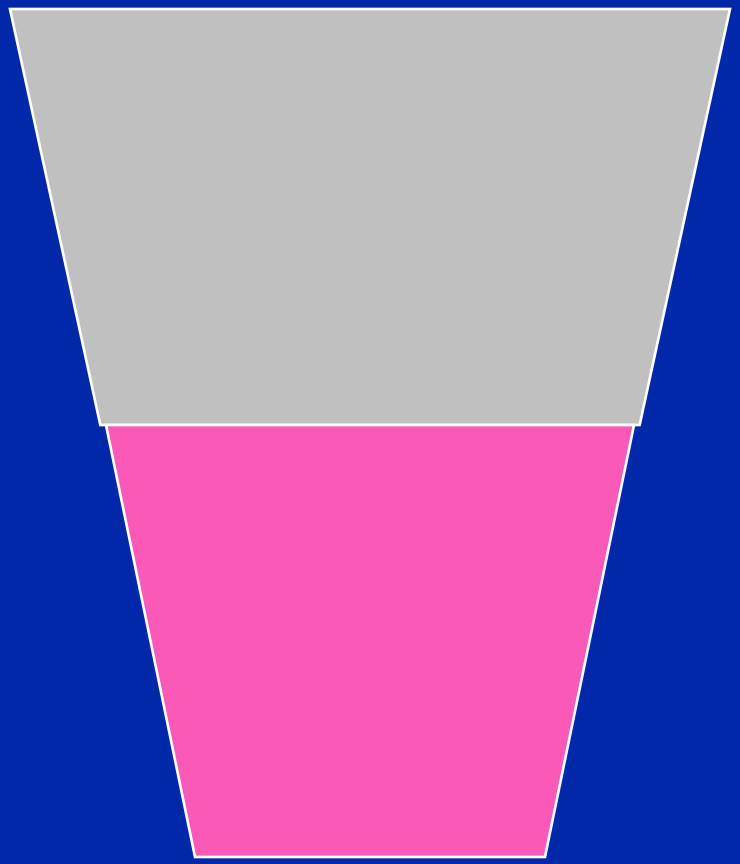
Study Designs in Epidemiologic Research


ONEXOX

How we view the world…..



- *Pessimist:* The glass is half empty.
- *Optimist*: The glass is half full.
- *Epidemiologist*: As compared to what?

Study Designs in Epidemiologic Research

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?**

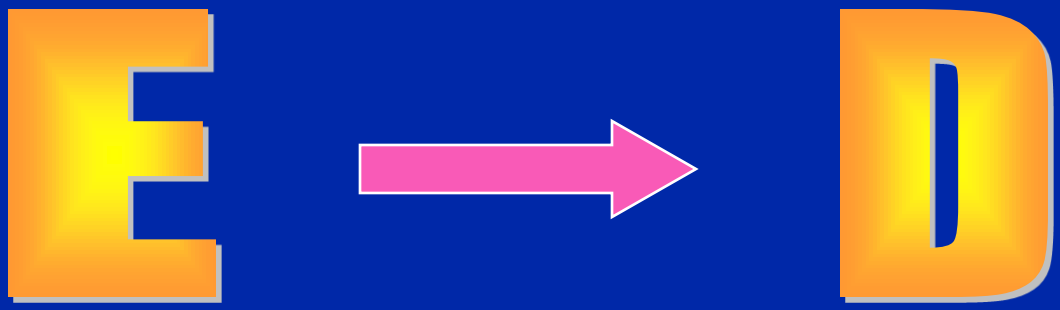
  ➔Formulation of hypotheses concerning causal and preventive factors
- **Identify determinants of disease**
  - **Hypotheses are tested using epidemiologic studies**

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?



Exposure **Disease**

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?**

Wijngaarden

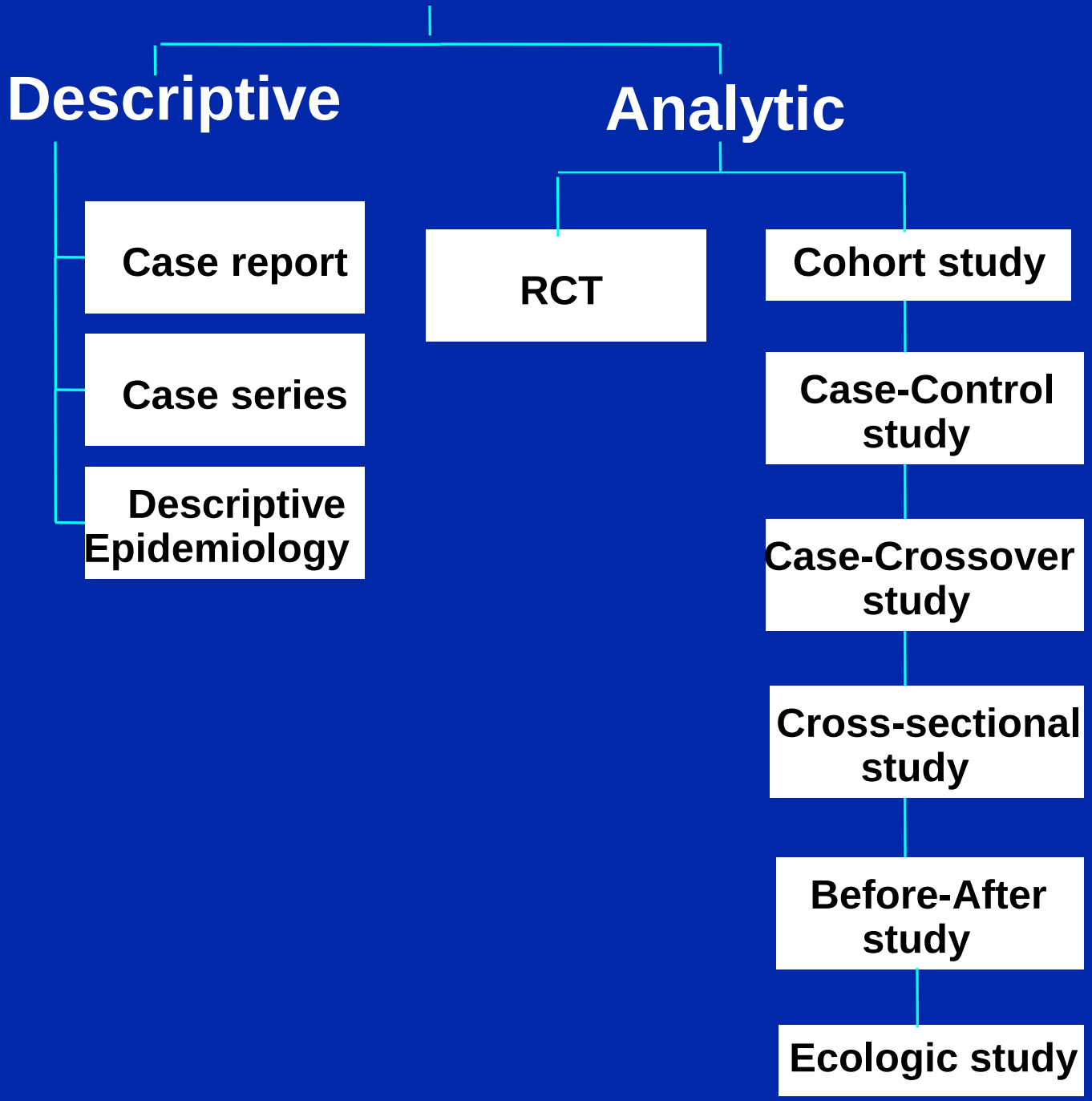
Basic Research Study Designs and their Application to Epidemiology

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

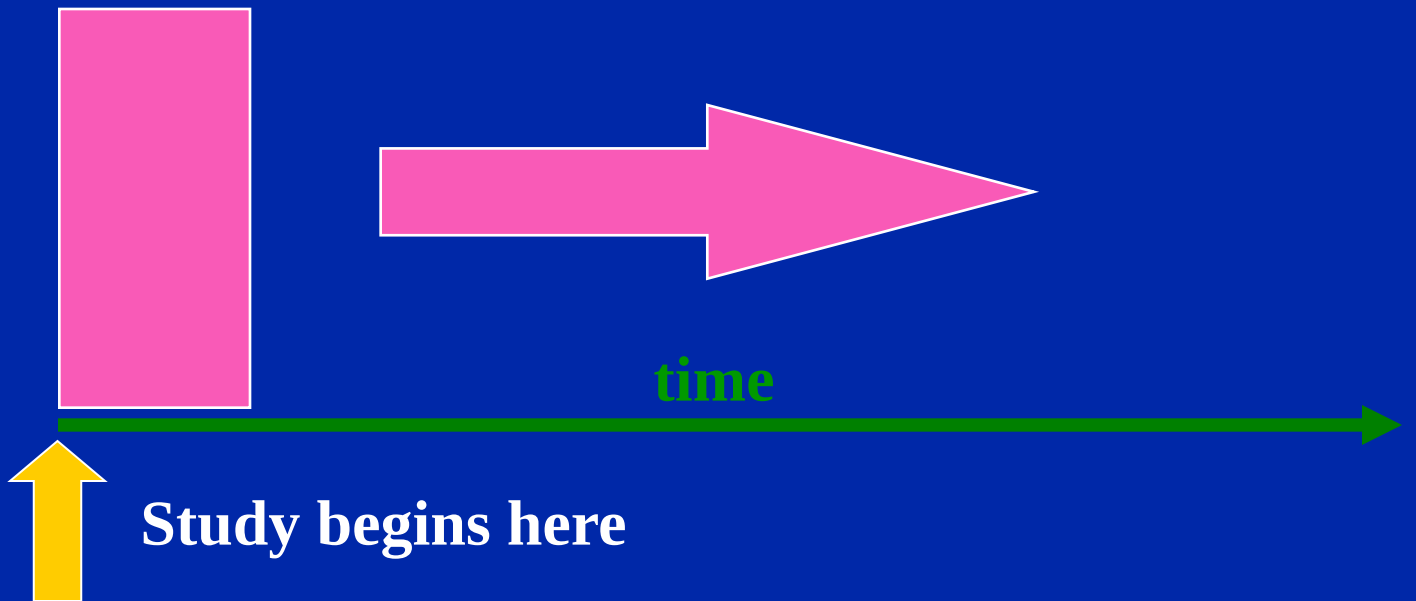
What designs exist to identify and investigate factors in disease?

Study Designs



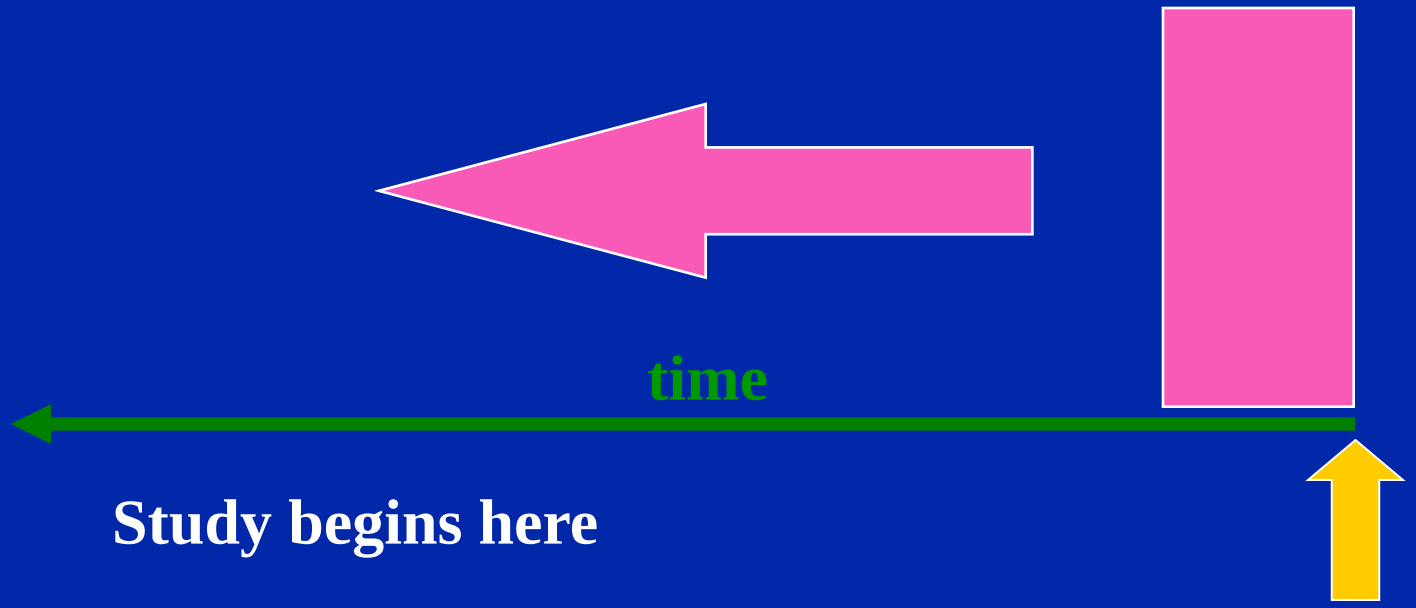
Timeframe of Studies

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

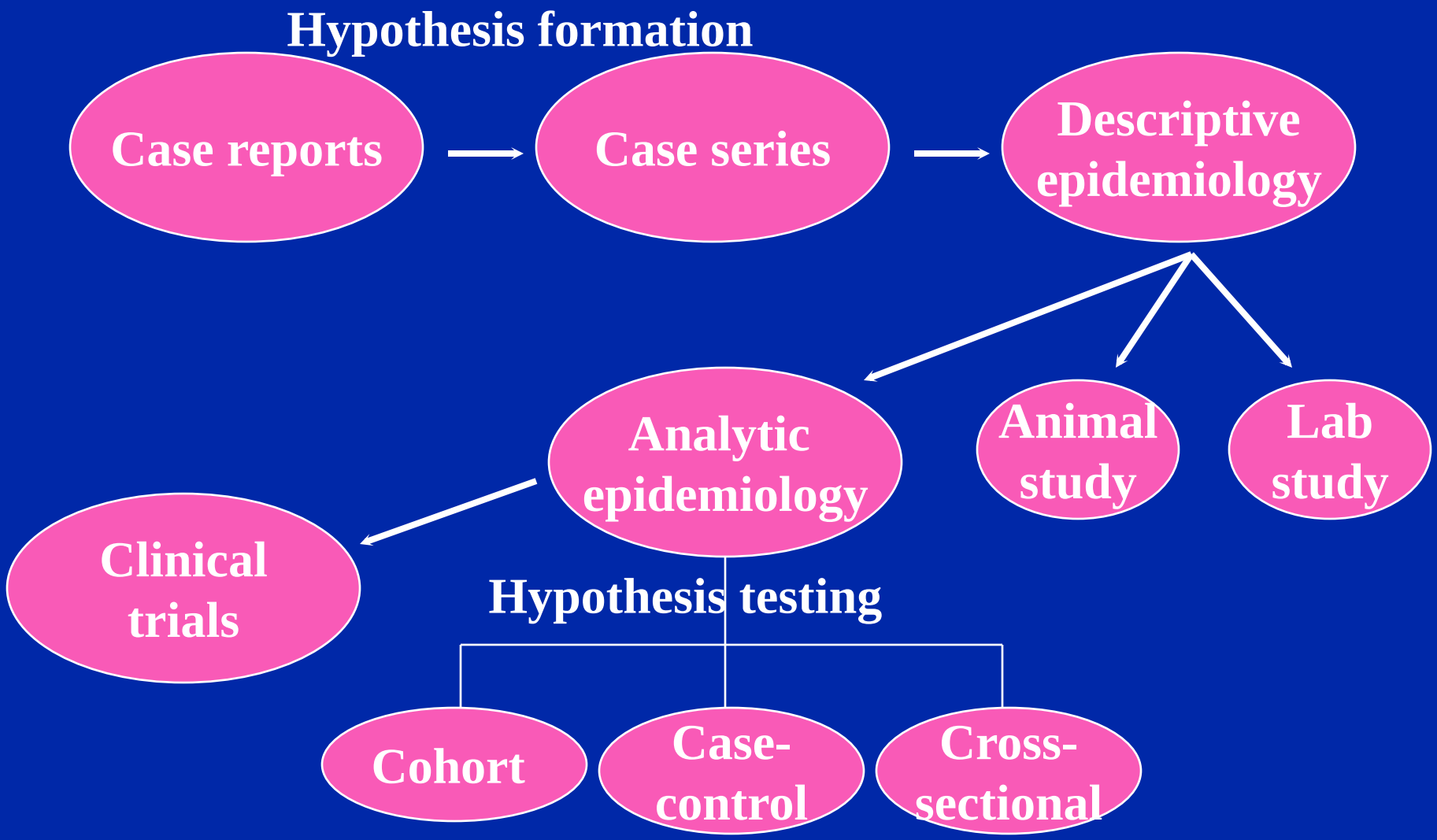


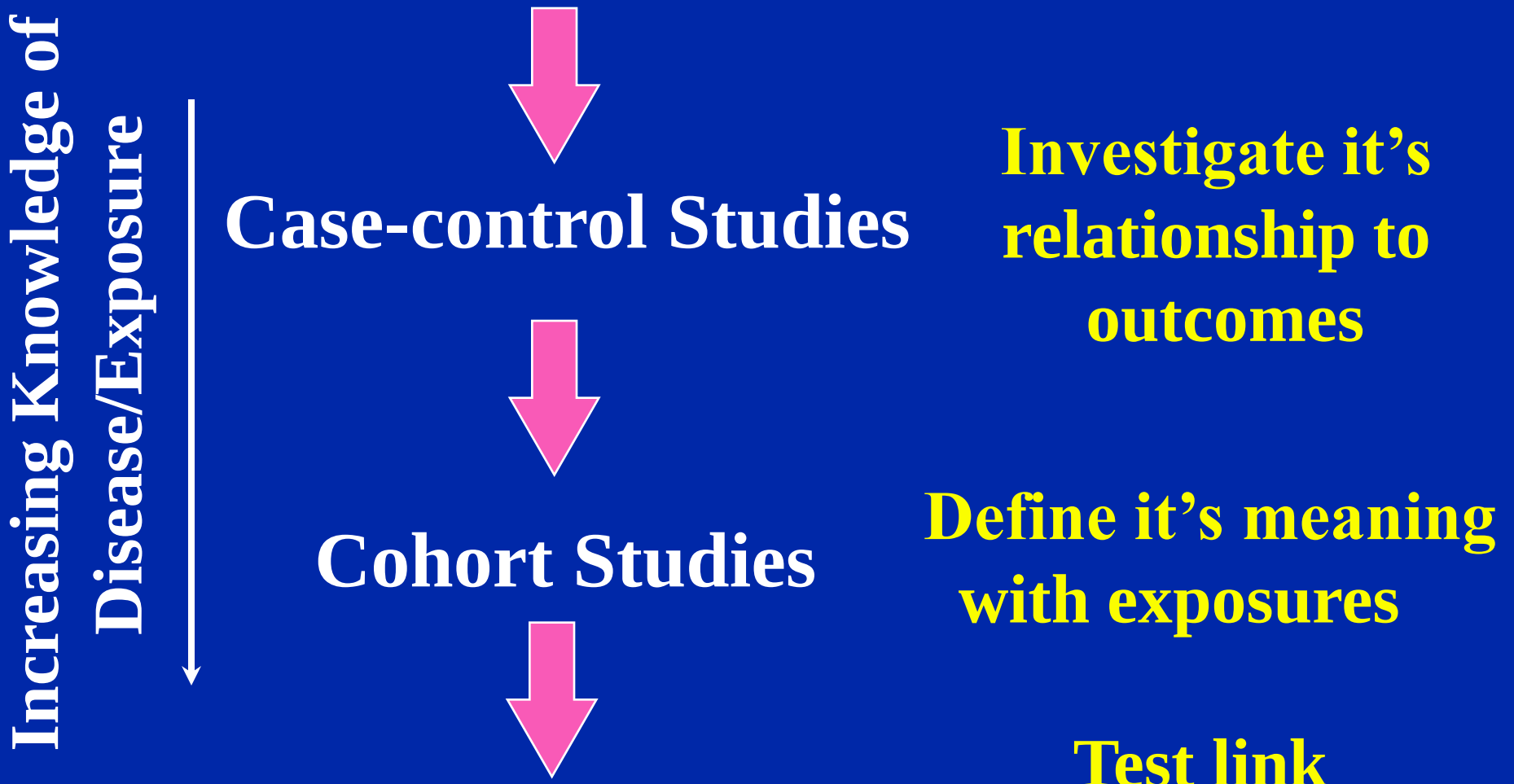
Timeframe of Studies

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



Study Design Sequence




Descriptive Studies
Develop hypothesis
Increasing Knowledge of Disease/Exposure
Case-control Studies
Investigate it's relationship to outcomes
Cohort Studies
Define it's meaning with exposures
Clinical trials
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

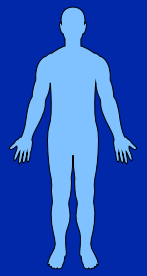
Experimental Studies

- 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

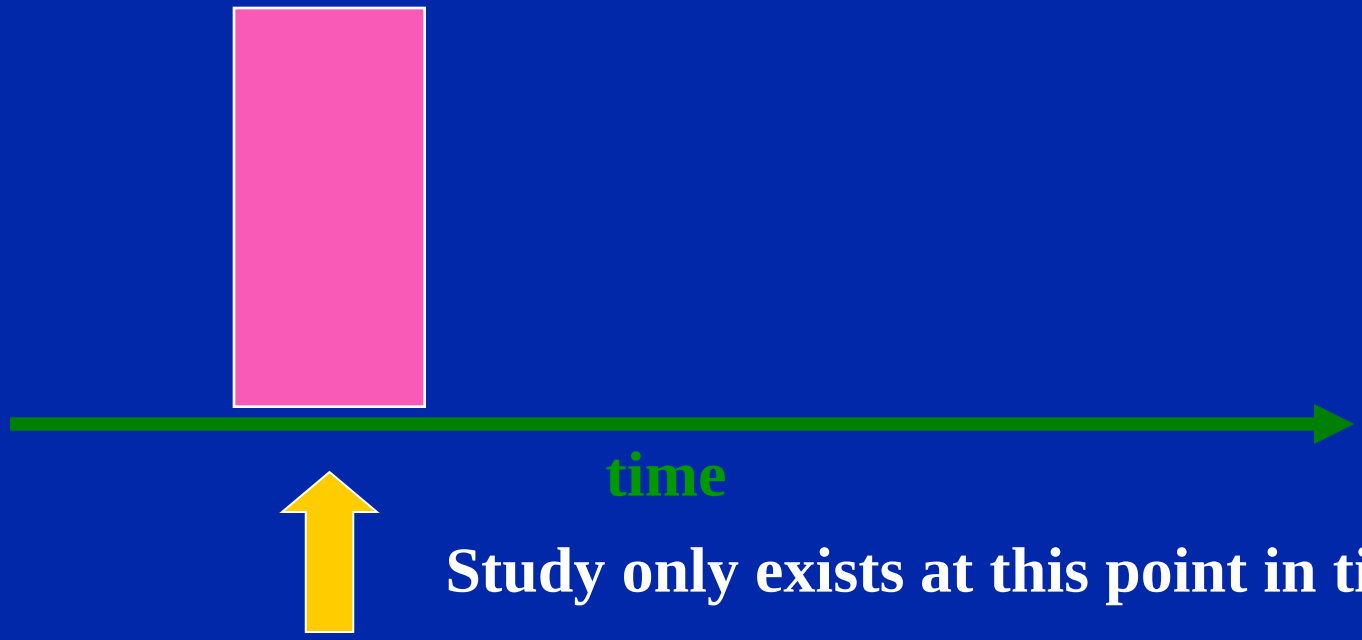
Observational Studies

- 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

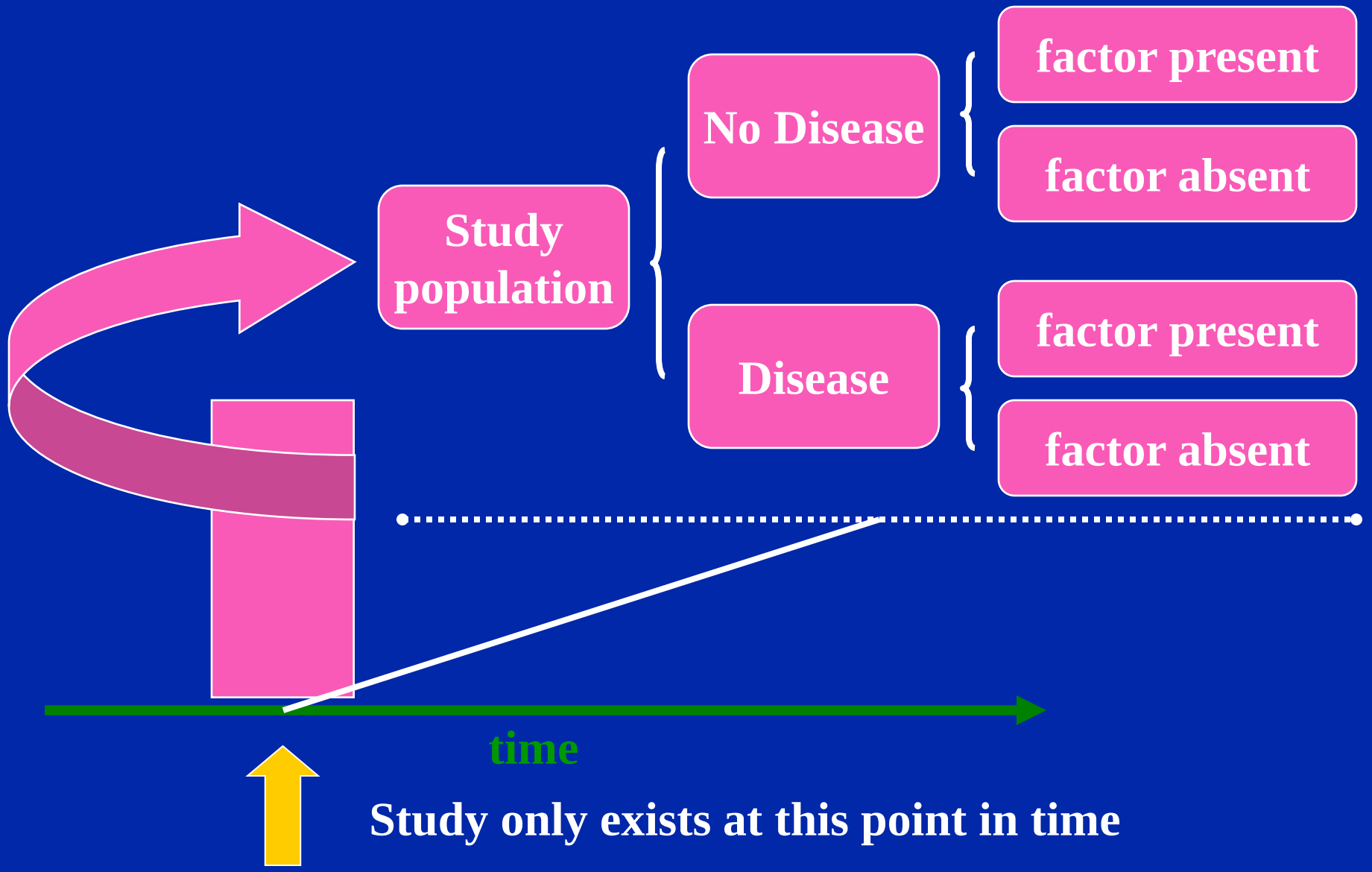
Cross-sectional studies



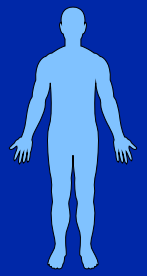
- An “observational” design that surveys exposures and disease status at a single point in time (a cross-section of the population)



Cross-sectional Design

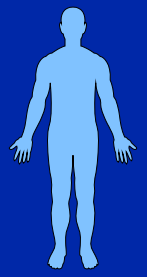


Cross-sectional Studies



- 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



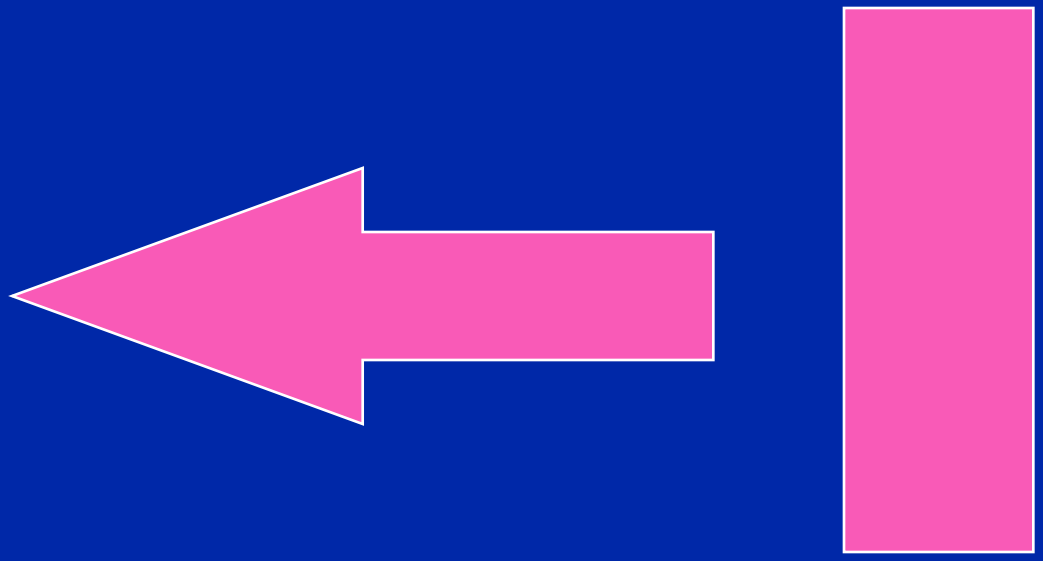
- **Disadvantages**
  - **Weakest observational design, (it measures prevalence, not incidence of disease). Prevalent cases are survivors**
  - **The temporal sequence of exposure and effect may be difficult or impossible to determine**
  - **Usually don't know when disease occurred**
  - **Rare events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

Epidemiologic Study Designs

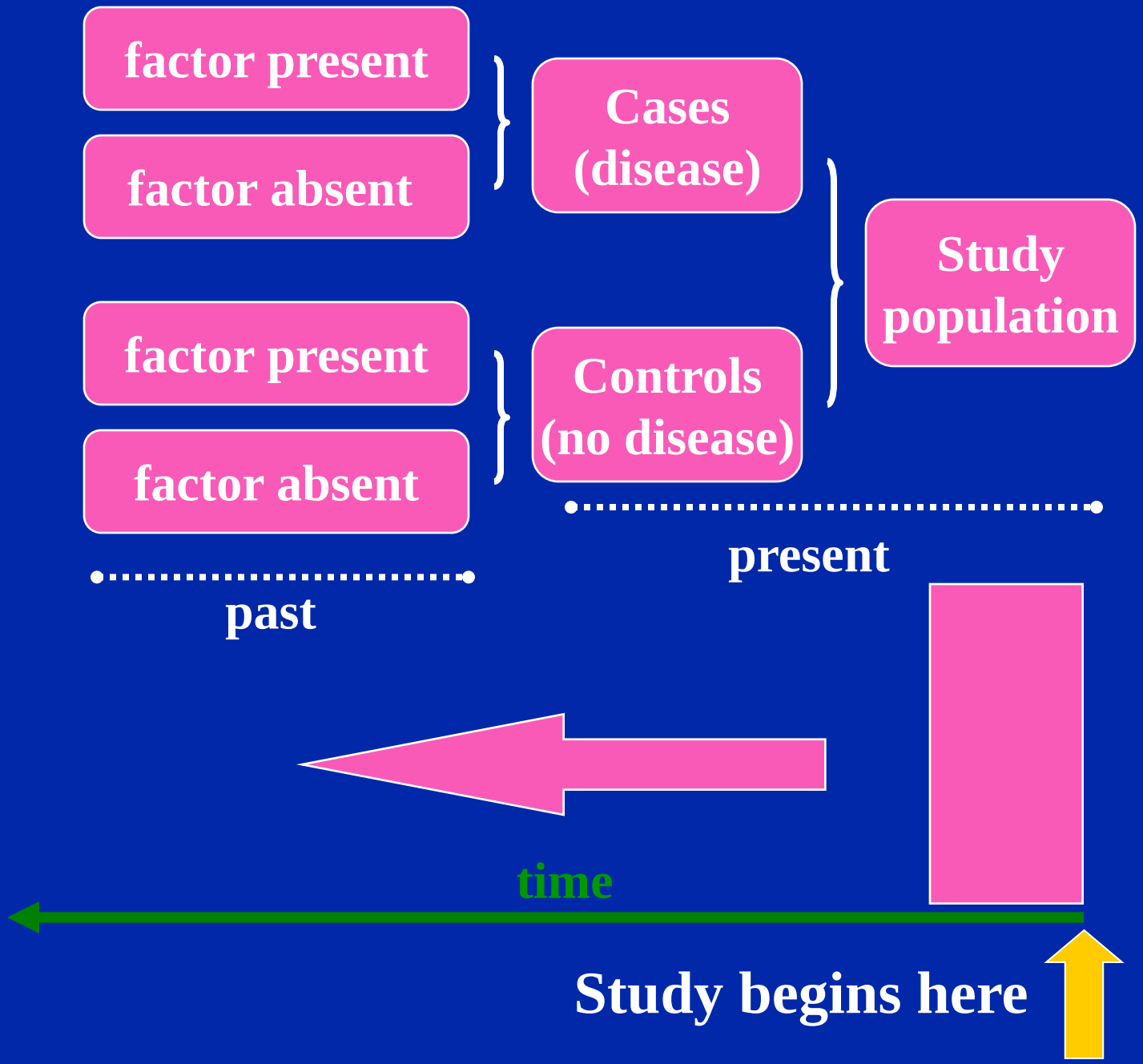
- **Case-Control Studies**
  - **an "observational" design comparing exposures in disease cases vs. healthy controls from same population**
  - **exposure data collected retrospectively**
  - **most feasible design where disease outcomes are rare**

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**

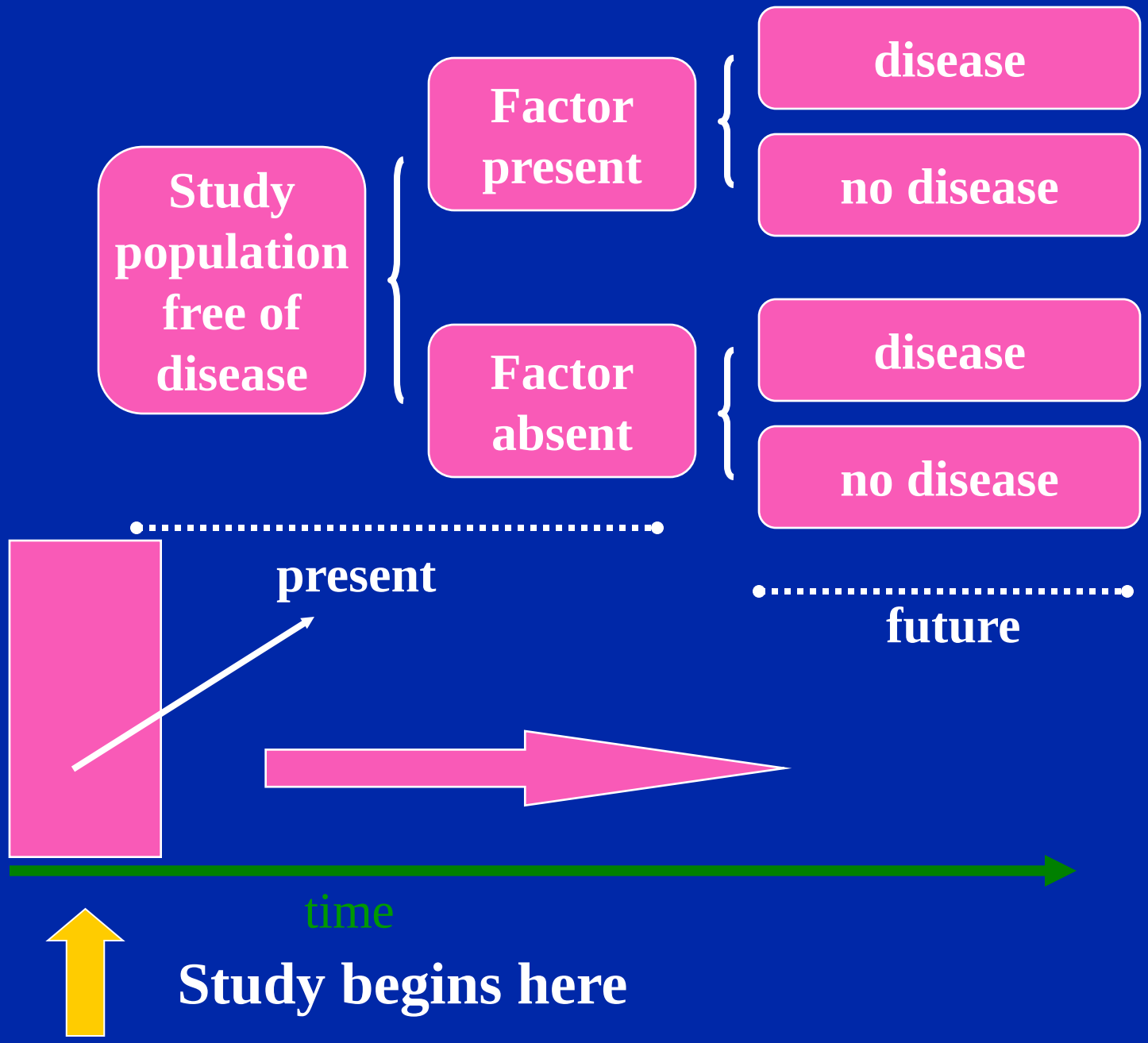
Hypothesis Testing: Case-Crossover Studies

- Study of “triggers” within an individual
- ”Case" and "control" component, but information of both components will come from the same individual
- ”Case component" = hazard period which is the time period right before the disease or event onset
- ”Control component" = control period which is a specified time interval other than the hazard period

Epidemiologic Study Designs

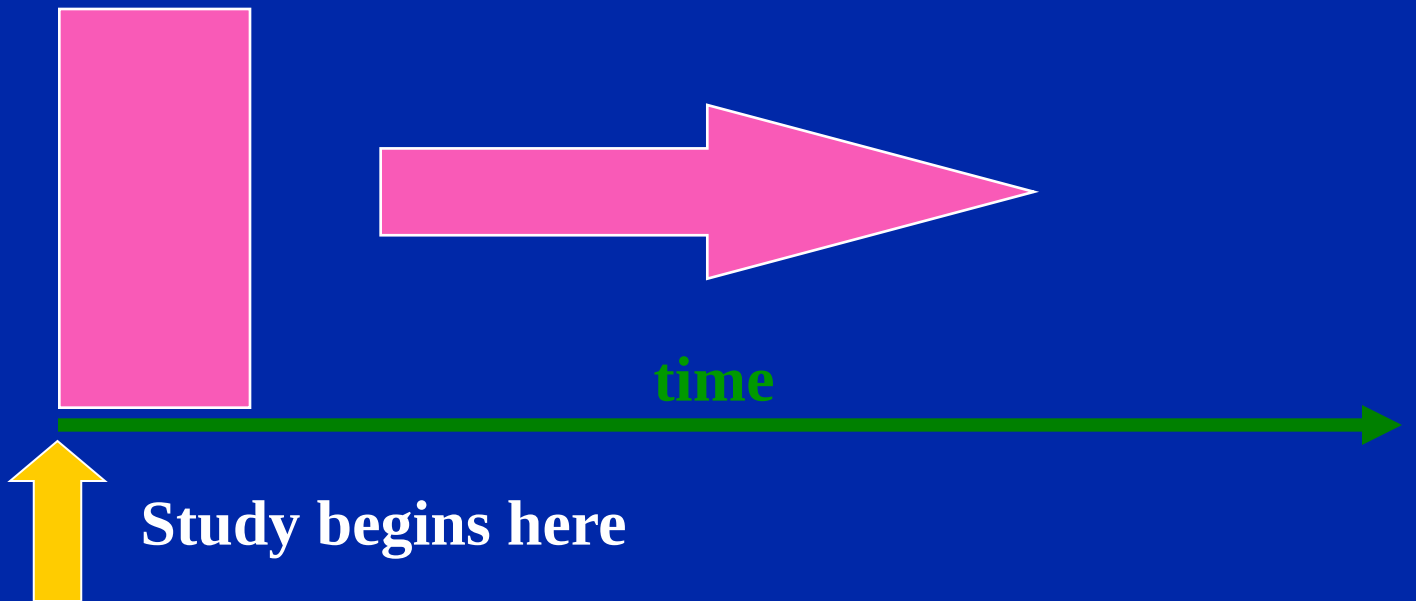
- **Cohort Studies**
  - **an "observational" design comparing individuals with a known risk factor or exposure with others without the risk factor or exposure**
  - **looking for a difference in the risk (incidence) of a disease over time**
  - **best observational design**
  - **data usually collected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

Cohort Design

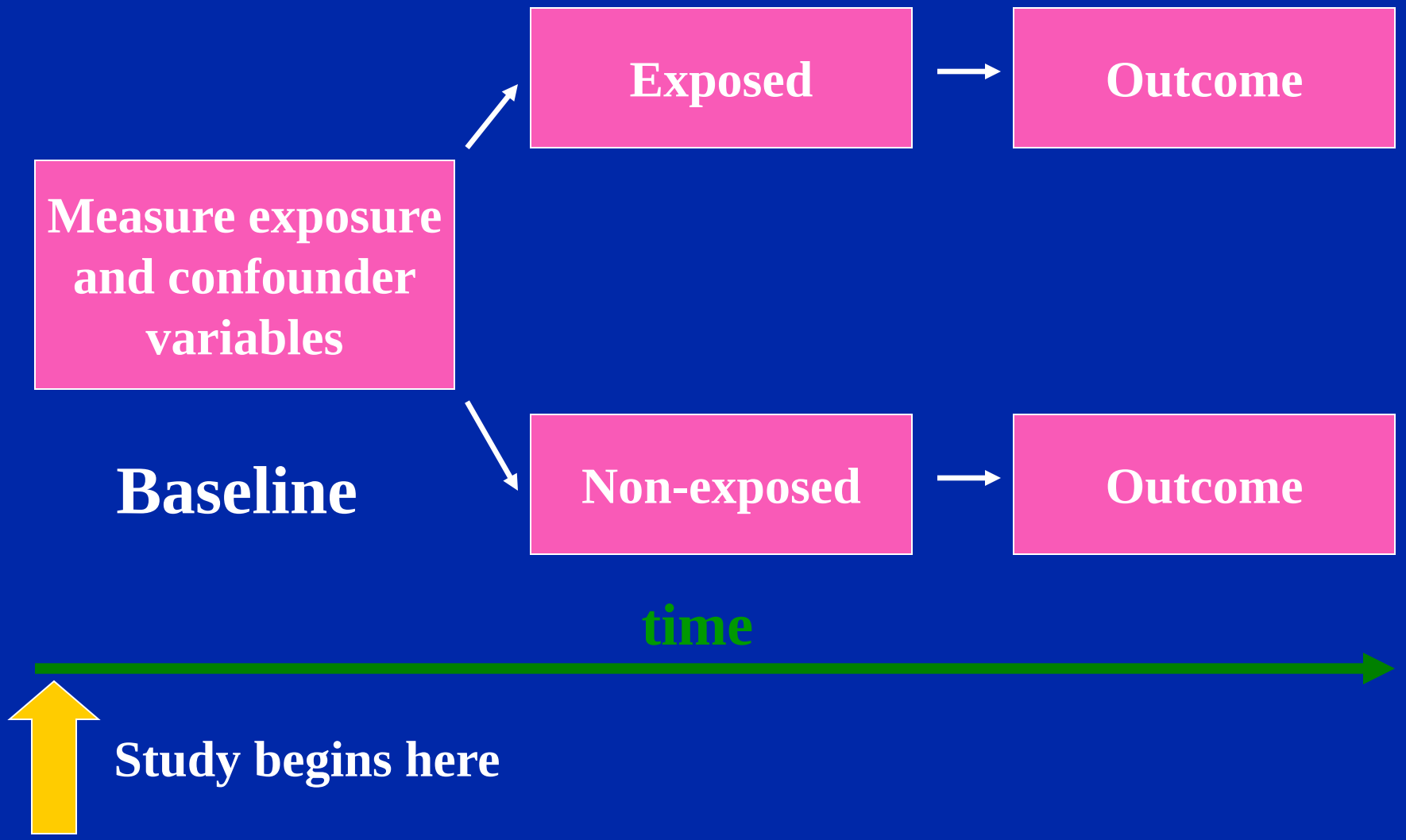


Timeframe of Studies

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

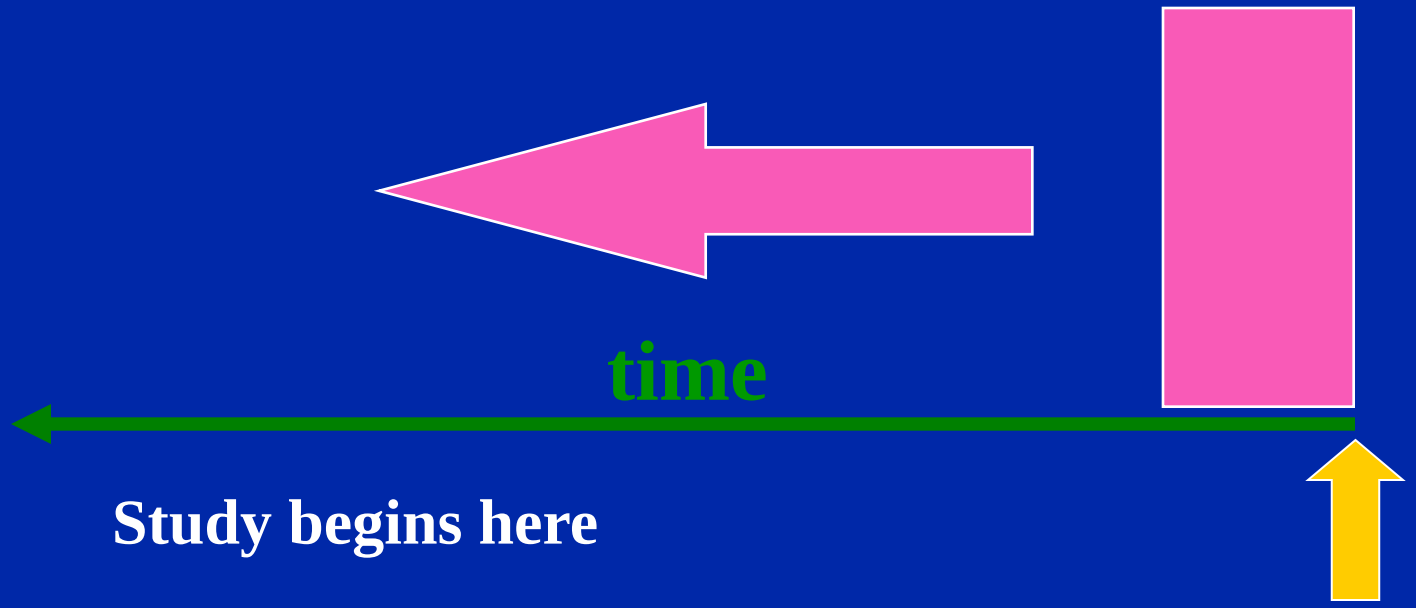


Prospective Cohort study

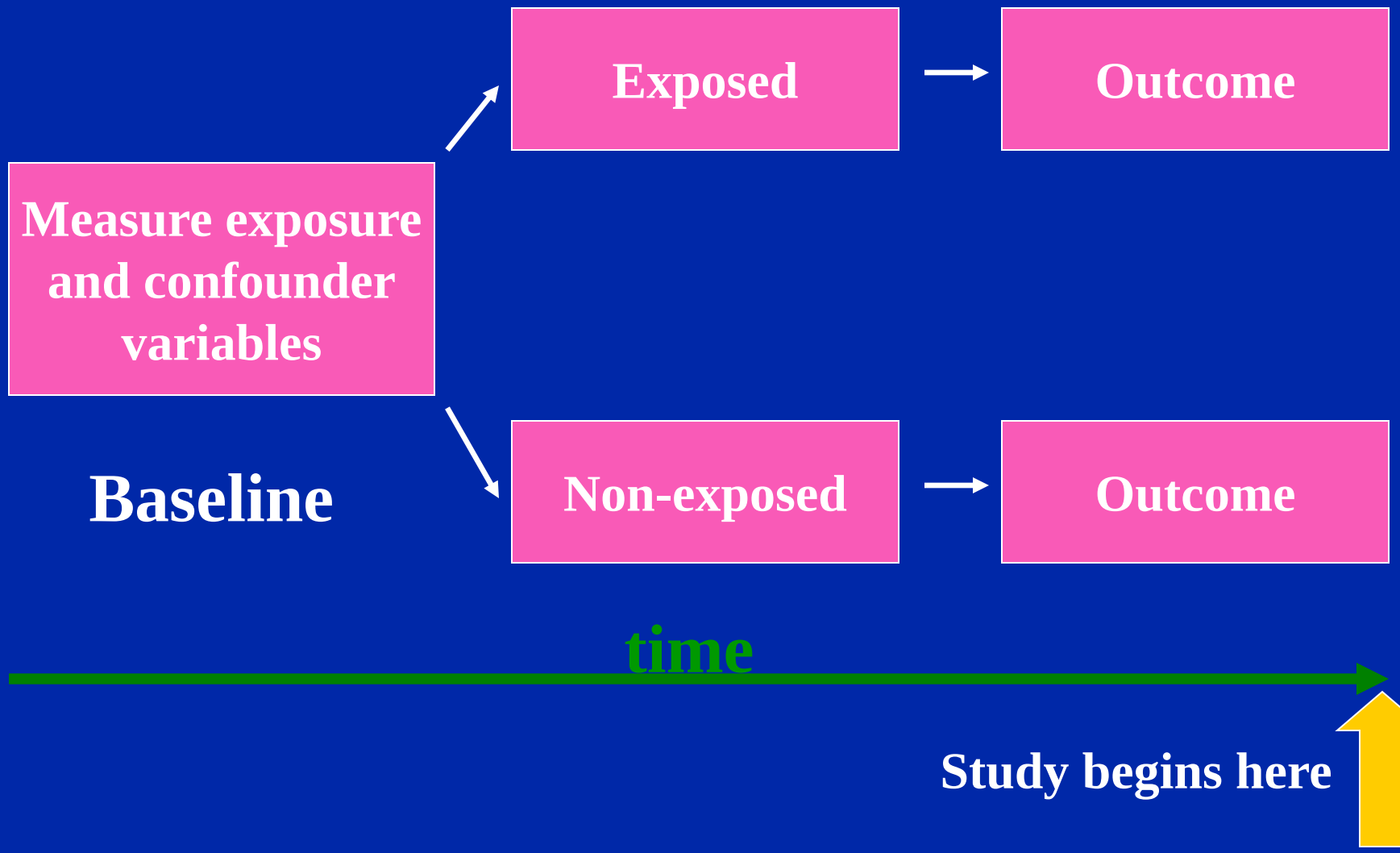


Timeframe of Studies

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



Retrospective 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

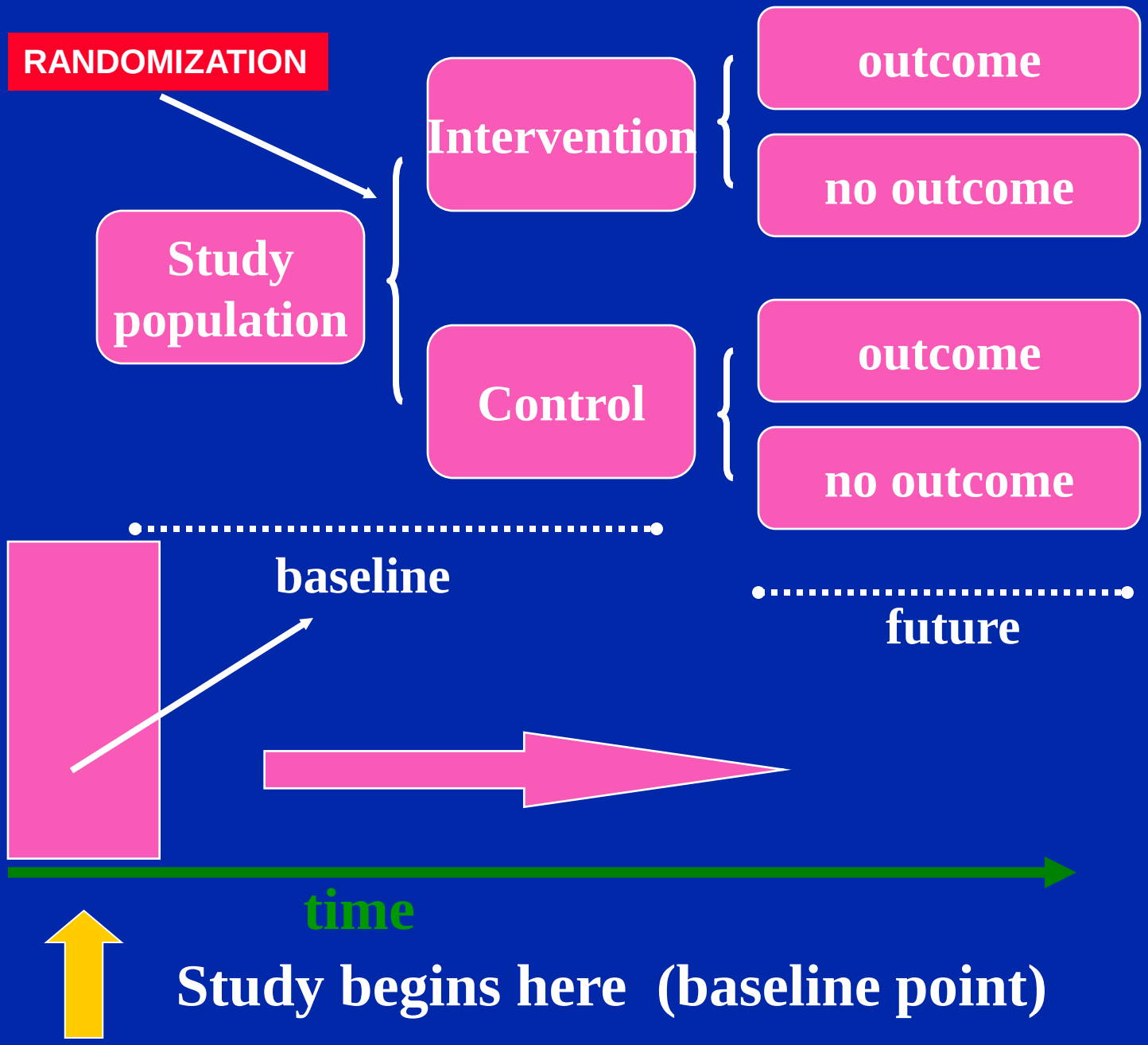
Experimental Studies

- **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**

Review Questions

- Describe the link between exposure and disease
- Describe study design sequence
- Describe strengths and weaknesses of each design

Thanks

- Thomas J. Songer
- University of Pittsburgh team