Multiple Ways to Participate in Wilson Disease Research Studies

Who We Are

Ultragenyx Pharmaceutical Inc. is a biopharmaceutical company working to develop new products for the treatment of rare and ultra-rare diseases. We are committed to both developing new treatments and helping advance the understanding of rare diseases.

How You Can Help

We are currently developing an investigational gene therapy (UX701) for Wilson disease. We are committed to partnering with patients, caregivers, and family members.

There are multiple ways you can help. Please consider whether one or more studies below may be a good fit for you or your loved one. All studies below are institutional review board (IRB)-approved.

	Goal	Ages	What's involved	Status
Interview Study	Better understand the experience of people living with Wilson Disease	18+	Questionnaires at home over 7 days	Enrolling now
Odyssey Study	Better understand how Wilson Disease appears and is managed medically	All	Get secure access to your medical records and contribute anonymized information from them for research	Enrolling now
Antibody Study	Better understand how many people have been exposed to a virus that is used in gene therapy	18+	1 home visit with blood draw	Enrolling soon
Clinical Survey Study	Better understand Wilson disease by clarifying measures for clinical trials	12+	1 clinic visit, 2 home visits over 35 days	Enrolling now
Gene Therapy Phase 1/2/3 Study	Evaluate an investigational gene therapy for the treatment of Wilson disease	18+	One-time gene therapy infusion with multi-year follow-up	Enrolling soon

If you are interested in learning more about how to get involved, please email PatientAdvocacy@Ultragenyx.com.