

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

**First published:** 29/08/2019

**Last updated:** 02/07/2024

Study

Ongoing

## Administrative details

### PURI

<https://redirect.ema.europa.eu/resource/46265>

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### EU PAS number

EUPAS31153

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### Study ID

46265

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### DARWIN EU® study

No

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## Study countries

- ☐ Argentina
  - ☐ Austria
  - ☐ Brazil
  - ☐ Canada
  - ☐ Denmark
  - ☐ Finland
  - ☐ France
  - ☐ Germany
  - ☐ Israel
  - ☐ Italy
  - ☐ Korea, Democratic People's Republic of
  - ☐ Mexico
  - ☐ Netherlands
  - ☐ Norway
  - ☐ Russian Federation
  - ☐ Saudi Arabia
  - ☐ Singapore
  - ☐ Sweden
  - ☐ Switzerland
  - ☐ Taiwan
  - ☐ Türkiye
  - ☐ United Arab Emirates
  - ☐ United Kingdom
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## 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.

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### **Study status**

Ongoing

## Research institutions and networks

### Institutions

#### **Novartis Pharmaceuticals**

**First published:** 01/02/2024

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**Institution**

## Contact details

### **Study institution contact**

Novartis Clinical Disclosure Officer

**Study contact**

[trialandresults.registries@novartis.com](mailto:trialandresults.registries@novartis.com)

### **Primary lead investigator**

Novartis Clinical Disclosure Officer

## Study timelines

### **Date when funding contract was signed**

Planned: 22/11/2018

Actual: 22/11/2018

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### **Study start date**

Planned: 31/12/2019

Actual: 18/12/2019

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### **Data analysis start date**

Planned: 01/11/2029

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### **Date of interim report, if expected**

Planned: 04/04/2020

Actual: 03/04/2020

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### **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)

[CLTW888A12401-v02--protocol\\_Redacted.pdf](#)(980.31 KB)

## Regulatory

### **Was the study required by a regulatory body?**

Yes

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### **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 type:**

Non-interventional study

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#### **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

**Name of medicine**

LUXTURNA

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

VORETIGENE NEPARVOVEC

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**Anatomical Therapeutic Chemical (ATC) code**

(S01XA27) voretigene neparvovec

voretigene neparvovec

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**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)

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## **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.

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## **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

### Data sources

#### Data sources (types)

Other

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#### Data sources (types), other

Prospective patient-based data collection

### Use of a Common Data Model (CDM)

#### CDM mapping

No

### Data quality specifications

#### Check conformance

Unknown

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#### Check completeness

Unknown

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**Check stability**

Unknown

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**Check logical consistency**

Unknown

Data characterisation

**Data characterisation conducted**

No