Safety and Efficacy Trial of HG004 for Leber Congenital Amaurosis Related to Rpe65 Gene Mutations (STAR)

NCT05906953

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	HuidaGene Therapeutics Co., Ltd.
Enrollment	20 participants

Key Eligibility Criteria

Inclusion (6)

- Male or females between 6 and 50 years of age at the time of signing the informed consent form.
- Willing to adhere to protocol as evidenced by written informed consent or parental permission and subject assent.
- Clinical confirmed diagnosis of Leber congenital amaurosis (LCA) and molecular diagnosis of LCA due to RPE65 mutations.
- Ability to perform tests of visual and retinal function.
- Visual acuity of d 20/80 or visual field less than 20 degrees in the eye to be injected.

... and 1 more (see full listing online)

Exclusion (7)

- Pre-existing eye conditions that would preclude the planned surgery or interfere with interpretation of study endpoints or complications of surgery (e.g., glaucoma requiring upcoming surgery, corneal or significant lenticular opacities).
- Presence of epiretinal membrane by OCT.
- Complicating systemic diseases or clinically significant abnormal baseline laboratory values.
- Complicating systemic diseases would include those in which the disease itself, or the treatment for the disease, can alter ocular function.
- Prior ocular surgery within six months.

... and 2 more (see full listing online)

Locations (3 total)

Research Site, Sacramento, California, United States
Research Site, Houston, Texas, United States
Research Site, Shanghai, Shanghai Municipality, China