1.2: Outline of contents

The chapters of this book can be considered in three main groupings. Chapters 2 through to 13 review issues to consider and steps to be taken before starting a trial. Chapters 14 through to 20 detail the tasks to be carried out during the conduct of a trial, with a focus on data collection. Chapters 21 and 23 discuss the analysis, interpretation, and reporting of trial results. We have also included a short chapter on Phase IV studies (Chapter 22), that are usually conducted after a product has been licensed and is in, or is about to go into, public health use. Phase IV studies are usually not randomized designs, because of the ethical issues in withholding a licensed intervention from participants, and such studies are not a main focus of this book. However, we have included this chapter because many of the design, conduct, and analysis issues discussed in other chapters have relevance for Phase IV studies and also because it will often be desirable for Phase III trials, which usually measure the efficacy of an intervention delivered in a highly controlled manner, to be followed by Phase IV evaluations in ‘real-life’ programmes.

Before embarking on a trial, the first steps are to define the goals, objectives, and key questions for the study. As background to this, the broad array of potential types of interventions is catalogued in Chapter 2. The importance of critically reviewing essential background information relevant to a trial, including trials of similar interventions, through a systematic review of literature is emphasized in Chapter 3. The heart of the book is concerned with the design of the trial, as outlined in Chapter 4, and making it of appropriate size (Chapter 5). Many of the design details are guided by ethical concerns (Chapter 6), regulatory requirements, and governance issues (Chapter 7). A major issue in planning a trial is generating the resources to carry it out, and guidance is given in Chapter 8 on the preparation of grant applications for trials to funding agencies.

Field trials are generally based in communities, and their successful conduct is highly dependent on investigators engaging appropriately with community members at all stages in the planning and execution of a trial (Chapter 9). Before a trial starts, the target population has to be defined and registered (Chapter 10), and then the interventions under test must be allocated to individuals or communities, in an unbiased way, by randomization, with the intervention
allocations being kept ‘blind’, if possible, to investigators and participants. Ways of achieving this are discussed in Chapter 11. Evaluation of the impact of an intervention depends upon appropriate definition of the outcomes that the intervention is expected to affect. Choice of appropriate outcome measures and unambiguous definition of these is considered in Chapter 12.

Undertaking a trial is often a major activity, involving a large trial team for several years. It is rarely possible to start a trial immediately the protocol has been written and the funding obtained. Almost always, it is necessary to have collected preliminary data to facilitate the planning of the trial and to conduct studies to test out the procedures that are proposed for use in the trial, and modifying them appropriately if they are not found to be fit for purpose. Such preliminary studies and pilot testing of procedures are covered in Chapter 13. Information about trial participants is commonly collected through the administration of questionnaires. The various forms that these might take and different methods of administering them are summarized in Chapter 14.

Most intervention trials involve some element of behaviour change, both on the parts of those administering the intervention (for example, workers in the health service) and of those taking it up—the trial participants. The extent of behaviour change required will vary, according to the intervention under test. Evaluating a new vaccine which is administered at the same time as routine vaccinations in the childhood immunization programme may require relatively little behaviour change, but implementing an intervention to reduce high-risk sexual behaviour to lower the risk of human immunodeficiency virus (HIV) infection, or promoting hand-washing to reduce the risk of diarrhoeal diseases, will involve substantial behaviour changes. Undertaking social and behavioural research to facilitate the design and implementation of interventions is reviewed in Chapter 15.

Quality control of all aspects of conducting a trial is crucial if the findings from the trial are to be used to make important public health decisions about the use, or otherwise, of an intervention, based on the trial results. These issues are discussed in Chapter 16, while Chapter 17 specifically focuses on methods and quality control in field laboratories, which are an important component of most trials.

Nothing can be done without financial support for the trial. The essentials for the preparation of budgets for grant applications are given in Chapter 8. The efficient planning and management of finances during a trial are also key to success, and a requirement of funding agencies. The necessary budgeting and accounting methods are outlined in Chapter 18. Chapter 19 affords an overview of the main methods used to assess the costs of health interventions and summarizes the types of economic analyses that can be conducted to assist decisions concerning resource allocation to health interventions.

In all but the smallest trials, substantial amounts of data are collected and have to be efficiently processed, both during the conduct of the trial and for the analysis of the results during, and at the end of, the trial. Methods of data management are summarized in Chapter 20, and an outline of methods of statistical analysis of trials is given in Chapter 21. In most trials it will be necessary to employ a statistician to oversee the analysis of the data from the trial, but the relatively simple methods summarized in this chapter should be sufficient to elucidate the main results from most trials.

Finally, Chapter 23 stresses the importance of communication at all stages of the trial, how best to communicate to the many different audiences who should be informed about the trial, and the necessary steps to translate research findings into policy and public health action.
We have deliberately not included large numbers of references, as the book is intended to stand largely on its own, without readers needing access to a well-stocked library. Referencing has been reserved for where a particular study has been described, or as a guide for readers who may require a more detailed explanation of a concept than can be included in this text. Whenever possible, we have favoured open access or relatively low-cost resources.

Copyright and permissions

© London School of Hygiene and Tropical Medicine 2015 This is an open access publication. Except where otherwise noted, this work is distributed under the terms of the Creative Commons Attribution NonCommercial 4.0 International licence (CC BY-NC), a copy of which is available at http://creativecommons.org/licenses/by-nc/4.0/. Enquiries concerning use outside the scope of the licence terms should be sent to the Rights Department, Oxford University Press, at the address above.