Prospective Observational Study to Monitor and Assess the Safety of Amvuttra® [Vutrisiran] in a Real-World Cohort of hATTR Amyloidosis Patients

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Last updated: 10/09/2024

Study Planned

Administrative details

PURI

https://redirect.ema.europa.eu/resource/199013

EU PAS number

EUPAS108904

Study ID

199013

DARWIN EU® study

No

Study countries

- [ ] Brazil
- [ ] Bulgaria
- [ ] Denmark
- [ ] France
- [ ] Germany
- [ ] Israel
- [ ] Italy
- [ ] Netherlands
- [ ] Portugal
- [ ] Spain
- [ ] Taiwan
- [ ] United Kingdom
- [ ] United States

Study description

This is a multinational, non-interventional, observational study based on secondary use of data and conducted over a period of 10 years to evaluate the safety of vutrisiran in hATTR amyloidosis participants exposed to vutrisiran under real-world conditions.

Study status

Planned

Research institutions and networks

Institutions

IQVIA

United Kingdom

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Institution | Non-Pharmaceutical company | ENCePP partner

Multiple centres: 41 centres involved in the study

Contact details

Study institution contact

Karien Verhulst

Study contact

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Primary lead investigator

Sophie Zhang

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 29/02/2024

Study start date
Planned: 29/02/2024

Date of final study report
Planned: 31/12/2034

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)

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Main study objective:

Primary aim = long-term(>2 years) safety of vutrisiran under real-world conditions including determining & comparing incidence of selected events of interest (clinical consequences of vitamin A deficiency, including delayed symptoms &hypersensitivity reactions) in hATTR amyloidosis participants exposed to vutrisiran as compared to other medications (except patisiran) treating hATTR amyloidosis.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medical condition to be studied

Acquired ATTR amyloidosis

Additional medical condition(s)

Transthyretin-mediated amyloidosis, Hereditary transthyretin-mediated amyloidosis

Population studied

Short description of the study population

Not yet known, study will enroll hATTR patients, who will be treated per standard of care.

Age groups

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)

Special population of interest

Hepatic impaired

Pregnant women

Estimated number of subjects

300

Study design details

Outcomes

1. Number of Participants With Adverse Events (AEs) due toClinical Consequences of Vitamin A Deficiency, IncludingDelayed Symptoms 2. Number of Participants With AEs due to HypersensitivityReactions, Number of Hepatic AEs in Participants With Moderate/Severe Hepatic Impairment; Pregnancy Outcomes (Live/Preterm Births, Spontaneous Abortions, Stillbirths & Elective/Therapeutic Abortions); Infant Outcomes, Congenital Malformations at Birth & Lactation & Infant Follow-up Data Through First Year of Life; Epidemiological &Clinical Characteristics of Patients Treated With Vutrisiran in

Real-world

Data analysis plan

Descriptive statistics for continuous variables including the number of observations, mean, standard deviation, median, interquartile range, and minimum/maximum; the associated 95% confidence interval (CI) will be provided, as appropriate. Categorical variables will be summarised as frequency and proportion of the relevant population. Summary statistics will be presented for the full study population and separately by subgroups (eg, exposure status, liver transplant status, hepatic impairment status, occurrence of specified safety events of interest) where appropriate.

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