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

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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.
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Study status
Finalised
Research institutions and networks
Institutions
Orphan Europe
Multiple centres: 8 centres are involved in the
study
Multilple centres: 8 centres are involved in the study

Contact details

Study institution contact

Medical Affairs Orphan Europe vedropregistry@orphaneurope.com

Study contact

vedropregistry@orphan-europe.com

Primary lead investigatorMedical 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

Medicinal product name

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

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)

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

Unknown Check completeness Unknown

Check stability

Check conformance

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

Unknown