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

EU PAS number
EUPAS31153

Study ID
46265

DARWIN EU® study
No

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

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.

Study status
Ongoing

Research institution and networks

Institutions

Novartis Pharmaceuticals
First published: 01/02/2024
Last updated 01/02/2024
Institution

Contact details

Study institution contact
Novartis Clinical Disclosure Officer
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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)

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 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

Name of medicine
Luxturna

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

Anatomical Therapeutic Chemical (ATC) code
(S01XA27) 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

Data sources

Data sources (types)
Other

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

Check completeness
Unknown

Check stability
Unknown

Check logical consistency
Unknown

Data characterisation

Data characterisation conducted
No