Philosophy of science and introduction to epidemiology

Introduction and learning objectives

In this chapter, we will begin by looking at different approaches to *scientific research*, how these have arisen, and the importance of recognising that there is no single, 'right way' to carry out investigations in the health field. We will then go on to explore the *research task*, discuss what is meant by *epidemiology* and *statistics*, and look at how these two disciplines are introduced and developed in the book. The next section introduces the concept of *rates* for measuring the frequency of disease or characteristics we are interested in, and in particular the terms *incidence* and *prevalence*. These definitions and uses of rates are fundamental ideas with which you should be familiar before we look in more detail at research methods and study design. In the final section, we will look at key concepts in disease prevention, including the commonly used terms *primary, secondary* and *tertiary* prevention. **and learning objectives**
 c will begin by looking at different approaches to *scientific* re

the importance of recognising that there is no single, 'right

the health field. We will then go on to explore the *research*

The reason for starting with a brief exploration of the nature of scientific methods is to see how historical and social factors have influenced the biomedical and social research traditions that we take for granted today. This will help you understand your own perceptions of, and assumptions about, health research, based on the knowledge and experience you have gained to date. It will also help you understand the scientific approach being taken in this book, and how this both complements, and differs from, that developed in books and courses on qualitative research methods – as and when you may choose to study these. Being able to draw on a range of research traditions and their associated methods is especially important for the discipline of public health, but also for many other aspects of health and health care.

Learning objectives

By the end of Chapter 1, you should be able to do the following:

Quantitative Methods for Health Research Nigel Bruce, Daniel Pope and Debbi Stanistreet © 2008 John Wiley & Sons, Ltd

- Briefly describe the philosophical differences between the main approaches to research that are used in the health field.
- Describe what is meant by epidemiology, and list the main uses to which epidemiological methods and thought can be put.
- Describe what is meant by statistics, and list the main uses to which statistical methods and thought can be put.
- Define and calculate rates, prevalence and incidence, and give examples of their use.
- Define primary, secondary and tertiary prevention and give examples of each.

1.1 Approaches to scientific research

1.1.1 History and nature of scientific research

Scientific research in health has a long history going back to the classical period. There are threads of continuity, as well as new developments in thinking and techniques, which can be traced from the ancient Greeks, through the fall of the Roman Empire, the Dark Ages and the Renaissance, to the present time. At each stage, science has influenced, and been influenced by, the culture and philosophy of the time. Modern scientific methods reflect these varied historical and social influences. So it is useful to begin this brief exploration of scientific health research by reflecting on our own perceptions of science, and how our own views of the world fit with the various ways in which research can be approached. As you read this chapter you might like to think about the following questions:

- What do you understand by the terms *science*, and *scientific research*, especially in relation to health?
- How has your understanding of research developed?
- What type of research philosophy best fits your view of the world, and the problems you are most interested in?

Thinking about the answers to these questions will help you understand what we are trying to achieve in this section, and how this can best support the research interests that you have and are likely to develop in the years to come. The history and philosophy of science is of course a whole subject in its own right, and this is of necessity a very brief introduction.

Scientific reasoning and epidemiology

Health research involves many different scientific disciplines, many of which you will be familiar with from previous training and experience. Here we are focusing principally on epidemiology, which is concerned with the study of the distribution and determinants of disease within and between populations. In epidemiology, as we shall see subsequently, there is an emphasis on *empiricism*, that is, the study of observable phenomena by scientific methods, detailed observation and accurate measurement. The scientific approach to epidemiological investigation has been described as:

- **Systematic** there is an agreed system for performing observations and measurement.
- **Rigorous** the agreed system is followed exactly as prescribed.
- **Reproducible** all the techniques, apparatus and materials used in making the observations and measurements are written down in enough detail to allow another scientist to reproduce the same process.
- **Repeatable** scientists often repeat their own observations and measurements several times in order to increase the reliability of the data. If similar results are obtained each time, the researcher can be confident the phenomena have been accurately recorded.

These are characteristics of most epidemiological study designs and will be an important part of the planning and implementation of the research. However, this approach is often taken for granted by many investigators in the health field (including epidemiologists) as the only way to conduct research. Later we will look at some of the criticisms of this approach to scientific research but first we need to look in more detail at the reasoning behind this perspective.

Positivism

The assumptions of contemporary epidemiological investigations are associated with a view of science and knowledge known as *positivism*. Positivism is a philosophy that developed in the eighteenth century in a period known as the Enlightenment, a time when scientists stopped relying on religion, conjecture and faith to explain phenomena, and instead began to use reason and rational thought. This period saw the emergence of the view that it is only by using scientific thinking and practices that we can reveal the truth about the world (Bilton *et al*., 2002).

Positivism assumes a stable observable reality that can be measured and observed. So, for positivists, scientific knowledge is proven knowledge, and theories are therefore derived in a systematic, rigorous way from observation and experiment. This approach to studying human life is the same approach that scientists take to study the natural world. Human beings are believed by positivists to exist in causal relationships that can be empirically observed, tested and measured (Bilton *et al*., 2002), and to behave in accordance with various laws. As this reality exists whether we look for it or not, it is the role of scientists to reveal its existence, but not to attempt to understand the inner meanings of these laws or express personal opinions about these laws. One of the primary characteristics of a positivist approach is that the researcher takes an objective distance from the phenomena so that the description of the investigation can be detached and undistorted by emotion or personal bias (Davey, 1994). This means that within epidemiology, various study designs and techniques have been developed to increase objectivity, and you will learn more about these in later chapters.

Induction and deduction

There are two main forms of scientific reasoning – *induction* and *deduction*. Both have been important in the development of scientific knowledge, and it is useful to appreciate the difference between the two in order to understand the approach taken in epidemiology.

Induction

With inductive reasoning, researchers make repeated observations and use this evidence to generate theories to explain what they have observed. For example, if a researcher made a number of observations in different settings of women cooking dinner for their husbands, they might then inductively derive a general theory:

All women cook dinner for their husbands.

Deduction

Deduction works in the opposite way to induction, starting with a theory (known as an *hypothesis*) and then testing it by observation. Thus, a very important part of deductive reasoning is the formulation of the hypothesis – that is, the provisional assumption researchers make about the population or phenomena they wish to study before starting with observations. A good hypothesis must enable the researcher to test it through a series of *empirical observations*. So, in deductive reasoning, the hypothesis would be:

All women will cook dinner for their husbands.

Observations would then be made in order to test the validity of this statement. This would allow researchers to check the consistency of the hypothesis against their observations, and if necessary the hypothesis can be discarded or refined to accommodate the observed data. So, if they found even one woman not cooking for her husband, the hypothesis would have to be reexamined and modified. This characterises the approach taken in epidemiology and by positivists generally.

One of the most influential science philosophers of recent times was Karl Popper (1902–1994), who argued that hypotheses can never be proved true for all time and scientists should aim to refute their own hypothesis even if this goes against what they believe (Popper, 1959). He called this the *hypothetico-deductive method*, and in practice this means that an hypothesis should be capable of being falsified and then modified. Thus, to be able to claim the hypothesis is true would mean that all routes of investigation had been carried out. In practice, this is impossible, so research following this method does not set out with the intention of proving that an hypothesis is true. In due course we will see how important this approach is for epidemiology and in the statistical methods used for testing hypotheses.

Alternative approaches to research

It is important to be aware that positivism is only one approach to scientific research. Positivism has been criticised by some researchers, in particular social scientists, who think it is an inappropriate approach to studies of human behaviour. From this perspective, they believe that human beings can behave irrationally and do not always act in accordance with any observable rules or laws. This makes them different from phenomena in the natural world, and so they need to be studied in a different way. Positivism has also been criticised because it does not allow for the view that human beings act in response to others around them; that is, that they interpret their own behaviour in response to others. As Green and Thorogood (2004, p. 12) argue:

Unlike atoms (or plants or planets), human beings make sense of their place in the world, have views on researchers who are studying them, and behave in ways that are not determined in law-like ways. They are complex, unpredictable, and reflect on their behaviour. Therefore, the methods and aims of the natural sciences are unlikely to be useful for studying people and social behaviour: instead of explaining people and society, research should aim to understand human behaviour.

Social scientists therefore tend to have a different belief about how we should research human beings. Consequently, they are more likely to take an *inductive* approach to research because they would argue that they do not want to make assumptions about the social world until they have observed it in and for itself. They, therefore, do not want to formulate hypotheses because they believe these are inappropriate for making sense of human action. Rather, they believe that human action cannot be explained but must be understood.

While positivists are concerned mainly with observing patterns of behaviour, other researchers principally wish to understand human behaviour. This latter group requires a different starting point that will encompass their view of the world, or different *theoretical positions* to make sense of the world. It turns out that there are many different positions that can be adopted, and while we cannot go into them all here, we will use one example to illustrate this perspective.

Interpretative approaches

An interpretative approach assumes an interest in the meanings underpinning human action, and the role of the researcher is therefore to unearth that meaning. The researcher would not look to measure the reality of the world but would seek to understand how people interpret the world around them (Green and Thorogood, 2004).

Let's look at an example of this in respect of asthma. A *positivist* approach to researching this condition may be to obtain a series of objective measurements of symptoms and lung function by a standard procedure on a particular sample of people over a specified period of time. An *interpretative* approach might involve talking in-depth to fewer participants to try to understand how their symptoms affect their lives. Obviously, in order to do this, these two 'types' of researchers would need to use very different approaches. Those planning the interpretative research would be more likely to use *qualitative methods* (interviews, focus groups, participatory methods, etc.), while positivists (for example, epidemiologists) would choose *quantitative methods* (surveys, cohort studies, etc., involving lung-function measurements and highly structured questionnaires). These two different approaches are called *research paradigms* and would therefore produce different types of information.

Interpretative researchers would also criticise positivists for their belief that researchers can have an objective, unimpaired and unprejudiced stance in the research that allows them to make value-free statements. Interpretative researchers accept that researchers are human beings and therefore cannot stand objectively apart from the research. In a sense they are part of the research, as their presence can influence the nature and outcome of the research. Whether or not you agree with the criticism of positivism, you need to be aware that there are alternative approaches to conducting research that neither prioritise objectivity nor set out to measure 'reality'.

One of the most influential writers on the scientific method was Thomas Kuhn (1922–1996) (Davey, 1994). He argued that one scientific paradigm – one 'conceptual worldview' – may be the dominant one at a particular period in history. Over time, this is challenged, and eventually replaced by another view (paradigm), which then becomes accepted as the most important and influential. These revolutions in science were termed 'paradigm shifts'. Although challenged by other writers, this perspective suggests that scientific methods we may take for granted as being the only or best way to investigate health and disease, are to an extent the product of historical and social factors, and can be expected to evolve – and maybe change substantively – over time.

Exercise for reflection

- 1. Make brief notes on the type of scientific knowledge and research with which you are most familiar.
- 2. Is this predominantly positivistic (hypothetico-deductive) or interpretative in nature, or is it more of a mixture?

There are no 'answers' provided for this exercise, as it is intended for personal reflection.

1.1.2 What is epidemiology?

The term *epidemiology* is derived from the following three Greek words:

Epi **– among** *Demos* **– the people** *Logos* **– discourse**

We can translate this in more modern terms into *'The study of the distribution and determinants of disease frequency in human populations'*. The following exercise will help you to think about the uses to which the discipline of epidemiology is put.

INK Self-Assessment Exercise 1.1.1

Make a list of some of the *applications* of epidemiological methods and thought that you can think of. In answering this, avoid listing types of epidemiological study that you may already know of. Try instead to think in general terms about the practical outcomes and applications of these methods.

Answers in Section 1.5

This exercise shows the very wide application of epidemiological methods and thought. It is useful to distinguish between two broad functions of epidemiology, one very practical, the other more philosophical:

- The range of epidemiological research methods provides a toolbox for obtaining the best scientific information in a given situation (assuming, that is, you have established that a positivist approach is most appropriate for the topic under study!).
- Epidemiology helps us use knowledge about the population determinants of health and disease to inform the full range of investigative work, from the choice of research methods, through analysis and interpretation, to the application of findings in policy. With experience, this becomes a way of thinking about health issues, over and above the mere application of good methodology.

You will find that your understanding of this second point grows as you learn about epidemiological methods and their application. This is so because epidemiology provides the means of describing the characteristics of populations, comparing them, and analysing and interpreting the differences, as well as the many social, economic, environmental, behavioural, ecological and genetic factors that determine those differences.

1.1.3 What are statistics?

A statistic is a numerical fact. Your height and weight and the average daily rainfall in Liverpool are examples of statistics. The academic discipline of statistics is concerned with the collection, presentation, analysis and interpretation of numerical information (also called *quantitative* information).

Statistics are everywhere!

We are surrounded by, and constantly bombarded with, information from many sources - from the cereal box, to unemployment figures, the football results and opinion polls, to articles in scientific journals. The science of statistics allows us to make sense of this information and is thus a fundamental tool for investigation in many disciplines, including health, education, economics, agriculture and politics, to name but a few. The next exercise encourages you to explore how statistics are used in everyday life.

INK Self-Assessment Exercise 1.1.2

Look at a recent newspaper (hard copy or website for a newspaper, or, for example, the BBC news website (http://news.bbc.co.uk)) and find up to five items in which statistics are used. List the ways in which numerical information is presented.

Examples in Section 1.5

The scientific term for pieces of information is *data*. The singular is *datum*, meaning a single piece of information, such as, for example, one person's weight. A set of data may consist of many items, such as the heights, weights, blood pressures, smoking habits and exercise level of several hundred people. In its raw state, this mass of figures tells us little. There are two ways in which we use statistics to help us interpret data:

- To *describe* the group about which the data have been collected. This may be a group of people, or a group of hospitals, or a group of laboratory cultures. We describe the group by summarising the information into a few meaningful numbers and pictures.
- To *infer* something about the population of which the group studied is a part. We often want to know something about a population, such as everyone aged over 65 in Liverpool, but, practically, can collect information about only a subset of that population. This 'subset' is called a sample, and is explored in Chapter 3 on surveys. With inference, we want to know what generalisations to the population can be made from the sample, and with what degree of certainty.

INK Self-Assessment Exercise 1.1.3

Can you find one example of *description*, and one example of *inference* in your newspaper or Web search? If you have found an example of making an inference, to which population does it apply?

Examples in Section 1.5

1.1.4 Approach to learning

We will explore the use and interpretation of statistical techniques through a number of published studies. Whether or not you go on to carry out research and use statistical methods yourself, you are certain to be a consumer of statistics through published research. We shall emphasise both the use of appropriate techniques and the critical interpretation of published results. You will also be learning about epidemiology and statistics in an integrated way. This approach recognises that the two disciplines embody many closely related concepts and techniques. There are also certain very distinct qualities, which you will find are emphasised through the more theoretical discussion relating to one or other discipline. Your learning of these research methods is based primarily on practical examples of data and published studies in order to help you to see how epidemiology and statistics are used in practice, and not just in theoretical or ideal circumstances.

Summary

- There is no single, 'right' philosophy of research. The approach taken is determined by many factors, including historical and social influences, and the nature of the problem being investigated.
- As an individual, your education, training and experience will strongly influence the scientific 'paradigm' that you are familiar, and comfortable with.
- A variety of approaches to, and methods for, research is both appropriate and necessary in the health field.
- Epidemiology provides us with a range of research tools, which can be used to obtain the information required for prevention, service provision, and the evaluation of health care. One of the most important contributions of epidemiology is the insight gained about the factors which determine the health of populations.
- Statistics is concerned with the collection, presentation, analysis and interpretation of numerical information. We may use statistical techniques to describe a group (of people, hospitals, etc.) and to make inferences about the population to which the group belongs.

1.2 Formulating a research question

1.2.1 Importance of a well-defined research question

This is arguably the most important section of the whole book. The reason for our saying this is that the methods we use, and ultimately the results that we obtain, must be determined by the question we are seeking to answer.

So, how do we go about formulating that question? This does not (usually) happen instantly, and there is a good deal of debate about how the question ought to be formulated, and how it is formulated in practice. Karl Popper argued that research ideas can come from all kinds of sources. But the idea is not enough on its own, and will usually need working on before it is a clearly formulated *research question*. Here are some of the factors we will have to take into account in fashioning a clear research question:

- What does all the other work on the topic tell us about the state of knowledge, and what aspects need to be addressed next? Our idea might actually arise from a review such as this, and therefore be already fairly well defined, but more often than not, the idea or need arises before we have had a chance to fully evaluate the existing body of knowledge. This will also depend on how far we are into researching a particular subject.
- Different types of problem and topic areas demand, and/or have been traditionally associated with, different research traditions. Does our idea require a positivist approach, with an hypothesis that can be falsified? If so, the research question will need to be phrased in a way that allows this. Alternatively, we might be trying to understand how a certain group of people view a disease and the services provided for them. This question must also be precisely defined, but not in the same way: there is nothing here to be falsified; rather, we wish to gain as full an understanding as possible of people's experience and opinions to guide the development of services.

• If our idea is overambitious, the necessary research may be too demanding, expensive or complex to answer the question(s) in one go. Perhaps it needs to be done in separate studies, or in stages.

In practice, defining the research question does not usually happen cleanly and quickly, but is a process that gradually results in a more and more sharply defined question as the existing knowledge, research options, and other practical considerations are explored and debated. There may appear to be exceptions to this – for instance, a trial of a new drug. On the face of it, the question seems simple enough: the drug is now available, so is it better than existing alternatives or not? However, as we will discover later, the context in which the drug would be used can raise a lot of issues that will play a part in defining the research question.

Although knowledge of appropriate research methods is important in helping you to formulate a clear research question, it is nevertheless useful to start the *process* of developing your awareness of, and skills in, this all-important aspect of research. The following exercise provides an opportunity for you to try this.

A research idea *---*

Your work in an urban area involves aspects of the care and management of people with asthma. You are well aware of the contemporary concern about the effect of air pollution on asthma, and the view that while pollution (e.g. ozone, nitrogen oxides) almost certainly exacerbates asthma, this may not be the cause of the underlying asthmatic tendency.

In recent years, you have noticed that asthmatics (especially children) living in the poorest parts of the city seem to suffer more severe and frequent asthma episodes than those living in better-off parts.

Although you recognise that pollution from traffic and industry might be worse in the poorer areas, you have been wondering whether other factors, such as diet (e.g. highly processed foods) or housing conditions such as dampness and associated moulds might be the real cause of the difference.

You have reviewed the literature on this topic and found a few studies which are somewhat conflicting, and do not seem to have distinguished very well between the pollution, diet and housing factors you are interested in.

Have a go at converting the idea described above into a well-formulated research question appropriate for epidemiological enquiry. Note that there is no single, right, research question here. You do not need to describe the study methods you might go on to use.

Specimen answer in Section 1.5

1.2.2 Development of research ideas

We have seen that research is a process which evolves over a period of time. It is influenced by many factors, including other work in the field, the prevalent scientific view, political factors, finance, etc. Research is not a socially isolated activity with a discrete (inspirational) beginning, (perfect) middle and (always happy) ending, Figure 1.2.1.

Figure 1.2.1 Research fantasy time

A more realistic way to describe the process of research development is cyclical, as illustrated in Figure 1.2.2. A well-defined and realistic question, which is (as we have seen) influenced by many factors, leads to a study which, it is hoped, provides much of the information required.

Figure 1.2.2 Research is a process that can usefully be thought of as being cyclical in nature, and subject to many influences from both inside and outside the scientific community

These findings, together with developments in the scientific field as well as social, political or other significant influences, will lead to further development of the research question.

Summary

- Defining a clear research question is a fundamental step in research.
- Well-defined research questions do not (usually) appear instantly!
- Research is a process, which can usefully be thought of as cyclical, albeit subject to many external influences along the way.

1.3 Rates: incidence and prevalence

1.3.1 Why do we need rates?

A useful way to approach this question is by considering the problem that arises when we try to interpret a change in the number of events (which could be deaths, hospital admissions, etc.) occurring during, say, a period of one year in a given setting. Exercise 1.3.1 is an example of this type of problem, and is concerned with an increase in numbers of hospital admissions.

INK Self-Assessment Exercise 1.3.1

Over a period of 12 months, the accident and emergency department of a city hospital noted the number of acute medical admissions for people over 65 had increased by 30 per cent. In the previous 5 years, there had been a steady increase of only about 5 per cent per year.

- 1. List the possible reasons for the 30 per cent increase in hospital accident and emergency admissions.
- 2. What other information could help us interpret the reasons for this sudden increase in admissions?

Answers in Section 1.5

In this exercise we have seen the importance of interpreting changes in numbers of events in the light of knowledge about the *population* from which those events arose. This is why we need *rates*. A rate has a *numerator* and a *denominator*, and must be determined over a specified *period of time*. It can be defined as follows:

1.3.2 Measures of disease frequency

We will encounter rates on many occasions, as these are a fundamental means of expressing information gathered and analysed in epidemiological research. We can view these as measures of the *frequency* of disease, or of characteristics in the population. Two important general ways of viewing disease frequency are provided by *prevalence* and *incidence*.

1.3.3 Prevalence

The prevalence tells us how many cases of a disease (or people with a characteristic, such as smoking) there are in a given population, at a specified time. The *numerator* is the number of cases, and the *denominator* the population we are interested in.

Prevalence = $\frac{\text{Number of cases at a given time}}{\text{Number in population at that time}}$

This can be expressed as a percentage, or per 1000 population, or per 10 000, etc., as convenient. The following are therefore examples of prevalence:

- In a primary care trust (PCT), with a population of 400 000, there are 100 000 smokers. The prevalence is therefore 25 per cent, or 250 per 1000.
- In the same PCT, there are known to be 5000 people diagnosed with schizophrenia. The prevalence is therefore 1.25 per cent, or 12.5 per 1000.

Note that these two examples represent 'snapshots' of the situation at a given time. We do not have to ask about people starting or giving up smoking, nor people becoming ill with (or recovering from) schizophrenia. It is a matter of asking, 'in this population, how many are there now?' This snapshot approach to measuring prevalence is known as *point prevalence*, since it refers to one point in time, and is the usual way in which the term prevalence is used. If, on the other hand, we assess prevalence over a period of time, it is necessary to think about cases that exist at the start of the period, and new cases which develop during the period. This measure is known as *period prevalence*, and this, together with point prevalence, is illustrated in Figure 1.2.3 and Exercise 1.3.2.

Figure 1.2.3 Period and point prevalence

Point prevalence (Figure 1.2.3a) is assessed at one point in time (time A), whereas period prevalence (Figure 1.2.3b) is assessed over a period (period B). The horizontal bars represent patients becoming ill and recovering after varying periods of time – the start and end of one episode is marked in Figure 1.2.3a. Period prevalence includes everyone who has experienced the disease at some time during this period.

Self-Assessment Exercise 1.3.2

- 1. In the above examples (Figure 1.2.3a and b), calculate the point prevalence and period prevalence.
- 2. Why do the point prevalence and period prevalence differ?

Answers in Section 1.5

1.3.4 Incidence

Whereas *prevalence* gives us a measure of how many cases there are in the population at a given time, *incidence* tells us the rate at which *new* cases are appearing in the population. This is a vital distinction. In order to determine the incidence, we need to know the number of new cases appearing over a specified period of time, and the number of people in the population who could become cases over that same period of time (the 'at-risk' population). This is called *cumulative incidence* (or *incidence rate*) and is calculated as follows:

Incidence rate (also termed 'cumulative incidence')\n
\n**Incidence** =
$$
\frac{\text{Number of new cases arising from defined population in specified time period}}{\text{Number in defined at risk population over same period of time}}
$$

The incidence can be expressed per 1000 per year (or other convenient time period), or per 10 000 per year, etc., as appropriate. Note that incidence is expressed over a specified time period, often taken as one year. Thus, if there were 20 new cases of a disease in a population of 2500 over a period of one year, the incidence, expressed per 1000 per year is as follows:

$$
Incidence = \frac{20}{2,500} \times 1,000
$$

This gives a rate of 8 cases per 1000 per year. The denominator, the 'defined population', must be exclusively that population which could become cases (termed *at risk*). For example, if we are considering the rate of hysterectomy (removal of the uterus) among UK women aged 50 years and above, the denominator population must be women over 50, excluding those who have previously had a hysterectomy.

Quite often, however, when a large group of the population is being studied, for example, all men in a city such as Liverpool, the number at risk in this defined population will not be readily available, and an estimate has to be made. In this case the corresponding midyear population estimate is often used as the denominator population, since it is usually available from published official statistics. This means that existing cases who are not 'at risk' of becoming new cases of the disease (because they are already affected) will be included in the denominator of the equation. In this case, the resulting incidence measure will not be much affected so long as the number of existing cases is relatively small in comparison to the total population, but you should be aware that the true incidence of the disease will be slightly underestimated. The following exercise will help you consolidate your understanding of *cumulative incidence*.

INK Self-Assessment Exercise 1.3.3

- 1. In the period prevalence diagram in Figure 1.2.3b, how many new cases arose during period B?
- 2. Surveillance data for episodes of food poisoning in a given population showed that among children aged 0–14 years there had been 78 new cases among boys, and 76 new cases among girls, over a period of 1 year. The population breakdown for the district is as shown in the following table.

Calculate the annual incidence rates per 10 000 for boys aged 0–14 years, and for girls aged 0–14 years. Comment on what you find.

Answers in Section 1.5

1.3.4.1 Person-time

Cumulative incidence assumes that the entire population at risk at the beginning of the study has been followed up for the same amount of time. It therefore measures the proportion of unaffected individuals who, on average, will contract the disease over the specified time period. However, in some study designs, such as cohort studies (described in Chapter 5), people may be entered into the study at different times and then be followed up to a specific end of study date. In addition, some may withdraw from the study, or die, before the end of the study. Study participants will therefore have differing lengths of follow-up.

To account for these varying times of follow-up, a denominator measure known as *person-time* is used. This is defined as the sum of each individual's time at risk while remaining free of disease. When person-time is used, incidence is calculated slightly differently, and is known as the *incidence density*. This is calculated as follows:

Since person-time can be counted in various units such as days, months or years, it is important to specify the time units used. For example, if 6 new cases of a disease are observed over a period of 30 person-years, then the incidence would be $6/30 = 0.2$ per person-year or equivalently, 20 per 100 person-years, or 200 per 1000 person-years. If people are lost to followup or withdraw from the study prematurely, their time at risk is taken to be the time they were under observation in the study. This next exercise will help with understanding incidence density.

INK Self-Assessment Exercise 1.3.4

Subject	Years	Disease	Subject	Years	Disease
1	19.6	N	16	0.6	Y
$\overline{2}$	10.8	Y	17	2.1	Y
3	14.1	Y	18	0.8	Y
$\overline{4}$	3.5	Y	19	8.9	N
5	4.8	N	20	11.6	Y
6	4.6	Y	21	1.3	Y
7	12.2	N	22	3.4	N
8	14.0	Y	23	15.3	N
9	3.8	Y	24	8.5	Y
10	12.6	N	25	21.5	Y
11	12.8	Y	26	8.3	N
12	12.1	Y	27	0.4	Y
13	4.7	Y	28	36.5	N
14	3.2	N	29	1.1	Y
15	7.3	Y	30	1.5	Y

Time of follow-up in study, or until disease develops, for 30 subjects

- 1. Assuming that all subjects in the table enter the study at the same time and are followed up until they leave the study (end of follow-up) or develop the disease, find the total observation time for the 30 subjects and estimate the incidence density. Give your answer per $1000 (10³)$ person-years.
- 2. It is more usual for follow-up studies to be of limited duration where not all the subjects will develop the disease during the study period. Calculate the incidence density for the same 30 subjects if they were observed for only the first 5 years, and compare this with the rate obtained in question 1.

Answers in Section 1.5

1.3.5 Relationship between incidence, duration and prevalence

There is an important relationship between incidence, illness duration, and prevalence. Consider the following two examples:

• Urinary tract infections among women are seen very commonly in general practice, reflecting the fact that the incidence rate is high. The duration of these infections (with treatment) is usually quite short (a few days), so at any one time there are not as many women with an infection as one might imagine given the high incidence. Thus, the (point) prevalence is not particularly high.

• Schizophrenia is a chronic psychiatric illness from which the majority of sufferers do not recover. Although the incidence is quite low, it is such a long-lasting condition that at any one time the prevalence is relatively high, between 0.5 per cent and 1 per cent, or 5–10 per 1000 in the UK.

Thus, for any given incidence, a condition with a longer duration will have a higher prevalence. Mathematically, we can say that the prevalence is proportional to the product of incidence and average duration. In particular, when the prevalence is low (less than about 10 per cent), the relationship between prevalence, incidence and duration can be expressed as follows:

Prevalence $=$ incidence \times duration

This formula holds so long as the units concerned are consistent, the prevalence is low, and the duration is constant (or an average can be taken). Thus, if the incidence is 15 per 1000 per year, and the duration is, on average, 26 weeks (0.5 years), then the prevalence will be (15×0.5) per $1000 = 7.5$ per 1000.

Summary

- Rates are a vitally important concept in epidemiology, and allow comparison of information on health and disease from different populations.
- A rate requires a numerator (cases) and a denominator (population), each relating to the same specified time period.
- The prevalence rate is the number of cases in a defined population at a particular point in time (point prevalence), or during a specified period (period prevalence).
- The incidence rate is the number of new cases which occur during a specified time period in a defined at risk population (cumulative incidence).
- Incidence density is a more precise measure of incidence and uses person-time of observation as the denominator.
- Without using rates, comparison of numbers of cases in different populations may be very misleading.
- The relationship between incidence and prevalence is determined by the duration of the condition under consideration.

1.4 Concepts of prevention

1.4.1 Introduction

In this section, we will look at the ways in which we can conceptualise *approaches to prevention*. This is a well-established framework that provides important background to many of the studies we will examine in learning about research methods, as well as for services such as screening. The following examples illustrate three different approaches to prevention. Please read through these, and complete exercise 1.4.1. We will then look at the formal definition of these approaches.

Example 1: Road accidents among children

Accidents are the most common cause of death among children in the UK, and the majority of these accidents occur on the roads. Traffic calming offers one way of reducing the number of childhood deaths arising from road accidents.

Example 2: Breast cancer

Breast cancer is one of the most common cancers among women. Despite this, we know little for certain about the causes beyond genetic, hormonal and possibly some dietary factors. In recent years, mammography, a radiographic examination of the breast, has been routinely offered to women 50–64 years of age. Abnormalities suggestive of cancer are investigated by biopsy (removal of a small piece of tissue for microscopic examination), and if the biopsy is positive, treatment for cancer is carried out.

Example 3: Diabetes and the prevention of foot problems

Diabetes, a disorder of glucose (blood sugar) metabolism, is generally a progressive condition. The actual underlying problem does not usually get better, and control of blood glucose has to be achieved through attention to diet, and usually also with medication, which may be in tablet form or as injected insulin. Associated with this disordered glucose metabolism are a range of chronic degenerative problems, including atherosclerosis (which leads to heart attacks, and poor blood supply to the lower legs and feet), loss of sensation in the feet due to nerve damage, and eye problems. Many of these degenerative processes can be slowed down, and associated problems prevented, by careful management of the diabetes. One important example is care of the foot when blood supply and nerves are affected. This involves education of the diabetic about the problem, and how to care for the foot, and provision of the necessary treatment and support.

INK Self-Assessment Exercise 1.4.1

For each of the above examples, describe in everyday language (that is, avoiding technical terms and jargon) how prevention is being achieved. In answering this question, think about the way in

which the prevention measure acts on the development and progression of the disease or health problem concerned.

Answers in Section 1.5

1.4.2 Primary, secondary and tertiary prevention

The three examples of prevention that we have just discussed are (respectively) illustrations of *primary, secondary* and *tertiary* prevention. These terms can be defined as follows:

For each of the following activities, state whether this is primary, secondary or tertiary prevention, giving brief reasons for your answer:

- 1. Measles immunisation.
- 2. Five-yearly smear tests for cervical cancer.
- 3. A well-managed programme of terminal care for a patient with cancer.
- 4. Use of bed nets impregnated with insecticide in malaria-endemic areas.
- 5. Smoking-cessation programme in middle-aged men recovering from a heart attack.

Answers in Section 1.5

1.5 Answers to self-assessment exercises

Section 1.1

Exercise 1.1.1

The uses of epidemiological methods and thought: this list is not necessarily exhaustive, but covers the most important applications:

- By studying populations rather than those already in the health-care system, one can gain a more *representative and complete picture* of the distribution of disease, and maybe identify the factors which determine who does, and does not, take up health care.
- Describing the frequency of a disease, health problem or risk factor; who is affected, where and when. This may be used for *planning*, as in epidemic control, service provision, etc.
- Understanding the *natural history* of health problems; that is, what happens if there is no treatment or other intervention.
- Understanding the *causes* of disease, thus laying the basis for prevention.
- Determining the *effectiveness* of health interventions, whether drugs, surgical operations, or health promotion.
- Through an understanding of the determinants of the health of populations, epidemiology contributes to the development of *prevention policy*.

Thus, while basic research may add to our biologic understanding of why an exposure causes or prevents disease, only epidemiology allows the quantification of the magnitude of the exposure – disease relationship in humans and offers the possibility of altering the risk through intervention. Indeed, epidemiologic research has often provided information that has formed the basis for public health decisions long before the basic mechanism of a particular disease was understood.

(Hennekens and Buring, 1987, p. 13)

Study of groups that are particularly healthy or vulnerable is often the beginning of the search for causes and so of prevention.

(Morris, 1957, p. 263)

Exercise 1.1.2

These are just a few examples of the use of statistics found in newspapers over two days in August 2005. Note the different ways that the information is presented.

Thatcher still holds iron grip on Downing Street

This panel shows results of a poll about the success of UK Prime Ministers. On the first line of the article, results are reported as a *proportion* (four out of five), while in the tables *percentages* are used.

This panel shows a *pie chart* (bottom left) and a *line graph* (bottom right). Data in the pie chart are presented as *percentages*.

The *table* in the upper section shows total **counts** (turnover, profit) and *averages* of earnings and dividends per share.

This panel shows quite a range of statistical information, presented as *line graphs* over time (by year).

Included are *average* petrol prices per litre, and the *percentage change* over 12 months in three indices of inflation (which are themselves calculated from the average prices of a range of commodities).

Exercise 1.1.3

Here is one example of *description* of groups (world drinkers), and one example of *inference* (German election poll).

Example 1

This is a *bar chart* presenting average alcohol consumption per adult, calculated from national data (on consumption and adult population). As these are data for the whole country, this represents *description* of groups (countries), not *inference*.

World Drinkers

Example 2

This panel shows results from German opinion polls. The results are in the form of *percentages*, displayed in *line graphs* to show the trends over time. As this is a poll, it is an example of a *sample* being used for *inference* about the opinions of those eligible to vote in the whole German *population*.

You will recognise that the example of the 'most successful prime minister' in Exercise 1.1.2 was also an example of *inference*. These statistics were obtained from a poll of 216 'opinion formers', so the intention is *inference* from this *sample* about the wider *population* of opinion formers.

German election poll

Section 1.2

Exercise 1.2.1

This is a complex problem, so don't worry if you have found it challenging. There are many different research questions that could arise from this, depending on the type of research your experience and interests relate to. For example, at one end of the spectrum, a laboratory-based scientist may wish to carry out some physiologically based animal experiments involving chemicals from traffic pollution, food additives and fungal spores (from damp housing). Any kind of laboratory experiment with humans is out of the question, so we will concentrate on epidemiological investigation. The following two research questions represent different levels of investigation. The first aims to start the research process off by describing the situation (from which associations between asthma and other factors can be investigated), while the second takes a more analytical approach. Which one is appropriate would depend on exactly what has already been studied, resources available, etc. You will note that both define the population as children aged 5–15 years, in order to help focus the study.

Research question 1

How are asthma, levels of air pollution, damp housing, consumption of (specified) processed foods, and socio-economic circumstances distributed among children aged 5–15 living in (named city)?

Research question 2

Among children aged 5–15 living in (named city), are the presence and/or frequency of asthma associated with (specified) processed foods or damp housing, after taking account of levels of air pollution?

Section 1.3

Exercise 1.3.1

The reasons for this very large increase could be as follows:

• A *chance* (random) variation (although this is unlikely given the large numbers involved). The role of chance variation is a very important concept in epidemiology and statistical methods, and we will begin to examine this in Chapter 2.

- An *artefact* of the system, such as a change, or error, in the system for recording admissions. This is also unlikely to cause such a dramatic increase, but needs to be considered. The quality of information and how it is defined and handled are also very important issues in research, which we will begin to look at in Chapter 2.
- A *real* increase, which could result from changes in referral procedures by GPs (although this is again unlikely to cause such a large increase in one year, especially given the more gradual increase seen in previous years). A more likely explanation is a sudden increase in the population that the accident and emergency department is serving. Natural increase in population is unlikely, unless there had been an event such as a rapid influx of refugees. Closure of another (smaller) accident and emergency department in the city is the most likely reason for the large increase in the population being served.

The key point here is that, in order to make some judgement about this increase, we need to know the size of the population from which the admissions are coming. If this population has increased by 30 per cent, then, all other things being equal, we would expect the number of admissions to increase by 30 per cent. Changes in numbers of events (whether deaths, cases, admissions, etc.) cannot be interpreted usefully without information on changes in the population from which these events arose.

Exercise 1.3.2

- 1. Calculation of point and period prevalence. *Point prevalence*: in this diagram, 7 cases were 'active' at time A. With a population of 1000, the point prevalence is $7 \div 1000 = 0.007$. This is a rather untidy way of expressing the prevalence, so we can state it as 0.7 per cent or 7 per 1000. *Period prevalence*: here we have a longer period to consider, during which some cases resolve, and new ones occur. We include the cases that got better, since they were cases at some time during period B. We must also include the new cases that start during period B. A total of 10 cases were 'active' during period B, so the period prevalence is $10 \div 1000 = 0.01$. This can be presented as 1 per cent or 10 per 1000.
- 2. The point and period prevalence rates differ because, for period prevalence, we included some cases that had not yet recovered and some new cases that appeared during period B.

Exercise 1.3.3

- 1. During period B, a total of seven new cases arose.
- 2. The incidence rate for boys aged 0–14 is $(78 \div 41\,000) \times 10000$ per year, = 19.0 per 10 000 per year. The incidence rate for girls aged 0–14 is $(76 \div 37100) \times 10000$ per year, = 20.5 per 10 000 per year. So despite the fact that there were more cases among boys, the incidence rate was actually higher among girls. The reason for this is that the population of girls was smaller. This again emphasises why rates are so important.

Exercise 1.3.4

1. The total observation time for the 30 subjects is 261.9 person-years, during which time $n = 20$ subjects developed the disease. The incidence rate is $20/261.9 = 0.0764$ per person-year or 76.4 per $1,000$ (10^3) person years.

2. In this case the total observation time is:

$$
5+5+5+3.5+4.8+4.6+5+\ldots +1.5=115.8
$$
 person-years

The total number of cases is now 11 (as we only include cases occurring within the first 5 years), so the incidence rate is $11/115.8 = 0.095$ per person-year or 95.0 per $10³$ person-years. This rate is somewhat higher than that for the longer follow-up.

Section 1.4

Exercise 1.4.1

Road accident prevention

Traffic calming slows vehicles to a speed which markedly reduces the chance of a collision. In addition, traffic calming makes drivers more aware of the likely presence of pedestrians, including children, and this further reduces the chance of an accident occurring. The slower speed also reduces the chance of serious injury or death if a collision between a child and a vehicle does occur. The prevention process here is principally through preventing the accident (injury/death) happening in the first place, but if it does happen, the severity and likelihood of death are reduced.

Breast cancer prevention

From the information we have, it is apparent that we do not yet know enough to prevent most cases of breast cancer from occurring in the first place. What we can do is detect the disease at a stage where, if treated promptly, it is possible to cure the disease in a substantial proportion of affected women. Thus, in contrast to the road accident example, the prevention begins after the disease process has started, but it is at a stage where it is still possible to cure the disease.

Prevention of foot problems in the diabetic

In this last example, the disease process is well established, and cannot be removed or 'cured'. That does not mean that the concept of prevention has to be abandoned, however. In this situation, preventative action (education, support, treatment, etc.) is being used to prevent damage to the skin of the feet, with the infection and ulceration that can follow. Prevention is carried out, despite the fact that the underlying disease remains present.

Exercise 1.4.2

(Continued)

