A Study of LX107 Gene Therapy in AIPL1-IRD Patients

NCT07063030

Status	RECRUITING
Phase	Early Phase 1
Sponsor	Shanghai General Hospital, Shanghai Jiao Tong University School of Medicine
Enrollment	13 participants

Plain Language Summary

This study tests a gene therapy (LX107) for people with a rare inherited eye condition called AIPL1-associated inherited retinal dystrophy, which causes progressive vision loss due to a faulty gene. The therapy aims to deliver a working copy of the gene directly into the eye.

You may be eligible if...
- You are 4 years old or older
- You have been diagnosed with inherited retinal dystrophy caused by mutations in both copies of the AIPL1 gene, confirmed by genetic testing
- The vision in your study eye is significantly reduced (visual acuity of 0.3 or less on standard testing)
- You or your guardian can consent to long-term follow-up

You may NOT be eligible if...
- You do not have confirmed AIPL1 gene mutations
- Your eye has certain structural conditions that could affect the safety or success of the injection
- You are unable to comply with long-term follow-up requirements

Talk to your doctor to see if this trial is right for you.

Key Eligibility Criteria

Inclusion (5)

- The subject and/or their guardian signs a written informed consent form and is willing to comply with the long-term follow-up protocol and supporting protocols.
- Adult or pediatric patients (aged e 4 years) diagnosed with AIPL1-IRD.
- Definitive molecular diagnosis of biallelic AIPL1 gene mutations confirmed by next-generation sequencing combined with Sanger validation.
- The study eye has a best-corrected visual acuity of no more than 58 letters (approximately equivalent to decimal visual acuity d 0.3) using the ETDRS visual acuity chart at baseline.
- Note: Only one eye will be designated as the "study eye" (i.e., the eye to receive treatment) at the investigator's discretion.

Exclusion (13)

- A history of ocular diseases that, in the investigator's judgment, may hinder the planned treatment or interfere with the interpretation of study endpoints (e.g., glaucoma, diabetic retinopathy, retinal vein occlusion, retinal detachment, posterior or panuveitis, etc.).
- Any eye with a history of gene therapy for IRD or other hereditary neuro-ophthalmic diseases (including but not limited to other viral vector-based gene therapies, mRNA therapies, etc.).
- A lack of sufficient viable retinal cells as determined by non-invasive methods such as OCT or ophthalmoscopy.
- Any active intraocular or periocular infection in the study eye (e.g., infectious conjunctivitis, keratitis, scleritis, endophthalmitis, infectious blepharitis, uveitis).
- A history of intraocular surgery (e.g., vitrectomy, cataract surgery, trabeculectomy, or other filtering surgery) in the 6 months prior to the screening visit.

... and 8 more (see full listing online)

Locations (1 total)

Shanghai General Hospital, Shanghai Jiao Tong University School of Medicine, Shanghai, China