ARCTURUS therapeutics

FREQUENTLY ASKED QUESTIONS

ARCT-810-04 STUDY

NCT06488313: Evaluate the safety and pharmacodynamics of multiple doses of ARCT-810 in adolescent and adult participants with OTC deficiency.

1. What is the ARCT-810-04 study?

The ARCT-810-04 study is a Phase 2a clinical study of an investigational mRNA drug called ARCT-810 for the treatment of OTC deficiency. The ARCT-810-04 study evaluates the safety of different dosing levels of ARCT-810 in individuals with OTC deficiency and explores its efficacy in restoring urea cycle function with multiple tests of activity.

2. What is ARCT-810?

ARCT-810 is an investigational mRNA treatment developed to target the root cause of OTC deficiency. The treatment consists of a lipid nanoparticle (LNP) and mRNA. The mRNA in ARCT-810 is designed to act as a template for making the OTC enzyme to help remove excess ammonia from the body.

3. How is ARCT-810 different from other therapies available on the market for OTC?

Treatment options available currently for OTC are ammonia scavengers, which help remove extra ammonia from the body. ARCT-810 is different in that it focuses on addressing the root cause of OTC, by providing a template for making the OTC enzyme to remove excess ammonia from the body.

4. What is different about mRNA therapy from gene therapy?

mRNA therapy does not change an individual's genetic makeup or cause any lasting genetic changes. Unlike DNA used in gene therapies, mRNA is a temporary "transcript" created from DNA that is broken down by your body over time.

5. How is ARCT-810 administered and how long does it take?

ARCT-810 will be given through intravenous (IV) infusion - a small tube inserted by a needle will deliver a slow 'drip' of study medication into a vein in the arm. The infusion will last approximately 2-3 hours.

6. Can I participate if I live outside of the USA?

Arcturus does not require that a person be a citizen or resident of the country where the trial site they enroll in is located. Those who qualify will receive all study related laboratory tests, medical exams, and travel at no cost.

7. What happens at a screening appointment?

You are provided with detailed information about the study and can decide at any time if you wish to continue to participate. Several tests may be performed such as vital signs, physical exam, blood work, and/or questionnaires to determine whether a person qualifies for the study. If the information provided and test results match the requirements for the study, you may be given the option to participate.

8. If I join the trial, how will my safety be monitored?

Study participants are monitored closely throughout the study by tests and assessments which measure and verify the safety of study participants. These include tests of blood (including chemistry, hematology, amino acids, and ammonia), urine, ECGs, physical examinations, and questionnaires.

9. When considering joining a clinical trial, what do I need to know?

Taking part in a clinical trial is an important choice and commitment. Speak with your doctor, family members, and the staff running the study. Consider asking some of the questions below that may be helpful in deciding whether you want to participate.

- What is the purpose of this study?
- How long will this study be?
- What tests or procedures will need to be done?
- What are the potential risks and benefits of participating in this study?
- What happens after the study is complete?

10. Who is sponsoring this study and why?

Arcturus Therapeutics is the company developing ARCT-810 and sponsoring this study to better understand how ARCT-810 affects individuals living with OTC deficiency.

11. Why should I (or my child) participate in a clinical trial?

Joining a clinical trial can be a meaningful way to benefit both yourself and others who are living with the same condition. Participation in clinical trials also allows you to:

- Play an active role in your own health journey.
- Gain access to investigational treatments before they are widely available.
- Help contribute to important research that advances therapies for your condition, potentially expanding treatments available to you and future generations.

12. Where can someone find support and information about OTC deficiency?

In addition to your doctor, you may want to seek support and resources from the following patient advocacy groups:

- Connecting Families UCD Foundation (United States)
- National UCD Foundation (United States)
- Metabolic Support UK (United Kingdom)

Scan the QR code to learn more about the ARCT-810-04 study or visit **ClinicalTrials.gov NCT06488313**

For all other questions, please contact **Community@ArcturusRx.com.**