

FAQs: NICE recommends Luxturna for use in the NHS in England

Why is this in the news?

It has been announced that the National Institute for Health and Care Excellence (NICE) has recommended Luxturna (*voretigene neparvovec*) for use in the NHS in England, making it the first available treatment for an inherited retinal dystrophy.

Luxturna, a gene therapy, is only for the treatment of Leber congenital amaurosis type 2 (LCA2) and retinitis pigmentosa caused by mutations in a specific gene called RPE65.

Who will be able to access the treatment?

People must have two faulty copies of RPE65, confirmed by genetic testing, as well as reasonable numbers of remaining viable retinal cells, in order to benefit from this treatment. Due to the severe, early-onset nature of this type of inherited retinal disease, eligible patients are likely to be children or young adults.

The NHS in England and Wales is legally obliged to fund medicines and treatments NICE recommends. NICE will publish its final guidance document in mid-October and we expect the treatment to be available from early 2020 in three treatment centres around the UK.

What about Scotland, Wales and Northern Ireland?

The NHS in Wales will follow the NICE recommendation and will be making Luxturna available at the beginning of 2020. We understand Northern Ireland is also likely to follow England's lead. Luxturna could be made available in Scotland in 2020; the Scottish Medicines Consortium will make a decision on this in December 2019.

Will this treatment restore my vision?

Clinical trial evidence shows that, in the short term, Luxturna can improve vision and prevent the condition from getting worse. In particular, the trial showed improvements in low light vision and mobility. There is no long-term clinical evidence, but it is biologically plausible that the treatment effect is likely to continue for a considerable time.

How can I find out if my child or I am eligible for treatment with Luxturna?

You will need to see your ophthalmologist in the first instance; if you don't currently have an ophthalmologist, your GP can refer you. The ophthalmologist may refer you for tests to check whether you have sufficient healthy retinal cells for the treatment to work. Luxturna provides healthy copies of the RPE65 gene but relies on retinal cells using their own molecular machinery to use these new genetic instructions; it won't help in cells that have already degenerated. Your ophthalmologist will also need to arrange for you to have a genetic test if you don't already know which gene is causing your sight loss, and the results for this may take some time. The test must show that you have two faulty copies of RPE65.

Around 86 people are likely to be eligible for treatment in England.

If I'm eligible, what happens next?

Your ophthalmologist will refer you to a treatment centre.

Luxturna is administered via an injection into the retina, which takes place under general anaesthetic. The treatment only needs to be given once but each eye is treated separately, with at least a week between the two operations; the treating ophthalmic surgeon will discuss with you how long this gap should be. You will then need several follow up appointments at the treatment centre.

There is a risk of side effects, including further sight loss. These will be explained to you at the treatment centre.

After treatment, is it still possible for me to pass on the faulty gene to children I might have in the future?

Yes. Your genetic code will still contain the error that caused your condition in the first place. Luxturna will provide your retina with the instructions to make healthy RPE65 protein but it will not change the underlying fault in your DNA that is present in every cell throughout your body.

We would suggest you speak with your GP or a genetic counsellor if you are thinking of starting a family and would like more information.

What role had Retina UK played in the process?

Retina UK had been involved throughout the NICE decision-making process and worked hard to ensure its community's voice was heard. The Retina UK community played a critical role in influencing NICE's decision.

We were able to present the decision-making committee with a number of findings on the burden of disease from our recent Sight Loss Survey, completed by almost 1,000 of our community.

The committee's evaluation document specifically quotes our survey's findings on mental health impact and concludes: 'The committee acknowledged that RPE65- mediated IRD is a rare, serious and debilitating condition that severely affects the lives of patients, families and carers.'