

Getting Started

The Research Team: Who's who



Video transcript –

Tasmeen Singh, MPH, NREMT-P: Who is part of a research team? Well, again, it differs by study, but you always have a principal investigator, and he or she is the person who is responsible for everything that goes on in the study. Then you may have a research nurse or a research assistant, who is responsible for the day-to-day stuff. So they're the ones who might be scheduling your appointments, or coming to do the blood draws, or calling you to check up on you, or maybe checking your diaries or checking your surveys, if you were asked to fill something out.

Victoria Pemberton, RNC: But oftentimes, the study offers a study coordinator or a research nurse, who really tends to be the pivotal point for that family, the contact person, the "go to" person, the "What do I do if my baby doesn't eat" person. "What happens when he spits out the study drug because he didn't want to swallow it today? Do I give another dose? Do I not?" And so generally, the study coordinators are the nurses that assist in the studies, are there to help reassure families, talk them through the bad times, be there to support them through the better times.

David Wendler, PhD: One of the crucial things is just to get to know those people, make sure that you're comfortable with those people, with the investigators and the research team.

Jill: I feel very comfortable with them, and I always feel like I can ask a question if I need to. And I have a phone number; I can even call them on the phone.

Nicole: The administration and the nurses, they were always there. I would call them for the simplest questions.

Britt: It helps you out as far as not worrying as much about everything, knowing that you've got somebody that you can pick up the phone and call.

Sherry: Susan is so warm, Bianca thinks that she's her personal nurse. And we can call her up at any time and talk to her. She'll call me any time. Whatever she had to ask me, if we had something to ask right there, she would be there. They are so supportive. We couldn't have a better team.

Every clinical study has its own research team...

It's made up of doctors, nurses, research coordinators and scientists, pharmacists, and developmental specialists, to name a few.

The most important members? Children and their parents.

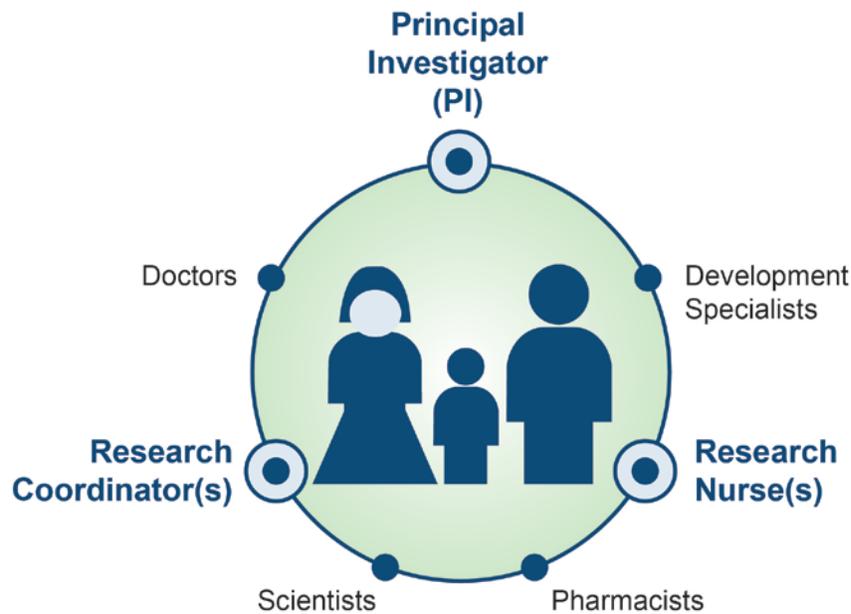
The medical and research teams make sure that your child is eligible to join a study, give instructions and monitor your child during the study. Not just anyone who is a nurse or doctor can be on the research team. In addition to their medical training they must take special classes and are highly trained in how to protect participants and conduct studies in an ethical, legal and safe way.

They are prepared to answer any and all of your questions...and always respect the parent's role as the one who knows your child best.

"Not only do you get to speak up, you should speak up. And in fact, this is one of both the rights and responsibilities, I would say, of parents..."

Dr. Gail Pearson, Pediatric Cardiologist

Parents are at the center of any research team.



Some studies have large teams and others are small with one or two individuals managing the study.

The **Principal Investigator (PI)** is often a medical doctor and is responsible for the entire study. He or she oversees the care of all the participants while they are in the study. The PI can sometimes be your child's regular doctor.

The **Research Nurse(s)** are responsible for patient recruitment. They also assess eligibility and, along with the study doctor, provide treatment. Part of their role is to collect data, and follow up with participants.

The **Research Coordinator(s)** make sure the study runs smoothly. They understand all parts of the study and make sure the staff has the right equipment, medication or devices needed to run the study.

Parents should be comfortable and familiar with the study team. Know who to call for general questions, and who to call in an emergency.

What happens if you run out of study drug? Who do you contact about side effects? What if it is 2 AM?

"Parents are in charge of their child's wellbeing. I mean, you have to take control."

Jackie, mother of child in Fabry disease study

"...talking to the nurses and finding more information, that made me more comfortable. And also the team itself, the nurses, the assistants."

Nicole, mother of child in heart defect study

There are many people involved in a research team that a parent may not talk to but who play a crucial role. **Institutional review boards**, study sponsors, oversight committees and federal regulators often interact with study teams during the study.

Siblings and other family members are also part of the team since they may be affected by the requirements of a study and may have special concerns.

"...the family can be very affected by enrolling into a clinical trial...it becomes what we call a family activity."

Victoria Pemberton, RN, Research Nurse

Good Questions to Ask



Video transcript –

Gail Pearson, MD: A way to become more comfortable with the process, I think, is to ask questions, and keep asking them until you get an answer that you like. And if you don't understand, ask again. Ask them to explain it again.

Victoria Pemberton, RNC: What is the time commitment? How many visits are there? Will I get parking? Do you have childcare available? What if I'm here all day? Are there meals that are provided? These are some of the more practical, simple questions that parents can ask, aside from the perhaps more important questions: What are the risks of being in a study? How long will it last? How adequately has this treatment been studied in other children before my child enrolls? Who's running the study?

Gail Pearson, MD: Not only do you get to speak up; you should speak up. And in fact, this is one of the both rights and responsibilities, I would say, of parents who are considering enrolling their child in a study. Nothing should ever be done that you're not comfortable with.

Sherry: I remember when I got this book, the FSGS book. The doctor wrote his cell number on the back of the book. "Call me any time," he said. And there was occasion when I had to call him. Bianca was having cramps, and she said, "I want to speak to my doctor." I am her registered nurse, but Bianca wanted to speak with her doctor. And I had to get him on the phone so that she could ask him, "Why am I having cramps?" She wanted to hear it from him. And he was able to talk to her and smooth away her worries.

It is difficult enough to decide to enroll in a study as an adult, but it is even harder to make that decision for a child, especially if the child is sick.

"As a parent you need to do your part...fear should not really control your actions. So you should be...proactive."

Jackie, mother of child in Fabry disease study

Clinical study documents have a lot of information, but there are questions that you may still have. And you need to ask them...**and ask them again** if the answers aren't clear to you.

There are many precautions in place to protect children in clinical studies. Safety is the most common concern for parents, along with what risks and benefits they can expect. But there are many other factors involved when joining a study, which parents need to look at and ask questions about. And sometimes when you ask a question, it might cause the research team to think about how to make the study better or easier for you and others. Don't forget, the research team will expect you to ask! Here are some questions:

- What will happen and how much time will it take?
- How will it affect other family members?
- Are there costs?
- How do I know what questions to ask?
- What if I have questions during the process?

"Do research, ask questions, tons of questions. Even if it could be like the silliest questions ever...Knowing is knowledge."

Nicole, mother of child in heart defect study

Enrolling in a study is a difficult decision. And the study team is there to answer any questions you have. Feeling comfortable that you can always ask questions should be a key part of the decision process. Before you make your decision, ask questions. Ask them again. Get the answers you need to feel that the decision you make is the right one for you...and your child.

"...just getting a comfortable relationship with the researchers is very important...that's an important part of the protections."

Dr. David Wendler, Bioethicist

There is a lot of information that is provided when thinking about enrolling in a clinical study, but you may have more questions. Here is list of possible questions you might want to consider asking your research team. Remember that you should ask questions until you are comfortable with the information provided.

For a printable version of these questions, go to
http://www.nhlbi.nih.gov/childrenandclinicalstudies/downloads/english/pdf/questions_to_ask.pdf.

The Study

1. Why is the study being done?
2. Why do researchers think the approach may be effective?
3. Who is paying for and supporting the study?
4. Who has reviewed and approved the study?
5. How are study results and safety of participants being checked?
6. How long will the study last?
7. What will our responsibilities be if we participate?

Possible Risks and Benefits

1. What are the possible benefits?
2. What are the short-term risks, such as side effects?
3. What are the possible long-term risks?
4. What other options do people with similar conditions have?
5. How do the possible risks and benefits of this trial compare with other options?

Participation and Care

1. What kinds of therapies, procedures or tests will my child have during the trial?
2. Will they hurt, and if so, for how long?
3. How do the tests in the study compare with those my child would have outside of the trial?
4. Will my child be able to take his or her regular medications while in the clinical trial?
5. Where will my child have his or her medical care?
6. Who will be in charge of my child's care?

Personal Issues

1. How could being in this study affect the daily life of my child or my family?
2. Can I talk to other people in the study?

Cost Issues

1. Will I have to pay for any part of the trial such as tests or the study drug?
2. If so, what will the charges likely be?
3. If I have health insurance, what is it likely to cover?
4. Who can help answer any questions from my insurance company or health plan?
5. Will there be any travel or child care costs while my child is in the trial?

Tips for Asking your Doctor About Trials

When you talk with your doctor or members of the research team:

1. Consider taking a family member or friend along, for support and for help in asking questions or recording answers.
2. Plan ahead what to ask - but don't hesitate to ask any new questions you think of while you're there.
3. Write down your questions in advance, to make sure you remember to ask them all.
4. Write down the answers, so that you can review them whenever you want.

Source: Modified from NCI/NIH,

<http://www.cancer.gov/clinicaltrials/learning/questions-to-ask-about-participating>

The Cost of Participation



Video transcript –

Tasmeen Singh, MPH, NREMT-P: A lot of parents are worried about what happens with the costs that they might incur if they have to come back for appointments, like cab fare or Metro fare. Or what about babysitting for other kids, if they have to come back to the hospital? Those types of things, you really should discuss with the research team before you sign up for the study. Usually in the informed consent document there's a section called Compensation. And under Compensation, they will tell you how much money they will give you to participate in the study. Usually the compensation is designed to help parents with the costs that they may incur while coming to the hospital or to the clinic for their appointments, or for babysitting and other things that they may need to pay for. But they are only estimating how much those costs are going to be. So if you think that it's going to cost you more money to come to the hospital, you need to talk to the research team ahead of time, and see if they can reimburse you for those expenses. In some cases they may, in some cases they may not. But that's something that you should think about right up front, and talk to the research team to make sure that you're not incurring any expenses. Sharda: They would pay our transportation to get back and forth from there. So they were really good with that as well, because it was expensive to go to the hospital, so they helped us out as much as they can.

So you may want to enroll your child in a clinical study.

You have asked all the questions and understand what will be involved. You've met the team and feel comfortable about what you are asked to do and the amount of time it will take.

But you may still have questions about cost. Some information may be in the **informed consent form**, but it is always good to ask:

- Will I have to pay for travel (gas, bus, subway or cab fare) and parking?
- What if I need a sitter for my other children?
- 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, if I have it?

These are practical questions. But they are as important as the study facts that you need to know.

Be sure to ask!

"...you need to talk to the research team ahead of time and see if they can reimburse you for [certain] expenses. In some cases they may; in some cases they may not. But that's something that you should think about right up front, and talk to the research team..."

Tasmeen Singh, Research Coordinator

Find out whether there are costs when you get a study medication and what happens when a study ends - do you need to pay for medicines, for instance? There is no single answer because it depends a lot on the study. But getting the details is the best way to make sure that your participation in a study is successful.

"...the best thing is to try to get the information as early on as you can, in making decisions for your children."

Dr. David Wendler, Clinical Bioethicist

The study team will want to make it as easy as possible by working with you to do things like scheduling study visits at times when you will already be seeing the doctor. There may be other things that can make it convenient, so it is important to ask.

*"In some cases, I've taken my crew and my equipment and gone to their home in the evening.
So we can make it a little bit more convenient for them."*

Victoria Pemberton, RN, Research Nurse

Do Children Get to Consent ?

Your child's role

Parents have to give legal consent for their child to join a research study, in almost all cases. So you've read the information and asked the questions of the study team. You think you might want to enroll your child. Now it's time to think about how your child feels about being in a study.

"...you might also want to involve your child in the decision as well. That's going to depend a lot on what sort of disease your child has, how sick your child is, how old your child is."

Dr. David Wendler, Clinical Bioethicist

- At what age should you ask a child if they want to enroll?
- What if your child feels differently than you about enrolling?
- How do you reach an agreement about what is best?

There is a process called "**assent**." In most cases, this means that children are given basic facts about a research study and are asked to be part of the decision. Children can be asked to give assent from as young as six or seven. Sometimes they can be older or, depending on the study, assent may not be required.

"...well [I thought], if my parents say yes then I'll say yes. But my parents were like, 'Well, it really is your decision!'"

Bianca, child in kidney disease study

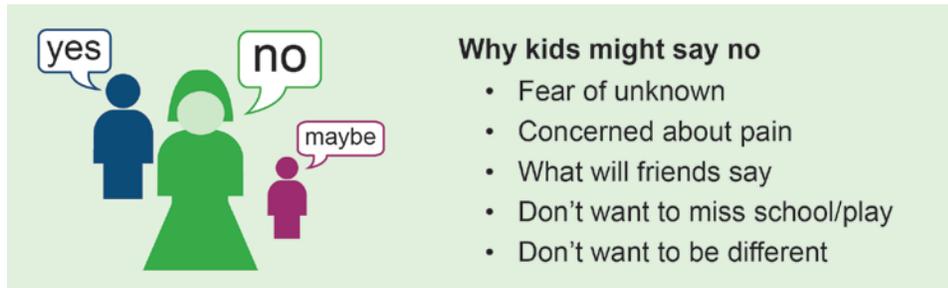
All kids are different.

Some kids may want to be part of the process, others may not. Some may be uncertain or fearful. Others may wonder about pain...or how it will affect school and friends. Some children may be too young to be involved while others can understand as an adult would.

Kids as young as 2 or 3 won't be involved in the decision process, but when children get to 14 or 15, data suggest they understand a lot about the process. That leaves a group of children in between that understand at different levels...some may understand very little, while others focus on

what is going to happen to them. At any age, the important thing is that they are comfortable and their questions are answered.

What seems to be true for all kids, though, is that their input should be valued.



"It was my choice completely whether I wanted to do it or not. I had to- I read over exactly what the disease was. Actually I think I do that every now and then too, just to make sure I want to stay on it."

Sawyer, child in Fabry disease study

Sometimes a parent and child can't agree. But often disagreements can be worked out with the help of the study team. In fact, there are advocates and ethics experts involved in most studies who can help with just these situations.

It's about talking...you and your child. And remember that the study team is there to help too.

"...you know, they're smart kids. If they don't want to participate they can really deep-six the whole thing in terms of their level of cooperation. It also isn't respectful of them. I mean, they need to be able to have it explained to them, and see if it's something they really want to commit themselves to."

Dr. Renee Jenkins, Pediatrician, American Academy of Pediatrics President, 2007-2008