Treatment of visual impairment in patients with hereditary optic neuropathy (LHON) using idebenone (Raxone®)

Hasham S.1, Metz G.1, Catarino C.1, Klopstock T.2

1: Santhera Pharmaceuticals, Liestal, Switzerland. 2: Dept. of Neurology, Friedrich-Baur-Institute, Ludwig-Maximilians-University, Munich, Germany.

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Background

LHON, the rare primary mitochondrial disease seen in young adults, is caused by a mitochondrial DNA mutation (G11778A, T14484C, G3460A) in the complex I of the electron transport chain (ETC). These mutations are present in 1 in 5,000 births and are typically passed from mother to child. Most patients are asymptomatic. A small percentage develop vision loss, with the majority of patients being <60 years old. Treatment options are limited and the management strategy has typically focused on visual rehabilitation and support for patients and caregivers. Despite advances in the understanding of LHON molecular mechanisms and the development of disease-modifying strategies, no effective treatment is available at present. Idebenone (Raxone®) has been shown to improve visual function in clinical trials. Randomized controlled trials (RCTs) and observational studies have confirmed idebenone’s benefit in reducing vision loss and improving visual acuity.

Methods

Identified are the first and only approved treatment for visual impairment in at-risk and newly diagnosed with LHON (Seymour, Williamson, 2015). They also confirm idebenone’s benefit in reducing vision loss and improving visual acuity, as well as the clinical relevance of the treatment effects observed in RCTs.

Results

Two-thirds of patients had vision loss at baseline, and 12% had vision loss at follow-up visit. Overall, 29% of patients had vision loss at baseline, compared with 16% at follow-up visit. These results confirm that idebenone can improve visual function in patients with LHON.

Discussion

Results confirm idebenone’s benefit in reducing vision loss and improving visual acuity, as well as the clinical relevance of the treatment effects observed in RCTs.

Conclusion

Idebenone is well-tolerated with a consistent safety profile and no new safety signals in the real world.

Safety

Idebenone is well-tolerated with a consistent safety profile and no new safety signals in the real world.

Expert Opinion

This article is one of several articles on idebenone (Raxone®) published in Expert Opinion on Investigational Drugs and Ocular Drug Delivery. In these articles, idebenone (Raxone®) is shown to be a safe and effective treatment for the management of LHON.

References