Registry of Pediatric Patients Treated with Vedrop® (Tocofersolan) in Europe for Vitamin E Deficiency due to Digestive Malabsorption in Congenital or Hereditary Chronic Cholestasis

First published: 09/10/2015

Last updated: 01/04/2024

Study | Finalised

Administrative details

EU PAS number

EUPAS11209

Study ID

26087

DARWIN EU® study

No

Study countries

- [ ] France
- [ ] Germany

☐ Netherlands

☐ Spain

☐ Sweden

Study description

This study is conducted in Europe. The purpose of the study is to evaluate the safety and efficacy of tocofersolan (Vedrop) in pediatric patients suffering from vitamin E deficiency due to chronic or hereditary chronic cholestasis leading to digestive malabsorption.

Study status

Finalised

Research institutions and networks

Institutions

Orphan Europe

Multilple centres: 8 centres are involved in the study

Contact details

Study institution contact

Medical Affairs Orphan Europe vedropregistry@orphan-europe.com

Study contact

vedropregistry@orphan-europe.com

Primary lead investigator

Medical Affairs Orphan Europe

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 20/07/2010

Study start date

Actual: 23/08/2010

Date of interim report, if expected

Actual: 05/10/2016

Date of final study report

Planned: 02/10/2017

Actual: 07/04/2017

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Orphan Europe

Regulatory

Was the study required by a regulatory body?

Yes

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

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Drug utilisation

Effectiveness study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Main study objective:

To collect data about the demographic profile of patients, the use of Vedrop® and its efficacy and safety profile.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

VEDROP

Medical condition to be studied

Vitamin E deficiency

Population studied

Short description of the study population

Pediatric patients suffering from vitamin E deficiency due to chronic or hereditary chronic cholestasis leading to digestive malabsorption.

Age groups

Preterm newborn infants (0 – 27 days)

Term newborn infants (0 – 27 days)

Infants and toddlers (28 days – 23 months)

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Special population of interest

Other

Special population of interest, other

Vitamin E deficient patients

Estimated number of subjects

500

Study design details

Data analysis plan

All data will be analyzed in a descriptive manner, no formal hypotheses will be tested. Continuous variables will be summarised with descriptive statistics and categorical variables will be displayed in frequency tables.

Documents

Study results

VEDROP Registry Synopsis_07Apr2017.pdf(692.15 KB)

Study publications

Thébaut A, Nemeth A, Le Mouhaër J, Scheenstra R, Baumann U, Koot B, Gottrand F,...

Data management

Data sources

Data sources (types)

Electronic healthcare records (EHR)

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

Unknown