How do Clinical Trials Work?

Each clinical trial has a study sponsor and a protocol. Potential drugs must move through four well-defined phases of testing in patients.

The trial sponsor, often the pharmaceutical company that develops the therapy or medication, prepares the protocol for the clinical trial.

One type of clinical investigation known as interventional research involves testing new drugs on volunteers under extremely strict government safety criteria and protocols.

Researchers observe how the drug affects the body under highly controlled conditions and whether the treatment is helpful.

A protocol is a plan that explains how the trial will work, what will be done during the trial and why. Each medical centre that conducts the same type of trial uses the same protocol.

Key information in a protocol includes:

- How many patients will participate
- What is known about the intervention from previous trials
- Who is eligible
- What tests patients will get and how often
- What type of data will be collected
- Detailed information about the treatment plan, if appropriate

Study-related medical care is often provided to you at no cost.

Clinical researchers work to ensure they avoid bias in clinical trials. By this, we mean “bias” relating to human choices or other factors unrelated to the protocol that might affect the trial's results. For example, if doctors could choose which patients to assign to comparison groups in a study, some might assign severely afflicted patients to the treatment group and healthier patients to the control group - the one not receiving treatment. The doctors might not even realise they are doing this and it could affect trial results.

Randomisation helps ensure that researchers don't introduce bias into the trial. In many clinical trials that test the effectiveness of a medication, half of the participants receive the medication in question. The other half receives a placebo, which contains no treatment. Randomisation involves assigning patients to these comparison groups by chance, rather than choice.

Researchers also may use “blinding” to help avoid bias. In a “blinded trial”, researchers won’t know which patients are receiving treatment and which ones are receiving a placebo.

The Four Phases of Clinical Research

For any new drug to become available to the public in Australia it must be approved by The Therapeutic Goods Administration (TGA), part of the Federal Australian Government’s Department of Health. The TGA is responsible for regulating therapeutic goods including prescription medicines, vaccines, sunscreens, vitamins and minerals, medical devices, blood and blood products.

Almost any product for which therapeutic claims are made must be entered in the Australian Register of Therapeutic Goods (ARTG) before it can be supplied in Australia. The drug must first pass through three phases of interventional clinical trials to show that it is safe and effective in treating the disease. If the drug passes these tests it will continue to be monitored for safety and effectiveness in what is known as a Phase 4 study.

Clinical research encompasses signing the informed consent form, the process of joining a clinical trial, after meeting all necessary criteria and following all necessary steps to the project’s conclusion.

Who Can Participate?

All clinical trials have guidelines about who can join. Some enrol healthy people. Others enrol only those with certain conditions, such as CF.

A CF clinical trial may have other guidelines in addition to requiring that the study volunteer has CF. These guidelines are known as “inclusion” and “exclusion” criteria and help ensure the research results are reliable and at the same time reduce the risk for those enrolling in the trial.

Inclusion criteria and exclusion criteria refer to the standards used to decide whether a person may or may not enrol in a clinical trial. Criteria are based on such factors as age, gender, disease, previous

treatment history and other medical conditions.

The benchmarks depend on the type of trial. For example, the age the person with CF must be to participate varies according to each trial because drugs work differently in young children than they do in adults. Before a drug can be tested in young children, it must be shown to be safe and effective in adults with CF.

For example if researchers are testing how well a particular antibiotic works in fighting Pseudomonas aeruginosa, then the trial would have inclusion criteria specifying that only people with CF who are infected with that bacteria can join the trial. Those who do not have that bacterial infection would be excluded from the trial.

Who Sponsors Clinical Research?

Clinical research can be sponsored (that is it's paid for) in part or entirely by any number of organisations or individuals. For example, medical institutions, universities, foundations, voluntary groups, drug companies and federal government agencies can play important roles in this regard.

The sponsor chooses principal investigators to run the trials. Study-related medical care is often provided to the participant at no cost. CFA is the primary supporter of CF research in Australia.