UNDERSTANDING RESEARCH STUDY DESIGNS

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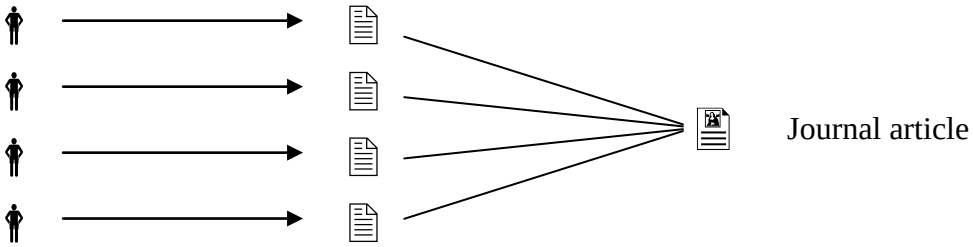
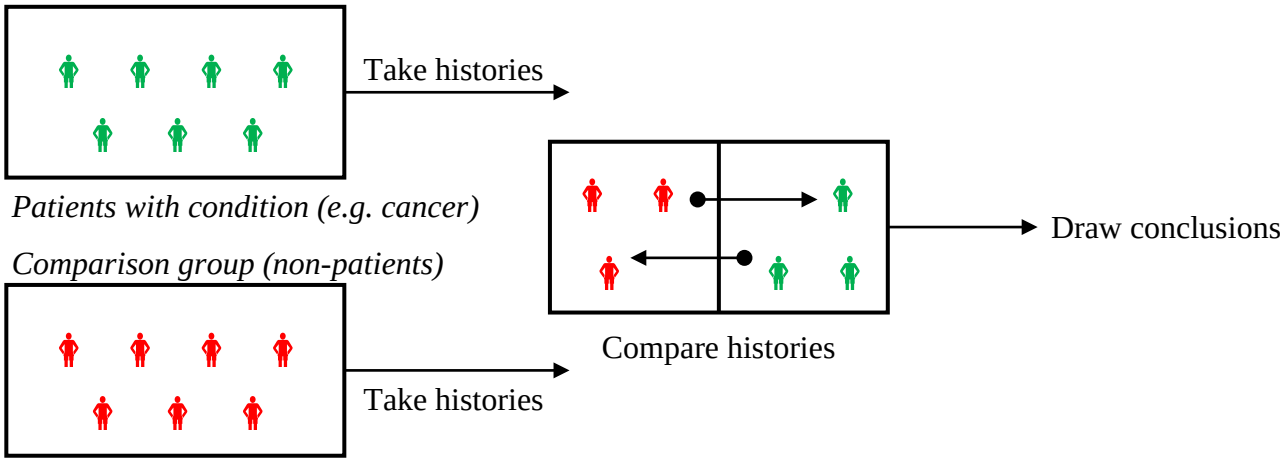
In order to find the best possible evidence, it helps to understand the basic designs of research studies. The following basic definitions and examples of clinical research designs follow the "hierarchy of evidence."

Case Series and Case Reports: These consist either of collections of reports on the treatment of individual patients with the same condition, or of reports on a single patient. Case series/reports are used to illustrate an aspect of a condition, the treatment or the adverse reaction to treatment.



Example: You have a patient that has a condition that you are unfamiliar with. You would search for case reports that could help you decide on a direction of treatment or to assist on a diagnosis. Case series/reports have no control group (one to compare outcomes), so they have no statistical validity. The benefits of case series/reports are that they are easy to understand and can be written up in a very short period of time.

Case Control Studies: Patients who already have a certain condition are compared with people who do not.



Case control studies are generally designed to estimate the odds (using an odds ratio) of developing the studied condition /disease. They can determine if there is an associational relationship between condition and risk factor.

Example: A study in which colon cancer patients are asked what kinds of food they have eaten in the past and the answers are compared with a selected control group. Case control studies are less reliable than either randomized controlled trials or cohort studies. A major drawback to case control studies is that one cannot directly obtain absolute risk (i.e. incidence) of a bad outcome. The advantages of case control studies are they can be done quickly and are very efficient for conditions/diseases with rare outcomes.

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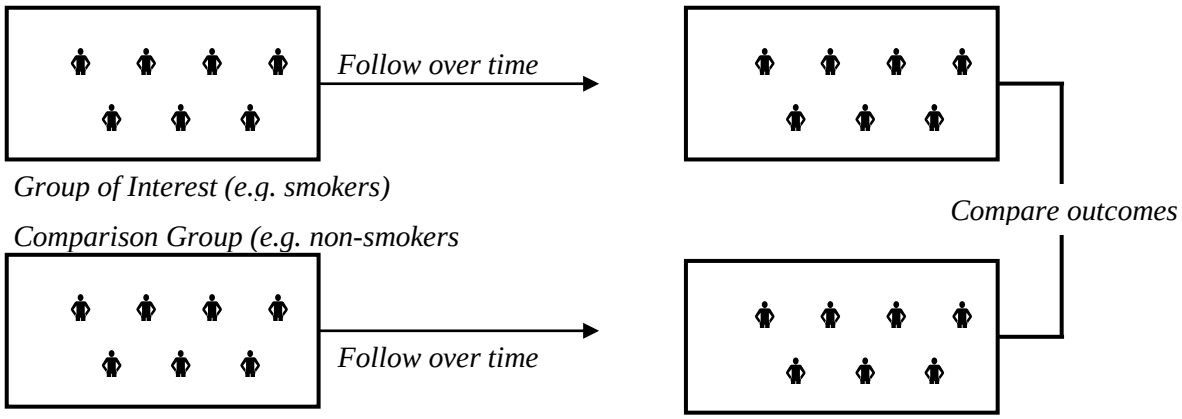
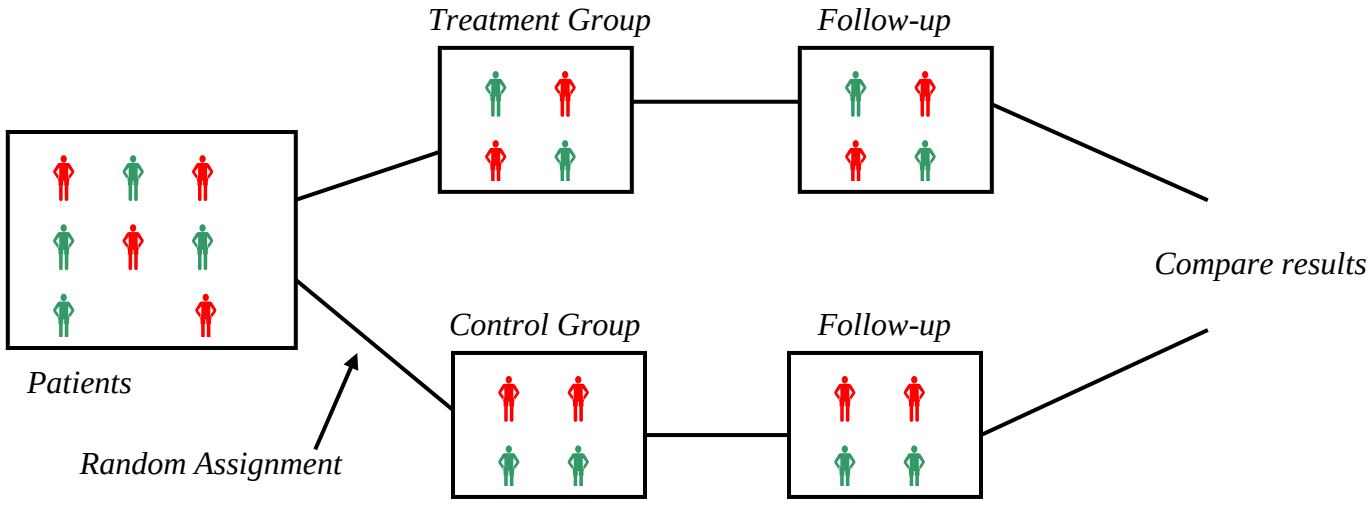
Cohort Studies: or *longitudinal* studies involve a case-defined population who presently have a certain exposure and/or receive a particular treatment that are followed over time and compared with another group who are not affected by the exposure under investigation.



Cohort studies may be either prospective (i.e., exposure factors are identified at the beginning of a study and a defined population is followed into the future), or historical/retrospective (i.e., past medical records for the defined population are used to identify exposure factors). Cohort studies are used to establish causation of a disease or to evaluate the outcome/impact of treatment, when randomized controlled clinical trials are not possible.

Example: One of the more well-know examples of a cohort study is the Framingham Heart Study, which followed generations of residents of Framingham, Massachusetts. Cohort studies are not as reliable as randomized controlled studies, since the two groups may differ in ways other than the variable under study. Other problems with cohort studies are that they require a large sample size, are inefficient for rare outcomes, and can take long periods of time.

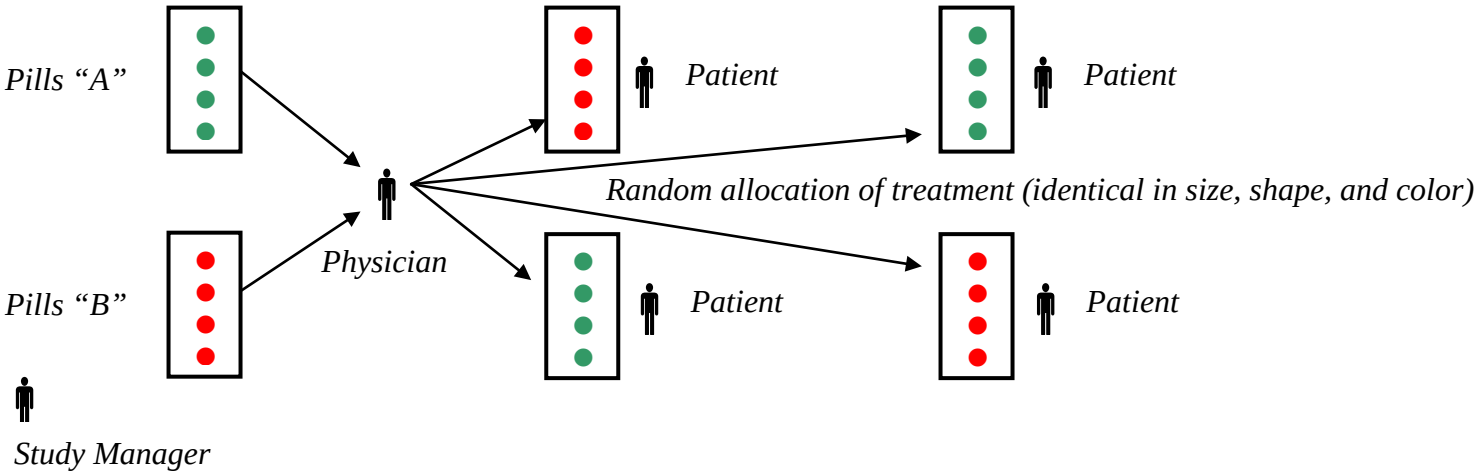
Randomized Controlled Studies: a study in which 1) There are two groups, one treatment group and one control group. The treatment group receives the treatment under investigation, and the control group receives either no treatment (placebo) or standard treatment. 2) Patients are randomly assigned to all groups.



Randomized controlled trials are considered the "gold standard" in medical research. They lend themselves best to answering questions about the effectiveness of different therapies or interventions. Randomization helps avoid the bias in choice of patients-to-treatment that a physician might be subject to. It also increases the probability that differences between the groups can be attributed to the treatment(s) under study. Having a

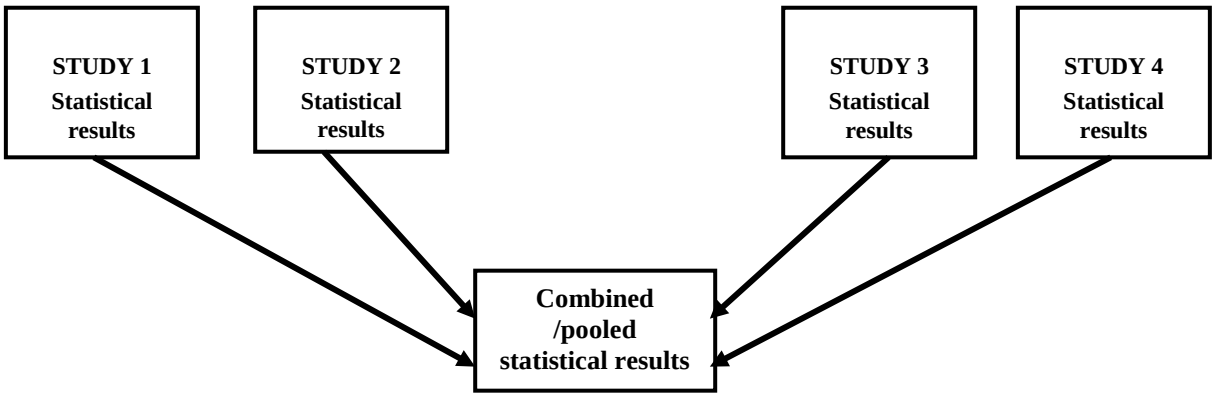
control group allows for a comparison of treatments – e.g., treatment A produced favorable results 56% of the time versus treatment B in which only 25% of patients had favorable results. There are certain types of questions on which randomized controlled studies cannot be done for ethical reasons, for instance, if patients were asked to undertake harmful experiences (like smoking) or denied any treatment beyond a placebo when there are known effective treatments.

Double Blind Method: A type of randomized controlled clinical trial/study in which neither medical staff/physician nor the patient knows which of several possible treatments/therapies the patient is receiving.



Example: Studies of treatments that consist essentially of taking pills are very easy to do double blind – the patient takes one of two pills of identical size, shape, and color, and neither the patient nor the physician needs to know which is which. A double blind study is the most rigorous clinical research design because, in addition to the randomization of subjects, which reduces the risk of bias, it can eliminate or minimize the placebo effect which is a further challenge to the validity of a study.

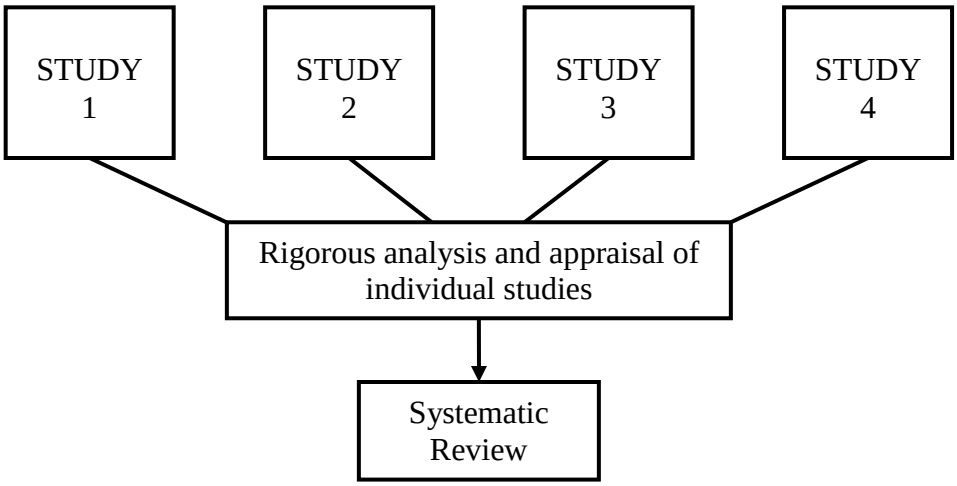
Meta-Analyses: Meta-analysis is a systematic, objective way to combine data from many studies, usually from randomized controlled clinical trials, and arrive at a pooled estimate of treatment effectiveness and statistical significance. Meta-analysis can also combine data from case/control and cohort studies. The advantage to merging these data is that it increases sample size and allows for analyses that would not otherwise be possible. They should not be confused with reviews of the literature or systematic reviews.



Two problems with meta-analysis are publication bias (studies showing no effect or little effect are often not published and just "filed" away) and the quality of the design of the studies from which data is pulled. This can lead to misleading results when all the data on the subject from "published" literature are summarized.

Systematic Reviews : A systematic review is a comprehensive survey of a topic that takes great care to find all relevant studies of the highest level of evidence, published and unpublished, assess each study, synthesize the findings from individual studies in an unbiased, explicit and reproducible way and present a balanced and impartial summary of the findings with due consideration of any flaws in the evidence. In this way it can be used for the evaluation of either existing or new technologies and practices.

Includes both published and unpublished studies



A systematic review is more rigorous than a traditional literature review and attempts to reduce the influence of bias. In order to do this, a systematic review follows a formal process:

- Clearly formulated question
- Published & unpublished (conferences, company reports, “file drawer reports”, etc.) literature is carefully searched for relevant research
- Identified research is assessed according to an explicit methodology
- Results of the critical assessment of the individual studies are combined
- Final results are placed in context, addressing such issues are quality of the included studies, impact of bias and the applicability of the findings

The difference between a systematic review and a meta-analysis is that a systematic review looks at the whole picture (qualitative view), while a meta-analysis looks for the specific statistical picture (quantitative view).