Luxturna® (voretigene neparvovec-rzyl)
Approved April 2022

Background:

Retinal dystrophies (RDs) are degenerative diseases of the retina which have marked clinical and genetic heterogeneity. Common presentations among these disorders include night or color blindness, tunnel vision and subsequent progression to complete blindness.

***Luxturna** is an adeno-associated virus vector-based gene therapy indicated for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy*

Criteria for approval:

1. The patient has a diagnosis of confirmed biallelic RPE65 mutation-associated retinal dystrophy; **AND**
2. Patient must have viable retinal cells as determined by the treating physician(s)
3. Medication is prescribed by or in consultation with an ophthalmologist
4. Patient is at least 12 months of age but less than 65 years of age
5. Patient has not previously been treated with Luxturna in the requested treatment eye(s)
6. Patient has not received intraocular surgery within prior 6 months
7. Luxturna will be administered to each eye on separate days within a close interval, but no fewer than 6 days apart
8. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Approval Duration: Date of Service

References:

1. Luxturna [prescribing information]. Spark Therapeutics, Inc. Philadelphia, PA 19104, 2017
2. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2019. Updated periodically
3. Russel S, Bennet J, Wellman JA, et al. Efficacy and safety of voretigene neparvovec (AAV2-hRPE65v2) in patients with RPE65-mediated inherited retinal dystrophy: a randomized, controlled, open-label phase 3 trial. Lancet 2017; 390:849-860