



Ultragenyx’s Wilson Disease Gene Therapy Program is NOW ENROLLING!



CYPRUS²⁺ is a randomized, double blind, placebo controlled, seamless Phase 1/2/3 study to assess safety, tolerability, and efficacy of a one-time intravenous (IV) infusion of UX701, an investigational AAV-Mediated Gene Therapy, for the treatment of Wilson Disease. Enrollment will include adults living with Wilson Disease currently being well managed on standard of care.

UX701 is an investigational gene therapy product for the treatment of Wilson Disease. This one-time intravenous administration is intended to correct the underlying defect of ATP7B and establish normal copper metabolism and balance in individuals living with Wilson Disease.

This study will be enrolling patients globally across 40+ clinical sites in the United States (University of Michigan, Northwestern University, Indiana University, etc), Canada, Latin America, The European Union, and Japan.

More information about the study, list of sites, and contact information are available at:

<https://clinicaltrials.gov/ct2/show/NCT04884815>

If you are interested in learning more about how to get involved in this program or any of the additional Wilson Disease programs listed below, please email PatientAdvocacy@ultragenyx.com

Additional Wilson Disease Studies Sponsored by Ultragenyx

	Goal	Ages	What’s involved	Status Update
CYPRUS²⁺ Gene Therapy Study	Evaluate an investigational gene therapy for the treatment of Wilson disease	18+	One-time gene therapy infusion with multi-year follow-up	Enrolling now
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	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 now
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