Children with CF and clinical trials

What every parent needs to know.

Patient safety is always the top priority in clinical trials and exceptional, rigorously developed precautions are taken to ensure children's safety. Even with this in mind, it is important to involve your child in the decision-making process about whether to join a trial.

Medical treatments that have been tested in adults often act differently in children. Medications require different dosages and sometimes produce different side effects in children than adults. This drives the need for more and more studies focusing on children's health to develop treatments, drugs and procedures specific to children.

Many different people are monitoring your child's safety in a clinical trial, just as they monitor the safety of adults in clinical trials. Clinical trials involving children must follow all the safety precautions in adult clinical trials. In addition, researchers must first collect and review safety data in adults before testing adolescents and then, finally, children.

Informed Consent

The process of learning about a clinical study or trial before deciding whether to join is called Informed Consent. Doctors and nurses involved in the trial are there to explain the study and answer your questions. The goal is to ensure that as a participant you are informed about the study or trial.

Parents must give legal consent for their child to participate in a research study. Children also can be asked to agree to participate, although some studies do not require their assent. This means that children are given basic facts about a research study and are asked to be part of the decision. Children as young as 7 years old can be asked to give assent.

If you are thinking of enrolling your child in a study – after you have given it careful consideration - ask how your child feels about it. Some children will want to be part of the process while others may not. Some may be uncertain or afraid, worried about pain or concerned about how it will affect school or friendships. Some will be interested in helping other kids.

At any age the important thing is that your child is comfortable and his or her questions are answered. When children are asked if they want to join a study, it shows that you respect them. Children understand a lot and it's important that their voices are heard.

The research team may be able to help if you and your child disagree about participating. Some studies have advocates and ethics experts involved who can help address any and all concerns you or your child may have.

Ask Questions

Your study team is prepared to answer your questions, so don't hesitate to ask the principal investigator or the research coordinator about anything that's on your mind. In fact there's no such thing as a silly question, no matter how seemingly trivial it is! Some questions you may want to ask include:

- What is the purpose of the clinical trial?
- Why do researchers think that this particular CF drug or treatment might work?
- Who is paying for and supporting the study?
- Who has reviewed and approved the study?
- Who will be in charge of my child's care?
- How long will the study last?
- What will my responsibilities be if my child participates?
- What kinds of therapies, procedures or tests will my child have during the trial?
- Will any of the therapies, procedures or tests hurt, and, if so, for how long?
- How do the tests in the study compare with those my child would have outside of the trial?
- Will my child be able to take his or her regular medications while in the clinical trial?
- What are the risks?
- What are the benefits?
- How do the possible risks, side effects and benefits compare with my child's current treatment?
- How could being in this study affect the daily life of my child or my family?
- Can I talk to other people who are participating in the study?
- Will I be compensated for my child's participation in the clinical trial and/or travel expenses?
- What is the time commitment for us?
- Will results of the clinical trial be given to me and, if so, when?
- Whom should I communicate with during the trial - the research team, my CF care team or both?

Write down your questions before you talk with your doctor or members of the research team to make sure you remember to ask them all. Also, try to write down the answers or record them with your smartphone, so that you can review them whenever you want.

Costs and Compensation

Even as a parent it's only natural to be concerned about the cost of participating in a clinical trial. The sponsor of the clinical trial typically covers costs so there are no fees to participate.

An Informed Consent document will be made available to you. This describes the rights of clinical research participants and details about the study or trial. It includes the study's purpose, length, required procedures and staff contact information. It also explains risks and potential benefits. As a participant or parent or guardian we encourage you to ask the study staff any questions you may have before signing this.

In this informed consent document a section on compensation will tell you how much money you will get to participate. The compensation is usually given to pay for your time and to help offset the costs of participating.

Some studies offer additional reimbursement for expenses such as meals, travel costs and even parking. If you anticipate other costs let your research team know ahead of time to see if they can reimburse you for those expenses.

Here are some questions you may want to ask:

- Will I have to pay for travel (petrol, road tolls, parking, public transport, taxis)?
- Are there funds to help me if I have to miss work without pay?
- What if there is an overnight stay?
- Will meals be provided?
- What will the study pay for and what is my insurance expected to pay for?
- What if I don't have health insurance - what will I have to pay for?
- Will I need to pay for the study medication once the study is complete?

Accommodating Your Schedule

To help make participation as easy as possible for you, care centres and researchers often can work together to coordinate schedules so that regular care centre visits can take place at the same time as the research check-up. Often appointments can be moved to the beginning or end of the day to accommodate your work day.

What You Can Expect

You need to be aware of the fact that you or your child will undergo more testing than would be done during clinical care visits because researchers must document changes in your child's health status. These assessments could include blood tests and extra breathing analysis.

Your CF centre will continue to be responsible for your child's primary care. If your child experiences any problems, you should let your care team and the research coordinators know straight away.

When a study ends it may take a while for the researchers to analyse data and publish results. If your child was testing a medication, he or she may have to stop the study medication at the end of the trial unless there is an "open-label extension" to the trial or the drug becomes approved by the TGA.

An "open-label extension" study means the doctors and participants know which treatment is being provided. As a participant – or as the parent or guardian - you are invited to enrol so additional safety information can be gathered about long-term use while the relevant regulatory bodies review the treatment for approval. This option is not always available.

A clinical trial is where all parties including physicians and participants know whether they are using an investigational drug. Once the safety and effectiveness of the investigational drug has been established, participants can take it before it is formally approved.