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Media and Public Relations

Press release

New gene therapy for rare inherited disorder causing vision loss recommended for approval

Luxturna is the first treatment option for hereditary retinal dystrophy with mutations of the RPE65 gene

The European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has recommended granting a marketing authorisation for the gene therapy Luxturna (voretigene neparvovec), for the treatment of adults and children suffering from inherited retinal dystrophy caused by RPE65 gene mutations, a rare genetic disorder which causes vision loss and usually leads to blindness.

The mutations of the RPE65 gene, which encodes one of the enzymes involved in the biochemistry of light capture by the cells of the retina, hinder the patient's ability to detect light. It is a severely debilitating disease, characterised by a progressive loss of vision. Most patients will be blind by the time they are young adults. There is currently no treatment for this disease; support to patients is limited to measures allowing the management of the disease such as aids for low vision.

Luxturna is meant for patients with confirmed biallelic mutations of the RPE65 gene (i.e. patients who have inherited the mutation from both parents) and who have sufficient viable retinal cells. It is the first gene therapy to be recommended for approval for a retinal disease. Luxturna works by delivering a functional RPE65 gene into the cells of the retina through a single retinal injection, which restores the production pathway for the required enzyme thereby improving the patient's ability to detect light.

Luxturna was studied in 41 patients. In the main clinical trial supporting the approval of Luxturna, patients treated with the medicine showed a significant improvement of night vision, one of the typical symptoms of the disease, after one year, while no improvement was seen in the control group. The most common side effects were conjunctival hyperaemia (eye redness), cataracts and increased intraocular pressure.

Given the novelty of the treatment and the limited number of treated patients, the CHMP requires the company to ensure the long-term follow-up of patients to confirm Luxturna's continuing efficacy and safety. Follow-up studies were agreed, including a post-authorisation safety study (PASS) based on a disease registry in patients with vision loss due to inherited retinal dystrophy caused by confirmed biallelic RPE65 mutations, as well as a 15-year follow-up programme of efficacy and safety outcomes

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for all patients treated in the clinical programme.

The CHMP's opinion is based on the assessment by EMA's expert committee on Advanced Therapy Medicinal Products (ATMPs), the Committee for Advanced Therapies (CAT). Luxturna was designated as an orphan medicine and an ATMP and EMA provided protocol assistance to the applicant during the development of the medicine.

The opinion adopted by the CHMP is an intermediary step on Luxturna's path to patient access. The opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once the marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Luxturna is Spark Therapeutics Ireland Ltd.
3. As always at time of approval, EMA's Committee for Orphan Medicinal Products (COMP) will review the orphan designation to determine whether the information available to date allows maintaining Luxturna's orphan status and granting this medicine ten years of market exclusivity.
4. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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