Clinical Research at The Children’s Hospital of Philadelphia®

Hope lives here.

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What is clinical research?

Clinical research is an important way to learn more about human health and disease. It involves developing new ways to prevent and treat illnesses. Doctors, nurses, psychologists and other healthcare professionals conduct clinical research in adults and children. The following are a few of the many types of clinical research studies:

- Studies of new drugs or medical devices to see if they are safe and effective
- Studies to find the best doses of medications to treat children with certain illnesses
- Nursing research studies that look at ways to improve nursing care, educate the public on prevention of diseases, and teach families how to care for their child
- Studies that try to understand how children's bodies and minds work. In such studies, children and/or parents agree to be observed, complete an interview or take a medication or series of tests over a period of time
- Studies designed to figure out better ways to provide care, such as how best to administer anesthesia and pain drugs to children
- Studies in which researchers review medical information in patient charts

How does clinical research help lead the way?

Research is an important part of our mission at The Children's Hospital of Philadelphia. Clinical research at Children's Hospital and other hospitals has led to better ways of diagnosing, treating, and often curing, pediatric diseases. For example, in the 1980s research here led to the development of a vaccine that prevents a common form of meningitis. Clinical research at Children's Hospital also led the way in treatments of pediatric leukemia.

If you choose to allow your child to take part in clinical research, your child may or may not benefit from the study. But it is possible that the research may result in new and better care for children all over the world.

Is clinical research with children really necessary?

Children are not simply little adults. While most new treatments are first tested in adults, testing in children is necessary to learn if a new or existing treatment is safe and effective for children. Testing in children also helps healthcare providers figure out what dosage of medication is safe and effective for children of different sizes and ages.

Children have special developmental care needs. Learning how to help children understand and cope with tests and treatments by communicating at an age appropriate level is another important research goal.
What’s important to know?

During your visit here at Children’s Hospital, you may be asked to consider enrolling your child in a clinical research study. It takes careful thought to decide to take part in clinical research. To help you better understand clinical research, here are answers to questions parents often ask. Keep in mind that each study is different. You should ask the researcher or your child’s doctor or nurse for any additional information you think you need.

How and why would I be asked to have my child take part in clinical research?

Every clinical research study has certain criteria for the types of children needed. For example, if your child has a disease or condition that is being studied here, your child’s healthcare provider may ask if you and your child are willing to take part in a clinical research study. There may be screening tests to determine if your child is qualified to take part.

In addition, healthy children are often asked to volunteer for studies that help healthcare providers understand how a healthy child grows, develops or fights common childhood diseases. Parents and siblings may also be asked to take part in many different types of research studies.

What is involved in a research study?

Every research study is different. Some are more involved than others. Before you agree to allow your child to take part, the details of the research study will be explained to you. Some research studies are for outpatient visits and some are for children staying in the Hospital. You should fully understand all the requirements for the research study, including such things as questionnaires, blood samples, medications and any other information required by the study. Make sure you understand what is being tested, the risks and benefits, and time and involvement that may be asked of you if you join the study. Be sure to ask any questions you may have until you feel that they have been fully answered.
How does Children’s Hospital reduce risk to children taking part in research studies?

Some research studies can involve risks to the people taking part. For example, there are risks involved in testing a new drug or device, but there are also risks in taking any medication. Not all of the possible risks of a research study are known ahead of time. Because of this, there are many safeguards. Our Hospital has a special committee, called the Institutional Review Board (IRB), which applies standards issued by the federal government to all clinical research studies. The IRB is a committee made up of Hospital physicians, researchers and other health professionals, as well as parents and people from the local community who are not associated with the Hospital. Together, they review research studies to make sure that the studies follow the proper regulatory and ethical standards.

In addition to reviews conducted by the Hospital, agencies of the federal government — including the Office of Human Research Protection (OHRP) and the Food and Drug Administration (FDA) — oversee some clinical research. The FDA works to ensure that new drugs and devices are tested on people only after laboratory and animal studies. Both the FDA and OHRP set strict standards for how clinical research studies should be conducted.

What is informed consent?

No one can make you or your child take part in a study and you may always change your mind. It is important to know all the facts about the study before making your decision. Your child’s healthcare provider will explain the entire study to you and provide you with information such as:

- The goal of the study
- What is being measured or tested
- Exactly what treatments and procedures will be done
- What risks might be involved
- What other options your child may have if you do not want him/her to take part in the study
- Whether or not there may be any benefit to your child
- Whether or not any extra costs are involved

In most cases, you will also receive a consent form to read. It sums up the research study. The researchers try to make sure the consent form is written in language you can easily understand. Should you decide to take part, you will be asked to sign the form. Depending on your child’s age, he or she may be asked to sign it as well. Your signature means that you freely agree to have your child take part in the study and that you have been informed of the details. You will be given a copy of the consent form so that you can refer to it at any time during the study.
If you are not sure you understand the study, do not sign the consent form. Wait until you are satisfied that all of your questions have been answered. Take the time you need to make sure that you understand how the study could affect your child and what risks might be possible.

**Do I decide for my child?**

In most cases, parents are asked to decide about taking part in a research study and sign the consent form for their children. It is important that your child knows about the study and understands what a research study is and what is involved. Generally, children ages 7 and older are asked to provide their agreement to taking part in a clinical research study. This is called “assent.”

**What if I’m not sure I want my child to take part in a research study?**

Listen to what the research staff has to say. Get as much information as you can. Read through all the materials they give you. Feel free to consult with your child’s healthcare provider and with other medical professionals. Ask the researcher how long you have to make a decision. Remember that it’s OK to say “no” if the study is not a good match for you or your child or if you have any doubts about being in the study. Keep in mind that you can leave the research study at any time, for any reason. It will not change the medical care your child receives at The Children’s Hospital of Philadelphia or elsewhere.

**What if I change my mind?**

Being in a study is completely voluntary. You can withdraw your child at any time for any reason, even if you have already signed the consent form. Your child will still continue to receive medical care. However, some experimental treatments are not yet approved and will not be available to children who are not taking part in a study. In other cases, early withdrawal from a study may result in changes in your child’s medical or nursing care. If you want to withdraw your child from a study, you should discuss this with your child’s healthcare provider.

**What if something goes wrong during the study?**

Studies can present risks to people taking part. Sometimes things happen that were not expected and may cause harm to participants. Some studies have outside experts that review study information along the way to help decide if the study should continue. These experts have experience with unexpected risks. There is an internal review of each research study at Children’s Hospital at least once a year. Different studies have different arrangements about payment to someone who is hurt during a study. You should ask questions about those arrangements for any study you are considering.
Will we be paid for taking part?

Some research studies offer payment for the time and effort involved, but not all studies do. Payment depends on different factors, such as time, number of visits or travel. Some studies will provide payment for parking or lunch. Others may give a small gift or a gift certificate to thank participants for their time and effort. If travel is required, those expenses may also be covered.

Who pays for the study?

This depends on the study. Some studies may involve only procedures that are part of the regular care for a child with a particular condition. In those cases, you or your insurance company will usually be asked to cover the charges for that regular care, just as you would if there were no study.

Other studies involve procedures or care done only because of the research study. Usually, the study will pay those costs, but insurance companies are sometimes willing to pay the costs of certain research study procedures, as is the case with many cancer studies.

Funding for research studies may come from a drug or medical device company, a government agency such as the National Institutes of Health, a research foundation such as the America Cancer Society or the Hospital. Your child's healthcare provider can explain which procedures of the study are paid for and which are not. Be sure to ask the healthcare provider or researcher about possible costs. Contact your insurance company if you have any questions.

What happens to study results?

In most cases, the people doing the research study hope to report the results to the medical community. Study results are usually reported without identifying participants by name. However, in many cases the organizations funding the study or government agencies have the right to review study records that do identify the participants. Please ask about who can review study records in any study you are considering.

How can I find out more?

Talk to your child's healthcare provider to get information about specific clinical research studies being conducted at The Children’s Hospital of Philadelphia. You and your child can make a difference in advancing pediatric healthcare. Be sure to make the most informed decision, should you be asked to take part in a research study.
Keep this checklist handy. Should you be asked to be in a clinical research study, these are some of the questions you might want to ask about the research and your child’s participation.

Some possible questions about the background of the research study include:

- Why is this research being done?
- What is the purpose of the study?
- Who has reviewed and approved this study?
- Why does the research team think the treatment, drug or medical device should be tested?
- Are other organizations taking part in this research with Children’s Hospital?

Some possible questions about your child’s participation in the research study include:

- Where will the study be performed?
- What kinds of therapies, procedures, and/or tests will my child have during the study?
- Will they hurt? If so, for how long? What can be done to minimize any pain?
- How would the tests, procedures and treatments in the study compare to those my child would have if he/she were not in the study?
- Have these therapies, procedures and/or tests already been approved in adults? Have they been approved by the Food and Drug Administration (FDA)?
- How long will the study last?
- How often will we have to go to the study site?
- Will my child be able to take regular medications, over-the-counter medications, herbs or food and drink during the trial?
- What medications, procedures or treatments must my child avoid while in the study?
- What are my responsibilities during the study?
- Will my child have to be admitted to the Hospital during the study? If so, how long will he/she have to stay?
- Can I stay with my child if an overnight stay is required?
- Can anyone find out if my child is taking part in a study?
- Can I talk to other people in the study?
- Will I be able to find out the results of the study?
- Who will I contact if I have a question or an emergency?

Some possible questions about risks and benefits include:

- How do the possible risks and benefits of the study compare with treatments for my child that are already being used?
- What are the possible immediate and long-term side effects?
- Will my child benefit by being a part of this research?

Other possible questions include:

- What other treatment options does my child have?
- Will I have to pay anything to have my child be in the study?
- What are the charges likely to be?
- Is my insurance likely to cover these expenses?
- What if something goes wrong?
- Will my child’s identity be disclosed in the study?
- Will medical records be available to other people?
- Will I be able to get access to my child’s medical records from the study?
Founded in 1855, The Children’s Hospital of Philadelphia is the birthplace of pediatric medicine in America. Throughout its rich history, a passionate spirit of innovation has driven this renowned institution to pursue scientific discovery, establish the highest standards of patient care and train future leaders in pediatrics. For a century and a half, Children’s Hospital has served as a haven of hope for countless children and families worldwide.

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