Prospective observational study to monitor and assess the safety of Onpattro® patisiran-LNP in a real-world cohort of hATTR amyloidosis patients

First published: 02/04/2021

Last updated: 07/05/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS40417

Study ID

40418

DARWIN EU® study

No

Study countries

Brazil

Bulgaria

Denmark

France
Germany
Israel
Italy
Netherlands
Portugal
Spain
Taiwan
United Kingdom
United States

Study description

The purpose of this observational study is to assess the safety of patisiran-lipid nanoparticle (LNP) (Onpattro®) in real-world clinical practice by creating and monitoring a longitudinal cohort of hereditary transthyretin-mediated (hATTR) amyloidosis patients, including both patients treated with patisiran-LNP and comparator patients (treated with a competitor and untreated), following local standard of care.

The primary objective of the study is to characterise the safety of patisiran-LNP under real-world conditions, including determining and comparing the incidence of protocol-specified events of interest in patients exposed to patisiran-LNP.

Study status

Ongoing

Research institutions and networks

Institutions

United BioSource Corporation (UBC)

Switzerland

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Institution | Non-Pharmaceutical company | ENCePP partner

Multiple centres: 40 centres are involved in the study

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Emily Brouwer

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 21/03/2019

Study start date
Actual: 23/11/2020

Date of interim report, if expected
Planned: 31/03/2024

Date of final study report
Planned: 31/03/2032

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Alnylam

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Other study registration identification numbers and links

ALN-TTR02-009

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Disease epidemiology

Main study objective:

The main objective of this study is to characterise the safety of patisiran-LNP under real-world conditions, including determining and comparing the incidence of selected events (e.g., hepatic) in hATTR amyloidosis patients exposed to patisiran-LNP.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name, other

Onpattro

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

PATISIRAN

Medical condition to be studied

Familial amyloidosis

Hereditary neuropathic amyloidosis

Amyloidosis

Acquired ATTR amyloidosis

Cardiac amyloidosis

Additional medical condition(s)

Hereditary transthyretin-mediated (hATTR) amyloidosis

Population studied

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)
- 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

300

Study design details

Outcomes

The primary outcome of interest is the incidence of selected events of interest. These events include: hepatic events, cardiovascular events, renal events and ocular events, as well as infusion-related reactions (including hypersensitivity reactions).

• The incidence of selected events in sub-populations (eg, patients administered home infusions, patients with prior liver transplant, hepatic impairment, and renal impairment).

• Epidemiological and clinical characteristics of hATTR amyloidosis patients, and patients treated with patisiran-LNP in a real-world setting.

• Pregnancy outcomes and selected infant outcomes.

Data analysis plan

Epidemiological cohort techniques will be used to analyse the occurrence of safety events by exposure status. Incidence rates will be calculated in patient-years with respect to both exposure status and observational time. Relative risk numbers (incidence rate ratios) will be derived for composite endpoints of selected safety events of interest.

To account for varying exposure windows and varying observational time, the proportion of patients experiencing an event may also be estimated using time-to-event methodology.

Data management

Data sources

Data sources (types)

Disease registry

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