21.1: Introduction to methods of analysis

This chapter describes simple statistical methods that are likely to be most useful for the basic analysis of intervention trials. Usually, a statistician will be closely involved in the design and analysis of a trial, and the more advanced analytical techniques that they might employ are not covered in this chapter. For more information on such techniques, the reader is referred to statistical texts such as Armitage and Berry (1987), Kirkwood and Sterne (2003), and Rothman et al. (2008). However, the methods presented in this chapter should enable the analysis of the main results of a trial. More advanced statistical techniques usually result in relatively small changes in the estimates of effect sizes through multivariate and associated analyses. Also, armed with the methods in this chapter, the reader should be in a good position to interpret and check the analyses reported in published studies.

The methods that are going to be used to analyse a trial should be considered at the time the trial is set up, so all of the appropriate data are collected and are assembled in a form suitable for the planned analyses. It is a common requirement nowadays for the statistical analysis plan to be fully developed, before any blinding in a trial is broken and in advance of a ‘frozen’ data set being prepared for analysis. Such plans are discussed in Section 3.

The choice of an appropriate method of analysis of a trial depends on the type of outcome measure which is of interest. The different types of outcome measure are discussed in Section 2, which also includes a brief review of the concepts of confidence intervals (CIs) and statistical tests. In Sections 4, 5, and 6, methods are described which are appropriate for the analyses of data in the form of proportions, rates, and means, respectively. RCTs have been recommended as the method of choice for determining the effects of an intervention, because such trials generally avoid the problem of confounding. Sometimes, however, particularly in small trials, there may be differences between the randomized groups, with respect to factors that might affect the outcome of interest, but which are unrelated to the intervention under test. If there has been a proper randomization process, any such differences should rise by chance only. If the trial is large, it is unlikely that there will be any important imbalance in this respect between the randomized groups. In small trials, such chance differences may have a larger effect, and, in such circumstances, it may be important to adjust for
any potential confounding due to these chance differences. In addition, where randomization is not feasible, any attempt to draw conclusions about the effects of an intervention must make allowance for possible confounding factors, and simple methods for doing this are described in Section 7. The analysis of trials in which interventions are allocated to groups, rather than individuals, is discussed in Section 8. How the results of a trial may be used to assess the possible public health impact of an intervention is considered in Section 9.