



Chapter 1

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Introduction to Study Design

The science of medical care is advanced through research, and we are consumers of that research, both in the classroom and later in providing care for our own patients. This section provides an overview of epidemiologic concepts and study designs to facilitate a better understanding of this vast and productive field of research. Epidemiology is based on the fundamental assumptions that disease does not occur at random, and that both causal and preventive factors can be identified through a systematic investigation of different groups.¹ These investigations, predicated on accurate measurements of disease and outcome frequencies, involve an array of study designs, each with their own unique advantages and limitations.



4 Methods and Designs for Outcomes Research

Knowledge of both the frequency and distribution of a disease is necessary to understand, formulate, and test hypotheses about its determinants, whether causal or preventive. These measurements of disease and outcome frequency, including incidence, prevalence, and mortality rates, are discussed in Chapter 2.

The chapters on study designs are presented in order of the ascending rigor of their design. We begin with a chapter on ecologic analyses (Chapter 3), which involve comparisons of groups and are especially well-suited to exploring relationships between a disease and its potential causes. Typically, the groups are derived from geographically defined areas, such as nations, states, or counties, and the analyses are based on existing data from these populations.

In contrast, case-control, cohort, and randomized clinical trials all focus on comparisons of individuals. Information for these analyses is often gathered through individual interviews, direct observation, or medical records, and is specific for each person included in the study. These approaches are more labor-intensive and expensive, but the pay-off is that they allow researchers to predict individual risk or odds.

Case-control and cohort analyses are observational study designs and are discussed in Chapter 4. Under a case-control study, subjects are selected based on whether they do (cases) or do not (controls) have the disease under investigation. The two groups are then compared based on their respective history of exposures. Case-control analyses tend to be less expensive and less time-consuming than cohort or randomized clinical trials.

Under a cohort study, subjects are selected based on whether or not they were exposed to a suspected risk factor for a disease. At the time exposure status is defined, potential subjects must be free from the disease under investigation. Eligible participants are then followed over a period of time to capture the number of subjects in both groups who develop the disease. Since cohort studies often involve following a large number of subjects for a long time period, they tend to be more time-consuming and expensive than case-control studies.

As discussed in Chapter 5, a randomized clinical trial (RCT) is the most rigorous study design. Patients are assigned to a particular treatment based on chance and are then monitored for a period of time to determine their disease experience.

Finally, we conclude this section with a chapter on meta-analyses (Chapter 6), statistical analyses of a collection of studies. They allow researchers to combine data from a number of previously conducted studies of the same subject to obtain an overall estimate of effect. However, it is important to remember that the quality and usefulness of any meta-analysis is dependent on the quality and comparability of the individual studies included therein.

Reference

1. Hennekens CH, Buring JE. *Epidemiology in Medicine*. Boston: Little, Brown, and Company; 1987.