Clinical Edit Criteria

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

New Criteria
Revision of Existing Criteria

Executive Summary

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

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.
Clinical Edit Criteria Proposal

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
- ☐ Non-Preferred Agent
- ☑ Appropriate Indications
- ☐

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

2. FDA News Release, December 19, 2017