# Should My Child Join a Clinical Trial?

Clinical trials are research studies. They are designed to learn more about ways to prevent, detect, or treat diseases. Clinical trials can also help people with chronic (long-term) illnesses find better ways to live each day with their illness. Read on for information from the American Academy of Pediatrics about clinical trials.

The goal of a clinical trial for children is to find out whether a treatment is safe, helpful, and well tolerated by children. Researchers may study new drugs, vaccines, devices, or procedures, or new ways to use current treatments. Before you and your child decide whether a clinical trial may be a good option for your child, it's important to know more about the study, including the risks and benefits.

- Why are clinical trials for children needed?
- How are clinical trials done?
- What are the benefits and risks of a clinical trial?
- What do I need to know before I sign up my child for a clinical trial?
- What questions should I ask about a clinical trial?

## Why are clinical trials for children needed?

It's important to study new treatments in children because children are not little adults. Children have different treatment needs than adults, and sometimes they have different diseases than adults. Also, dosages of medicine, sizes of devices, or types of therapy may change over time according to each child's needs and stage of growth and as more information about the side effects and usefulness of a drug becomes available over time.

A successful clinical trial can create a new standard of care for children with certain conditions. Without a standard of care for children with certain conditions, treatment for children may need to be based on what works for adults. For example, most medicines given to children as part of standard treatments have been tested only in adults. When medicine that has been tested only in adults is given to children, this is called *off-label use* of the medicine.

In recent years, laws have been passed that require pediatric clinical trials for certain conditions. As a result, the number and range of clinical trials for children have expanded.

## How are clinical trials done?

Each clinical trial follows a detailed plan called a *protocol.* A protocol is carefully designed to minimize the risks of the study, to increase the chance of getting useful results, and to answer specific research questions. It includes a description of who can and cannot participate, the likely length of the study, information about what is being tested, and how information will be collected.

> **NOTE:** Before clinical trials of new medicines begin, these medicines are first tested and studied in a laboratory and in animals.

## What are the benefits and risks of a clinical trial?

Clinical trials are done because we do not know the best treatments of many diseases or because researchers believe that a new treatment may work better than current treatments. However, as with any new or existing treatment, there may be certain benefits and risks. Also, sometimes the risks and benefits are unknown.

### Possible Benefits

- My child may have access to new drugs or treatments that are not yet available and may be more effective.
- My child will help provide information that will benefit children in the future.
- My child may receive extra care from caregivers.
- My child may receive closer monitoring or extra testing that may not be a part of regular care.
- My child (we) may have access to more information about the condition or illness.
- Financial rewards or other incentives may be offered.

### Possible Risks

- My child may find the treatment unpleasant or there may be harmful side effects that can range from minimal to serious or life-threatening.
- Treatment may be ineffective.
- Treatment may involve a lot of time, including visits to the study site, more blood tests, more treatments, or hospital stays.
- My child may need to follow complex medicine dosing or procedures.

## What do I need to know before I sign up my child for a clinical trial?

If you and your child are interested in a clinical trial or have been asked to join one, it's important that you know all the facts. As part of the clinical trial process, informed consent ensures that parents are fully informed and can ask questions about the clinical trial. Informed consent grants permission but is not a contract. Parents and children can leave a clinical trial at any time. However, for some children, doctors may recommend that certain steps are taken before treatment is completely ended, depending on what is safest for the child.

In addition to informed consent from parents, assent from children in many clinical trials is also required. *Assent* is the term used to describe when a child agrees to be in a clinical trial. Before children are asked

whether they want to assent to join a clinical trial, they must first be old enough to understand basic facts about the clinical trial and be able to ask questions about the trial.

Your care team would be involved in helping you and your child make those important decisions.

## What questions should I ask about a clinical trial?

Before you and your child join a clinical trial, it's important that each of you understands the process from start to finish. The following questions about clinical trials can help:

### About the Trial

- What is the purpose of the trial?
- Is there an age range that qualifies my child for the trial?
- Who is sponsoring or funding the trial? (Sponsors may be organizations or individuals—including medical doctors, foundations, medical institutions, voluntary groups, and biopharmaceutical companies—as well as federal agencies.)
- Why do the researchers believe that the treatment being studied may be better than the one being used now? Why may it not be better?
- What kinds of tests and treatments are involved?
- How will the doctor know whether the treatment is working?
- How and when will we be told about the trial's results?
- Will we be told about changes in the study that might make us want to quit the study?
- When do we need to decide about joining this trial?
- Who can answer questions before, during, and after the trial?
- Who will be in charge of care?

### Benefits and Risks

- What are possible benefits?
- What are possible side effects or risks?
- How do possible risks and benefits of this trial compare with those of standard treatment?
- If my child receives a placebo, could that be harmful to his or her care?

### Privacy and Rights

- Will I have access to my child's test results throughout the trial?
- How will my child's health information be kept private? Who will have access to my child's information?
- What happens if I want to take my child out of the trial?

### Costs

- Will I have to pay for any of the treatments or tests?
- What costs will my health insurance cover?
- Who pays the medical bills if my child is injured in the trial?
- Who can help answer questions from my insurance company?
- Are any personal costs such as meals or gas paid for by the organization or individual running the clinical trial?

## Words to Know

The following medical and technical words are used by researchers and

other professionals involved with clinical trials.

**assent**—When a child agrees to be in a study.

**blinding or masking**—When participants and usually study investigators do not know which medicine or treatment participants are getting until the end of the trial period.

**informed consent**—Parents' permission for their child to join a research study after they have read an informed consent form, spoken with the investigators, read other materials about the study, and asked questions.

**interventional study**—Another name for a clinical trial.

**investigators**—People doing the research study. People on the research team may include doctors, nurses, research coordinators, social workers, and other health care professionals. Each clinical study is led by a principal investigator, who is often a medical doctor.

**Institutional Review Board (IRB)**—An independent committee who ensures that clinical trials are ethical and that the rights of the people in the study are protected. Each clinical trial in the United States must be approved and monitored by an IRB. Members of an IRB include doctors, statisticians, and members of the community.

**participants or subjects**—Volunteers who enroll in a research study.

**placebo**—A pill, liquid, or powder without active medicine in it. Placebos are given to children only when withholding treatment poses minimal risks.

**protocol**—Detailed plan of the research study.

randomization—A way to choose what treatment each person in a study gets so that there is less chance of bias. It's like flipping a coin or rolling dice—the results are random and are by chance, not choice.

### Time

- How long is the trial?
- How often and for how long will my child have to go to the hospital or clinic?
- Will my child have to stay in the hospital during the clinical trial? If so, how often and for how long?
- How much time will be spent traveling to and from the hospital or clinic?
- Will my child have checkups after the trial?

### Other Choices

- What are other treatment choices, including standard treatments?
- How does treatment my child would receive in this trial compare with other treatment choices?
- What will happen to my child's illness or disease without treatment?

## For more information

**Children and Clinical Studies**

www.childrenandclinicalstudies.org

**ClinicalTrials.gov** (a service of the US National Institutes of Health)

http://clinicaltrials.gov

**National Cancer Institute**

http://cancer.gov/clinicaltrials

**National Heart, Lung, and Blood Institute**

www.nhlbi.nih.gov/health-topics/about-clinical-trials

**National Institutes of Health**

www.nih.gov/health/clinicaltrials

**US Food and Drug Administration**

www.fda.gov/ForConsumers/ConsumerUpdates/ucm048699.htm
*(Would Your Child Benefit from a Clinical Trial?)*

**From Your Doctor**

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