Prospective study on safety and efficacy of gene therapy with voretigene neparvovec (Luxturna®) in patients with RPE65-associated inherited retinal degenerations

No registrations found.

Ethical review	Positive opinion
Status	Pending
Health condition type	-
Study type	Observational non invasive

Summary

ID

NL-OMON22976

Source
NTR

Brief title
Luxturna® Follow Up

Health condition

RPE65-associated inherited retinal degenerations (IRDs).

Sponsors and support

Primary sponsor: The Rotterdam Eye Hospital
Source(s) of monetary or material Support: Stichting Wetenschappelijk Onderzoek Het Oogziekenhuis – Prof. dr. H.J. Flieringa

Intervention

Outcome measures

Primary outcome

Full-field stimulus testing (FST) at 1 year.

Secondary outcome

Multi-luminance mobility test (MLMT) .
Best corrected visual acuity.
Perimetry, kinetic (all patients) and static (if feasible).

Study description

Background summary

Rationale: RPE65-associated inherited retinal degenerations (IRDs) are rare, and account for 5−10% of all autosomal recessive childhood-onset IRDs. Visual function of these patients can vary early in life, but inevitably deteriorates towards blindness. Gene therapy with voretigene neparvovec (Luxturna®) was approved by the US Food and Drug Administration (FDA) in 2017 and by the European Medicines Agency (EMA) in 2018.
Objective: To collect long-term, real world data on safety and efficacy of gene therapy with voretigene neparvovec (Luxturna®).
Study design: Multi-center prospective, observational study; follow-up (FU): 5 years.
Study population: Patients with bi-allelic RPE65 mutations treated with voretigene neparvovec.
Intervention: The treatment with voretigene neparvovec itself is not considered as an intervention for the purpose of this study.
Main study parameters/endpoints: Full-field stimulus testing (FST) at 1 year.

Study objective

Voretigene neparvovec (Luxturna®) improves visual function.

Study design

Baseline, day 30, 90, 180, and year 1 to 5.

Intervention

Treatment with voretigene neparvovec is not considered as an intervention for the purpose of this study.

Contacts

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Eligibility criteria

Inclusion criteria

- Be able to cooperate.
- Informed consent.
- Clinical diagnosis of IRD with confirmed bi-allelic RPE65 mutations.
- Clinical evidence of viable retinal tissue (RPE cells, photoreceptors, and downstream ganglion cells) as target. Patients have to have more than one characteristic:
1. total retinal thickness > 100 µm in the posterior pole (OCT).
2. area without atrophy of at least three disc diameters (funduscopy).
3. residual island in the central visual field (within 30º of central fixation; Goldmann).
- Recordable FST.
- Scheduled to receive treatment with Luxturna®.

Exclusion criteria

- None specified.

Study design

Design

Study type: Observational non invasive

Intervention model:	Other
Allocation:	Non controlled trial
Masking:	Open (masking not used)
Control:	N/A , unknown

Recruitment

NL

Recruitment status:	Pending
Start date (anticipated):	22-03-2021
Enrollment:	20
Type:	Anticipated

IPD sharing statement

Plan to share IPD: Undecided

Ethics review

Positive opinion

Date:	26-02-2021
Application type:	First submission

Study registrations

Followed up by the following (possibly more current) registration

ID: 50929
Bron: ToetsingOnline
Titel:

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
NTR-new	NL9321
CCMO	NL76234.000.21
OMON	NL-OMON50929

Study results