Safety of Single and Repeat Dose of PYC-001 Eye Injections in People With Autosomal Dominant Optic Atrophy

NCT06970106

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	PYC Therapeutics
Enrollment	21 participants

Key Eligibility Criteria

Inclusion (27)

- Must give written informed consent before any study-related activity is carried out and must be able to understand the full nature and purpose of the study, including possible risks and adverse effects;
- Adult males and females, aged 18 years and above at screening;
- Body mass index e18.0 and d35.0 kg/m2
- Have a recent (within five years) genetic diagnosis of OPA1 mutation-associated (haploinsufficiency) ADOA and/or confirmed diagnosis during pre-screening or screening, as determined by the PI. In case of complex mutation profile, eligibility will be determined in consultation with the Sponsor. Rollover participants are exempt from this criterion as their genetic diagnosis was confirmed in PYC-001-101;
- Treatment naïve participants with best-corrected visual acuity (BCVA) of between d20/40 (d70 Early Treatment of Diabetic Retinopathy Study \[ETDRS\] letters) and e20/200 (e35 ETDRS letters). If both eyes meet this eligibility criteria, the eye with better fixation as determined by the PI in consultation with the Sponsor will be selected as the study eye and the other eye will be designated as the fellow eye. In the event that both eyes are eligible and have adequate fixation to reliably perform all study assessments, the worse eye as determined by the physician will be taken as the study eye.

... and 22 more (see full listing online)

Exclusion (26)

- Participant has a known allergy to PYC-001 or any of its excipients;
- Demonstrated clinically significant co-morbidities, which, in the opinion of the PI, would interfere with the participant's ability to participate in the study and/or confound study outcomes;
- Females who are breastfeeding or planning to breastfeed;
- Based on recent (within five years of screening \[for rollover PYC-001-101 participants, within five years of entry into PYC-001-101\]) genetic testing, the participant has mutations in genes that cause ADOA, other than OPA1 (for example in case of dominant negative ADOA and ADOA Plus) or has other pathological variants that result in an ADOA-like optic atrophic phenotype or other pathologic genetic findings indicating presence of additional confounding ocular diseases based on comprehensive genetic screening. Eligibility will be determined by the PI in consultation with the Sponsor as needed;
- Have received any prior cell or gene therapy for a retinal condition, excluding participation in study PYC-001-101;

... and 21 more (see full listing online)

Locations (3 total)

Save Sight Institute - Sydney Eye Hospital, Sydney, New South Wales, Australia
Cerulea Clinical Trials, East Melbourne, Australia
Retina Specialists, Auckland, New Zealand