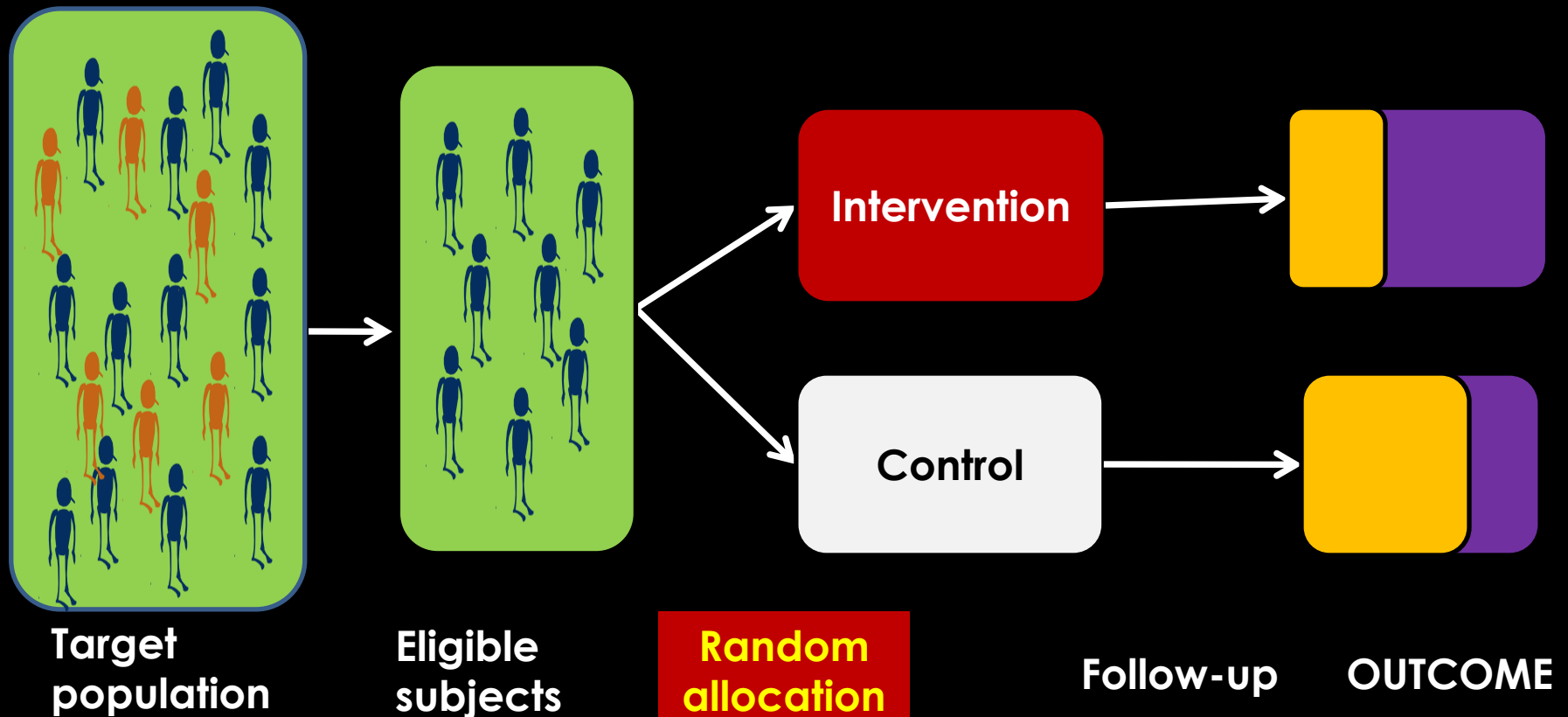
## ❖ **Example:** Study of Anti-hypertensive treatment

- Random error: variation in BP due to variable observer technique (observer)
- Systematic error: BP increase due to proximity to attractive technician (subject)

# Overview of Research Designs



# A Typical RCT



# Confounder

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- ❖ A variable that is known to relate with the outcome of interest
- ❖ Differentially distributed between comparison groups
- ❖ Example:
  - Outcome: death from trauma
  - Comparison groups: Consultant versus junior house officer
  - If % of severe cases in consultant group is higher than in junior house officer, then the severity factor is a confounder

# RCT is the gold standard

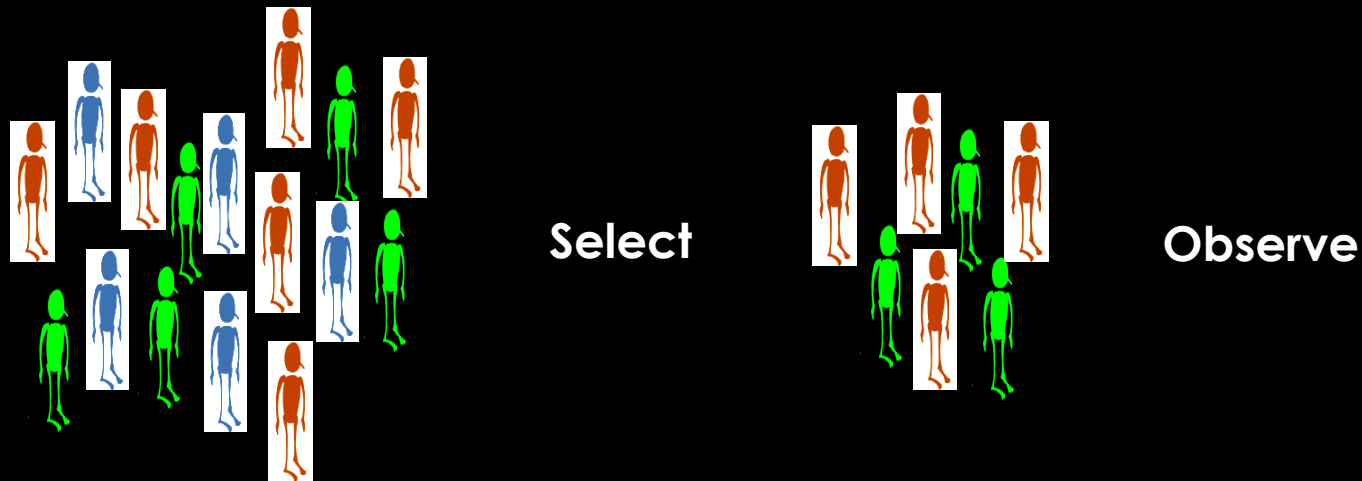
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- ❖ But not always best to perform
  - Unnecessary
    - ✓ Penicillin for bacterial infections
  - Inappropriate
    - ✓ Accident prevention schemes
    - ✓ HRT to prevent femoral fractures
  - Impossible
    - ✓ Ethical issues
  - Inadequate
    - ✓ Surgery ( where low external validity of results is likely)



# Observational Designs

- ❖ A researcher does not **manipulate in any way the conditions** under which the study is performed



- ❖ Descriptive or analytic

# Cohort study

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- A cohort is a **group** of individuals who share a **common experience or condition or characteristic**
- A cohort study involves the **follow-up** of at least one **exposed** and **non-exposed** cohorts to determine the **causal effect** (etiology) of exposure on **a future event**
- Cohort with the characteristic ==> **exposed cohort**

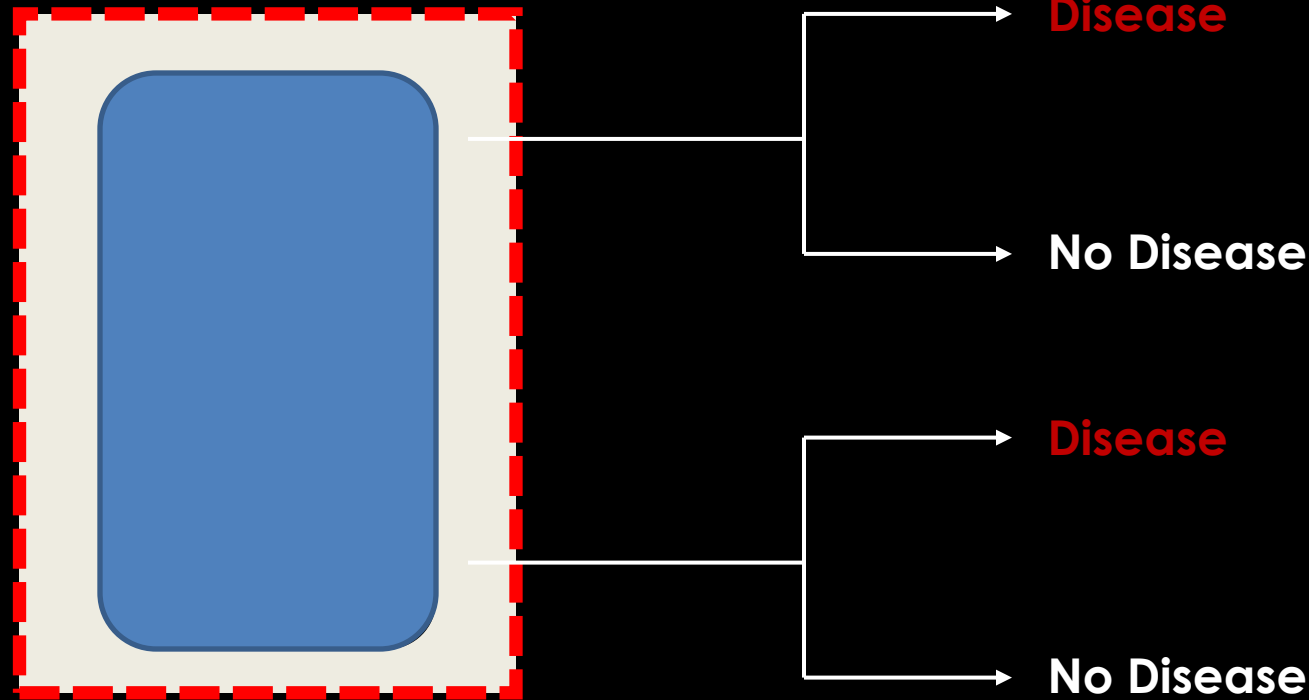
# Prospective Cohort study

START (sampling)

Follow-up

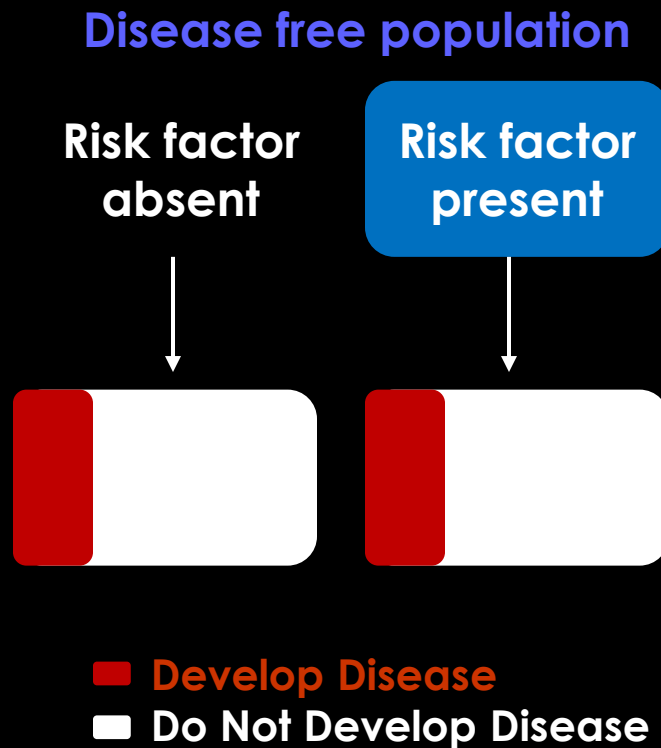
OUTCOME

TIME

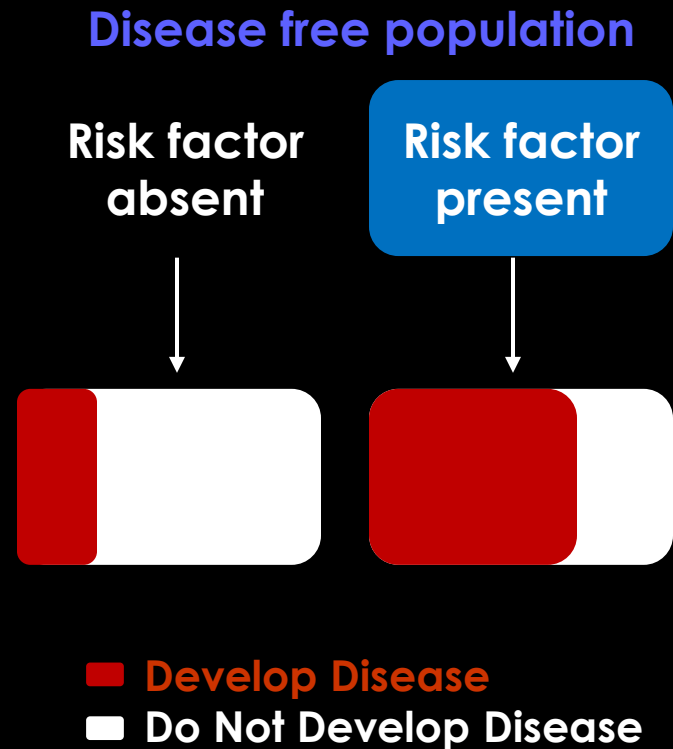


Disease-free population

## NO Association



## Association



# Types of questions

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- ❖ Which risk factors predict 28-day mortality in elderly ED patients admitted for infection?
- ❖ How do 3 ankle rules: Ottawa Ankle Rules, Low risk Exam and Malleolar Zone algorithm compare in predicting fractures in children with acute ankle trauma?
- ❖ In non-traumatic adult OOHCA patients, does ETI improve survival-to-hospital discharge when compared to BVM?

# Strengths and Weaknesses

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## ❖ Plus

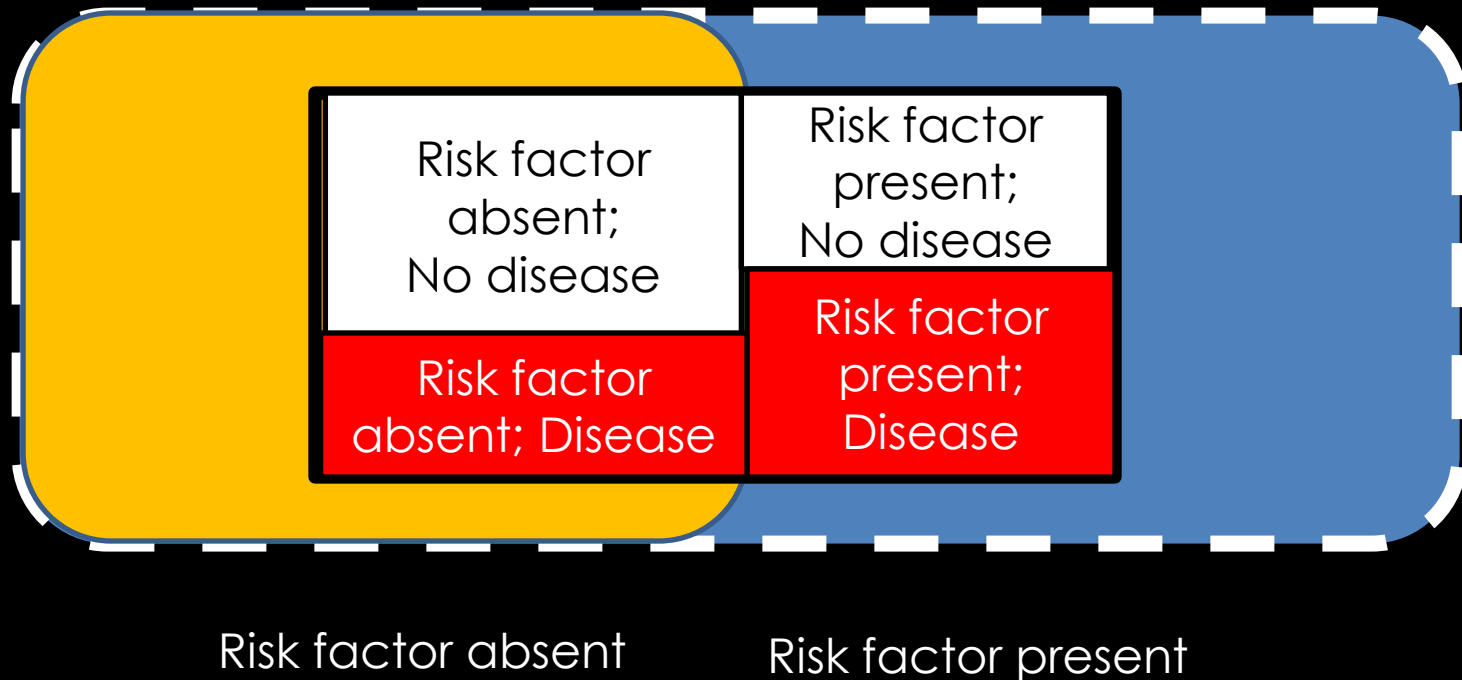
- Suitable to describe incidence or natural history of a condition
- Measurement of exposure before the outcome controls bias in measurement
- Possible to collect multiple exposures

## ❖ Minus

- Can be expensive
- May take decades to complete
- Losses to follow-up may invalidate results

# Cross-sectional studies

- Both the **exposure** and the **outcome** are assessed at the **same time**



# Types of questions

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- ❖ What is the national LWBS rate in Singapore in 2010?
- ❖ What are the characteristics of non-urgent patients seeking medical attention at an ED?
- ❖ How reliable is the clinical examination of ED physicians in the diagnosis of SSTIs?
- ❖ What is the prevalence of anxiety and depressive disorders in patients presenting with chest pain to the Emergency Department (ED)? Does the prevalence differ between cardiac and non-cardiac chest pain?



# Strengths and Weaknesses

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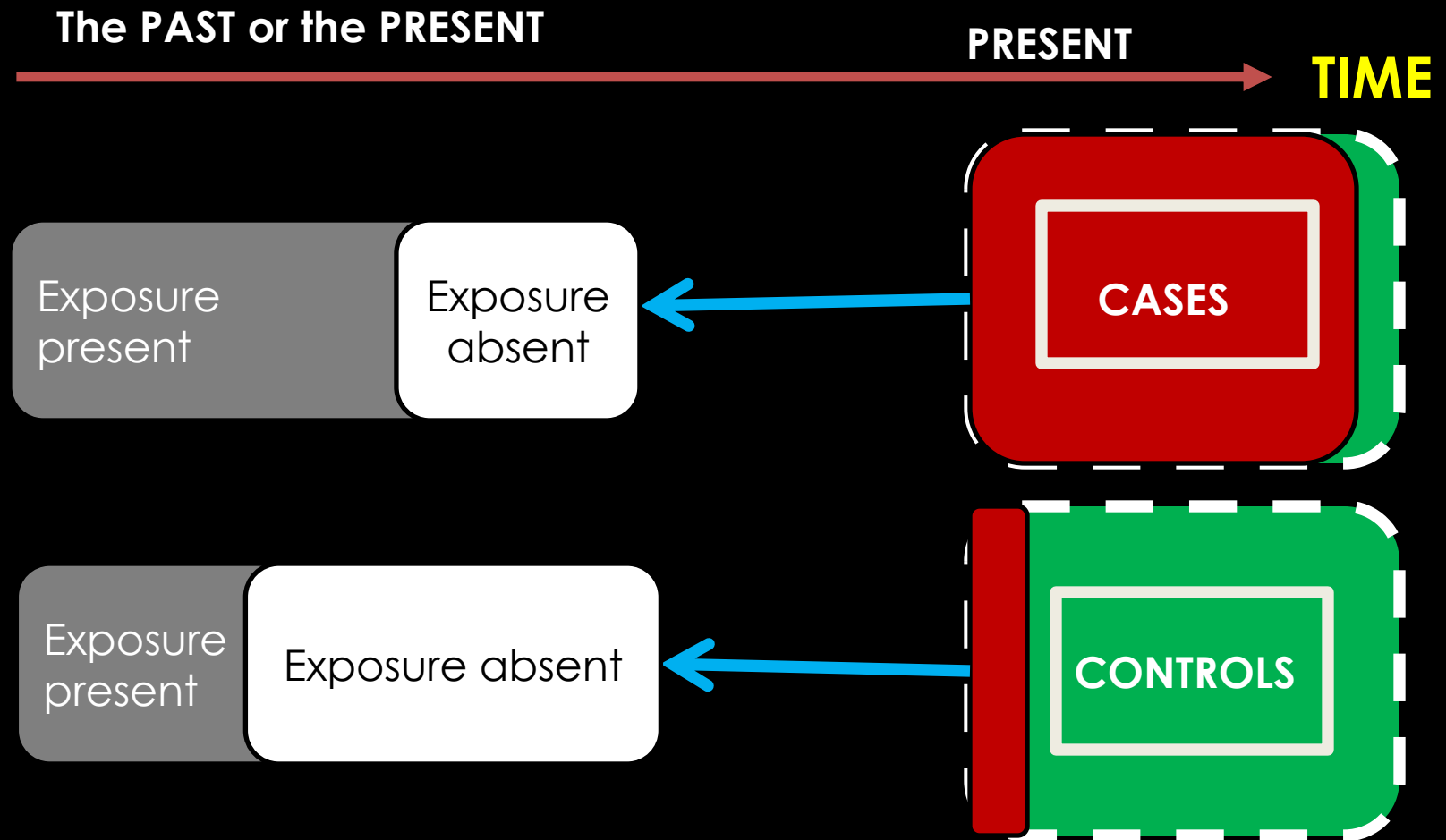
## ❖ Plus

- suitable for assessing prevalence of a disease or outcome
- Avoids time, expense, and drop-out problems of a follow-up design

## ❖ Minus

- Cannot be used to assess causality
- Cannot determine incidence
- Mainly descriptive

# Case-Control studies



# Types of questions

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- ❖ What features discriminate between SARS and severe non-SARS community-acquired viral infection in an ED setting?
- ❖ What factors predict laryngospasm during ED ketamine sedation in children?
- ❖ Is sleeping position associated with an increase in cot death (a.k.a. SIDS)?

# Strengths and Weaknesses

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## ❖ Plus

- sensible for study of rare, harmful outcomes
- Reasonably economical
- No loss to follow-up

## ❖ Minus

- Uncertain if exposure preceded disease
- Potential for recall bias
- Selection bias
- Unable to estimate disease incidence

# Pros and Cons of study designs

Study Design	Advantages	Disadvantages
RCTs	Unbiased distribution of confounders	Expensive; may be ethically problematic
Cohort studies	Establishes sequence of events, multiple predictors and outcomes, yields incidence, relative risk	Often requires large sample sizes; less feasible for rare or harmful outcomes; losses to follow-up
Cross-sectional	Yields prevalence or multiple predictors and outcomes; relatively short duration; good first step for cohort or RCT	Does not establish sequence of events; not feasible for rare outcomes; does not yield incidence
Case-control	Useful for rare outcomes; short duration, small sample size, relatively inexpensive	Bias and confounding from sampling from two populations; differential measurement bias; limited to one outcome variable; sequence of events unclear

# Matching questions to study design

P	I & C	O	Study design	Topic
Adults with migraine headache in the ED	Metoclopramide vs systemic DHE	Pain relief and relapses after discharge	RCT	Therapy
Adults with new onset COPD	Exposure to work-related or environmental irritants	Development of COPD	Prospective cohort	Etiology
Adults in the ED with acute swollen leg and chest pain	Use of Well's criteria vs unstructured clinical exam	Diagnosis of DVT/PE	Prospective cohort	Diagnosis
Infants	Sleeping position	Cot death	Case-control	Harm

# Which study design?

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- ❖ Is the study design ethical?
- ❖ What resources do I have
  - Time
  - Money
  - personnel
- ❖ Is there a more efficient way of reliably answering the same question?