READ THIS FOR SAFE AND EFFECTIVE USE OF YOUR MEDICINE

PATIENT MEDICATION INFORMATION

PrLUXTURNA®
[Lucks-turn-a]

voretigene neparvovec

Read this carefully before you start taking **LUXTURNA®**. This leaflet is a summary and will not tell you everything about this drug. Talk to your healthcare professional about your medical condition and treatment and ask if there is any new information about **LUXTURNA**.

What is LUXTURNA?

LUXTURNA is a gene therapy product that contains the active substance voretigene neparvovec.

LUXTURNA will be given to you only if genetic testing shows that your vision loss is caused by confirmed biallelic mutations in the *RPE65* gene.

What is LUXTURNA used for?

LUXTURNA is used for the treatment of adults and children with vision loss due to inherited retinal dystrophy caused by mutations in the *RPE65* gene. These mutations prevent the body from producing a protein needed for vision which can lead to loss of sight and eventual blindness.

How does LUXTURNA work?

The active substance in LUXTURNA, voretigene neparvovec, is a modified virus that contains a working copy of the *RPE65* gene. After injection it delivers this gene into the cells of the retina, the layer at the back of the eye that detects light. This enables the retina to produce the proteins needed for vision. The virus used to deliver the gene does not cause disease in humans.

If you have any questions about LUXTURNA, how it works or why this medicine has been prescribed for you, ask your doctor.

What are the ingredients in LUXTURNA?

Medicinal ingredients: Voretigene neparvovec
Non-medicinal ingredients: *Concentrate and Diluent*: Disodium hydrogen phosphate dihydrate (for pH adjustment), sodium chloride, sodium dihydrogen phosphate monohydrate (for pH adjustment), poloxamer 188, water for injections.

LUXTURNA contains no preservatives.

LUXTURNA comes in the following dosage form:

LUXTURNA concentrate and the diluent are both clear, colorless liquids. LUXTURNA vial contains 5×10^{12} vector genomes (vg) per mL of voretigene neparvovec.

Do not use LUXTURNA if:

- you are allergic (hypersensitive) to voretigene neparvovec, any of the other ingredients of LUXTURNA or component of the container.
- you have an eye infection.
- you have an eye inflammation.

To help avoid side effects and ensure proper use, talk to your healthcare professional before starting the treatment. Talk about any health conditions or problems you may have, including if you:

- think you may be allergic.
- have signs of an eye infection or eye inflammation, for example if you have eye redness, sensitivity to light, eye swelling or eye pain.
- have an active infection of any sort. Your doctor may delay your treatment until your infection is gone because this medicine may make it more difficult for you to fight an infection. See also "**How LUXTURNA is given**".

Warnings and precautions you should know about:

LUXTURNA will be injected into your eye in an operating room by surgeons experienced in performing eye surgery.

After receiving LUXTURNA:

- Get immediate care from your doctor if your eye or eyes become red, painful, sensitive to light, you see flashes or floaters in your vision, or if you notice any worsening or blurred vision.
- Permanent decline in visual acuity may occur following subretinal injection of LUXTURNA. Contact your doctor or pharmacist if you experience any changes in vision.
- You should rest laying on your back as much as possible for 24 hours after discharge.
- You should avoid air travel or travel to high elevations until advised by your doctor. During treatment with this medicine, the doctor inserts an air bubble in the eye, which is slowly absorbed by your body. Until the bubble is fully absorbed, air travel or travel to high elevations may make the bubble expand and lead to eye damage, including vision loss. Please talk to your doctor before traveling.
- You should avoid swimming because of an increased risk of infection in the eye. Please talk to your doctor before you resume swimming.
- Some people develop cataracts. A cataract is clouding of the natural lens inside the eye that can make it harder to see clearly. The development or worsening of cataracts is a known complication of the eye surgery that will be required before you receive LUXTURNA. There is an additional risk of cataract if the lens inside the eye is damaged by the needle used to inject the medicine into the back of the eye.
- You and your caregiver, especially if pregnant, breastfeeding or with a suppressed immune system, should wear gloves during dressing changes and when disposing of the dressings and other waste material. Follow these precautions for 14 days after the treatment.
- You and your caregiver should place any used dressings and waste material with tears and nasal secretions in sealed bags before disposing of them. You and your caregiver should follow these precautions for 14 days.
- You will not be able to donate blood, organs, tissues and cells for transplantation. This is

because LUXTURNA is a gene therapy product.

Children (below 4 years of age)

LUXTURNA has not been studied in children under four years of age.

Pregnancy and breast-feeding

If you are pregnant or breastfeeding, think you might be pregnant, or are planning to have a baby, ask your doctor or nurse for advice before being treated with LUXTURNA.

The effects of this medicine on pregnancy and your unborn child are not known. As a precaution, you should not receive LUXTURNA while you are pregnant.

It is not known whether LUXTURNA passes into breast milk. Ask your doctor whether you should stop breastfeeding after receiving LUXTURNA.

Tell your healthcare professional about all the medicines you take, including any drugs, vitamins, minerals, natural supplements or alternative medicines.

How LUXTURNA is given:

LUXTURNA will be injected into your eye in an operating room by surgeons experienced in performing eye surgery.

LUXTURNA is given under anesthesia. Your doctor will talk to you about the anesthesia and how it will be given to you.

Your doctor will carry out eye surgery to remove the clear gel inside the eye, and then inject LUXTURNA directly under your retina, the thin light-sensing layer at the back of that eye. This may be repeated in your other eye at least 6 days afterwards. You will need to stay for post-operative observation for a few hours after each procedure to monitor your recovery and watch for any side effects from the surgery or the anesthesia.

Before LUXTURNA treatment is started, your doctor may prescribe a medicine that will suppress your immune system (the body's natural defenses) so that it will not try to fight the LUXTURNA when it is given. It is important that you take this medicine according to the instructions given. Do not stop taking the medicine without first talking to your doctor.

If you have any further questions on the use of this medicine, **ask your doctor**.

Usual dose:

You will receive a single dose of 1.5×10^{11} vg of LUXTURNA in each eye. Each dose will be injected directly under your retina in a total volume of 0.3 mL. LUXTURNA is given to each eye on separate days, at least 6 days apart.

Overdose:

If you think you have been given too much LUXTURNA, contact your healthcare professional, hospital emergency department or regional poison control centre immediately, even if there are no symptoms.

What are possible side effects from using LUXTURNA?

As with all medicines, patients treated with LUXTURNA may experience side effects, although not everybody gets them. The side effects associated with the administration of LUXTURNA are either due to the medicine itself, the injection procedure, or the use of corticosteroids and mostly affect the eye. If you experience any side effects not listed here, contact your healthcare professional.

If these side effects become severe, please tell your doctor.

Very common: *may affect more than 1 in 10 people*
- Redness of the eye
- Cataract (clouding of the lens)
- Increased pressure in the eye

Common: *may affect up to 1 in every 10 people*
- Deposits under the retina
- Break in the retina (retinal tear)
- Abnormalities in the back of the eye
- Thinning of the surface of the eye (dellen)
- Eye pain
- Eye swelling
- Eye irritation
- Eye inflammation
- Foreign body sensation in the eye
- Detachment of the retina

Damage to the tissues of the eye may be accompanied by bleeding and swelling and an increased risk of infection. There is reduced vision in the days after surgery that usually improves; tell your doctor if vision does not return.

Serious side effects and what to do about them			
Symptom / effect	Talk to your healthcare professional		Get immediate medical help
	Only if severe	In all cases	
COMMON Inflammation, infection or allergic reaction of the eye: • a sudden decrease or change in vision, • an increase in pain, discomfort or redness in your eye.			X

If you have a troublesome symptom or side effect that is not listed here or becomes bad enough to interfere with your daily activities, talk to your healthcare professional.

Reporting Side Effects

You can report any suspected side effects associated with the use of health products to Health Canada by:

- Visiting the Web page on Adverse Reaction Reporting (http://www.hc-sc.gc.ca/dhp-mps/medeff/report-declaration/index-eng.php) for information on how to report online, by mail or by fax; or
- Calling toll-free at 1-866-234-2345.

NOTE: Contact your health professional if you need information about how to manage your side effects. The Canada Vigilance Program does not provide medical advice.

Storage:

LUXTURNA will be stored by the healthcare professionals at your healthcare facility. You will not store LUXTURNA yourself.

If you want more information about LUXTURNA:

- Talk to your healthcare professional.
- Find the full product monograph that is prepared for healthcare professionals and includes this Patient Medication Information by visiting the Health Canada website; the manufacturer's website http://www.novartis.ca or by calling 1-800-363-8883.

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