Title Cerebrotendinous Xanthomatosis Registry: Long term Non-Interventional Follow-up of Safety and Effectiveness of Chenodeoxycholic Acid Leadiant.

First published: 08/03/2019

Last updated: 22/02/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS27028

Study ID

46836

DARWIN EU® study

No

Study countries

France

Italy

Netherlands

United Kingdom

Study description

Research question and objectives The objective of the registry study is to collect long-term safety and effectiveness of Chenodeoxycholic Acid Leadiant in the treatment of Cerebrotendinous Xanthomatosis.

Study status

Ongoing

Research institutions and networks

Institutions

CWZ Nijmegen

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Institution

Contact details

Study institution contact

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Study contact

ctxregistry@leadiantbiosciences.com

Primary lead investigator

Surabhi Verma

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 14/12/2017

Actual: 19/10/2017

Study start date
Planned: 15/12/2019

Actual: 09/03/2020

Date of interim report, if expected
Planned: 15/03/2023

Date of final study report
Planned: 30/03/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Leadiant Biosciences Ltd

Study protocol

riskmgtsystem-clinstudprotocolv20.pdf (503.34 KB)

Leadiant_2017_LBL_NIS_01_Protocol_Version 5.0_20230710_FINAL clean_PAA (002).pdf (578.19 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 2 (specific obligation of marketing authorisation)

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:

To study long term effectiveness and safety of Chenodeoxycholic Acid Leadaint for the treatment of Cerebrotendinous Xanthomatosis

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Registry

Study drug and medical condition

Medicinal product name

CHENODEOXYCHOLIC ACID

Medical condition to be studied

Leukodystrophy

Population studied

Age groups

- Infants and toddlers (28 days – 23 months)
- 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

60

Study design details

Outcomes

The primary endpoint of change from baseline in serum cholestanol level will be summarised using descriptive statistics, Urinary bile alcohols, disability scales, ataxia scales

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Data analysis plan

The primary endpoint of change from baseline in serum cholestanol level will besummarised using descriptive statistics. The mean change from baseline at each postbaselineassessment will be presented with 95% confidence intervals. A paired t-testwill be used to test for a significant change from baseline.

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)

Disease registry

Other

Data sources (types), other

Exposure registry

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