Caring for someone with CDKL5 deficiency disorder?

Consider the GEMZ Study for children and young adults who have uncontrolled seizures from CDKL5 deficiency disorder

Introducing the GEMZ Study

Zogenix International Limited is conducting a phase 3 research study called the GEMZ Study, which will test an investigational medication called fenfluramine hydrochloride (ZX008) in those who experience frequent epileptic seizures associated with cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder (CDD). Fenfluramine hydrochloride is taken daily by mouth (orally).

The GEMZ Study is a 2-part trial that lasts up to 98 weeks (approx. 2 years) and includes up to 18 visits to the study site. During part 1, which lasts 20 weeks, participants will be randomised (like tossing a coin) to receive either fenfluramine (ZX008) or placebo, which looks like fenfluramine hydrochloride but contains no medically active ingredients. During part 2 of the study, which lasts up to 78 weeks, all participants will receive fenfluramine hydrochloride.

There are tests and procedures required for this study, some of which are part of the normal care for those with CDD, such as having vital signs (e.g., blood pressure, heart rate) taken and having physical examinations. Blood and urine samples will also be collected.

About 80 patients will be enrolled in this global study at approximately 70 study sites.

GEMZ

Who can participate in the GEMZ Study?

In order to participate, patients must:

- Be between 2 and 35 years of age
- Have a confirmed mutation in the CDKL5 gene which is thought to be causing their disease
- Have a diagnosis of CDD with epilepsy first starting in the first year of life
- Have motor and developmental delays
- Have uncontrolled seizures despite previous or current use of 2 or more anti-seizure treatments
- Currently be receiving at least 1 anti-seizure treatment
- Currently not be receiving more than 4 anti-seizure medications (excluding rescue medications)
- Have 4 or more motor seizures per week
- Have not previously been treated with Fintepla® (fenfluramine hydrochloride)

There are additional criteria that need to be met, which the study team will discuss with potential participants and their caregivers.

What are the potential risks of the GEMZ Study?

There are potential risks in taking part in any research study, including side effects from the investigational medication. Any drug has a possible risk of an allergic reaction. All participants will be monitored carefully for any side effects and allergic reactions.

What are the potential benefits of participating in the GEMZ Study?

There is no guarantee that participants will get any health benefit from being in the GEMZ Study. The results of this study may provide information about CDD and may help others in the future by helping to understand more about the disease. The data gathered from this research study may provide information to advance treatments, make new discoveries and determine if the investigational medication fenfluramine (ZX008) is well-tolerated and effective in children and young adults with CDD.

Consider the GEMZ Study

To learn more about the GEMZ Study and see if someone you care for may be able to join, please contact:

GEMZ