



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Luxturna (*voretigene neparvovec*)

An overview of Luxturna and why it is authorised in the EU

What is Luxturna and what is it used for?

Luxturna is a medicine that is used to treat adults and children with loss of vision due to inherited retinal dystrophy, a rare genetic disorder of the retina (the light sensitive membrane at the back of the eye).

Luxturna can only be used while patients still have enough functioning cells left in the retina and when the disease is caused by mutations (changes) in the gene *RPE65*. *RPE65* is responsible for the production of an enzyme called all-trans retinyl isomerase, which is necessary for the normal functioning of retinal cells.

Luxturna contains the active substance voretigene neparvovec and is a type of advanced therapy medicine called a 'gene therapy product'. This is a type of medicine that works by delivering genes into the body.

Inherited retinal dystrophy is rare, and Luxturna was designated an 'orphan medicine' (a medicine used in rare diseases) for two forms of the disease on various dates (retinitis pigmentosa: 28 July 2015; Leber's congenital amaurosis: 2 April 2012). Further information on the orphan designations can be found on the European Medicines Agency's website ema.europa.eu/medicines/human/orphan-designations.

How is Luxturna used?

Luxturna can only be obtained with a prescription and treatment should be given by a surgeon experienced in performing eye surgery.

Luxturna is given as a single injection into the back of each eye, under the retina. The second eye should be treated at least 6 days after the first. Patients should start receiving immunosuppressant medicines (medicines that reduce the activity of the immune system, the body's natural defences) 3 days before Luxturna is injected into the first eye, to lower the risk of the medicine being rejected by the body, and this treatment should continue for 14 days after injection.

For more information about using Luxturna, see the package leaflet or contact your doctor or pharmacist.



How does Luxturna work?

Luxturna consists of a virus that contains normal copies of the *RPE65* gene. When Luxturna is injected into the eye the virus carries the *RPE65* gene into the retinal cells and enables them to produce the missing enzyme. This helps the cells in the retina to function better, slowing down the progression of the disease.

The type of virus used in this medicine (adeno-associated virus) does not cause disease in humans.

What benefits of Luxturna have been shown in studies?

Luxturna was investigated in one main study involving 31 patients with inherited retinal dystrophy due to *RPE65* mutations. The main measure of effectiveness was how well patients performed in a mobility test, where they were required to navigate a route with turns and obstacles under various light settings. Patients were considered to have passed the test if they completed the route within 3 minutes and bumped into no more than 3 obstacles.

After one year of treatment, patients treated with Luxturna improved their scores by 1.8 points, while patients who were not given Luxturna improved their scores by 0.2 points, meaning that patients treated with Luxturna were able to move better along the route. Additionally, 13 of the 21 patients (62%) treated with Luxturna passed the mobility test at the lowest light level of 1 lux (similar to conditions of a poorly lit pavement at night), while none of the patients not given the medicine were able to do so. The improvement in patients' vision was maintained for at least three years.

What are the risks associated with Luxturna?

The most common side effects with Luxturna (which may affect more than 1 in 20 people) are conjunctival hyperaemia (increased blood supply to the eye, leading to redness of the eye), cataract (clouding of the lens of the eye) and increased pressure inside the eye. For the full list of side effects of Luxturna, see the package leaflet.

Luxturna must not be used in patients with eye infection or inflammation. For the full list of restrictions, see the package leaflet.

Why is Luxturna authorised in the EU?

Luxturna has been shown to improve patients' vision and ability to move around obstacles, particularly in dim light, and is expected to improve their quality of life. This was considered an important clinical benefit, taking into account the lack of authorised treatments for this progressive, degenerative condition. The safety of Luxturna was considered acceptable and side effects manageable. Therefore, the European Medicines Agency decided that Luxturna's benefits are greater than its risks and it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Luxturna?

The company that markets Luxturna will set up an educational programme and prepare educational material aimed at doctors and pharmacists expected to use and handle Luxturna, in order to ensure the correct use of the medicine and minimise the risks associated with the medicine and its administration. Luxturna will only be available from centres where the educational programme is in place. An information package for patients and their carers will also be provided.

In addition, the company will have to follow-up all patients who received Luxturna in the main studies for 15 years, in order to characterise the long-term effectiveness and safety of the medicine, and establish a registry to collect long-term safety data in patients treated with Luxturna.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Luxturna have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Luxturna are continuously monitored. Side effects reported with Luxturna are carefully evaluated and any necessary action taken to protect patients.

Other information about Luxturna

Luxturna received a marketing authorisation valid throughout the EU on 22 November 2018.

Further information on Luxturna can be found on the Agency's website:

ema.europa.eu/medicines/human/EPAR/luxturna.

This overview was last updated in 11-2018.