

Clinical trials: what, why, how and who?

What is a clinical trial?

A clinical trial is scientific study designed to collect evidence on the safety and effectiveness of an experimental intervention in a particular population of individuals. Interventions are not restricted to drug treatments and can include surgical techniques, diet and exercise programs and medical devices, just to name a few. Often a trial will involve measuring and comparing the relative merits of the experimental intervention against what is considered to be current standard practice. All clinical trials must be conducted in accordance with relevant regulations and ethical principles governing scientific research activities involving volunteers. The References section of the Outreach website provides links to some of these.

Why are clinical trials important?

The evidence obtained through appropriately designed and conducted clinical trials (especially randomised controlled trials) may be applied in practice to optimise all manner of health care decisions. Improvements in health outcomes are achieved through the preferential use of interventions that have been proven to be safe and effective over other options that have a poorer or an uncertain level of effectiveness or safety. Trials with inappropriate study designs or processes are more likely to produce inconclusive or unconvincing results and be ineffective in terms of generating useful evidence. Seeking expert advice on the design and planned execution of a trial helps maximise the potential of a trial to make a substantive contribution to existing evidence and ultimately lead to improved health outcomes.

How is a proposal for a clinical trial developed?

The development of a brief document that outlines a summary of the trial is recommended as a first step from which a full protocol and/or funding applications can be developed. A concept outline begins by establishing the motivation for a trial on the basis of the prevalence and severity of the health problem and the size of the benefit that the intervention could plausibly impart. The general

research question must be distilled to a specific set of measurable objectives and hypotheses that can feasibly be tested in an appropriate target population. A suitable experimental design must be selected, and all study procedures, data collection and analysis methods must be carefully planned. Approaches for recruiting participants, project managing and overseeing the trial must also be developed. The Outreach program is available to help translate a research idea into a formal plan for a clinical trial. Contact the Outreach team via email (trials@ctc.usyd.edu.au) or through the website. Guidelines on the preparation of a concept outline are available in the resources section of the Outreach website. Participation in one of the Outreach concept development workshops is another way of obtaining support (information on the next planned workshop is available on the Outreach website under the events section).

Who does what?

A good strategy for ensuring that a trial is appropriately planned and executed is to assemble a multidisciplinary study team that is able to provide expertise across the clinical, statistical, and operational aspects of the research. Additional expertise may also be required in other areas, such as clinical epidemiology, pharmacology, and health economics. For a clinical trial to be conducted effectively and efficiently, it can also be desirable to establish committees with specific responsibilities. More information on the statistical and operational considerations and responsibilities relating to undertaking clinical trials as well as the roles of various clinical trial committees and subcommittees is available in the Resources section of the Outreach website.

Contact the Outreach team (trials@ctc.usyd.edu.au) or through the website (www.outreachclinicaltrials.org.au) for further advice on this topic.