5.3: Epidemiology Studies

What Are Epidemiology Studies?

Epidemiology studies are conducted using human populations to evaluate whether there is a **correlation or causal relationship** between exposure to a substance and adverse health effects.

These studies differ from clinical investigations in that individuals have already been administered the drug during medical treatment or have been exposed to it in the workplace or environment.

Epidemiological studies measure the risk of illness or death in an exposed population compared to that risk in an identical, unexposed population (for example, a population the same age, sex, race and social status as the exposed population).


Illustration of arbitrary bar charts, a line chart, and a population represented by stick figures

Figure 5.3.1. Epidemiology studies tend to produce graphs and charts for data analysis and presentation
(Image Source: Adapted from iStock Photos, ©)

Types of Studies

There are four primary types of epidemiology studies. They are:

1. **Cohort studies** — A cohort (group) of individuals with exposure to a chemical and a cohort without exposure are followed over time to compare disease occurrence.
2. **Case control studies** — Individuals with a disease (such as cancer) are compared with similar individuals without the disease to determine if there is an association of the disease with prior exposure to an agent.
3. **Cross-sectional studies** — The prevalence of a disease or clinical parameter among one or more exposed groups is studied, such as:
    - The prevalence of respiratory conditions among furniture makers.
4. **Ecological studies** – The incidence of a disease in one geographical area is compared to that of another area, such as:
    - Cancer mortality in areas with hazardous waste sites as compared to similar areas without waste sites.

Cohort Studies

Cohort studies are the most commonly conducted epidemiology studies and they frequently involve occupational exposures. Exposed persons are easy to identify and their exposure levels are usually higher than in the general public. There are two types of cohort studies:

1. **Prospective**, in which cohorts are identified based on current exposures and followed into the future.
2. **Retrospective,** in which cohorts are identified based on past exposure conditions and study "follow-up" proceeds forward in time; data come from past records.

Common Statistical Measures

Standard, quantitative measures are used to determine if epidemiological data are meaningful. The most commonly used measures are:

- **Odds Ratio (O/R)** — The ratio of risk of disease in a case-control study for an exposed group to an unexposed group. An odds ratio equal to 2 (O/R = 2) means that the exposed group has twice the risk as the non-exposed group.
- **Standard Mortality Ratio (SMR)** — The relative risk of death based on a comparison of an exposed group to non-exposed group. A standard mortality ratio equal to 150 (SMR = 150) indicates that there is a 50% greater risk.
- **Relative Risk (RR)** — The ratio expressing the occurrence of disease in an exposed population to that of an unexposed population. A relative risk of 175 (RR = 175) indicates a 75% increase in risk.

Study Design

When designing an epidemiology study, the most critical aspects include:

- An appropriate control group.
- An adequate time span.

- The statistical ability to detect an effect.

More specifically, the control population used as a comparison group must be as similar as possible to that of the test group, for example, same age, sex, race, social status, geographical area, and environmental and lifestyle influences.

Many epidemiology studies evaluate the potential for an agent to cause cancer. Because most cancers require long latency periods, the study must cover that period of time.

The statistical ability to detect an effect is referred to as the **power** of the study. To gain precision, the study and control populations should be as large as possible.

Bias Errors

Epidemiologists attempt to control errors that can occur in the collection of data, which are known as bias errors. The three main types of bias errors are:

1. **Selection bias**, which occurs when the study group is not representative of the population from which it came.
2. **Information bias**, which occurs when study subjects are misclassified as to disease or exposure status. Recall bias occurs when individuals are asked to remember exposures or conditions that existed years before.
3. **Confounding factors**, which occur when the study and control populations differ with respect to factors which might influence the occurrence of the disease. For example, smoking might be a confounding factor and should be considered when designing studies.

Postmarketing Studies

Finally, for consumer products, **postmarketing** epidemiological studies can be performed. Examples include studies developed by Poison Control Centers, companies, academia, and other sources to look at the "real world" health reports of effects associated with consumer use of a product or article under reasonably foreseeable conditions.

Knowledge Check

While animal testing was historically the primary method used in testing for toxicity, modern testing methods prefer:

- ◯ *In silico*
- ◯ *In vitro*
- ◯ Refined animal testing
- ◯ All of the above

Answer

All of the above - **This is the correct answer.**
Modern approaches to testing for toxicity include *in silico*, *in vitro*, and improved (refined) animal testing.

In testing a pharmaceutical to comply with FDA requirements, the initial testing consists of:

- ◯ Clinical investigations
- ◯ Non-clinical laboratory studies
- ◯ Epidemiology studies
- ◯ All of the above

Answer

Non-clinical laboratory studies - **This is the correct answer.**
Investigational New Drug Applications (IND) require clinical investigations. Before clinical investigations begin, a minimal battery of non-clinical laboratory studies must be completed.

The primary goal of a Phase 1 clinical investigation is to:

- ◯ Obtain information to design a more definitive Phase 2 clinical investigation

- ◯ Evaluate the effectiveness of a drug for treating disease
- ◯ Provide the basis for pharmaceutical labeling

Answer

Obtain information to design a more definitive Phase 2 clinical investigation - **This is the correct answer.**
The primary goal of a Phase 1 clinical investigation is to obtain information that is used to design more extensive, Phase 2 studies.

Determining the overall risk versus the benefit of a new pharmaceutical is part of:

- ◯ Phase 2 clinical investigation
- ◯ Phase 3 clinical investigation
- ◯ Epidemiology study

Answer

Phase 3 clinical investigation - **This is the correct answer.**
Determining the overall risk versus the benefit of a new pharmaceutical is part of Phase 3 clinical study. The risk versus benefit is one of the last steps in the drug evaluation process.

The type of epidemiology study in which individuals are identified according to exposure and followed to determine subsequent disease risk is known as:

- ◯ Cohort study
- ◯ Case control study
- ◯ Cross-sectional study
- ◯ Ecological study

Answer

Cohort study - **This is the correct answer.**
The type of epidemiology study in which individuals are identified according to exposure and followed to determine subsequent disease risk is known as a cohort study. In a cohort study individuals are selected to be part of the group based on their exposure to a particular substance.

An epidemiological study in which the individuals that make up the test cohort are identified according to past exposures is known as:

- ◯ Case control study
- ◯ Prospective cohort study
- ◯ Retrospective cohort study

Answer

Retrospective cohort study - **This is the correct answer.**
This is known as a retrospective cohort study. As the name implies, retrospective cohorts are identified according to past exposure conditions and the follow-up study proceeds forward in time.