Luxturna (voretigene neparvovec-rzyl)
PAM – 020

Iowa Medicaid Program	Prior Authorization	**Effective Date**	04/01/2021
Revision Number	6	**Last Reviewed**	04/17/2026
Reviewed By	Medicaid Medical Director	**Next Review**	04/16/2027
Approved By	Medicaid Clinical Advisory Committee	**Approved Date**	04/16/2021

Overview

Medication: [1]	voretigene neparvovec-rzyl
Brand Name:	Luxturna®
Pharmacologic Category:	Adeno-associated virus vector-based gene therapy
FDA-Approved Indication(s):	Treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy
How Supplied:	Single-dose 2 mL vial (0.5 mL extractable volume) for a single administration in one eye • The supplied concentration ($5x10^{12}$ vg/mL) requires a 1:10 dilution prior to administration • The diluent is supplied in two single-use 2-mL vials
Dosage and Administration:	• For subretinal injection only. • Recommended dose for each eye is 1.5×10^{11} vector genomes (vg), administered by subretinal injection in a total volume of 0.3 mL. • Perform subretinal administration of Luxturna® to each eye on separate days within a close interval, but no fewer than 6 days apart. • Luxturna® should be administered in the surgical suite under controlled aseptic conditions by a surgeon experienced in performing intraocular surgery.
Benefit Category:	Medical

Descriptive Narrative

Retinal dystrophies are a group of degenerative disorders of the retina with clinical and genetic heterogeneity. Common presentations include color blindness or night blindness, peripheral vision abnormalities, and subsequent progression to complete blindness in progressive conditions. Loss of vision can begin as early as the first few months of life or during

childhood or adolescence. The exact incidence of retinal dystrophies is unknown, but the most common form, retinitis pigmentosa, affects around 1 in 5,000 individuals worldwide.[2]

Retinal dystrophies are not particularly rare; however, due to their genetic heterogeneity with more than 200 disease genes identified to date, each genetic subtype may be exceedingly rare.[3] In the US, approximately 1,000 to 2,000 patients have biallelic retinal pigment epithelial 65 kDa protein (RPE65) mutation-associated retinal dystrophy.[4]

Multiple causative gene defects have been identified. Mutations in the *RPE65* gene lead to reduced or absent levels of *RPE65* isomerohydrolase activity, blocking the visual cycle and resulting in impaired vision. Luxturna® is a gene replacement therapy designed to deliver a normal copy of the gene encoding the human RPE65 to cells of the retina in persons with reduced or absent levels of biologically active RPE65, providing the potential to restore the visual cycle.

Gene replacement therapy can provide a healthy copy of the defective gene and replace the missing protein. However, timing of gene replacement in the progression of the disease is critical, as adequate numbers of viable photoreceptor cells must still be present to produce enough of the missing protein to restore visual function.

Use in infants under 12 months of age is not recommended because of potential dilution or loss of Luxturna® after administration due to retinal cell proliferation still occurring in this age group.

Guidelines

In the United Kingdom, the National Institute for Health and Care Excellence has recommended Luxturna® for eligible patients who have enough viable retinal cells.[5]

Criteria

Prior authorization is required.

Luxturna® is considered medically necessary when **ALL** of the following are met:

1. Diagnosis of retinal dystrophy confirmed by genetic diagnosis of biallelic* retinal pigment epithelial 65 kDa protein (RPE65) gene mutations; **AND**
2. Member is 1 year of age or older; **AND**
3. For each eye indicated for treatment, presence of sufficient viable retinal cells as determined by non-invasive means, such as optical coherence tomography (OCT) and/or ophthalmoscopy as evidenced by **ONE** of the following:
    a. An area of retina within the posterior pole of greater than 100 µm thickness shown on OCT; **OR**
    b. Three or more disc areas of retina without atrophy or pigmentary degeneration within the posterior pole; **OR**
    c. Remaining visual field within 30 degrees of fixation as measured by a III4e isopter or equivalent; **OR**
    d. Fundus photography (i.e., presence of neural retina); **AND**
4. No intraocular surgery on the eye to be treated in the previous 6 months; **AND**
5. Member has not been previously treated with RPE65 gene therapy in the intended eye; **AND**
6. Prescribed and administered by an ophthalmologist or retinal surgeon with experience providing sub-retinal injections; **AND**
7. Dose per eye does not exceed 1.5×10^{11} vector genomes (vg).

* **biallelic –** a mutation in both copies of a particular gene, one from each parent

Luxturna® is considered NOT medically necessary for use in infants under 12 months of age because of potential dilution or loss of Luxturna® after administration due to retinal cell proliferation still occurring in this age group.

Repeat treatments with Luxturna® (on the same eye) are considered investigational and are not covered by Iowa Medicaid**.**

Approval Duration and Quantity Limits

	Initial Authorization (subsequent authorizations not applicable)
Approval Duration	• Approval of Luxturna® is for one treatment for each eye per lifetime • Repeat injections of Luxturna® in the same eye are considered investigational and not medically necessary in all cases
Quantity Limits	• Per eye: one treatment, not to exceed a dose of 1.5×10^{11} vector genomes (vg)

Coding and Product Information

The following list(s) of codes and product information are provided for reference purposes only and may not be all inclusive. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment, nor does the exclusion of a code imply that its association to the HCPCS code is inappropriate.

HCPCS	**Description**
J3398	Injection, voretigene neparvovec-rzyl, 1 billion vector genomes
0810T	Subretinal injection of a pharmacologic agent, including vitrectomy and 1 or more retinotomies

ICD-10	**Description**
H35.50	Unspecified hereditary retinal dystrophy
H35.52	Pigmentary retinal dystrophy
H35.54	Dystrophies primarily involving the retinal pigment epithelium

NDC	**Labeler**	**Dosage**	**Pkg Size**	**Pkg Qty**	**Units/ Pkg**
71394-0065-01 (single-dose vial, 0.5 mL extractable volume)* • Supplied in a carton (71394-0415-01) containing one vial of Luxturna and 2 vials of diluent (71394-0716-01)	Spark Therapeutics, Inc. (71394)	1 billion vector genomes	0.5	1	150

* Luxturna contains 5×10^{12} vector genomes (vg) per mL and requires a 1:10 dilution prior to administration.

Compliance

1. Should conflict exist between the policy and applicable statute, the applicable statute shall supersede.
2. Federal and State law, as well as contract language, including definitions and specific contract provisions or exclusions, take precedence over medical policy and must be considered first in determining eligibility for coverage.
3. Medical technology is constantly evolving, and Iowa Medicaid reserves the right to review and update medical policy on an annual or as-needed basis.

Medical necessity guidelines have been developed for determining coverage for member benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. Medical necessity guidelines are developed for selected physician-administered medications found to be safe and proven to be effective in a limited, defined population or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. Criteria are revised and updated annually, or more frequently if new evidence becomes available that suggests needed revisions.

References

[1] Luxturna® prescribing information (05/2022). Spark Therapeutics, Inc.: Philadelphia, PA. Available online: luxturnahcp.com. Accessed January 28, 2026.

[2] Chawla H, Vohra V. Retinal Dystrophies. [Updated March 16, 2023]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2023 Jan. Available from: www.ncbi.nlm.nih.gov/ books/NBK564379/.

[3] Talib M, Boon CJF. Retinal Dystrophies and the Road to Treatment: Clinical Requirements and Considerations. Asia Pac J Ophthalmol (Phila). 2020 May-Jun;9(3):159-179. PMID: 32511120.

[4] Gardiner MF. Retinitis pigmentosa: Treatment. Li H, ed. UpToDate. Waltham, MA: UpToDate Inc. www.uptodate.com. Accessed March 4, 2025.

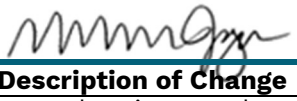
[5] National Institute for Health and Care Excellence. Voretigene neparvovec for treating inherited retinal dystrophies caused by RPE65 mutations. Published October 9, 2019. Available online at www.nice.org.uk/guidance/hst11.

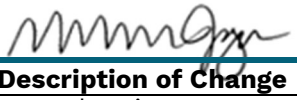
Development of utilization management criteria may also involve research into other state Medicaid programs, other payer policies, consultation with experts and review by the Medicaid Clinical Advisory Committee (CAC). These sources may not be referenced individually unless they are specifically published and are otherwise applicable to the criteria at issue.

Criteria Change History

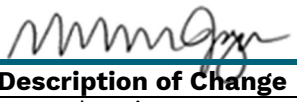
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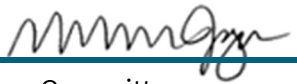
Change Date	Changed By	Description of Change	Version
04/17/2026	CAC	Annual review. No changes.	6
Signature William (Bill) Jagiello, DO			

Change Date	Changed By	Description of Change	Version
04/18/2025	CAC	Annual review. Replaced C9770 with 0810T (subretinal injection). Updated references.	5
Signature William (Bill) Jagiello, DO			

Change Date	Changed By	Description of Change	Version
04/19/2024	CAC	Annual review. Corrected NDC in Billing and Coding Information.	4
Signature William (Bill) Jagiello, DO			

Change Date	Changed By	Description of Change	Version
04/21/2023	CAC	Annual review. Updated references. Added C9770 to code section *(C9770: Vitrectomy, mechanical, pars plana approach, with subretinal injection of pharmacologic/ biologic agent).*	3
Signature William (Bill) Jagiello, DO			

Change Date	Changed By	Description of Change	Version
04/15/2022	CAC	Annual review.	2
Signature William (Bill) Jagiello, DO			

Change Date	Changed By	Description of Change	Version
04/16/2021	CAC	Criteria implementation.	1
Signature William (Bill) Jagiello, DO			

CAC = Medicaid Clinical Advisory Committee