Summary of opinion[1] (initial authorisation)

Luxturna

voretigene neparvovec

On 20 September 2018, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Luxturna, intended for the treatment of retinal dystrophies caused by RPE65 mutations.

As Luxturna is an advanced therapy medicinal product, the CHMP positive opinion is based on an assessment by the Committee for Advanced Therapies. Luxturna was designated as an orphan medicinal product on 02 April 2012. The applicant for this medicinal product is Spark Therapeutics Ireland Ltd.

Luxturna will be available as concentrate (5×10^{12} vector genomes/ml) and solvent for solution for injection. The active substance of Luxturna is voretigene neparvovec, a gene transfer vector which works by replacing the mutated gene in retinal cells.

The benefits with Luxturna are its ability to improve night vision. The most common side effects are conjunctival hyperaemia, cataract and increased intraocular pressure.

The full indication is: "Luxturna is indicated for 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."

It is proposed that Luxturna be administered by a retinal surgeon experienced in performing macular surgery.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

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[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

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