Why Participate in a Clinical Trial?

Clinical trials (or studies) are vital to developing new treatments for cystic fibrosis. All clinical trials that will be listed here will have undergone a careful independent review by an ethics committee to ensure the trial will be conducted safely and appropriately, which includes protecting your privacy. In addition, clinical researchers are bound by a duty of care to perform their research in a way that protects your rights and health. Nonetheless, while your safety will be paramount and protected at all times when you are considering participation in a clinical trial you must consider the risks and the benefits.

There have never been more opportunities to help develop new therapies for CF than there are today. This means that because of all the promising new research avenues opening to us, as many individuals with CF as possible are needed to participate in clinical trials.

Another challenge our researchers face with the accelerating rate of change is known as under-enrolment. It is a critical issue because it can slow the development of a potential treatment and its approval for use by regulatory authorities. As there are only around 3,200 people with CF in Australia there is a limited pool of people to draw upon for a clinical trial. That is why it is so important that those who are interested and able to participate find an appropriate clinical trial – for their potential benefit and the advantages it could bring to others.

The Benefits of Participating in a Clinical Trial

Participating in a clinical trial can be a very fulfilling and worthwhile experience. In turn, you or your child with CF may choose to participate in clinical trials for any number of different reasons. Some possible benefits include:

- Taking an active role in managing your own CF care or that of your child
- Gaining access to new treatments before they are more widely available
- Helping advance our knowledge of CF

What About the Risks?

Although there are many benefits to participating in a clinical trial, it is important to remember there may also be some possible risks including:

- Side effects of the medications or treatments being studied
- Unwanted events during the trial that may or may not be related to the study drug
- Failure of a treatment to work

Every trial is different but all trials will supply you with a patient/consumer information form. This form must clearly explain the aims of the trial and the requirements that you and the investigators have specifically in regard to the trial you are considering. This form should be written in a way that is easily understood by you and your family. It should fully disclose the potential risks of the research and your and the investigator's obligations in the trial. It is important that you carefully read this form and understand it. You should be provided with sufficient time to consider this and should be able to discuss it with your treating health professionals before making a decision. The investigators should answer any questions that you may have.

Remember that by signing an Informed Consent Document you are not signing any form of contract and you can leave the study or trials at any time. In turn those who are monitoring the trial – such as the principal trial investigator or the study medical monitor – can withdraw you from the study if they believe your health is at risk.

In general, for all trials, the research team will monitor your or your child's health and safety throughout the trial, whether you are receiving the drug being studied or a placebo.

A placebo is a pharmaceutical preparation that contains no active substance (a sugar pill) and looks like the drug that is being tested.

If the research team notes any worsening of your health during the trial they will notify your regular CF care team and the study sponsor to determine if it is related to the study drug. Equally importantly, if you notice any worsening of your or your child's health or have any concerns during the clinical trial, please do not hesitate to contact the study team.