Enhance 3 Gene Therapy for the Treatment of OTC Deficiency

Clinical Study for Patients With Late-Onset OTC Deficiency

Participate in OTC Research and Help Make an Impact.

Why are we doing this study?

DTX301 is an investigational gene therapy that is being developed for the treatment of ornithine transcarbamylase (OTC) deficiency. The purpose of this study is to determine the efficacy and confirm the safety of DTX301 in patients 12 years of age and older with late-onset OTC deficiency.

Consider participating if you:

- Are 12 years of age or older
- Have a confirmed diagnosis of late-onset OTC deficiency
- Are willing and able to comply with study procedures and requirements, including periodic inpatient hospitalizations
- Require daily treatment with ammonia scavenger(s) medication(s) and/or protein-restricted diet

What is involved?

6.5 years

Study duration:

- Up to 6.5 years
- Screening Period: up to 8 weeks
- Follow-up Period: about 4 or 5 years, depending on when you receive DTX301
  - This long-term follow-up is important for patients receiving investigational gene therapy products and consistent across gene therapy trials
- Primary Efficacy Analysis Period: 15 months

Study treatment:

- All participants will receive DTX301, either on Day 1 or at Week 64 (if still eligible).

Study visits:

- You will have lab tests, clinical assessments, and a review of your electronic study diary at inpatient clinic visits and outpatient visits throughout the study. The number of visits you will have will depend on when you receive DTX301.

What to expect:

- Infusion of study medication into your arm
- Blood and urine tests
- Physical exams and vital signs
- Cognitive tests
- Electronic diary entries and review*
- Health and disease questionnaires
- Entry and exit interviews

* Electronic daily diary entries are a key component of understanding a participant's experience. These involve **daily** entries for your diet, your OTC medication, and how you are feeling from the start of screening through approximately 2.5 years into the study.

For additional OTC study information, please contact
TrialRecruitment@Ultragenyx.com

At Ultragenyx, we are committed to bringing patients new treatments for rare and ultra-rare diseases.

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