

## Study procedures come at no cost to the family

Clinical research studies like this one provide information to determine whether investigational study medications are safe and effective. Participation in a clinical study does not guarantee a benefit to the study participant.

All clinical study participants will receive thorough study-related physical, psychological and behavioral evaluations by a study physician and psychologist.

In addition, the research team will monitor each study participant throughout the course of the study. All study-related clinical care is provided at no cost.

While there is no guaranteed benefit to anyone who participates in a clinical research study, participation may help to advance scientific knowledge in the area that is being studied.



## About the Study Sponsor

This study is sponsored by Neuren Pharmaceuticals Limited, a public biopharmaceutical company focusing on the development of new therapies for brain injury, neurodevelopmental and neurodegenerative disorders.

Current trials target chronic conditions such as Fragile X and Rett syndromes as well as acute neurological injuries.

neuren

pharmaceuticals

[www.neurenpharma.com](http://www.neurenpharma.com)

## FOR MORE INFORMATION

visit [www.fragilexstudy.org](http://www.fragilexstudy.org)

or

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DOES SOMEONE YOU KNOW HAVE

# Fragile X Syndrome?

If so, he may be eligible to participate in a research study



## Researchers want to learn about an investigational medication for Fragile X Syndrome

Fragile X syndrome is a genetically determined neurological disorder in which most affected individuals have intellectual disabilities of varying degrees and display a variety of associated psychiatric symptoms.

Treatment for the disorder focuses on the management of symptoms and supportive care.

This study investigates a novel oral medication called NNZ-2566. NNZ-2566 is a modified version of a biologically active part of Insulin-like Growth Factor ((1-3) IGF-1). IGF-1 is a protein in the human body that helps cells grow.

In the brain, IGF-1 supports the growth of brain cells and the connections between them (synapses) as well as brain function. NNZ-2566 may help improve brain function by enhancing IGF-1 levels, which may be low in people with Fragile X syndrome, and by restoring the function of certain brain cells. NNZ-2566 has an entirely novel mechanism of action relative to other drugs that have been tested for Fragile X syndrome.

The purpose of this study is to determine whether NNZ-2566 is safe and well tolerated for the treatment of Fragile X syndrome in adolescent and adult males. The study also will investigate efficacy during treatment.

The study will look at two dose levels of NNZ-2566 compared to placebo (fake medication).



## Who might be eligible for participation?

- ✓ Has a diagnosis of Fragile X syndrome
- ✓ Is a male between 12-45 years old
- ✓ Is able to swallow a liquid medication
- ✓ Has some verbal ability

## What does this study involve?

### Study participants will:

- Have study visits every 1-2 weeks for a total of 8 study visits
- Be given medication or a placebo (fake medication)
- Have blood and laboratory sampling
- Have Fragile X syndrome symptoms monitored
- Have heart activity monitored
- Have a physical exam

### Participate in three short activities:

- Do a computer-based activity testing cognitive abilities
- Do a computer-based activity involving viewing pictures
- Do a language task which involves answering questions and telling a story from a picture book

