

Basic Epidemiology

Study Designs in Epidemiologic
Research

Epidemiology = A branch of medical science

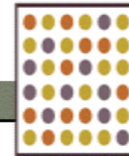
- **Definition:** Epidemiology :is defined as the study of the distribution and determinants of health, disease and injuries in human population
- Derived from Greek **epi (upon)**, **demos (people)**, **logos (knowledge)** = the knowledge of what happens to people

Definitions

1. **Epidemic**: A disease that clearly exceeds normal or expected frequency in a community or region.
2. **Pandemic**: Epidemic with worldwide distribution (for e.g. plague or AIDS)
 - In England alone, approximately one fourth of the population died from the plague.
3. **Endemic**: Continuing presence of a disease or infectious agent in a given geographic area means the disease is endemic to that area.

The Five Ws of Epidemiologic Studies

The Five Ws of Epidemiology Studies



- What = Clinical
 - Who = Person
 - Where = Place
 - When = Time
- } Descriptive Epidemiology

-
- Why / How = Causes
Risk factors
Modes of transmission
- } Analytic Epidemiology



Fundamental Assumption in Epidemiology

- **Disease doesn't occur in a vacuum**
 - * **Disease is not randomly distributed throughout a population**
 - **Epidemiology uses systematic approach to study the differences in disease distribution in subgroups**
 - **Allows for study of causal and preventive factors**

Components of Epidemiology

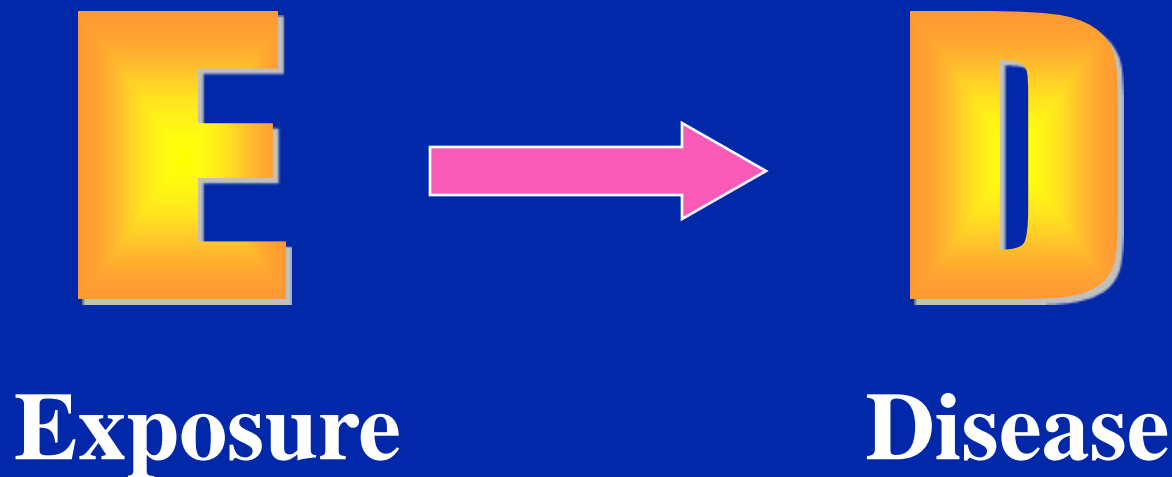
- **Measure disease frequency**
 - Quantify disease
- **Assess distribution of disease**
 - Who is getting disease?
 - Where is disease occurring?
 - When is disease occurring?
 - **Formulation of hypotheses concerning causal and preventive factors**
- **Identify determinants of disease**
 - Hypotheses are tested using epidemiologic studies

Types of primary studies

- **Descriptive studies**
 - describe occurrence of outcome
- **Analytic studies**
 - describe **association** between exposure and outcome

Basic Question in Analytic Epidemiology

- Are exposure and disease linked?



Basic Questions in Analytic Epidemiology

- Look to link exposure and disease
 - What is the exposure?
 - Who are the exposed?
 - What are the potential health effects?
 - What approach will you take to study the relationship between exposure and effect?

**Basic Research Study
Designs and their
Application to Epidemiology**

Big Picture

- **To prevent and control disease**
- **In a coordinated plan, look to**
 - **identify hypotheses on what is related to disease and may be causing it**
 - **formally test these hypotheses**
- **Study designs direct how the investigation is conducted**

**What designs exist to
identify and investigate
factors in disease?**

Study Designs

Descriptive

Case report

Case series

Descriptive
Epidemiology

Analytic

RCT

Cohort study

Case-Control
study

Case-Crossover
study

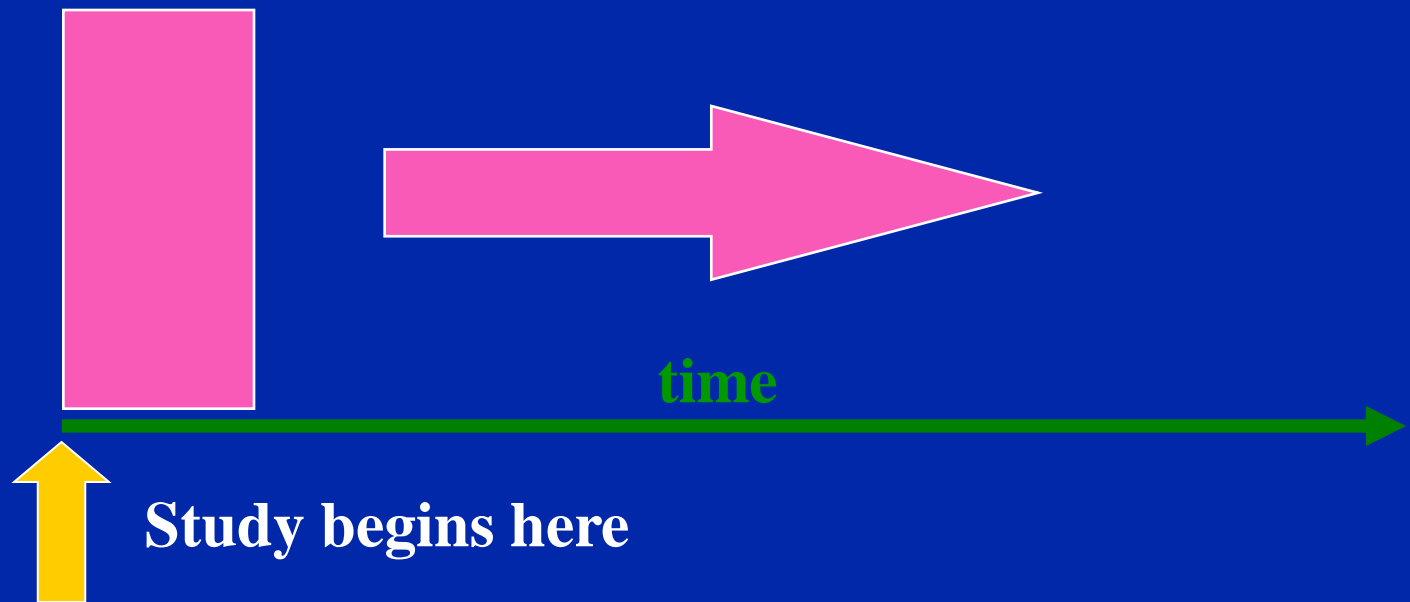
Cross-sectional
study

Before-After
study

Ecologic study

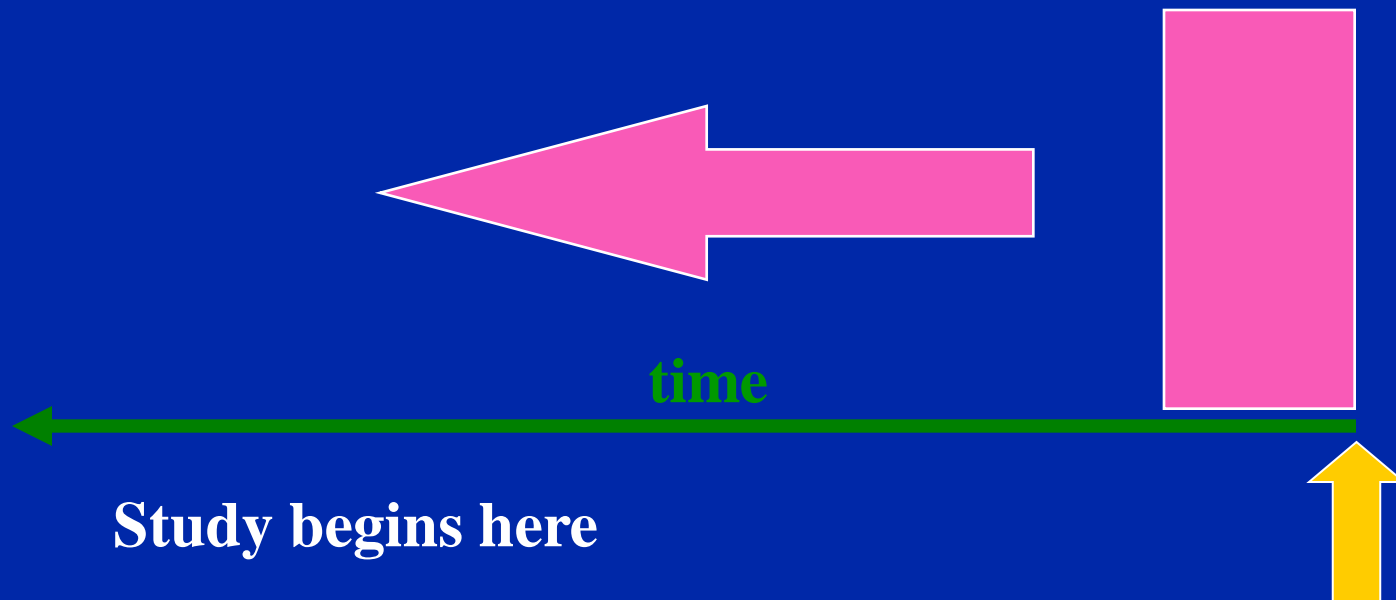
Timeframe of Studies

- **Prospective Study** - looks forward, looks to the future, examines future events, follows a condition, concern or disease into the future



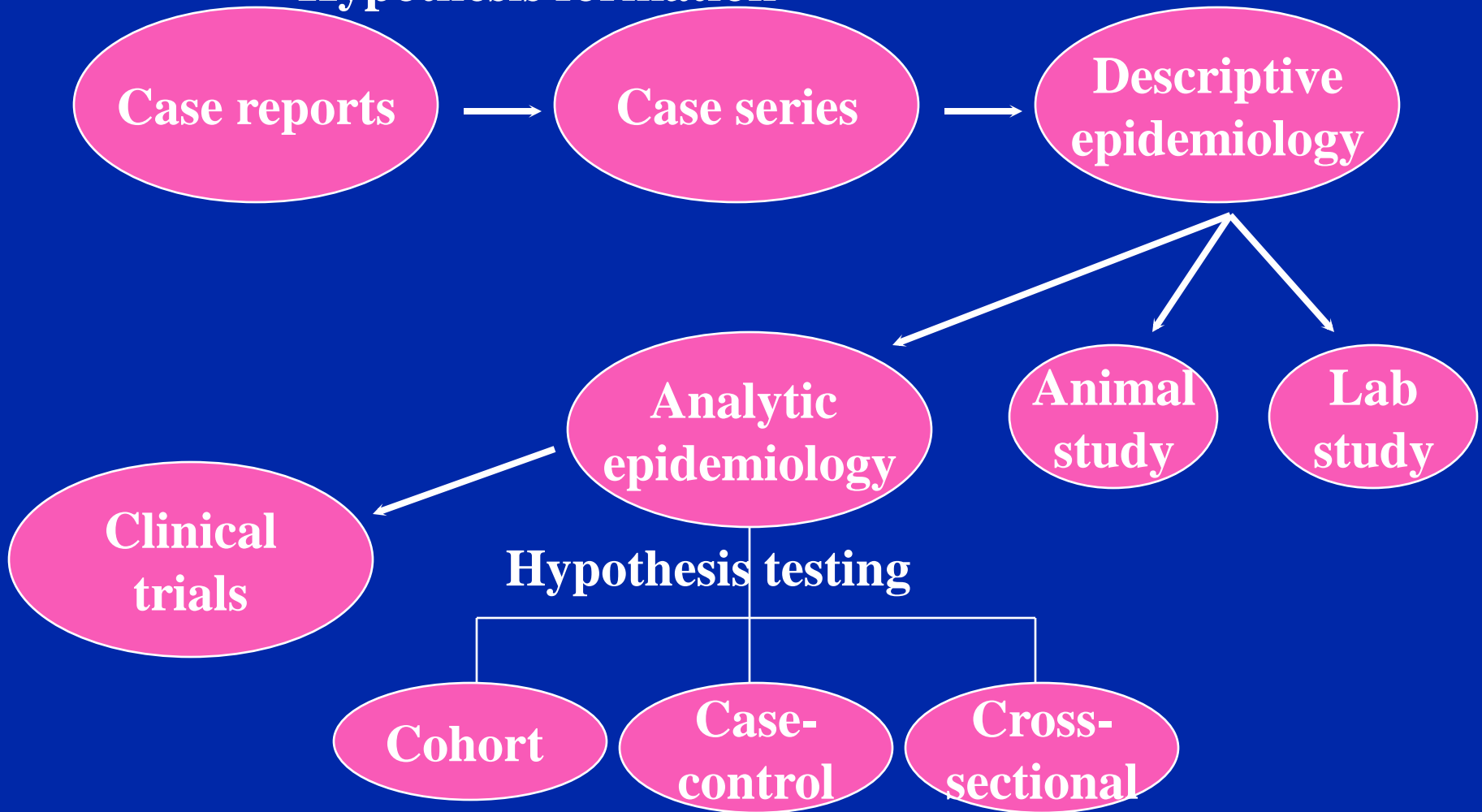
Timeframe of Studies

- **Retrospective Study** - “to look back”, looks back in time to study events that have already occurred



Study Design Sequence

Hypothesis formation



**Increasing Knowledge of
Disease/Exposure**



Descriptive Studies



Case-control Studies



Cohort Studies



Clinical trials

**Develop
hypothesis**

**Investigate it's
relationship to
outcomes**

**Define it's meaning
with exposures**

**Test link
experimentally**

Descriptive Studies

Case Reports

- **Detailed presentation of a single case or handful of cases**
- **Generally report a new or unique finding**
 - e.g. previous undescribed disease
 - e.g. unexpected link between diseases
 - e.g. unexpected new therapeutic effect
 - e.g. adverse events

Case Series

- **Experience of a group of patients with a similar diagnosis**
- **Assesses prevalent disease**
- **Cases may be identified from a single or multiple sources**
- **Generally report on new/unique condition**
- **May be only realistic design for rare disorders**

Case Series

- **Advantages**

- Useful for hypothesis generation
- Informative for very rare disease with few established risk factors
- Characterizes averages for disorder

- **Disadvantages**

- Cannot study cause and effect relationships
- Cannot assess disease frequency

Case Report



One case of unusual findings

Case Series



Multiple cases of findings

**Descriptive
Epidemiology Study**



Population-based cases with denominator

Analytical Studies

Study Designs - Analytic Epidemiology

- **Experimental Studies**
 - Randomized controlled clinical trials
 - Community trials
- **Observational Studies**
 - Group data
 - Ecologic
 - Individual data
 - Cross-sectional
 - Cohort
 - Case-control
 - Case-crossover

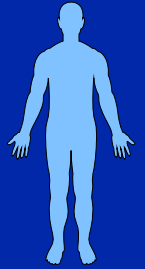
Experimental Studies

- **treatment and exposures occur in a “controlled” environment**
- **planned research designs**
- **clinical trials are the most well known experimental design. Clinical trials use randomly assigned data.**
- **Community trials use nonrandom data**

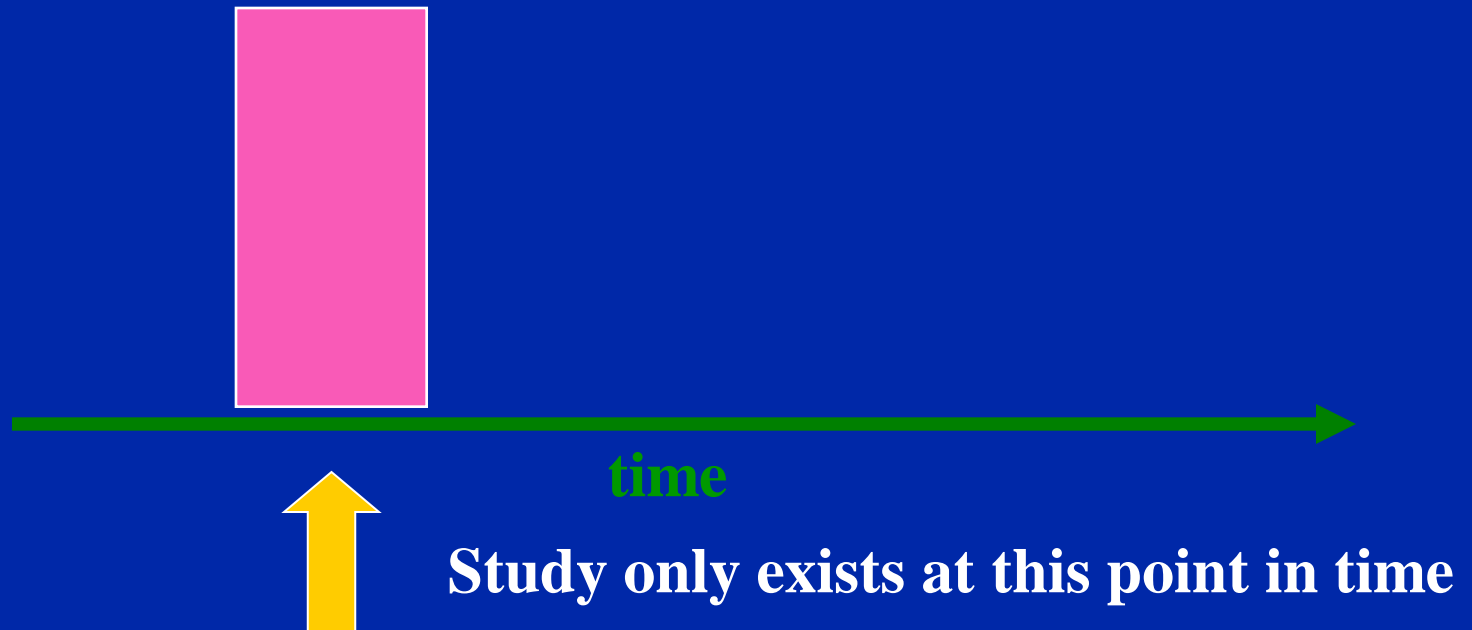
Observational Studies

- **non-experimental**
- **observational because there is no individual intervention**
- **treatment and exposures occur in a “non-controlled” environment**
- **individuals can be observed prospectively, retrospectively, or currently**

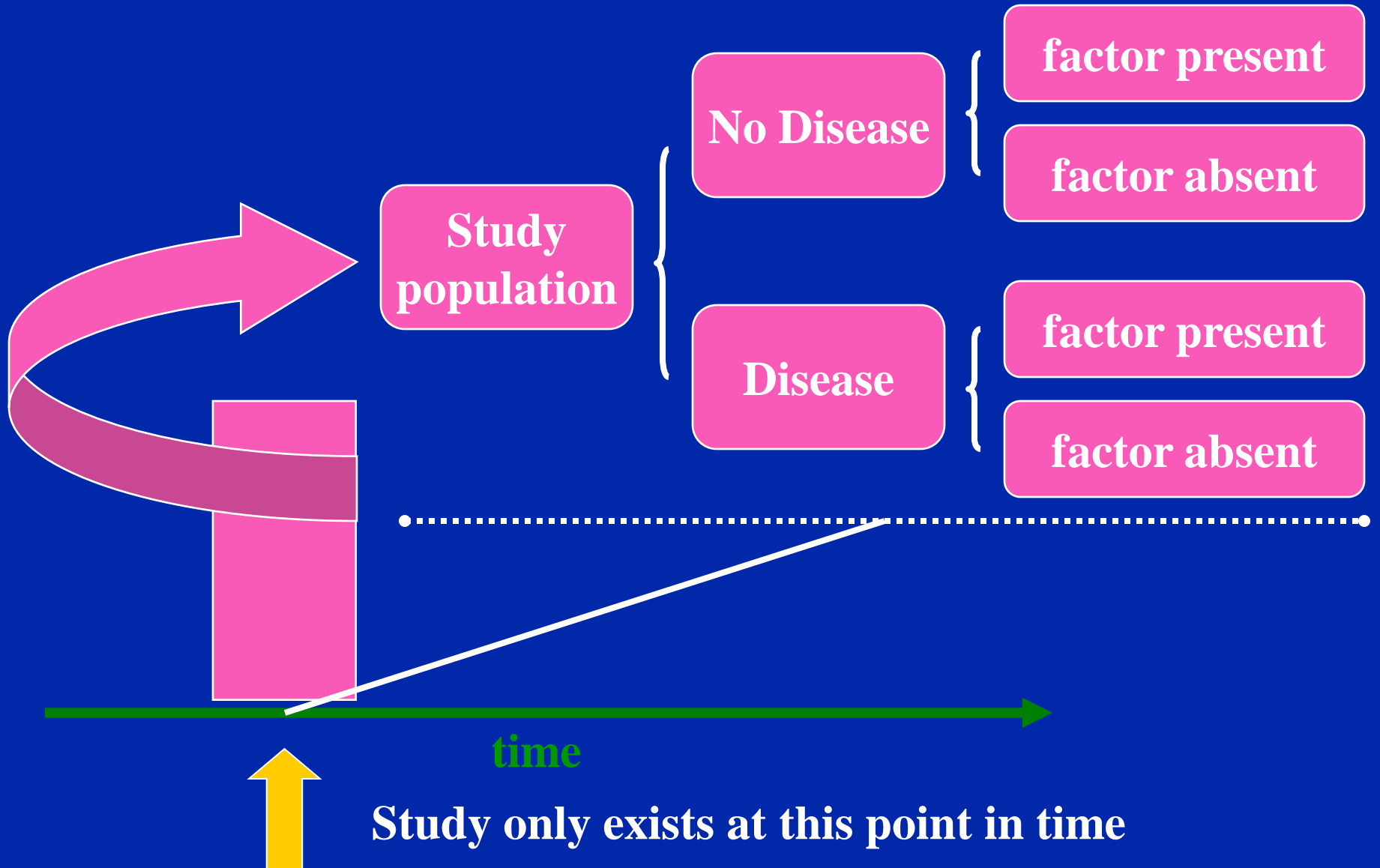
Cross-sectional studies



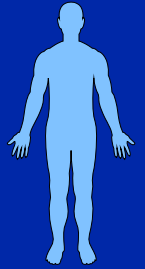
- An “observational” design that surveys exposures and disease status at a single point in time (a cross-section of the population)



Cross-sectional Design

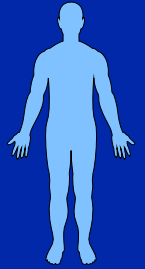


Cross-sectional Studies



- **Often used to study conditions that are relatively frequent with long duration of expression (nonfatal, chronic conditions)**
- **It measures prevalence, not incidence of disease**
- **Example: community surveys**
- **Not suitable for studying rare or highly fatal diseases or a disease with short duration of expression**

Cross-sectional studies



- **Disadvantages**

- **Weakest observational design, (it measures prevalence, not incidence of disease). Prevalent cases are survivors**
- **The temporal sequence of exposure and effect may be difficult or impossible to determine**
- **Usually don't know when disease occurred**
- **Rare events a problem. Quickly emerging diseases a problem**

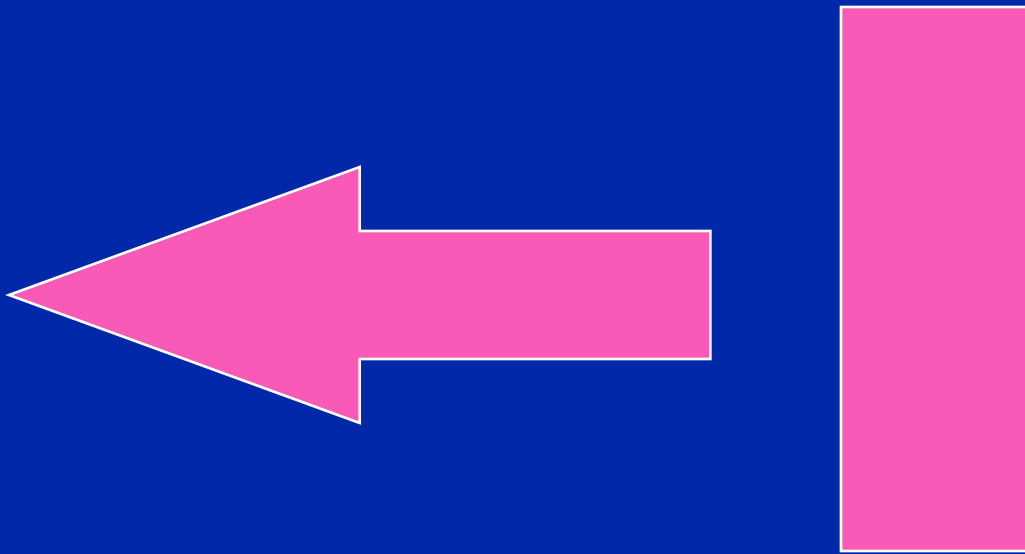
Epidemiologic Study Designs

- **Case-Control Studies**
 - an “observational” design comparing exposures in disease cases vs. healthy controls from same population
 - exposure data collected retrospectively
 - most feasible design where disease outcomes are rare

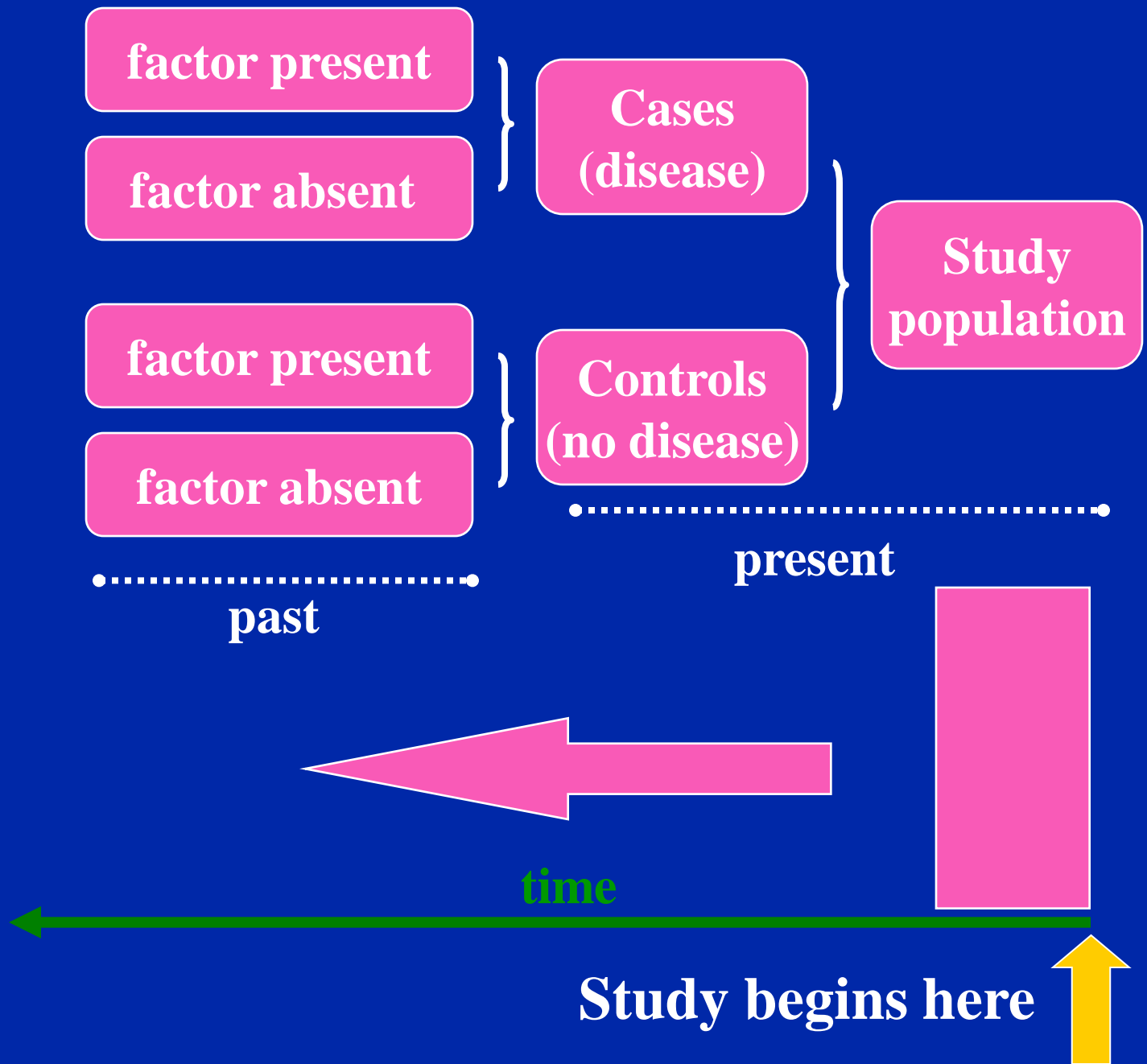
Case-Control Studies

Cases: Disease

Controls: No disease



Case-Control Design



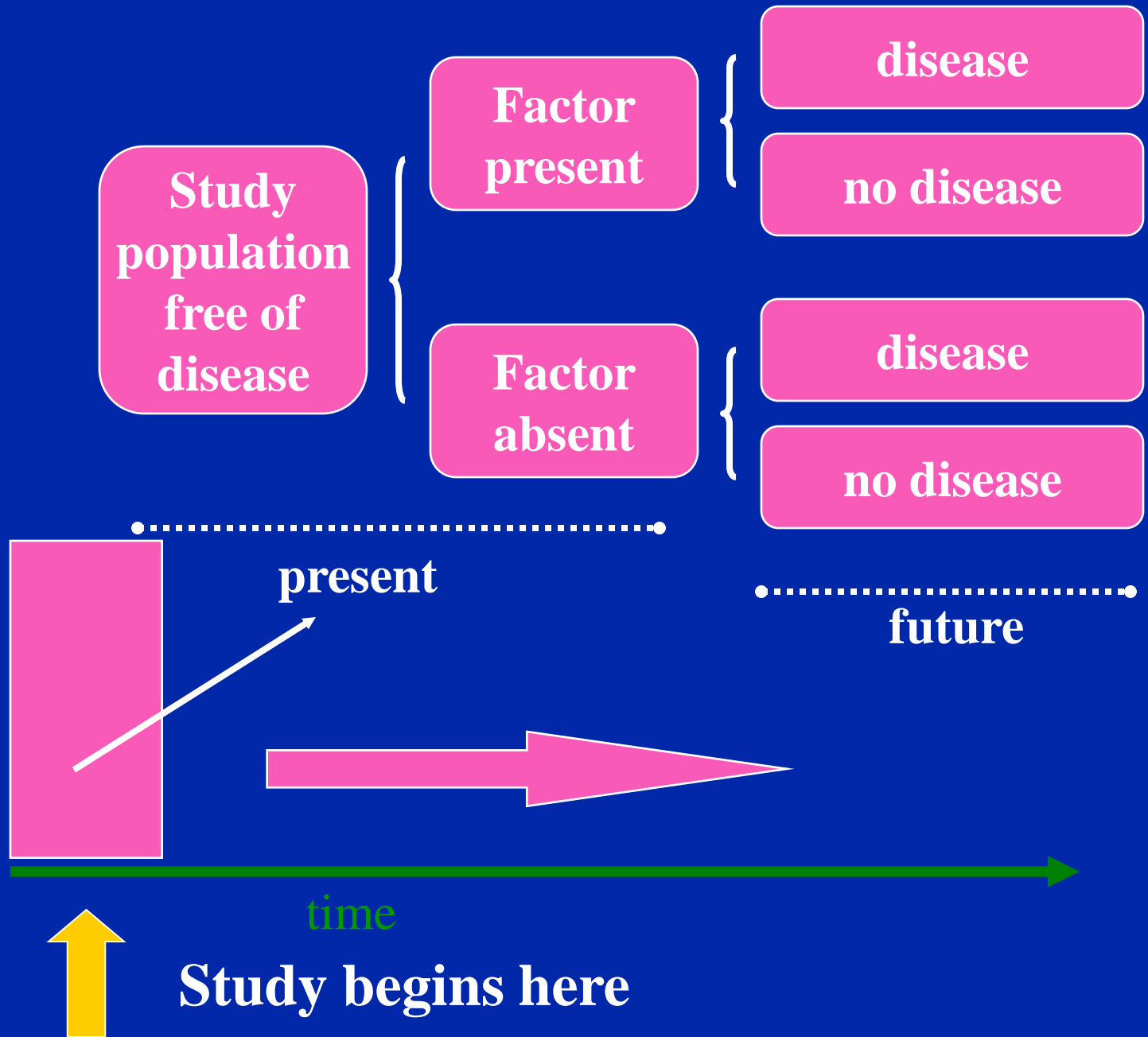
Case-Control Study

- **Strengths**
 - **Less expensive and time consuming**
 - **Efficient for studying rare diseases**
- **Limitations**
 - **Inappropriate when disease outcome for a specific exposure is not known at start of study**
 - **Exposure measurements taken after disease occurrence**
 - **Disease status can influence selection of subjects**

Epidemiologic Study Designs

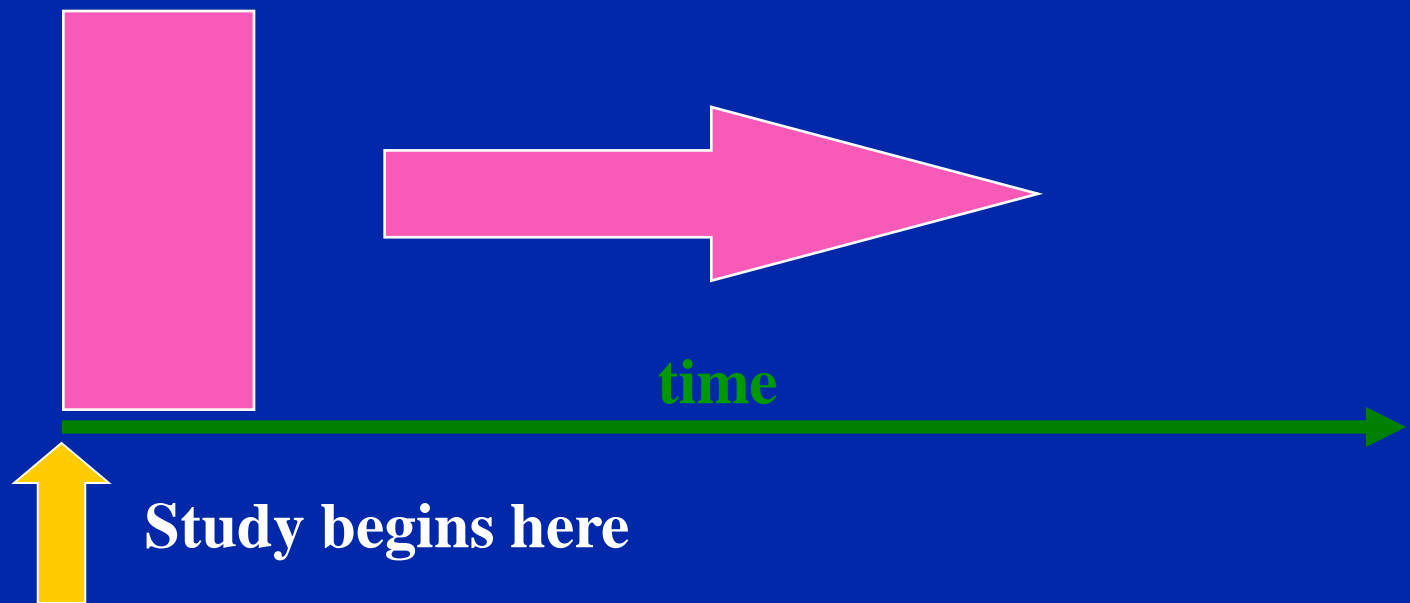
- **Cohort Studies**
 - an “observational” design comparing individuals with a known risk factor or exposure with others without the risk factor or exposure
 - looking for a difference in the risk (incidence) of a disease over time
 - best observational design
 - data usually collected prospectively (some retrospective)

Cohort Design

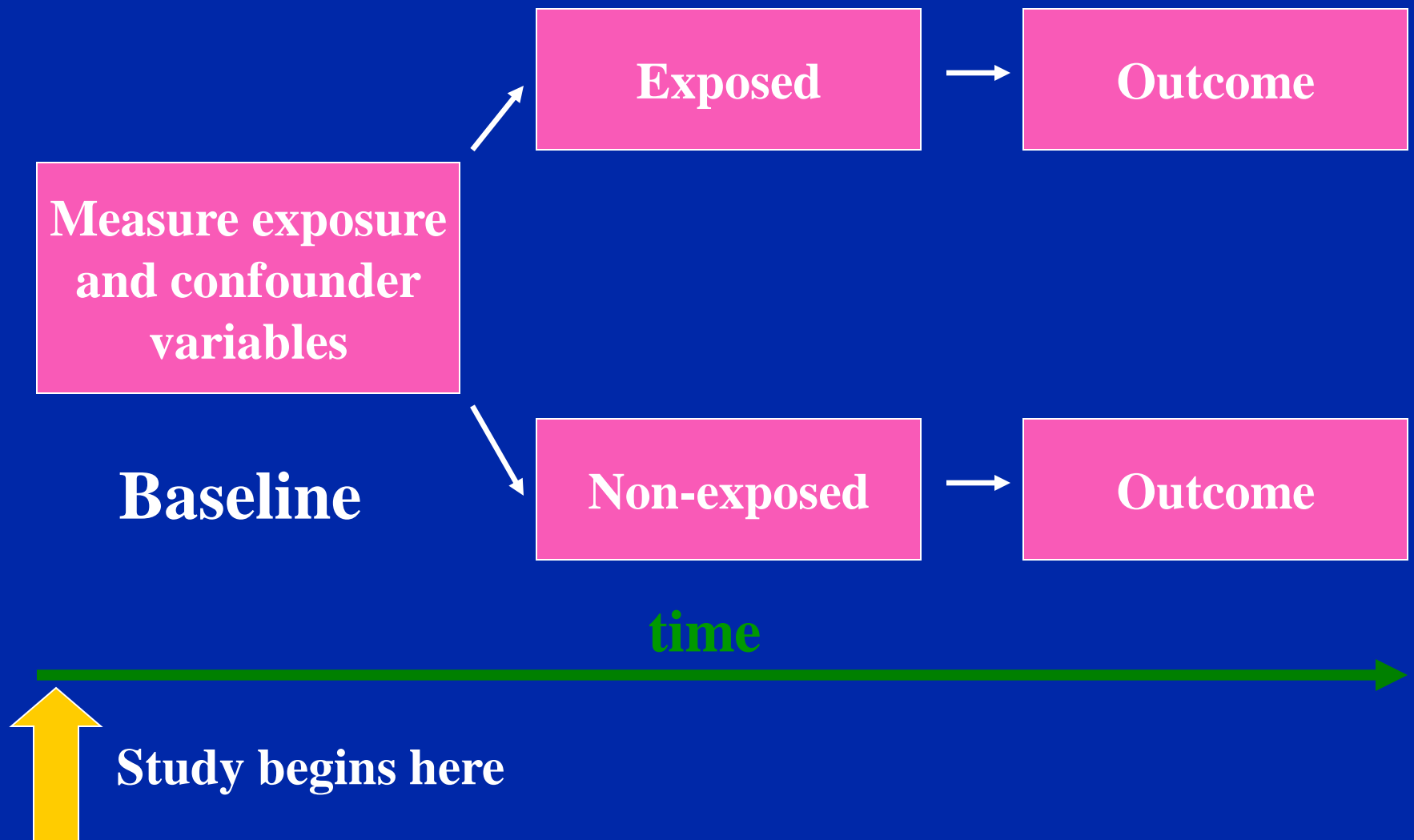


Timeframe of Studies

- **Prospective Study** - looks forward, looks to the future, examines future events, follows a condition, concern or disease into the future



Prospective Cohort study



Cohort Study

- **Strengths**

- **Exposure status determined before disease detection**
- **Subjects selected before disease detection**
- **Can study several outcomes for each exposure**

- **Limitations**

- **Expensive and time-consuming**
- **Inefficient for rare diseases or diseases with long latency**
- **Loss to follow-up**

Experimental Studies

- investigator can “control” the exposure
- akin to laboratory experiments except living populations are the subjects
- generally involves random assignment to groups
- clinical trials are the most well known experimental design
- the ultimate step in testing causal hypotheses

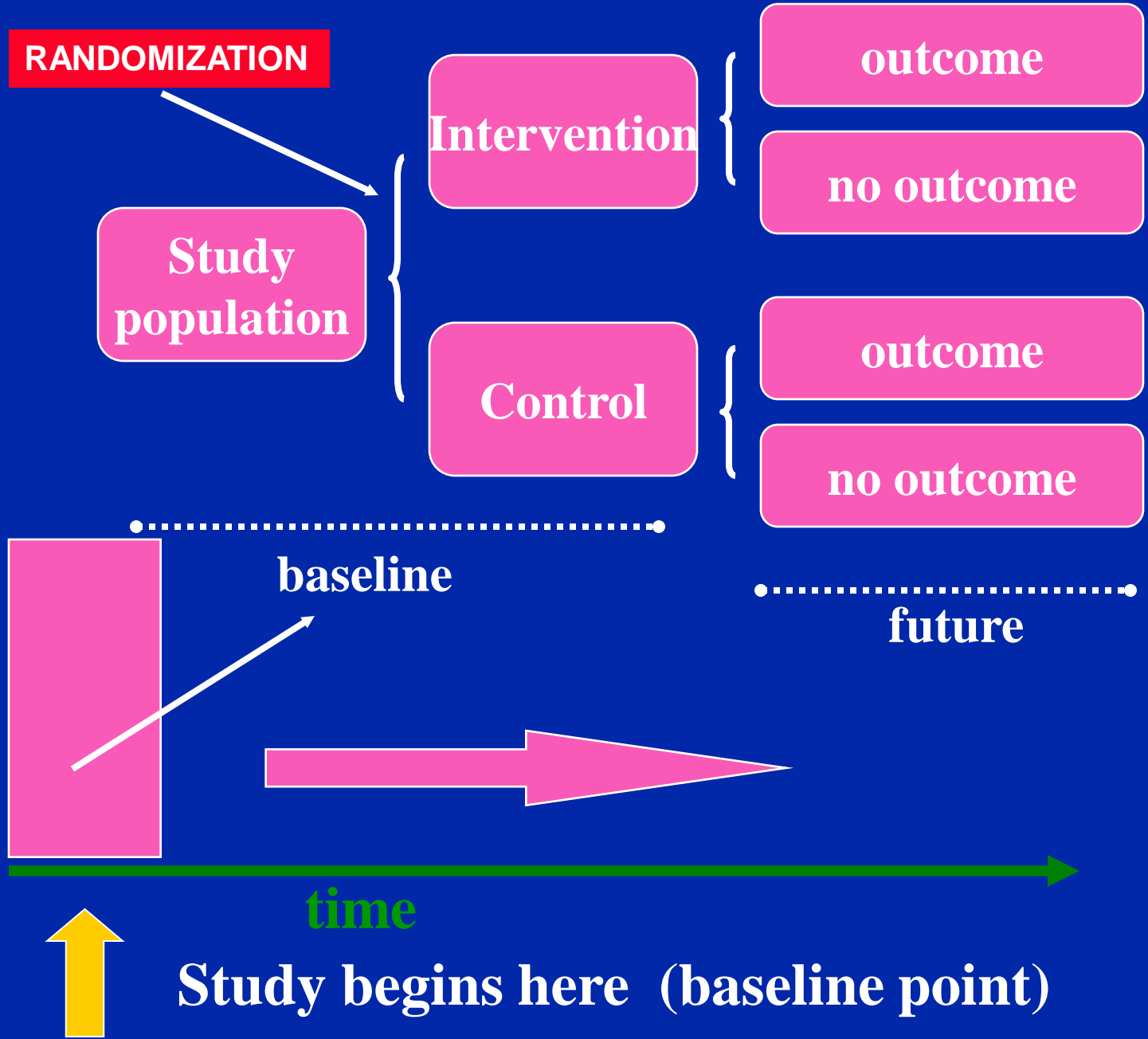
Experimental Studies

- In an experiment, we are interested in the consequences of some treatment on some outcome.
- The subjects in the study who actually receive the treatment of interest are called the **treatment group**.
- The subjects in the study who receive no treatment or a different treatment are called the **comparison group**.

Epidemiologic Study Designs

- **Randomized Controlled Trials (RCTs)**
 - a design with subjects randomly assigned to “treatment” and “comparison” groups
 - provides most convincing evidence of relationship between exposure and effect
 - not possible to use RCTs to test effects of exposures that are expected to be harmful, for ethical reasons

Experimental Design





Epidemiologic Study Designs

- **Randomized Controlled Trials (RCTs)**
 - the “gold standard” of research designs
 - provides most convincing evidence of relationship between exposure and effect
- *trials of hormone replacement therapy in menopausal women found no protection for heart disease, contradicting findings of prior observational studies*

Randomized Controlled Trials

- **Disadvantages**
 - **Very expensive**
 - **Not appropriate to answer certain types of questions**
 - **it may be unethical, for example, to assign persons to certain treatment or comparison groups**