

# Real-World Outcomes of Voretigene Neparvovec for RPE65-Mediated Retinal Dystrophy: A Systematic Review and Meta-Analysis

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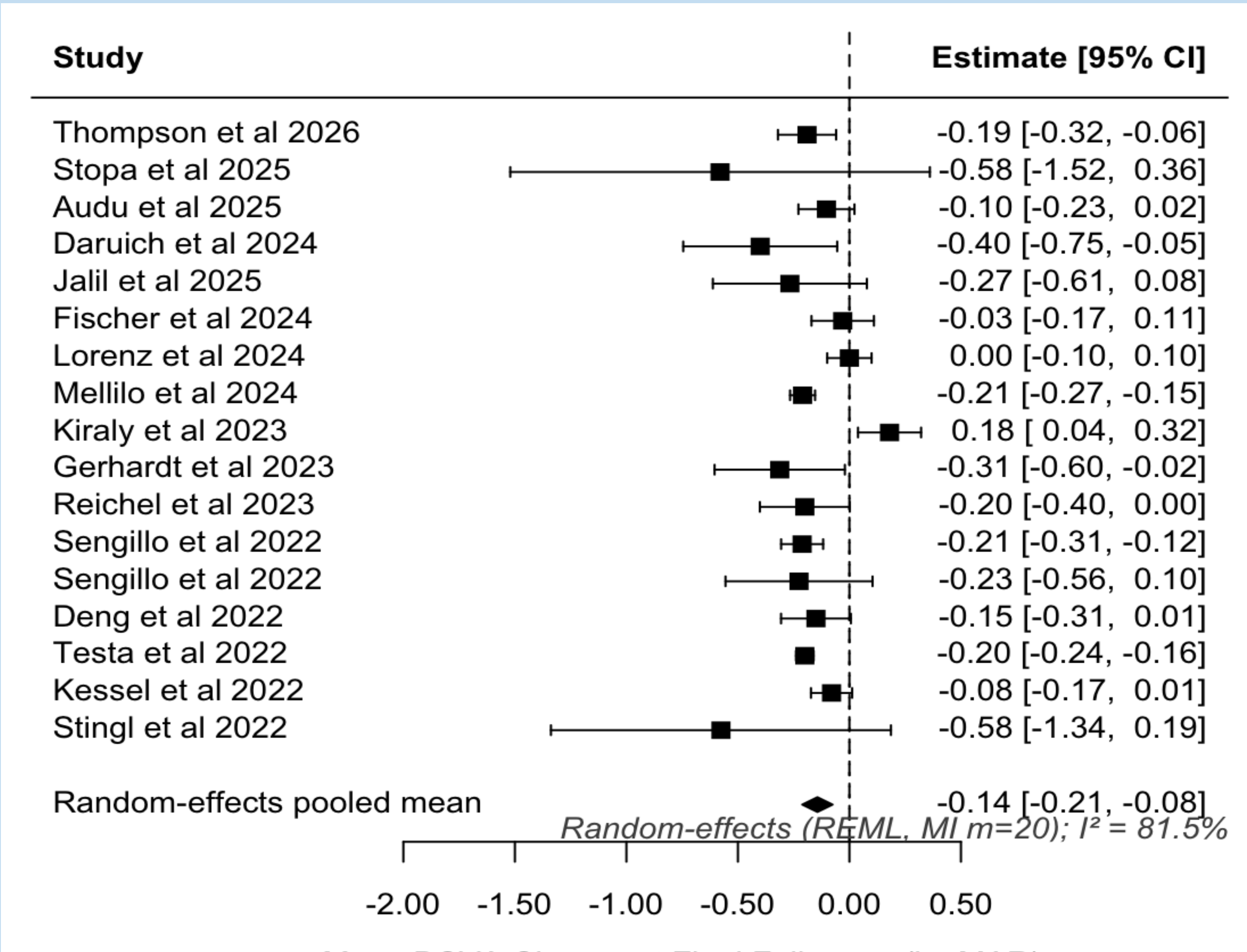
## PURPOSE:

- Systematically review **real-world outcomes** of Voretigene Neparvovec therapy.
- Assess **anatomical and functional outcomes** in patients with **biallelic RPE65 mutation-associated IRD**.
- Evaluate reported **adverse outcomes and safety profile** following treatment.

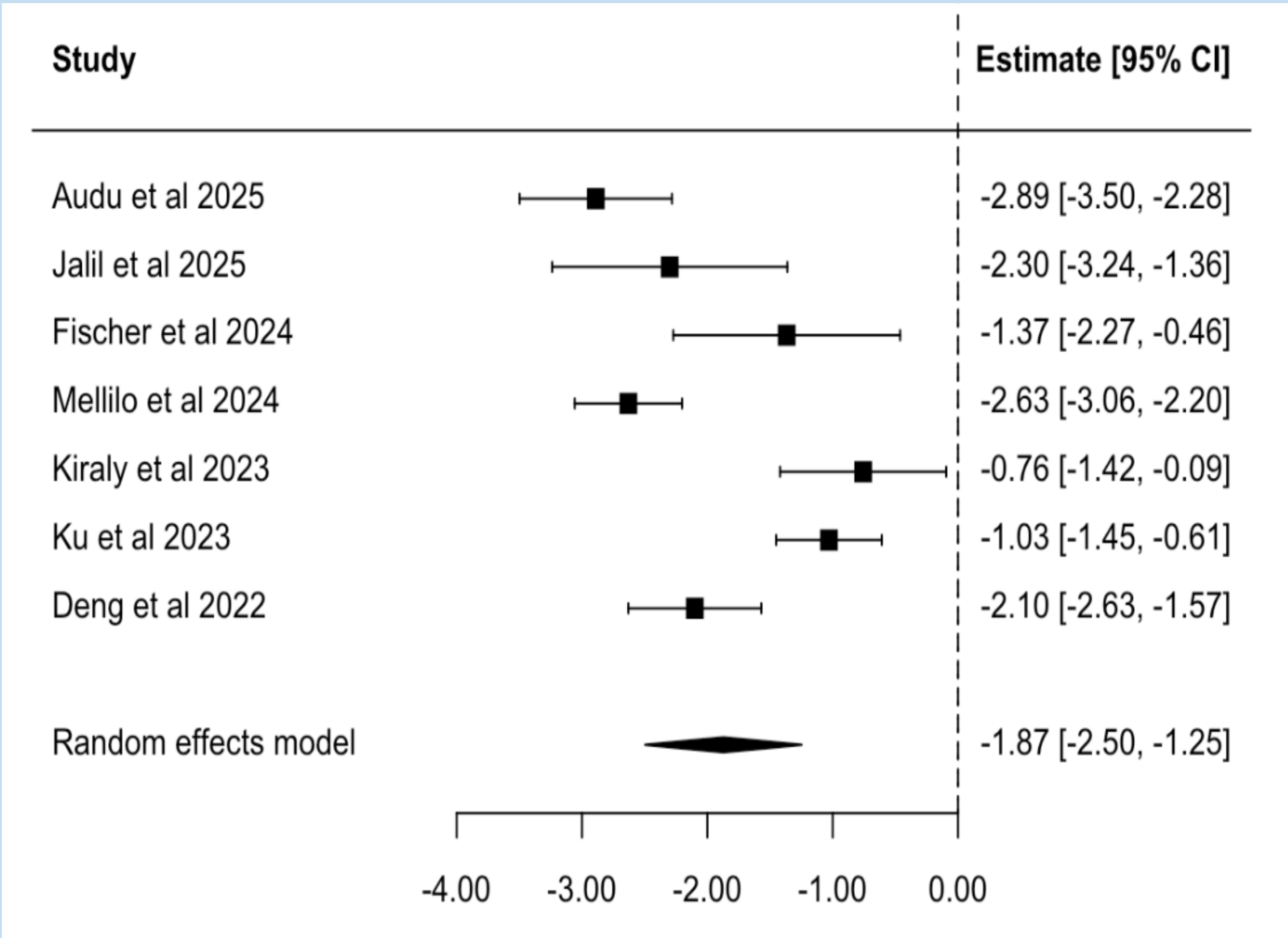
## METHODS:

- Conducted a **PROSPERO-registered systematic review and meta-analysis**.
- Searched **PubMed, Embase, Scopus, CENTRAL, and Web of Science** for studies reporting VN outcomes in **RPE65-associated inherited retinal dystrophy**.
- Included studies reporting **real-world clinical outcomes** following Voretigene Neparvovec therapy.
- **Primary outcomes** were **best-corrected visual acuity** and **full-field stimulus testing**.
- **Secondary outcomes** included **MLMT, LLVA, visual fields, adverse events**, and other reported outcomes.
- All statistical analyses were performed using **R statistical software**.

## RESULTS:



Forest plot of mean FST (white) change at Final FU



Forest plot of mean FST (white) change at Final FU

## CONCLUSION:

- Real-world visual, anatomical, and functional outcomes of VN therapy appear broadly consistent with pivotal clinical trial findings.
- Chorioretinal atrophy (CRA) is an increasingly recognised post-treatment finding that was not fully characterised in early trials.
- Real-world studies report higher rates of CRA, highlighting the need for longer-term monitoring and safety reporting.



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