LUXTURNA (PREAUTHORIZATION REQUIRED)

X.96

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POLICY

Luxturna (voretigene neparvovec) may be considered **medically necessary** for the treatment of Inherited Retinal Dystrophies (IRD) caused by mutations in the retinal pigment epithelium-specific protein 65kDa (RPE65) gene in patients who meet **ALL** of the following criteria:

I. Patient is greater than 12 months of age or greater at the time of therapy initiation; **AND**

II. Patient has a diagnosis of a confirmed biallelic RPE65 mutation-associated retinal dystrophy (e.g. Leber's congenital amaurosis [LCA], Retinitis pigmentosa [RP] Early Onset Severe Retinal Dystrophy [EOSRD], etc.); **AND**

III. Genetic testing has been performed documenting biallelic mutations of the RPE65 gene; **AND**

IV. Patient has viable retinal cells as determined by retinal thickness on spectral domain optical coherence tomography (>100 microns within the posterior pole), fundus photography, and clinical examination; **AND**

V. The patient has remaining light perception in the eye(s) that will receive treatment. **AND**

VI. The medication is prescribed and administered by ophthalmologist or retinal surgeon with experience providing sub-retinal injections; **AND**

VIII. If **ALL** criteria are met, Luxturna may be approved for 1 injection per eye per lifetime

Luxturna (voretigene neparvovec) is considered **investigational** when:

I**.** Patient has previously been treated with Luxturna.
II. The medication is used for inherited retinal diseases not due to an RPE65 mutation.
III. The medication is used after, or, in combination with any other gene therapy.

Dates

Original Effective
11-08-2017
Last Review
11-06-2024
Next Review
11-12-2025

DESCRIPTION

Luxturna is an adeno-associated virus vector-based gene therapy indicated for the treatment of patients with confirmed biallelic *RPE65* mutation-associated retinal dystrophy. Luxturna is a live, non-replicating adeno-associated virus serotype 2 which has been genetically modified to express the human *RPE65* gene. Luxturna is derived from naturally occurring adeno-associated virus using recombinant DNA techniques.

Luxturna is designed to deliver a normal copy of the gene encoding the human retinal pigment epithelial 65 kDa protein (RPE65) to cells of the retina in persons with reduced or absent levels of biologically active RPE65. The RPE65 is produced in the retinal pigment epithelial (RPE) cells and converts all-*trans*-retinol to 11-*cis*-retinol, which subsequently forms the chomophore, 11-*cis*-retinal, during the visual (retinoid) cycle. The visual cycle is critical in phototransduction, which refers to the biological conversion of a photon of light into an electrical signal in the retina. Mutations in the *RPE65* gene lead to

Luxturna was studied in a randomized, controlled, open-label, phase 3 study. The study included 31 individuals aged 3 years or older with a confirmed genetic diagnosis of biallelic RPE65 gene mutations with visual acuity of 20/60 or worse, or visual field less than 20 degrees in any meridian, or both in both eyes. Individuals had to have sufficient viable retinal cells as determined by retinal thickness on spectral domain optical coherence tomography (>100 microns within the posterior pole), fundus photography, and clinical examination; and they were able to perform a standardized multi-luminance mobility test (MLMT) within the luminance range evaluated, but unable to pass the MLMT at 1 lux, the lowest luminance level tested. Individuals were excluded if they had participated in previous gene therapy or invitational drug study, used high-dose (7500 retinol equivalent units [or >3300 IU] per day of vitamin A) retinoid compounds in the past 18 months, had known hypersensitivity to medications planned for use in the peri-operative period, or had ocular or systemic conditions that would interfere with study interpretation. Luxturna was injected in the first eye and then injected on day 6-18 in the second eye. Individuals in the control group were eligible to receive Luxturna after 1 year after baseline evaluations.[2]

For the primary endpoint of MLMT change scores at year 1 compared to baseline, Luxturna had an average of 1.9 to 2.1 improvement in MLMT scores while placebo had an average of 0.1 to 0.2 score improvement depending on the eye. Differences between Luxturna and controls scores were statistically significant. Improvements in MLMT scores for Luxturna was stable throughout year 1.[2]

For the secondary endpoint of full-field light sensitivity threshold (FST) testing average over both eyes, Luxturna had a greater than 2 log unit improvement by day 30 in light sensitivity that remained stable over 1 year. The control group had no meaningful change in this measure over 1 year. The difference between Luxturna and control of -2.11 (95% CI -3.19 to -1.04) was statistically significant (p=0.0004).[2]

For another secondary endpoint of best corrected visual acuity (BCVA) averaged over both eyes, Luxturna had a mean improvement of 8·1

In clinical trial, Luxturna exhibited no product related serious adverse events and no deleterious immune responses. The most common ocular adverse events were transient mild ocular inflammation, transient elevated intraocular pressure, and intraoperative retinal tear

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REFERENCES

retinal dystrophy: a randomized, controlled, open-label, phase 3 trial. The Lancet. 2017;390: 849-860.

2017

Luxturna prescribing information. Spark Therapeutics, Inc. December 2017.

REVISIONS

12-01-2023

Policy reviewed at Medical Policy Committee meeting on 11/8/2023 – no changes to policy.

12-30-2020

Added new 2021 HCPC code C9770 for administration

12-29-2020

Removed unlisted codes C9399, J3490, J3590 and deleted code C9032

01-03-2019

Added new code for 2019 J3398

03-19-2018

Added criteria