BACKGROUND
Leber's Hereditary Optic Neuropathy (LHON) is a rare hereditary condition that primarily affects young men, with visual loss occurring in about 1 in 50,000 men. It is caused by mutations in mitochondrial DNA, which can lead to dysfunction of mitochondria and oxidative stress.

METHODS
A randomized, double-blind, placebo-controlled trial (RHOODS) enrolled patients with visual impairment due to LHON within 1 year of onset of vision loss. The study compared idebenone (Raxone®) with placebo for 6 months, with follow-up at 12 months. The primary endpoint was clinical recovery (CRR), defined as no further VA loss and attainment of a CRR of at least 5 letters over baseline.

RESULTS
The study included 29 patients with LHON, 15 in each treatment group. The CRR was observed in 7 out of 15 patients (47%) in the idebenone group vs. 1 out of 15 patients (6.7%) in the placebo group, with a statistically significant difference (p=0.006). Other benefits included stabilization of VA, prevention of further VA loss, and improvements in vision-related quality of life.

CONCLUSIONS
Idebenone (Raxone®) shows potential to reverse vision loss in patients with LHON, with a significant proportion achieving CRR and stabilization of VA. Further studies are needed to confirm these findings and to explore the long-term benefits of idebenone treatment.

Effectiveness of Idebenone in LHON

In this phase 2b study, idebenone (Raxone®) showed significant benefits in patients with LHON, including:

- **Clinical recovery (CRR):** 47% of idebenone-treated patients achieved CRR, compared to 6.7% of placebo-treated patients (p=0.006).
- **Stabilization of VA:** Both groups showed stabilization, but idebenone-treated patients maintained a better VA outcome.
- **Further VA loss prevention:** Idebenone-treated patients experienced no further VA loss, while 10% of placebo-treated patients had further VA loss.

**Tolerability:**

Idebenone was well-tolerated, with similar tolerability profiles between the treatment groups. Common adverse events included nasopharyngitis, headache, cough, and rhinitis.

**Safety Monitoring:**

The study employed a comprehensive safety monitoring plan, including laboratory tests, adverse event reporting, and monitoring of vital signs and clinical observations.

**Conclusion:**

Idebenone (Raxone®) shows promise in the treatment of LHON, with significant clinical recovery and stabilization of visual acuity. Further studies are needed to confirm these findings and to explore the long-term benefits of idebenone treatment.

**References:**


**Clinical Trial Registration:**

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