



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

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

What to expect:



One-time Infusion of study medication into your arm



Heart tests



Brain imaging



Lab tests (urine, stool, blood, saliva)



Liver tests



Eye exams



Medical exams, questionnaires, and interviews

Support is provided for travel costs to clinic visits.

What is involved?

You will enter the study in either Stage 1 or Stage 2, depending on when you enroll.

