Cyprus 2+™
Gene Therapy for the Treatment of Wilson Disease

Research Study for Investigational Wilson Disease Treatment

Your participation can shape the future of Wilson Disease treatment.

Why are we doing this study?

An investigational gene therapy called UX701 is being tested for the treatment of Wilson disease. "Investigational" means the study drug is not approved by the US Food and Drug Administration (FDA) and is still being tested in research studies.

Ultragenyx is sponsoring this global, IRB-approved study to find out if the UX701 gene therapy is a safe and effective treatment for adults with Wilson disease.

Consider participating if you:

- Have been diagnosed with Wilson disease
- Are 18 years of age or older
- Are currently on copper chelator and/or zinc therapy
- Limit your intake of high copper-containing foods

What is involved?

1 year

Stages 1 and 2

- You will receive:
  - a single intravenous infusion of either UX701 or placebo
  - oral corticosteroids or placebo for ~2 months
- 3-4 clinic visits for screening. Once eligible, 1 overnight clinic stay for dosing and then 5 clinic visits.
- 1 home health visit for screening. Once eligible, then 14 home health visits. Visits may be conducted by telephone or video.

5 years

Stage 3 (Long-Term Follow-up)

- If you received placebo in Stage 1 or 2, you will receive UX701 in Stage 3, if still eligible, followed by ~2 months of oral corticosteroids
- Ongoing clinic and home health visits to evaluate the long-term safety and efficacy of UX701

Support is provided for travel costs to clinic visits.

What to expect:

- One-time Infusion of study medication into your arm
- Lab tests (urine, stool, blood, saliva)
- Medical exams, questionnaires, and interviews
- Heart tests
- Brain imaging
- Liver tests
- Eye exams

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

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