

# Study Design - Part 1

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# Evidence-based Medicine

## Key Terms:

Evidence based medicine

Primary studies

Descriptive/Observational studies

**Case report (and Case series)**

**Prevalence Survey**

Analytic

**Case-control study (retrospective)**

**Cohort study (prospective)**

Experimental/Interventional Studies

**Clinical trials (randomized controlled trials)**

Secondary Studies

**Meta-analysis**

Hierarchy of Evidence

Reading: Glaser. High-Yield Biostatistics, 3rd edition

# Evidence Based Medicine (EBM)

## Definition:

The enhancement of a clinician's traditional skills in diagnosis, treatment, prevention, and related areas through the systematic framing of relevant and answerable questions and the use of mathematical estimates of probability and risk.

Donald A, Greenhalgh T. A hands on guide to evidence based health care: practice and implementation. Blackwell Science, Oxford, UK. 2000

# EBM - Steps

Convert your information needs into answerable questions

Find the best evidence (systematic search of the medical literature)

Critically appraise the evidence with respect to its validity and utility

Implement changes based on your appraisal

Evaluate the results

# Primary vs. Secondary Studies

## **Primary studies**

*Descriptive/Observational studies*

**Case report (and Case series)**

**Prevalence Survey**

*Analytic studies*

**Case-control study (retrospective)**

**Cohort study (prospective)**

*Experimental/Interventional Studies*

**Randomized controlled clinical trials**

## **Secondary Studies**

**Meta-analysis, Systematic reviews**

# Primary Studies

## ■ Descriptive / Observational

- These studies describe the occurrence or distribution of disease. They do not attempt to explain a causal relation or test a hypothesis. Descriptive statistics are generated

## ■ Analytic

- *These studies observe groups of individuals and various characteristics are recorded for analysis. There is no controlled intervention (experiment), but they attempt to test hypotheses or explain causal relations through comparisons using inferential statistics.*

## ■ Experimental

- *A controlled intervention is performed on a group of patients (clinical trials) or small numbers of volunteers (experiments)*

# Primary Studies – Descriptive

## ■ Case report or case series:

- A careful, detailed description of a single patient (case report) or a series of patients (case series) with a specific disease or characteristic of interest. There is no comparison group. These studies are generally used to report a new disease, a new manifestation of a known disease, an adverse response to a drug, or a new possible treatment for a disease. The scientific evidence from these studies is generally thought to be weak. They can be used to *generate hypotheses* about the cause of disease, its diagnosis, or treatment. They cannot be used to *establish* the cause, diagnosis or treatment of the disease in question with any validity.

# Primary Studies – Descriptive

## ■ Prevalence surveys:

- Data are collected at a single time point (rather than over a period of time) yielding a "snapshot" of what is happening in the population at that time. These studies are most useful for estimating the frequency of disease in a population (prevalence). These studies are also used to study the utility of diagnostic tests (they can determine the percentage of people with a given disease have a positive or negative test).
- Again, they can be used to *generate hypotheses* about the cause of disease in a population but they cannot *establish* the cause with great validity.

# Primary Studies – Analytic

## ■ Cohort studies:

- These studies are generally concerned with determining the etiology of disease. They follow a carefully defined population that has been or may become exposed to a factor or factors that are thought to contribute to the occurrence of disease or other outcome. Subsets of the population (called cohorts) are grouped according to their exposure status. Cohort studies may be prospective or retrospective, but most are **prospective** (and the cohorts are followed over time). Cohort studies are most useful for establishing risk factors for disease.

# Primary Studies – Analytic

## ■ Case-control studies:

- These studies are also generally concerned with determining the etiology of disease. They use data from case patients (*who are known to have the characteristic or disease of interest at the outset of the study*) and control patients (who do not have the disease of interest) to test theories about disease occurrence by looking at past exposures and risk factors. Thus, case-control studies are **retrospective**.

# Experimental Studies

## ■ **Randomized Controlled Trial:**

- A large interventional study in which participants (with a defined set of characteristics) are randomly assigned to two or more treatment groups and followed for a specified period. The randomization should ensure that the treatment groups are equal at the outset. This allows the best chance of studying the effect of a single (treatment) variable. These studies are considered the gold standard of medical research, but not all questions can be studied in this fashion.

# Secondary Studies:

- Studies or reports that summarize and draw conclusions from a review of multiple primary studies.
  - Systematic reviews (a careful review of each of the primary studies in accordance with pre-established criteria)
    - **Meta-analysis** – A particular type of systematic review that involves the synthesis of numerical results from several studies that investigated the same question. By combining the data from several studies, a meta-analysis can usually make conclusions that are more powerful than a single study.

# Hierarchy of Evidence

- The relative weight given to the evidence from each type of study is generally ranked in the order below (from best evidence to least best evidence)
  - Meta-analyses and systematic reviews
  - Randomized controlled trials
  - Cohort studies
  - Case control studies
  - Prevalence surveys
  - Case reports and case series