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

Published: 26-01-2021
Last updated: 15-05-2024

To collect long-term, real world data on safety and efficacy of gene therapy with voretigene neparvovec (Luxturna®).

Ethical review	Approved WMO
Status	Recruiting
Health condition type	Congenital eye disorders (excl glaucoma)
Study type	Observational non invasive

Summary

ID

NL-OMON50929

Source
ToetsingOnline

Brief title
Luxturna® Follow Up

Condition

- Congenital eye disorders (excl glaucoma)

Synonym
RPE65-associated inherited retinal degenerations

Research involving
Human

Sponsors and support

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

Intervention

Keyword: efficacy, gene therapy, RPE65 mutations, voretigene neparvovec

Outcome measures

Primary outcome

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

Secondary outcome

Visual acuity

Visual field

Mobility course

Study description

Background summary

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.

Study 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 burden and risks

Assessments in this study are part of the clinical routine. Extra time for each MLMT will be 4h; risks are negligible.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)
Elderly (65 years and older)

Inclusion criteria

Scheduled to receive treatment with Luxturna®

Informed consent

Exclusion criteria

None

Study design

Design

Study type: Observational non invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	15-03-2021
Enrollment:	20
Type:	Actual

Ethics review

Approved WMO Date:	26-01-2021
Application type:	First submission
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	25-02-2021
Application type:	First submission
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

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

ID: 22976
Source: NTR
Title:

In other registers

Register	ID
EudraCT	EUCTR2021-000265-33-NL
CCMO	NL76234.000.21
OMON	NL-OMON22976