

Medicine: voretigene neparvovec (brand name: Luxturna[®]) for inherited retinal dystrophy due to biallelic *RPE65* mutations

Novartis Pharmaceuticals UK Ltd

Voretigene neparvovec meets the SMC definition of an ultra-orphan medicine, which is a medicine to treat an extremely rare condition. This document summarises the initial SMC assessment of voretigene neparvovec.

What does this mean for patients?

If your healthcare professional thinks that voretigene neparvovec is the right medicine for you, you should be able to have the treatment on the NHS in Scotland within the **ultra-orphan pathway** (see next page).

This is provided the company submits a plan to the Scottish Government describing how further data, including on the patient and carer lived experience, will be collected over the next 3 years. After this, SMC will reassess the medicine and make a decision on routine availability.



What is voretigene neparvovec used for?

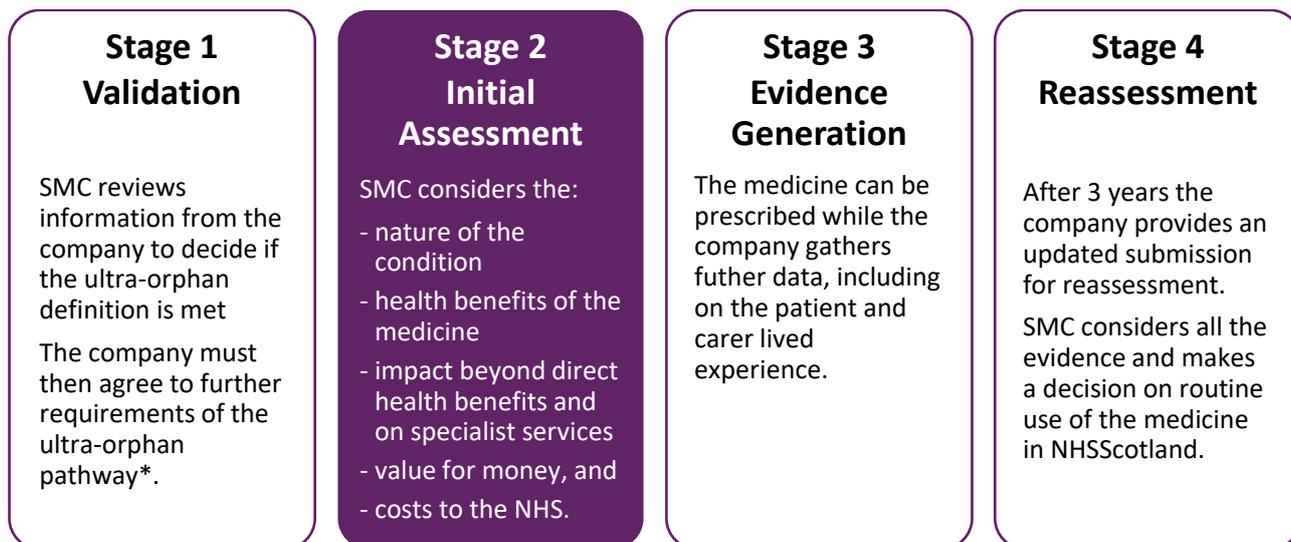
Voretigene neparvovec is used to treat adults and children with loss of vision due to inherited retinal dystrophy, which is a rare genetic condition that affects the light sensing cells at the back of the eye (retina). Voretigene neparvovec is for use in patients who still have enough working cells left in the retina and when the disease is caused by mutations (genetic changes) in the *RPE65* gene (a gene that is necessary to make a protein needed for vision) which are biallelic (passed down from both parents).

How does voretigene neparvovec work?

Voretigene neparvovec is a type of medicine called a gene therapy. It is given as a one-off treatment in each eye and allows a functional (working) *RPE65* gene to act in place of a mutated *RPE65* gene. This helps the cells in the retina to function better, slowing down the progression of the disease.

How do we assess ultra-orphan medicines?

SMC uses a broad assessment framework for ultra-orphan medicines. This is part of a new ultra-orphan pathway in NHSScotland which has four stages:



*provide a confidential discount known as a Patient Access Scheme (PAS) to increase the cost-effectiveness of the medicine, and provide a data collection plan.

What have we said in this assessment?

- Clinical studies show that voretigene neparvovec improves vision compared with no treatment. However currently it is unclear how long this benefit lasts and how it impacts on overall quality of life.
- Despite the confidential discount (PAS) offered by the company, the cost in relation to the health benefits of voretigene neparvovec remains high.

For further information please see the SMC ultra-orphan medicine initial assessment report (SMC2228).



More information

The organisations below can provide more information and support for people with inherited retinal dystrophy and their families. SMC is not responsible for the content of any information provided by external organisations.

Retina UK



www.retinauk.org.uk



0300 111 4000

Fight for Sight



www.fightforsight.org.uk

You can find out more about voretigene neparvovec in the European public assessment report (EPAR) summary for the public by searching for the medicine name on the European Medicines Agency (EMA) website.



www.ema.europa.eu