MAD of IVT VP-001 in PRPF31 Mutation-Associated Retinal Dystrophy Subjects (Wallaby)

NCT06455826

Status	RECRUITING
Phase	Phase 1
Sponsor	PYC Therapeutics
Enrollment	12 participants

Key Eligibility Criteria

Inclusion (12)

- Male or female sex; e12 years of age at Baseline (Visit 2).
- Have a molecular (genetic) diagnosis of PRPF31 mutation.
- Have a clinical diagnosis of PRPF31 mutation-associated retinal dystrophy, that is, RP11. The following conditions are allowed for inclusion if due to RP11, if in the opinion of the investigator they will not interfere with study evaluations or have resolved: macular edema (intraretinal, sub-retinal or other fluid) requiring regular treatment at a frequency of less than every 6 weeks; macular edema must be stable for at least 3 months prior to Screening (Visit 1). The investigator must consult with the study Medical Monitor.
- If e18 years of age, understand the language of the informed consent and are willing and able to provide written informed consent prior to any study procedures. If a minor (12 to \<18 years of age), a parent or legal guardian willing and able to provide written permission for the minor's participation prior to performing any study related procedures and pediatric participant able to provide age appropriate assent for study participation.
- If e18 years of age, are willing to comply with the instructions and attend all scheduled study visits. If a minor (12 to \<18 years of age), able to complete all study assessments, comply with the protocol, and has a parent or caregiver willing and able to follow study instructions and attend study visits with the participant as required, in the opinion of the Investigator.

... and 7 more (see full listing online)

Exclusion (14)

- Have any uncontrolled systemic disease that, in the opinion of the Investigator, would preclude participation in the study that include but are not limited to infection, uncontrolled elevated blood pressure, cardiovascular disease, or glycemic control issues, or any other medical condition that may put the participant at risk due to study procedures.
- Mutations in genes that cause autosomal dominant RP, Xlinked RP, or presence of biallelic mutations in autosomal recessive RP/retinal dystrophy genes other than PRPF31 mutations.
- Have used anti-vascular endothelial growth factor (VEGF) agents within 2 months or corticosteroid injections within the last 3 months.
- Have had Ozurdex
- implants placed within 3 months or Retisert

... and 9 more (see full listing online)

Locations (5 total)

University of Florida Health, Jacksonville, Florida, United States
University of Michigan Kellogg Eye Center, Ann Arbor, Michigan, United States
Oregon Health and Science University - Casey Eye Institute, Portland, Oregon, United States
... and 2 more locations