

## FOCAS Trial Recruitment Video Script

### Introduction Video

Blood vessels called arteries bring oxygen and nutrients to the brain. If an artery to the brain is narrowed or blocked, then part of the brain does not receive the blood it needs. That can cause permanent brain injury, called a stroke.

Although strokes are much more common in adults, they can happen in children, even previously healthy children.

Strokes in children can be caused by diseases or injury of the arteries to the brain, or by blood clots in the heart that travel to the brain and block an artery. Either way, part of the brain does not receive enough blood, which can cause a stroke.

In otherwise healthy children, the most common cause of a stroke is a rare condition called “focal cerebral arteriopathy,” or FCA. In FCA, the wall of an artery in the brain becomes inflamed. As the inflammation gets worse over days, the artery becomes more and more narrow, making it harder for the blood to get to the brain.

Pediatric stroke doctors commonly treat FCA with high doses of steroids, which help calm the inflammation. But they do not know the *best time* to start the steroid treatment because there have been no clinical trials of FCA treatment.

Doctors know a child has FCA when they see arteries get more narrow over a matter of days. Before that, they are often unsure of the diagnosis. The child *might* have FCA, or might have another cause of a stroke, like a blood clot that came from the heart.

This leads to two different ways that doctors currently treat FCA, each with possible pros and cons.

Sometimes doctors start steroids as soon as they think a child *might* have FCA. We will call this Option A: the “just in case” approach. If the child has FCA, it might be better to start the steroids right away and prevent the disease from getting any worse. But...if the child has another cause for their stroke, then they will have gotten high doses of steroids for no reason, and steroids can have serious side effects.

More often, doctors will wait to start steroids until they are *certain* the child has FCA. They can be certain when the disease gets worse, meaning the artery gets narrower, within about 3 to 7 days. We will call this Option B: the “wait and see” approach. This way, only children with FCA get treated with steroids, and no child gets it without needing it. But...doctors still worry that if they wait for the FCA gets worse, the child might have more injury to the brain.

Doctors simply don’t yet know which option is best. Give steroids “just in case” a child has FCA? Or “wait and see” if the child really has FCA before starting steroids?

To find out, researchers at more than 25 children's hospitals throughout the United States and Canada are conducting a study. The study is called FOCAS, the Focal Cerebral Arteriopathy Steroid Trial. It is paid for by the National Institutes of Health because they know how important it is to protect children from brain injury.

We are looking for volunteers to take part in the study. You and your child are being invited to participate because doctors believe *your* child might have FCA. By joining the study, you and your child will help doctors learn the best time to start steroids for future children in the same situation.

Taking part in the study is optional. It will not affect the rest of your care and does not cost any additional money.

The way the study works is that, if a family decides to participate, the child will randomly be assigned to either Option A, steroids "just in case," or Option B, "wait and see." A computer, not a doctor, will make the assignment, and the decision will be random. Like flipping a coin, the child will have the same chance of ending up in either group.

Children assigned to Option A will get started on high-dose steroids right away, without waiting to see if their arteries get worse. Children assigned to Option B will *not* get steroids right away. Doctors will follow the child's exam closely and repeat pictures of the brain within 3 to 7 days. If the child's arteries get more narrow, the doctors will start the steroids right away.

With either Option, *all children that have FCA will get treated with steroids*. The difference is that, with Option A, they will get it sooner. And with Option B, they will get them later. The children who turn out *not* to have FCA will *not* get steroids because they did not need them.

You may ask, what's the big deal about steroids? What are the downsides of a child with a stroke getting steroids, even if they do not really need them? Those are other questions that the FOCAS study will try to answer. The steroids given to treat FCA are high doses given through an IV, meaning directly into the bloodstream. High dose steroids *can* have serious side effects, like raising blood pressure, irritating the stomach, and affecting the body's ability to fight off infection. You can learn more about this in one of the later videos.

We hope this video will help you understand the FOCAS trial, and why it is so important. If you are interested in learning more, your child's doctors and the FOCAS study team at your hospital can tell you more about it. You can also watch more of our short videos that explain different parts of the study.

## Short Video Clips

**1) Why is the FOCAS study being done?** Focal cerebral arteriopathy, or FCA, is a rare condition of inflammation of certain blood vessels in the brain. When blood cannot get to the brain, it might result in a stroke, which causes permanent injury to the brain.

Pediatric stroke doctors commonly treat FCA with steroids to try to stop the progression of the disease and protect a child's brain from more injury. But they do not know the best time to start the steroid treatment because there have been no clinical trial studies of FCA treatment.

The purpose of the FOCAS trial is to compare two steroid treatment options to determine if one option is better. Option A is to start steroid treatment right away even if your child might not have FCA. Option B is to watch your child closely to see if there is any disease progression before starting steroid treatment.

**2) Why enroll in this study?** By participating in the FOCAS study, you will help doctors learn how best to treat future patients like your child.

The data from FOCAS can also potentially be used to optimize your child's clinical care. Your child may benefit from extra pictures of the brain, called MRI scans, paid for by the research study. These scans could give your doctors additional information about your child's brain and blood vessels.

In addition, the study will test for some viruses that may play a role in causing FCA. This may help you and your doctors understand why this happened to your child. Right now, doctors do not know why some healthy children develop this rare disease and participating in FOCAS can help doctors understand why.

**3) How many participants will be in this study? Who is leading the study?** About 80 children in the U.S. and Canada will participate in this multi-center research study. They will be enrolled at children's hospitals across both countries. The study is being led by a pediatric stroke neurologist at the University of California, San Francisco, and the NIH-funded stroke clinical trials network, called StrokeNet.

**4) What happens if I agree to participate in this study?** If you agree to participate in this study, your child will be randomly assigned to either treatment option A or B. A computer, not a doctor, will decide which of the two treatments to give your child. The decision will be random and due to chance alone, and not based on the patient's or doctor's decision.

Depending on the group your child is randomly assigned to, they will either start the steroid treatment right away or doctors will wait for disease progression before starting steroid treatment. An MRI machine will take pictures of your child's brain. The MRI machine looks like a big doughnut and makes loud noises. An MRI scan will be done within 5 days of stroke diagnosis

to determine disease progression. If no MRI scan is ordered by your doctors for clinical reasons, then your child will have an extra research scan done. Research scans are the same as clinical scans—the only difference is that the study pays for research scans because they are not needed for clinical care.

While your child is in the hospital, we will collect about a teaspoon of blood for the study. To avoid extra needle pokes, this sample, will be collected alongside clinical care like a needle poke for tests the doctors need.

In addition, we will assess how your child is doing while in the hospital by asking questions. If your child receives steroids, we will also give them a questionnaire to measure how well the drug is tolerated.

Your child will need to return in 1 month for a clinical assessment and MRI scan. *This is the most important assessment of the study since it will be used by the study researchers to decide which option is better.* This may be an extra, research-only MRI scan, paid for by the study, if your child’s doctor does not order this for clinical reasons. Either way, the FOCAS trial will help cover the costs of your travel and time to return at 1-month.

After the 1-month visit, we will schedule two more visits so we can perform additional assessments and questionnaires. These visits will occur at 6 and 12 months after the stroke. These visits can be completed either virtually or in-person. The 12- month visit is optional and will be completed based on clinical care guidance. We will also add some clinical information from your child’s medical chart into the FOCAS study database.

All other aspects of your child’s care will be standard and the same in either Option A or Option B. All children in the study will be treated with aspirin, which has mild blood thinning effects and is a standard medication to prevent stroke. All children will be monitored closely and given fluids through an IV to keep them well hydrated.

**5) What are the side effects of steroids?** There are different types of steroids. The ones used to treat inflammation are called “corticosteroids.” These are the steroids used to treat FCA. In FOCAS, the children who get steroids will first receive high-dose steroids through an IV, meaning directly into the blood stream, for 3 days. This will happen in the hospital setting with close observation by nurses and doctors. After that, they will take steroid pills by mouth that they can continue to take at home. Children experience more side effects from steroids when they are receiving the high doses through an IV. The side effects are less when receiving the low dose, and go away when the steroids are stopped.

The most common side effect is a change in mood or behavior. Children can become more irritable, short-tempered, moody, or tearful. The steroids can also disrupt their usual sleep, which is often already disrupted when a child is sleeping in a hospital. These are usually not serious side

effects, but can be bothersome to both the children and their families.

Other common but minor side effects are increased appetite and increased water retention. Steroids can also affect the salt and sugar balance in the blood, and doctors will monitor for this.

More serious side effects include irritation to the stomach lining, which can cause stomach pain and even stomach bleeding. However, doctors can use other medications to prevent or treat this side effect. Steroids can cause an increase in blood pressure, called hypertension. Very high blood pressures can increase the risk of bleeding into the brain in a person with a recent stroke. Doctors can use other medications to control blood pressure, if needed, and this side effect goes away when steroids are stopped.

Steroids can reduce the body's ability to fight off infection. If a child develops an infection while on steroids, they may develop a more serious infection than they would have if they were not taking steroids.

Prior reports of corticosteroids for the treatment of FCA have not described any bad side effects and suggests serious side effects are rare.

**6) Are there any benefits to participating in this study?** By participating in the FOCAS study, you will help doctors learn how best to treat future patients like your child. Information learned from the study may help us better understand when doctors should begin steroid use in children with a stroke.

The data from FOCAS can also potentially be used to optimize your child's clinical care. Your child may benefit from extra pictures of the brain paid for by the research study, that could give your doctors additional information about your child's brain and blood vessels. The study will pay for some of your time and travel expenses to return for the 1-month pictures of the brain. This may benefit you if you need to return at that time for a doctor's appointment or another test ordered by your doctor.

Your child may also benefit from the advice of the FCA experts who are the doctors leading the study. If requested by your doctors, the FCA experts will review your child's imaging and speak to your doctors confidentially about your child's case.

**7) How much time do I have to decide whether or not to participate in the study? What else should I know about the research study?** To participate in FOCAS, a child must be within 96 hours of the onset of their stroke. The study team at your hospital will let you know the deadline for enrollment based on your child's particular situation.

There are a few final things that we would like you to know about the FOCAS study.

- Someone on the study team will explain this research study to you and answer your questions.
- Whether or not you decide to participate in this study is up to you and your family.
- You can agree to participate and change your mind at any time and withdraw.
- Your decision will not be held against you.
- You can ask all the questions you want before you decide.
- You can talk about your decision to participate with anyone you would like (family members, friends, etc.) before you make your decision.
- There is no cost to you for taking part in the study.