

A Post-Authorization, Multicenter, Multinational, Longitudinal, Observational Safety Registry Study for Patients Treated with Voretigene Neparvovec

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Study

Ongoing

Administrative details

EU PAS number

EUPAS31153

Study ID

46265

DARWIN EU® study

No

Study countries

- Argentina
- Australia
- Austria

- Belgium
- Brazil
- Bulgaria
- Canada
- Croatia
- Czechia
- Denmark
- France
- Germany
- Greece
- Hungary
- Israel
- Italy
- Japan
- Korea, Democratic People's Republic of
- Netherlands
- Norway
- Poland
- Portugal
- Singapore
- Slovakia
- Spain
- Sweden
- Switzerland
- Taiwan
- United Arab Emirates
- United Kingdom

Study description

This is a global (ex-US) non-interventional registry-based, post-authorization study (PASS) in pediatric and adult patients who have received Luxturna® sub-retinal injections in a real-world setting. Patients will be treated according to the local prescribing information and routine medical practice.

The study will collect all AEs and SAEs including AEs of special interest (AESIs), information about pregnancy occurrence and outcomes, and ophthalmic examination results.

A five year enrollment duration is expected to provide a minimum of 40 participants who are to be followed annually for 5 years.

Extended follow up until the end of study will be required for patients who will develop chorioretinal atrophy.

Study status

Ongoing

Research institutions and networks

Institutions

[Novartis Pharmaceuticals](#)

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[Institution](#)

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Novartis Clinical Disclosure Officer

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 22/11/2018

Actual: 22/11/2018

Study start date

Planned: 31/12/2019

Actual: 18/12/2019

Data analysis start date

Planned: 01/11/2029

Date of interim report, if expected

Planned: 04/04/2020

Actual: 03/04/2020

Date of final study report

Planned: 12/04/2030

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Novartis

Study protocol

[LTW888A12401 PASS Protocol_July 2019_Redacted_21 Aug 2019.pdf](#) (861.51 KB)

[Protocol Amendment_-_V03_Redacted_09 Jun 2025.pdf](#) (650.46 KB)

[CLTW888A12401-v02--protocol_Redacted.pdf](#) (980.31 KB)

Regulatory

Was the study required by a regulatory body?

Yes

Is the study required by a Risk Management Plan (RMP)?

EU RMP category 1 (imposed as condition of marketing authorisation)

Other study registration identification numbers and links

CLTW888A12401

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

Main study objective:

The objective of this post-authorization observational study is to collect long-term safety information (i.e. for 5 years after treatment) associated with voretigene neparvovec (vector and/or transgene), its subretinal injection procedure, the concomitant use of corticosteroids, or a combination of these procedures and products.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

LUXURNA

Study drug International non-proprietary name (INN) or common name

Anatomical Therapeutic Chemical (ATC) code

(S01XA27) voretigene neparvovec

voretigene neparvovec

Medical condition to be studied

Hereditary retinal dystrophy

Population studied

Age groups

- Children (2 to < 12 years)
- Adolescents (12 to < 18 years)
- Adults (18 to < 46 years)
- Adults (46 to < 65 years)
- Adults (65 to < 75 years)
- Adults (75 to < 85 years)
- Adults (85 years and over)

Estimated number of subjects

40

Study design details

Outcomes

Frequency of adverse events, serious adverse events and adverse events of special interest, Pregnancy outcomes, and visual function measures.

Data analysis plan

This study is intended to descriptively document the frequency and severity of events of interest related to voretigene neparvovec (vector and/or transgene), the subretinal injection procedure, the concomitant use of corticosteroids, or a combination of these procedures and products.

The Full Analysis Set (FAS), all enrolled individuals with a signed informed consent/assent, will be used to summarize all data.

Continuous variables will be summarized in terms of mean, standard deviation, median, minimum and maximum (and other descriptive statistics when appropriate).

Categorical variables will be summarized using frequency counts and percentages. Unless otherwise noted, the denominator for the percentages will be the FAS population.

Demographics and pretreatment/baseline disease characteristics will be summarized.

Ophthalmic examination and patient/caregiver questionnaire results will be presented at pre-treatment/baseline and post-administration/annually.

Data management

ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025.

The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

Data sources

Data sources (types)

Non-interventional study

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No