

Once in a Study

The Effect on the Family



Video transcript –

David Wendler, PhD: Some studies can be very short, and there's very few, very low demands on both the child and the parent. Other studies can take years. They can involve having to come back to a clinic every month, maybe even every week. Some studies involve children having to stay in the hospital for a couple of days, even a week at a time, while they're getting a medication, so that the research team can keep an eye on them to see how they're doing, to see how the medication is affecting them. Now obviously, those kinds of studies can have a lot more burden on a parent. Particularly when it's a younger child, the parent wants to stay with the child, and also when they're away from home.

Victoria Pemberton, RNC: The family can be very affected by enrolling into a clinical trial. It becomes what we call a family activity. The mom probably has a lot of other things to do, outside of the illness itself. Maybe she's been asked to keep medication diaries, or diaries about side effects of some medications that her child is on in a study. She may miss work. She may need to find childcare. Daddy may have to fill in, if at all possible, to help her.

Sherry: I had to make sure that she took the medication every single day, and then to ask her how she was feeling so I can make some documentations, if she had swelling, if she had pain. And sometimes she had irritability. She was very irritable. And pain also. And that was hard. But the drive in, her father helped with that. And appointments, we had no problem.

Gail Pearson, MD: A big concern is the effect on other siblings. So already there's an effect, particularly if a child has a chronic condition and requires just a great deal more attention. And other siblings may or may not get the attention they think they want or need. Add to that

participation in clinical research, an additional time sink for the family. And this becomes perhaps even more of a concern. On the other hand, I think the family can embrace the idea that their sister or brother or daughter or son is participating in something that may not have a benefit for them but may have a benefit for other children with a similar condition.

Having a child in a clinical study can affect the whole family. Parents must think about the amount of time that will be required, the emotional impact on the family and how to give and get support when their child is in a study.

Time

The time and the number of demands on the family can vary widely between studies.

- A lot of studies simply ask a child a few questions after they get a medication or treatment which could take only a few minutes.
- Some studies can be short with few demands like one extra blood draw.
- Other studies can take years. They may have repeat visits - weekly or monthly. They may require a hospital stay. Those studies can have a bigger time burden than others.

The time commitment and effect on the family depends a lot on the nature and length of the study, the age of the child and the condition. It is important that you understand what will be asked of you and your child.

"It's just a couple of extra trips down to the hospital...But it really just involved a matter of a few hours time."

Jose, father of child in heart defect study

"...it takes a lot of planning and...getting things lined up where you can take off to come up here and do things you need to do. You just make plans ahead of time and make the arrangements you need to make..."

Britt, parent of child in chronic granulomatous disease study

You may want to ask:

- Will I need to take time off of work?

- Will any overnight stays be required?
- Will we need to do any long-distance travel?
- How much time will I spend at home keeping diaries, answering surveys or giving medicines and doing procedures for the study?
- How much time will any procedures require?

Emotional Impact

Being in a study can require effort and understanding from every member of the family...and may take time away from other children and normal family activities. Added to this is the emotional stress of realizing that your child may be sick...may, for a time anyway, not be like other kids.

"...it's devastating again at the beginning. Everybody had their tears to shed. But it's brought everybody closer. Everybody knows everything. Family's a good- a good support."

Dawn, mother of child in chronic granulomatous disease study

"...within the space of an hour or two, we may be talking about some very difficult decisions that a family needs to make...if there is a research protocol, getting engaged in that..."

Dr. Joe Wright, Pediatrician

You may want to ask:

- How much information should I give to my child about the study and what will happen?
- How can my family cope with being in a study especially if my child is sick?
- Who can care for my other children during study visits or overnight stays?
- How will I manage my family and work responsibilities if we are in a study?
- Will my child be able to participate in regular school and social activities?

Giving and Getting Support

Seeking out help and sharing the burden with trusted family, friends, teachers, and community members can help parents and children throughout a study.

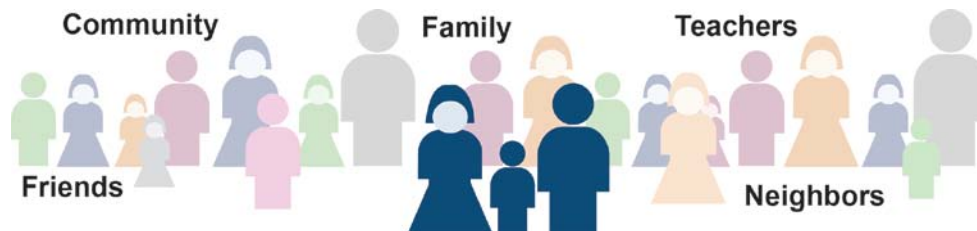
"Get to know your child's teacher. Let the teacher know that your child is on a certain medication...She has special needs and this is why she's carrying this whole bag of medication."

Sherry, mother of child in kidney disease study

Study teams are part of that network too, and have resources to help with the stress of having a sick child or with the additional activities you may have been asked to do. Talk to your research team for help.

You may want to ask:

- How can I support my child and my other children during a study?
- Who can help support me during this time?
- Who can help me make decisions?
- Are there support groups, other parents or social workers I can speak with?
- How can the study team make it easier for us to join and stay in the study?



What Children Say about Being in a Study



Video transcript –

Sharda: She was always comfortable whenever she went to the doctor. And she herself would ask questions as to what you're going to do; why you're going to do; is it going to hurt; do it easy; I want a tie like this; I don't want you to click the needle. So she was always comfortable herself. So once she was comfortable, I was comfortable.

Gail Pearson, MD: It's amazing what they can understand and what questions they have about how something is going to affect them. Mostly they want to know if it's going to hurt or not. That's a big concern. But they also are able to think about how this might affect their life.

David Wendler, MD: Then once you finally ask the children, they love being asked the questions. These are things they had been thinking about anyway. Having the opportunity to think about them is useful for them, being able to interact with adults, being taken seriously by researchers.

Sawyer: Actually it has affected a lot of stuff. Like I've missed some stuff, but I've been willing to do it because I think it would be good if I kept going. And it also helps me a lot, and it's going to be worth it in the long run, because I'm helping myself and future generation and people who have the disease now. So if we can get this medicine approved fully and so it's open to everybody, that'd be great.

Caroline: Sometimes you have to go to the doctor. And sometimes we have to go to a hospital, and I have to wear like a holter monitor to like check my heart and make sure everything is okay. People are like, "How do you swallow those big pills?" And I'm like, "They're not big. I just swallow them. I don't even need like a drink or anything. I just swallow them."

Zach: We get up there, we get our blood drawn, and then after that we'd go to CT scans. Then we'd get our height and weight checked, and then we'd just talk to the doctors about what's

been going on. Has anything been wrong? Stomach been hurting? Anything like that. And they'll just check your heart and stuff. That's pretty much it.

Parents often try to determine what will be difficult, or what won't be, for their child. But sometimes parents and researchers get it wrong.

Depending on age and maturity, many children have a great ability to understand basic facts about a clinical study...and often want to have a say. Parents should always ask questions and get the details they need, but it is important to talk with your child and let him or her ask questions too.

But what *do* kids say about being in a study?

"So I guess the reason why I was so comfortable with it because they told me everything off the bat and they didn't keep me in the dark about anything."

Bianca, child in kidney disease study

"And I chose to because I wanted to do pretty much anything I could to help and also help with the study to help for future generations as well."

Sawyer, child in Fabry disease study

While parents and doctors may focus on drugs, or needles, or asking difficult questions, kids may be worried about staying overnight, how much school they will miss or if there are other kinds of testing or physical exams. There may be things that scare your child about being in a study. Ask your child to talk with you about them and answer as honestly as you can. Across the board, children want very much to be able to ask their own questions...and feel at ease with the answers.

"You bet they (kids) get to ask questions! You're surprised about the number of questions they ask...And you have to be ready to answer those questions honestly."

William Rodriguez, Pediatrician

"Bianca was having cramps and she said, I want to speak to my doctor. You know I'm a registered nurse but Bianca wanted to speak with 'her' doctor!"

Sherry, mother of child in kidney disease study

The point is, kids get it.

And because they do, it's important to listen to their concerns and questions...to give them a voice. One thing researchers have found is that often children in clinical studies were more willing and more interested in participating in future research to help other kids. They understand that it is a charitable activity.

Whatever their point of view is, **find out**. More than likely, they'll have a lot to say.

Leaving a Study



Video transcript –

Tasmeen Singh, MPH, NREMT-P: You can stop at any point. This is true of any research study that's done under federal regulations. The parents have the right to withdraw at any time.

Gail Pearson, MD: I think people worry about making the doctors mad, or making the staff mad, if they withdraw from the study. It would be disappointing, but it in no way affects the care that they're going to get before or after.

Victoria Pemberton, RNC: And when parents tell us that they want to withdraw from a study, we really try to sit down with them and find out what are the reasons. Are there things that we could do to change, to make it easier for them to participate? It may be a convenience issue. "I just got a new job. I can no longer make these study visits."

Tasmeen Singh, MPH, NREMT-P: So what you do is, you call the contact person that you've been dealing with, or you look on the informed consent document and see where it says who to contact for questions or comments, and you tell them, "I want to withdraw my child from participation," or "I don't want to participate anymore." And you can tell them why.

Victoria Pemberton, RNC: And why we would want parents to talk to us before they just withdraw and disappear from a study, is that oftentimes children need to be placed on a medication, or the medication that they're on needs to be down-titrated, if you will, or the dose lowered over a period of time, so that it's safe. Sometimes stopping a medication abruptly could be harmful to a child. So there are things that we would want to discuss with the family. But certainly we want to reassure them that if they felt uncomfortable and felt they needed to leave a study, that is their right.

If your child is in a study and you feel you need to leave, **that is your right**. You may leave the study at any time, for any reason. You will still get your regular care, and no one will be upset with your decision. The decision to withdraw may be hard; it may come from the parents or it may come from the child, but like every other part of a study, parents have control and can make the decision to withdraw if they feel that is best.

"Actually I think I do that every now and then too [read the study materials], just to make sure I want to stay on it. And I can get off of it whenever I want, which is actually a really good deal."

Sawyer, child in Fabry disease study

Talk with the team

Remember to talk with the research team if you want to leave a study.

Maybe you are moving to another state and think you cannot attend the study visits. The research team may have a solution that would allow your child to continue in the study. And if you must leave the study, the team will want to make sure that your child is safe when you do so.

Depending on the study, leaving can be very simple...with few requirements and easy transfer back to regular care. But in other cases, it may mean that your child has to be monitored until they are safely off of a study drug or until regular care can be resumed.

"...in some cases, if your child got a drug or something else, and they'd like to continue monitoring your child because they just want to make sure that he or she will be okay, it's probably a good idea to let them do that."

Tasmeen Singh, Research Coordinator

There are some important things to consider when thinking about withdrawing from a study - and, as with every part of a study, it is important to **ask questions**.

Here are some things you should consider finding out:

- After we leave the study what will happen to my child's study data?
- Will my child be able to get the medication or treatment he or she was taking in the study?

- What should I do with materials I was given for the study?
- Can I still contact the study team?
- What happens if my child's care is being paid by the study?
- Will my doctor get the study health records?

Remember: While it is absolutely okay to withdraw, make sure you know what happens next. When you leave a study, there are many pieces that may need to be addressed for the safety of your child.



When a Study Ends



Video transcript –

David Wendler, PhD: Parents probably need to be very proactive, because a lot of times the researchers are very focused on the study, and sometimes their horizon ends when the study ends, and they're not thinking as much about transitioning families back to regular medical care as they should be.

Victoria Pemberton, RNC: Sometimes patients will enter a study, and when their participation is finished, they may think that the medication was really working for their child. And we all have to bear in mind that one of the reasons that we do clinical research-in anyone-is that we may not have the answer as to which treatment is better. So we do need to look at that until the very end of the study. And once they were finished with the study, and we were able to see if there was beneficial effect, we would then notify the families.

David Wendler, PhD: It's one of the things probably that the parents need to be very aware about. They need to try to be very clear on how long the study lasts, when it ends, and then when they're getting towards the end, trying to work with the researchers and also the physicians at home to try to get as seamless of a transition as they can, getting medical records back to the home doctors, making sure the doctors at home know what medications-if it was a medication study-what medications the child was on as part of the study, what did that medication do, how might it interact with new medications the child is going to get.

The study is about to end...you've been involved for a few weeks, months, or years. Your child may be on a study drug or treatment. Perhaps the costs for additional medical care were covered by the study. Maybe your child hasn't been to their primary care doctor while they were in the study.

"They [parents] need to try to be very clear on how long the study lasts, when it ends, and then when they're getting towards the end, try to work with the researchers and also the physicians at home to try to get as seamless of a transition as they can."

Dr. David Wendler, Clinical Bioethicist

So what do you do now?

There are some important things you'll want to find out...

- Will the treatment stop?
- If it is helping, what about further treatment?
- What happens to my child's study data?
- How will our regular care team be involved?
- Will I get study results? When and how?
- Will our regular doctor get our medical records?
- Do my regular doctors know what medication my child was on?

Who do you ask?

Just as much as the study team was there to help you enroll, they are there to help you when the study ends. Safety and helping you to get back to your regular healthcare, after you have left the study or the study has ended, is very important to the study team. It is important to them that your child remains safe and they will help you to transition off the study. This may mean weaning off of a medicine, helping you get back to your regular healthcare provider or continuing to monitor your child for side effects. Your study team is there to help with this process.

After the study is over

Even after a study ends, there are times when the study team may need to talk with you. Maybe they want to tell you the results of the entire study. Or maybe they want to see if you are interested in joining a new study or a study that will follow the same group of children for a longer time. Sometimes there is a question they forgot to ask you during the study or they may want to discuss a medical or study matter with you. You may also find that you would like to speak with a member

of the study team or visit them when your child comes for regular visits. Most study teams will tell you that they become very attached to the parents and children who were in their studies and really enjoy seeing them after the study ends.

"We do become bonded with our families. We have sometimes had reunions and followed patients for 10, 15, and 20 years. And I think there is a special connection that is created and exists between the study team and the families."

Victoria Pemberton, RN, Research Nurse