

## What does this study involve?

Participants will be in this study for a total of 21 months. If your child participates, then he/she will

- Have a total of 15 in-person study visits
- Be given medication or a placebo (dummy medication)
- Have blood and laboratory sampling
- Have heart activity monitored
- Have a physical, neurological and eye exam
- Participate in activities involving:
  - Tests of cognitive abilities
  - Viewing pictures of people on a computer
  - Language tasks like answering questions and telling a story from a picture book
  - Interactive play sessions with study staff.
  - Brain wave test to measure sound processing

You will also be asked to participate in several activities including:

- Frequent visits over phone/video
- Language Intervention sessions
- Complete questionnaires regarding your child's language, behavior and overall function development and also their sleep habits.

## Study Locations

The study is being conducted at the following centers

- Boston Children's Hospital- Boston, MA
- Children's Health - Children's Medical Center (UTSW)- Dallas, TX
- Children's Hospital of Colorado- Denver, CO
- Children's Hospital of Pittsburgh- Pittsburgh, PA
- Children's National Medical Center- Washington D.C.
- Cincinnati Children's Hospital Medical Center- Cincinnati, OH
- Columbia University - New York Presbyterian- New York, NY
- Emory University-Atlanta, GA
- Nationwide Children's Hospital- Columbus, OH
- Rush University Medical Center- Chicago, IL
- St. Louis Children's Hospital- St. Louis, MO
- University of California- Davis, CA
- University of Kansas Medical Center- Kansas City, KS
- Vanderbilt University Medical Center- Nashville, TN



## Does your child have FRAGILE-X SYNDROME?

If yes, your child may be eligible to participate in the FX-LEARN clinical trial...

## Who is conducting the study?

The study is coordinated by NeuroNEXT, the Network for Excellence in Neuroscience Clinical Trials, with support and funding from the National Institute of Neurological Disorders and Stroke (NINDS), a division of the National Institutes of Health (NIH).

## Rush University Medical Center Contact Information



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## What is a clinical research study?

A clinical research study is a carefully designed scientific evaluation of an investigational drug conducted by doctors. Clinical research studies help to learn about how a new drug acts in the body, how it affects certain diseases or conditions, whether or not it is safe for use in people.

## What is Fragile X Syndrome (FXS)?

Fragile X syndrome is a genetic disorder, which means that your child was born with it. Fragile X syndrome is caused by damage to the FMR1 gene located on the X-chromosome. The FMR1 gene produces a protein called FMRP, which is required for brain cells to respond to messages properly.



Normal X chromosome



Fragile-X chromosome

fragile site

## Why is the FX-LEARN Study being done?

There are two purposes of this study:

- To find out if the drug AFQ056, made by the pharmaceutical company Novartis, is safe and has beneficial effects on language learning in children who have fragile X syndrome (FXS)
- Find out if a structured language intervention can help children with FXS communicate better

AFQ056 is an investigational drug that is not approved for use for the treatment of people with FXS by the U.S. Food and Drug Administration (FDA). AFQ056 is an oral (given by mouth) study drug which may be able to reduce the symptoms of fragile X syndrome by compensating for some of the function of the missing FMRP.

**Participation in a clinical research study is always voluntary!**



## Who might be eligible for participation in this study?

Your child will be eligible to participate if he/she

- ✓ Has a diagnosis of Fragile X Syndrome
- ✓ Is between 32 months – 6 years of age
- ✓ Is able to swallow a liquid medication

The study coordinator will provide you a full list of requirements for participation

**Will I be paid for to participate in the study?**

You will receive \$34 for each study visit that you and your child attend and up to \$510 if you and your child attend all study visits.

**Study procedures come at no cost to the family!**

For more information, visit  
<https://clinicaltrials.gov/show/NCT02920892>  
<http://www.neuronext.org>

