

What is a clinical research study?

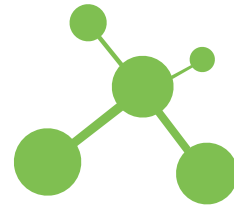
A clinical research study is a scientific study in which participants are assigned to one or more investigational drugs to answer questions about the drug to see if it works, works better than other treatments, and has side effects.

What is an investigational drug?

An investigational drug is a substance that is being tested in clinical research studies. It has been reviewed by an ethics committee for testing in people and is not approved by the government health agency for treatment for this condition.

Why should I take part in this study?

Clinical research studies are important for medical advances. Current treatments for diseases are only available because of research study volunteers.



Thank you for your interest
in this clinical research study
in cystic fibrosis.

For more information, please contact:
patient-information@eloxxpharma.com



EL-004_Patient_Brochure_v1_5Mar2020_English

Do you have cystic fibrosis (CF)?

Ask your doctor if you are eligible
for the Eloxx CF study.

PROTOCOL NUMBER: EL-004



Why is this study important?

Cystic fibrosis (CF) is a common and severe genetic disease. There are many different gene mutations that can play a role in the development of CF, and the types of mutations a person has can make a difference in which treatments work, and how well they work, in controlling CF. For people with CF who have a gene mutation called *G542X*, there are currently few treatment options available, and they are not very effective. Researchers continue to test different drugs for these people who are in need of additional and more effective therapies.

Pharmaceutical companies use clinical research studies like this one to learn more about investigational drugs before they are made available to the public. Study volunteers can help Eloxx in this important research. Thank you for considering participation in this trial.

What is the purpose of this study?

In this study, researchers are testing a possible treatment for CF in people with a *G542X* mutation. They want to find out if the investigational drug ELX-02 is safe, how much of it a person can take (the “dose level”) without experiencing too many side effects, and how it behaves inside the body.

Who can participate in this study?

To be eligible for this study, you must be:

- At least 18 years old
- Diagnosed with CF with a *G542X* mutation

This is not a complete list of study requirements. The study doctor will review the full requirements for this study with you.

How long will the study last?

Participation in this study is expected to last for a minimum of 15 weeks, including a 6-week screening period, a 5-week treatment period, and a 4-week follow-up period. The time interval between treatment periods will be minimized as possible.

What can I expect if I decide to participate?

If you agree to join this study and are eligible to be enrolled, you will receive the investigational drug ELX-02 for a total of 5 weeks. The dose level of ELX-02 will increase each week for the first three weeks. The study doctor will then determine the appropriate dose for you to take for the remaining 2 weeks based on the severity of any side effects you experienced during the first 3 weeks of study treatment.

You will take ELX-02 as an injection beneath the surface of the skin. This is called a subcutaneous injection. At your first study center visit, you will be trained on how to give yourself the injection. Your caregiver/family member can also be trained to give the injection instead of giving to yourself. You will take some of the injections at home, and some at the study clinic.

Throughout this study, you will be scheduled for 15 visits to the study clinic (including the screening and follow-up visits). Lab tests, a physical exam, and other assessments and questionnaires will be conducted at the in-clinic visits. Not all activities will occur at every visit.

What are my costs to take part in this study?

You do not have to pay for the investigational drug, study supplies, study visits, or tests that are part of this clinical research study.

What risks are involved if I decide to participate?

There are possible risks involved with any clinical research study. Your study doctor will review the risks with you, and you will be closely monitored throughout the study.

