Prescreening Study to Identify Potential Wilson Disease Participants for Gene-Editing Clinical Trial

NCT07226622

Status	RECRUITING
Sponsor	Prime Medicine, Inc.
Enrollment	30 participants

Key Eligibility Criteria

Inclusion (3)

- Confirmed Wilson Disease (WD) as determined by the following criteria:
- An established clinical diagnosis of WD
- Genetic analysis confirming the presence of biallelic pathogenic variants at ATP7B, at least one of which is EITHER p.H1069Q OR p.R778L OR Participants without a confirmed genetic diagnosis may enroll only with explicit approval from the Medical Monitor

Exclusion (8)

- Prior history of gene therapy, liver transplantation, hepatocyte (cellular) transplantation, or active listing for liver transplantation
- For individuals with known ATP7B genotype: individual does not have at least 1 ATP7B allele with either the p.H1069Q or p.R778L mutation.
- Significant neurological conditions within the prior 12 months which may impact participant safety or participation in the study, including ability to complete study requirements or procedures as outlined in the clinical study protocol.
- In patients with psychiatric involvement, current or fluctuant clinical instability with new or changing diagnoses or substantial medication regimen changes in the past 12 months that could limit their participation, in the opinion of the Investigator.
- History of cirrhotic decompensation within the past year.

... and 3 more (see full listing online)

Locations (7 total)

University of California Los Angeles Medical Center, Los Angeles, California, United States
University of California Davis Health, Sacramento, California, United States
Yale New Haven Hospital, New Haven, Connecticut, United States
... and 4 more locations