PHARMACY POLICY STATEMENT
Marketplace

DRUG NAME	Luxturna (voretigene neparvovec-rzyl)
BILLING CODE	J3398
BENEFIT TYPE	Medical
SITE OF SERVICE ALLOWED	Outpatient hospital
STATUS	Prior Authorization Required

Luxturna, approved by the FDA in 2017, is an adeno-associated virus vector-based gene therapy indicated for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy. Mutations in the RPE65 gene lead to reduced or absent levels of RPE65 isomerohydrolase activity, blocking the visual cycle and resulting in impairment of vision. Luxturna is designed to deliver a normal copy of the gene encoding RPE65 to persons with reduced or absent levels of biologically active RPE65 so that functional RPE65 protein can be produced to help restore the visual cycle and potentially improve vision. It is estimated that between 1,000 and 2,000 people in the United States are affected. With time, untreated patients lose the ability to detect any intensity of light.
In clinical trials, the efficacy of Luxturna was established based on multi-luminance mobility testing (MLMT) score change from Baseline to Year 1. The MLMT was designed to measure changes in functional vision, as assessed by the ability of a subject to navigate a course accurately and at a reasonable pace at different levels of environmental illumination. Response was rapid and sustained, with improvement noted by day 30, durable overall for at least 4 years. Some degree of numerical improvement in visual acuity was shown, but it was not statistically significant.

Luxturna (voretigene neparvovec-rzyl) will be considered for coverage when the following criteria are met:

For **reauthorization**:
1. Luxturna will not be re-authorized.

CareSource considers Luxturna (voretigene neparvovec-rzyl) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.