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Luxturna® (active substance: voretigene neparvovec)

First authorisation in Switzerland: 14.02.2020

Gene therapy medicinal product for the treatment of adults and children with inherited retinal dystrophy

About the medicinal product

Luxturna is a gene therapy medicinal product containing the active substance voretigene neparvovec. It was authorised on 14 February 2020 in Switzerland for the treatment of adults and children with vision loss due to inherited retinal dystrophy. Retinal dystrophy is a rare inherited abnormality of the retina caused by a mutation in the RPE65 gene. This genetic change prevents the body from forming a protein that is necessary for vision. The reduction in this protein leads to the loss of vision and, ultimately, to blindness.

Mode of action

The active substance in Luxturna, voretigene neparvovec, is a modified virus containing a copy of the RPE65 gene. After Luxturna is injected into the back of the eye, the virus transports this gene to the cells of the retina. The retina is the cell layer responsible for light detection. The gene enables the retina to form the proteins that are required for vision.

The virus, which transports the gene to the cells, does not cause any illnesses in humans.

Administration

Luxturna can be administered only if the patients still possess sufficient viable retinal cells. Treatment with Luxturna can be considered only if a genetic test confirms that the loss of vision is caused by a mutation in the RPE65 gene.
Luxturna is injected under the retina and may be administered only in a university hospital with a qualified treatment centre by a surgeon experienced in intraocular surgery. The procedure is performed under anaesthesia. Each eye is treated separately, with a mandatory interval of at least 6 days between each eye injection.

Meticulous long-term follow-up by specialist ophthalmologists is essential.

Efficacy

The efficacy of Luxturna was investigated in a total of 41 patients, all of whom were suffering from inherited retinal dystrophy. 11 adults (36%) and 20 children from 4 years of age (64%) took part in the main study. The average age was 15 years.

To demonstrate the efficacy of Luxturna, the parameter of "functional vision" was measured in the main study. Functional vision comprises visual acuity, visual field and the ability to perceive and/or see in dim light. For this assessment, the study participants navigated a course as accurately as possible and at a reasonable pace at different levels of environmental illumination.

After one year of treatment, the patients treated with Luxturna achieved a higher score than those who did not receive Luxturna. In other words, the patients treated with Luxturna were able to complete the course more accurately, faster and at lower light levels, indicating that an improvement in functional vision was obtained with the Luxturna treatment. This improvement was maintained over a period of at least three years.

Precautions, undesirable effects & risks

Luxturna may not be used in the event of hypersensitivity to the active substance or any of the excipients, or an infection or inflammation in or around the eye.

The most common side effects connected with Luxturna treatment are conjunctival hyperaemia (increased supply of blood to the eye leading to reddening of the eye), cataract (clouding of the lens of the eye), increased intraocular pressure, retinal tear, corneal dellen, macular hole (macular foramen), subretinal deposits, eye inflammation, eye irritation, eye pain, wrinkling on the surface of the macula, headache, nausea.

Temporary visual disturbances may occur after a subretinal injection.

Eye infections can lead to a permanent decline in visual acuity.

All precautions, risks and other possible undesirable effects are listed in the Information for healthcare professionals and the Patient information.

Why the medicinal product has been authorised

On the basis of the available evaluation results, Swissmedic has concluded that the benefit-risk profile for the medicinal product Luxturna with the active substance voretigene neparvovec is positive. The lack of treatment options for patients suffering from inherited retinal dystrophy is an argument in favour of authorisation in Switzerland. The prevention of disease progression, even in patients with advanced retinal dystrophy, is also an advantage. Whether the effect of Luxturna is maintained over many years and whether blindness can be prevented in the long term remains to be seen.

A 15-year follow-up of the 41 patients who took part in the pivotal study is in progress. A further 5-year study on the safety of Luxturna is a condition for the authorisation in Switzerland.

After considering all the above-mentioned points, Swissmedic has authorised the medicinal product Luxturna with the active substance voretigene neparvovec for the described indication in Switzerland.

Further information on the medicinal product

Information for healthcare professionals:
Information for healthcare professionals Luxturna®

Information for patients (package leaflet):
Patient information Luxturna®

This information is correct as at the date above. New information concerning the authorised medicinal product in question will not be incorporated into the Public Summary SwissPAR.

Swissmedic monitors medicinal products authorised in Switzerland. Swissmedic initiates the necessary action in the event of newly discovered adverse drug reactions or other safety-relevant signals.

New findings that could impair the quality, efficacy or safety of this medicinal product are recorded and published by Swissmedic. If necessary, the medicinal product information is adapted.