Clinical Edit Criteria

Drug/Drug Class:	**Luxturna® (voretigene neparvovec-rzyl) Subretinal Injection Clinical Edit**
Date:	**April 18, 2019**
Prepared for:	
Prepared by:	**MO HealthNet**

☒ **New Criteria** ☐ **Revision of Existing Criteria**

Executive Summary

Purpose: Ensure appropriate utilization and control of Luxturna® (voretigene neparvovec-rzyl).

Why was this Issue Selected: 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. It is the first FDA-approved gene therapy for a genetic disease, the first and only pharmacologic treatment for an inherited retinal disease and the first adeno-associated virus vector gene therapy approved in the U.S. Gene therapy is designed to introduce genetic material into cells to compensate for abnormal genes or to make a beneficial protein. Biallelic RPE65 mutation-associated retinal dystrophy affects approximately 1,000 to 2,000 patients in the U.S. Previously untreatable, RPE65-mediated inherited retinal dystrophy is an inherited retinal disease, and a natural history study has shown that people with it eventually progress to total blindness. People living with IRDs due to biallelic RPE65 gene mutations frequently suffer from nigh blindness as a result of decreased light sensitivity during childhood or early adulthood, as well as involuntary back and forth eye movements. The approval of Luxturna® further opens the door to the potential of gene therapies. The recommended dose of Luxturna® for each eye is 1.5 x 10^{11} vector genomes (vg), administered by subretinal injection in a total volume of 0.3 ml. Subretinal administration to each eye is performed on separate days within a close interval, but no fewer than 6 days apart.

Program-specific information:	**Drug** • Luxturna®	**Cost per Eye** *$425,000 WAC*
Setting & Population:	Patients ages 12 months and older	
Type of Criteria:	☐ **Increased risk of ADE** ☒ **Appropriate Indications**	☐ **Non-Preferred Agent** ☐
Data Sources:	☐ **Only administrative databases**	☒ **Databases + Prescriber-supplied**

Setting & Population

- Drug for review: Luxturna ® (voretigene neparvovec-rzyl)
- Age range: Patients ages 12 months and older
- Gender: Male and female

Approval Criteria

- Appropriate diagnosis for Luxturna ®
  - Confirmed biallelic RPE65 mutations-associated retinal dystrophy
  - Patient must have viable retinal cells as determined by the treating physician

Denial Criteria

- Lack of appropriate diagnosis

References

1. Prescribing Information, Spark Therapeutics, Inc., Philadelphia, PA; December 2017.
2. FDA News Release, December 19, 2017