Understanding Clinical Research:

A Guide for Parents and Caregivers

WHAT IS CLINICAL RESEARCH?

Clinical research is conducted to help researchers better understand diseases and possible ways to treat them. There are two types:

Observational Studies: Conducted to learn how a disease develops or changes over time. Researchers look at data such as test results, health history, or blood or tissue samples.

Clinical Trials: Conducted to learn if a new medicine, medical device or procedure is safe and if it works.

HOW DO CLINICAL TRIALS WORK?

In all clinical trials, also called clinical studies, researchers have questions and are looking for answers.

Researchers follow a detailed plan called a protocol to get answers in a scientific way that is not biased. The protocol also explains the study goals or endpoints.

Researchers learn about safety and effectiveness by comparing the experiences of participants who receive the study drug with those who receive another drug or a placebo. A placebo does not contain any active ingredients.

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WHAT ARE CLINICAL TRIAL PHASES?

Trials are conducted in phases. If the trial endpoints are met, the study may move to the next phase.

Phase 1 Conducted first in a small group of often only healthy people and studies safety.

Phase 2 The study drug is given to more people to research safety, side effects and if it works.

Phase 3 The study drug is given to more people and may be compared to a similar treatment. In rare disease studies, phases are often combined since fewer participants are available.

Phase 4 Conducted after a drug is approved so researchers can continue to monitor safety and learn more about the drug.

Clinical trials may last weeks, months or even years so study participant's long-term health and safety can be monitored.

WHO CAN JOIN A CLINICAL TRIAL?

- To join a clinical trial, study participants must meet the eligibility criteria for that trial.
- The criteria are different in every study and may include age, health status, disease severity or specific test results.
- Clinical trials for children study how a potential treatment impacts their health and safety. Researchers also try to find the right dose for their growing bodies.

WHAT IS INFORMED CONSENT?

Joining a clinical trial is an important decision and a commitment. Informed consent is the process of learning about all the potential benefits and risks of joining a certain trial.

If a child is joining a study, they may be asked to sign an Informed Assent Form. Their caregiver will sign the Informed Consent Form.

Before joining a study, participants or their caregivers must sign an Informed Consent Form that says the benefits and risks of this study are understood.

WHAT ARE THE POSSIBLE RISKS AND BENEFITS OF JOINING A CLINICAL TRIAL?

The safety of study participants is a top priority for researchers. There are systems in place to monitor safety and ethics, including an Institutional Review Board (IRB). The IRB makes sure the risks to participants are as low as possible and participant's rights are protected. If you ever have any concerns about your safety or the ethics of the research, you can contact the IRB listed in the Informed Consent Form. You may leave a study at any time for any reason but be sure to let the study staff know.

Every clinical trial has its own possible risks and benefits. Below are some common ones to consider.

POSSIBLE RISKS

- Possible side effects from the medication, from minor to serious complications
- Procedures, questionnaires, travel to study visits, etc., that may be time-consuming and physically or emotionally uncomfortable
- May need to stop taking other medications
- May need to follow requirements around things like birth control or diet
- Your privacy may not be kept. Many efforts are taken to secure your health data but it is a risk

POSSIBLE BENEFITS

+ Knowing that you are helping others and helping researchers learn more about the disease
+ An opportunity to play an active role in your healthcare
+ Early access to a new investigational treatment
+ Access to tests, care from experts, and ongoing health monitoring, usually at no extra cost

WHAT HAPPENS WHEN THE TRIAL IS FINISHED?

The study sponsor may continue to provide the study medication to study participants through an access program

You may be unblinded, which means you may learn if you were given the study treatment or a placebo

Study results will be shared in a medical journal or at a medical meeting. Study participants are de-identified, which means their names are not used.

A summary of the study results is posted on clinicaltrials.gov

The FDA or another regulatory agency may review the results and approve the treatment for public use, or more research may be done

INTERESTED IN JOINING A CLINICAL TRIAL?

- ✓ Discuss the decision with your doctor and family and carefully weigh all the benefits and risks. You do not have to join a clinical trial.
- ✓ Ask the study staff what to expect if you decide to join so you can decide if you have the time and can make a long-term commitment.
- ✓ Make sure your child understands how their participation will impact their life and how the potential treatment might or might not help them.

Questions to Ask When Considering a Clinical Trial for Your Child

STUDY OVERVIEW

- What is the purpose of this clinical trial?
- What kind of trial is this?
- Have similar studies been done before? What were the results of those studies?
- Has this study drug been studied in children before?
- What are the risks and benefits of joining this trial?
- If my child is eligible for the study, do they have to participate?
- My child's screening test results show they are not eligible for this trial; can you retest them? If so, how often?
- If my child is enrolled in a trial, and they are on the placebo, will I (as a parent/guardian) be able to unenroll my child from the trial?
- Will enrolling in this trial affect my child's ability to enroll in a future clinical trial?
- What will happen next if the study is successful?
- What will happen next if the study is unsuccessful?

STUDY TREATMENT

- Does my child need to stop their current treatment before or during the trial?
- Will my child be given medicine as part of this study?
- How will the study drug be given?
- Will my child take the study drug at home, during a trial visit or both?
- Is this trial double-blinded, which means neither the study staff or the study participant knows if they recieve a placebo or not? If so, what are the chances my child will receive the placebo?
- Will my child get a chance to be given the study drug after the study is finished?
- Why and how do researchers think this treatment will work?
- When the trial is over, will there be an open-label extension study so my child has free access to the treatment?

TESTS AND PROCEDURES

- What labs or other tests will be done?
- Are kidney biopsies a part of this trial? If so, how many are there?
- How often will blood samples be taken? Who will draw the blood?
- Is there a way to monitor things like blood pressure or body weight from home, without going to the clinic?

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LOGISTICS, REQUIREMENTS AND FINANCIAL COMMITMENTS

- o How long is the trial?
- o How will the trial impact my child's daily life and routine?
- o How will participating in this trial impact me as a caregiver?
- o How many visits will my child have to attend as part of the trial? Will there be options for office visits and/or telehealth or home visits?
- o Can my child go to their doctor's office instead of the trial site?
- o Where is the trial site closest to me?
- o How long can we expect study visits to take?
- o Can my child continue to see their regular doctor while participating in the trial?
- o Can I get a note I can share with my employer/ school to explain absences?
- o Do I need to collect urine or other samples at home before the visits?
- o Can my child quit the trial at any time?
- o Will costs for travel, lodging, meals, medical tests, childcare, etc. be covered by the trial?
- o How will we be recognized for participating in the study? Will there be any financial compensation?

HEALTH AND SAFETY

- o How will the study staff ensure my child's safety and well-being throughout the study?
- o What are the possible risks and side effects of the treatment being studied?
- o Do the possible benefits outweigh the risks?
- o Will my child receive regular medical care and monitoring from the study team during the trial?
- o How are side effects being checked and monitored? What should I do if I think my child has a side effect between visits?
- o How will I be told about new possible side effects that happen during the trial?
- o If my child has a side effect, will they have to leave the study?
- o How can my child's anxiety about blood sampling and other procedures be addressed?
- o How will the clinical trial impact the future of my child's well-being (school, physical features, fertility etc.)?

PERSONAL DATA AND PRIVACY

- o Will the study findings be shared with other doctors, my insurance company or with the government?
- o Is there any genetic information being collected and will it be shared?
- o If you collect data or samples from my child, where will they be stored and for how long?
- o Will my child's health data be used or shared outside of the trial?
- o Will I receive results from this trial during or after it ends?
- o Can I share my child's involvement in the trial on social media? With other families?

RESOURCES

- o Will I receive help completing the Informed Consent form?
- o As a parent/caretaker, is there someone who can guide me through the trial?
- o Who will I be interacting with during this trial?
- o Do you have videos or other resources that I can share with my child to explain the trial?
- o Are there materials available that I can use to track our trial visits and requirements?
- o Can I meet with families who have been part of the trial?
- o Are there any additional resources or support services available to my child and our family while participating in the trial?