Patient Safety Is a Priority

Nothing is more important than patient safety in developing new CF treatments. The Australian federal government requires that all clinical trials are supervised for safety.

A clinical trial must go through many layers of review before it can start enrolment. Every layer of the process is intended to protect patient well-being through monitoring aspects including the safety and effectiveness of all drugs, biologics, vaccines, and medical devices.

The sponsor of the study - usually the drug company - must submit an application for an investigational new drug. In this application, the TGA reviews the results from initial laboratory studies to determine that the potential drug poses very few risks to people and that any risks are worth the possible benefits.

Once the TGA approves the application for an investigational new drug, it must approve all clinical trial protocols and make sure all required guidelines are being followed. When a drug or therapy has finished three phases of research, the TGA determines whether the new drug or therapy will be approved for use by other people with CF.

To approve the new drug, the TGA requires that the study results show that the new therapy will be as effective as or better than medications or therapies that are already available. The potential financial cost to patients is not a factor in approval.

As part of the patient safety process, there are many other people who review clinical trials to make sure the possible benefits outweigh the risks, and this includes the principal investigator.

Study Sponsor and Medical Monitors

The study sponsor, often the pharmaceutical company that developed the drug, develops a study plan (a “protocol”), which includes a guide for how safety will be monitored. During the study, medical experts hired by the sponsor look out for and monitor adverse events in real-time.

Adverse events are considered negative side effects, and these include any unexpected results whether they are related to the drug or not. For example, an increased cough during a clinical trial would be considered an adverse event, and it would be monitored throughout the study.

A compound review Explained

A compound review is conducted when different drug compounds in the same treatment category (such as anti-inflammatories) are being developed for clinical trials. The goal of this evaluation is to independently assess whether the compound being considered is likely to be more effective than other compounds in the category. If it isn't, the compound will not move forward into clinical trials.

All multicentre clinical trials are required to go through a protocol review process, consisting of reviews that:

- Enhance study design
- Ensure efficient conduct within the CF centre network
- Protect patient safety
- Maintain the accuracy of study data and specimens
- See how their studies fit with the priorities of the CFA

Institutional Review Board

After a clinical trial has been approved by the TGA, the company developing the potential drug identifies the research institution(s) where it will test the drug.

The next level of approval must come from each research institution's established review board, an independent committee that reviews the entire study proposal to make sure that the benefits of the trial outweigh the risks and that volunteers will be monitored closely for any complications and safety.

This also ensures that anyone enrolling in the clinical trial has been given information that is easy to understand and allows them to make an informed decision about participation.

The selected Institutions will only approve research that deals with medically important questions in a scientific and responsible way. These Institutions continually monitor study conduct throughout the trial and is notified of any adverse events.

Data Safety

All clinical studies must ensure data safety at all times. A data monitoring committee is usually assigned to a CF clinical trial to confirm protocols are reviewed before enrolment begins to ensure it includes appropriate testing and monitoring to ensure safety is the paramount concern at all times.

An important feature of each data monitoring committee is that it scrutinises the clinical trial in real time and is able to see what is called “unblinded” data. In other words, when reviewing the data, the committee members know if a participant is being treated with the study drug or with a placebo (an inactive substitute). This close monitoring allows the committee to pick up early signs of potential side effects. If necessary, the committee will suggest changes or even stop the clinical trial to protect a participant’s well-being.

Many of the risks for people with CF who participate in clinical trials are inherent to the disease so they can be shared across different clinical trials. Maintaining a core of expertise specifically related to CF ensures effective monitoring CF clinical trials and protecting CF research participants as new drugs are developed.

Site Principal Investigator

In a trial that investigates CF The study site Principal Investigator (PI) is a doctor who specialises in the care of people with CF and is responsible for the oversight of a study at a particular site. The most important part of the PI's responsibility is to monitor the safety of the study participants at the PI's site.

Before enrolment, the PI reviews your or your child's health to determine safe participation in the study. During the study, the PI reviews all safety measurements including pulmonary function testing and blood work, watching for any changes in health. The PI also reviews any changes in symptoms.

The PI's first priority is always the participant's safety. If at any time the PI feels the study is not in your or your child's best interest, the PI has not only the right - but also the duty - to remove you or your child from the study.

Study Participant's Role

The process of learning about a clinical study or trial before deciding whether to join is a key part of your participation. Doctors and nurses involved in the trial are also there to explain the study and answer your questions. The deeper your understanding the better the trial will flow to its conclusions.

In the informed consent process, as a participant, you are given all the available information about the study process as well as possible risks and benefits. It is important for you to ask any and all the questions that you want answers to so you can feel comfortable about participating.

During the study, you must keep your doctor informed about how you or your child is feeling and any concerns you may have. It’s equally imperative that you follow the study plan as explained to you during the informed consent process.