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Title LUXTURNA – A Health Technology Assessment

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Reference link to full report in French:
https://www.has-sante.fr/portail/jcms/c_2964759/fr/luxturna-voretigene-neparvovec-therapie-genique

Aim

Assessment of LUXTURNA (voretigene neparvovec) with a view to funding by the French national health insurance system and of its clinical contribution compared to other strategies with a view to setting of its price by the French Healthcare Products Pricing Committee (CEPS) in the treatment of adult and paediatric patients with vision loss due to inherited retinal dystrophy caused by confirmed biallelic RPE65 mutations and who have sufficient viable retinal cells.

Conclusions of Transparency Committee

High actual clinical benefit in the indication, considering:

◗ The seriousness of the disease: inherited retinal dystrophies caused by biallelic RPE65 mutations are rare, highly incapacitating diseases, with an early onset in children or young adults and leading to legal blindness. A distinction is made between severe forms (such as Leber congenital amaurosis) with a very early onset, from birth, with loss of visual acuity ranging from 1/10 to the total absence of light perception, associated with nystagmus and searching nystagmus, and slow-progressing forms often starting in adolescence or earlier, evolving towards visual field loss, initially peripheral and then central, and, finally, blindness.

◗ The curative objective of LUXTURNA.

◗ The efficacy/adverse effects ratio, which is high.

◗ The role of the medicinal product in the therapeutic strategy: it is a first-line treatment in adult and paediatric patients with vision loss due to inherited retinal dystrophy caused by confirmed biallelic RPE65 mutations (homozygous and compound heterozygous) and who have sufficient viable retinal cells.

◗ The absence of alternative treatments.

◗ The possible public health impact of LUXTURNA.

Substantial clinical added value (CAV II) in the management of adult and paediatric patients with vision loss due to inherited retinal dystrophy caused by confirmed biallelic RPE65 mutations and who have sufficient viable retinal cells, taking into consideration:

- the quality of the demonstration of the efficacy of LUXTURNA in a randomised, multicentre phase III study versus the absence de treatment, despite the open-label nature of the study,
- the relevance of the primary endpoint assessing the visual function of patients using a Multi-Luminance Mobility Test (MLMT) and the clinical relevance of the effect observed after 1 year of follow-up compared to the absence of treatment,
- the unmet medical need in an incapacitating disease that progresses to blindness,
- the absence of a robust demonstration of the effect of LUXTURNA on quality of life.

Recommendations

The Transparency Committee issued its approval for the funding of LUXTURNA by the French national health insurance system (hospital use only) in the MA indication.

Methods

The assessment of LUXTURNA was founded on evidence-based medicine with a critical analysis of the clinical data. The assessment of the efficacy and safety of LUXTURNA is primarily based on a randomised, open-label, multicentre phase III study having compared LUXTURNA with the absence of treatment, after one year. In the context of an addendum, follow-up was extended to 2-3 years.

Written by

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