

Fact Sheet 5 – What is a Clinical Trial

What is a clinical trial?

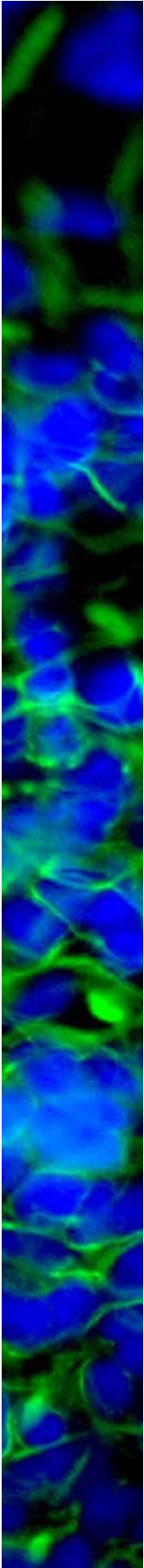
Clinical trials are where new treatments, drugs and devices are tested in volunteer patients, to see whether they are **safe** and **effective**. Clinical trial research is conducted by experienced medical staff under experimental conditions. All clinical trials must be approved by an independent Ethics Committee that monitors the conduct of the trial and be conducted within the guidelines set out by Australia's Therapeutic Goods Administration (TGA). The TGA's website outlines the regulation and processes that must be followed - www.tga.gov.au/ct/index.htm.

All pharmaceutical treatments in use today had to be proven effective and safe in clinical trials before they could be made available for widespread use within the community. A high quality clinical trial will be one which the proposed treatment has undergone extensive prior investigation in the laboratory and in animal studies and will have shown a strong repeatable effect.

A clinical trial is generally made up of four levels or phases, which must be passed, before the product or treatment is able to gain regulatory approval.

- Phase I – the first testing of a new drug, treatment or clinical device on a small group (20-80) of people in an attempt to evaluate safety. Phase I research studies can include drugs or treatments that have been tested in animals but never in humans. These trials are usually first conducted with 'healthy' volunteers (this is someone with no pre-existing medical condition).
- Phase II - generally involves a larger group of people (several hundreds) to further evaluate safety and explore the efficacy of the intervention. This involves one group of patients receiving the experimental drug, while a second 'control' group will receive a standard treatment or placebo (drug containing no active ingredient). Often these studies are double blinded, this is where neither the patients nor the researchers know who is receiving the experimental drug, or who is receiving the placebo. This is so that the study can provide a comparison between the relative safety and effectiveness of the experimental drug.
- Phase III – continues to investigate the efficacy of the intervention in larger groups of people (up to several thousand) by comparing against other similar interventions while monitoring for undesired effects. Once a Phase III study is successfully completed, it can be requested that the drug be approved for availability to general public.
- Phase IV – once the intervention has been marketed, further studies are performed to monitor effectiveness and collect information regarding undesired effects. Late Phase III/Phase IV studies often compare an investigational drug with other drugs already available on the market. This is called a 'bio-equivalence study'.

In Phase III and IV trials, patients are usually separated into two separate groups; a control group and a trial group. The control group does not receive the new treatment or medicine and act as a means of comparison for the trial group. The trial group is the one chosen to test the effects of the new drug, treatment or clinical device. Neither the patient nor clinicians



performing the clinical trial should know which group the patient is in. This is known as a double blind trial and is done to ensure there is no bias or placebo effect.

Why are clinical trials so important?

Clinical trials are important for a number of reasons. For the area of stem cell application it is important to know if a treatment is not only safe but also works. To comprehensively understand the workings of a new treatment for a human disease, it is necessary that it is tested on those the new treatment is intended to help.

Once data has been collected, the regulation of clinical trials must be approved by the TGA. The equivalent of the TGA in the United States of America is the Food and Drug Administration and in the United Kingdom, the Medicines and Healthcare Products Regulatory Agency.

Results from clinical trials can in turn lead to the development of new medicines and treatments for various diseases and conditions.

Without clinical trials it is impossible to fully understand if stem cells can act as an effective treatment for the condition it is being intended. Experimental treatments, even with evident successes, cannot be deemed worthy by the medical community until tested properly and scrutinised under peer review.

There are some clinical trials in Australia using adult stem cells to treat conditions such as arthritis, corneal conditions and long bone fractures.

For more information on clinical trials refer the Australian Stem Cell Centre's Patient Information Handbook which can be found on our website at [http://www.stemcellcentre.edu.au/For the Public/Patient/Handbook.aspx](http://www.stemcellcentre.edu.au/For_the_Public/Patient/Handbook.aspx).