

Luxturna™ (voretigene neparvovec-rzyl)

When requesting Luxturna™ (voretigene neparovvec-rzyl), the individual requiring treatment must be diagnosed with an FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

FDA-approved indication

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

Approved Off-label Compendial use(s)

None

Coverage Guidelines

The individual must meet **all** of the following criteria for approval:

- The diagnosis of retinal dystrophy is confirmed by genetic testing showing mutations in BOTH copies of the *RPE65* gene
- Has viable retinal cells as determined by the treating physician
- Is at least 1 year of age
- Has not previously received Luxturna therapy

Approval doses: 2 doses lifetime

Dosing Recommendation

The recommended dose of Luxturna for each eye is 1.5×10^{11} vector genomes (vg), administered by subretinal injection in a total volume of 0.3 mL.

The subretinal administration of Luxturna should be performed to each eye on separate days within a close interval, but no few than 6 days apart.

References

1. Luxturna prescribing information. Philadelphia, PA: Spark Therapeutics, Inc. 2017.
2. Russell S, Bennett 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(10097):849-860.
3. Ku CA, Pennesi ME. Retinal Gene Therapy: Current Progress and Future Prospects. *Expert Rev Ophthalmol*. 2015;10(3):281-299.
4. American Society of Gene and Cell Therapy. Inherited Retinal Diseases. Available at: https://www.asgct.org/education/disease_treatments/inherited-retinal-disease. Accessed February 05, 2018.

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