A cohort event monitoring study to characterise use of eplontersen in patients with prior liver transplant and pre-existing severe hepatic impairment and to assess long-term safety among all new users of eplontersen (sTTRing)

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Study Planned

Administrative details

EU PAS number

EUPAS1000000890

Study ID

1000000890

DARWIN EU® study

No

Study countries

Canada
China
Germany
Spain
United Kingdom
United States

Study description

The aim of this observational cohort study is to characterise use of eplontersen in patients with prior liver transplant or with pre-existing severe hepatic impairment, as well as to assess long-term safety among all new users of eplontersen. The sTTRing study is a retrospective observational cohort study of patients initiating eplontersen in the real-world (and compared to patients initiating other transthyretin amyloidosis [ATTR] treatments, if feasible) which combines deidentified data from several existing data sources. Primarily sTTRing will be a secondary data use of the ‘non-interventional prospective, multi-country study collecting real-world data on the characteristics, treatment patterns, and outcomes of patients with amyloid transthyretin (ATTR) amyloidosis’ (clinicaltrials.gov identifier NCT06465810). The sTTRing dataset will extract deidentified data from patients enrolled in NCT06465810 and who initiated eplontersen, and if comparative analyses are performed, a balanced cohort of patients unexposed to eplontersen will also be identified. Additionally, tokenized data for NCT06465810 patients in the USA will be linked to claims, if feasible, and deidentified individual case safety report (ICSR) data from AstraZeneca’s PV database will also be used, if feasible to link those data to NCT06465810.The primary objectives of the study are:

1. To describe demographic and clinical characteristics of patients at eplontersen initiation, including the prevalence of prior liver transplant (overall and by reason for liver transplant), and the prevalence of severe hepatic impairment; and to describe patients in these subgroups (prior liver transplant,

severe hepatic impairment).

2. To describe long-term safety in patients who initiate eplontersen treatment, including onset of new clinical events, abnormal laboratory values and serious adverse events.

Study status

Planned

Research institutions and networks

Institutions

AstraZeneca

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Institution

ICON Commercialisation & Outcomes

Germany

Ireland

First published: 19/03/2010

Last updated: 05/07/2024

Institution | Non-Pharmaceutical company | ENCePP partner

Contact details

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Primary lead investigator

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Study timelines

Date when funding contract was signed

Actual: 10/04/2025

Study start date

Planned: 15/11/2026

Data analysis start date

Planned: 15/05/2027

Date of interim report, if expected

Planned: 12/11/2027

Date of final study report

Planned: 15/02/2033

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

AstraZeneca

Study protocol

D8450R00022 - Protocol 2.0 Final_redacted.pdf (865.35 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

Non-EU RMP only

Other study registration identification numbers and links

Protocol number: D8450R00022

Link to Clinicaltrials.gov

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Study design:

The sTTRing study is a retrospective observational cohort study and analyses of patients initiating eplontersen in the real-world, combining data from several different data sources.

Main study objective:

The primary objectives of the study are as follows:

1. To describe demographic and clinical characteristics of patients at eplontersen initiation, including the prevalence of prior liver transplant (overall and by reason for liver transplant), and the prevalence of severe hepatic impairment; and to describe patients in these subgroups (prior liver transplant, severe hepatic impairment).

2. To describe long-term safety in patients who initiate eplontersen treatment, including onset of new clinical events, abnormal laboratory values and serious adverse events.

The secondary objectives of the study are as follows:

1.To describe eplontersen treatment use, including duration of treatment and

reasons for discontinuation, overall and by sub-populations of individualswith prior liver transplant or individuals with pre-existing severe hepatic impairment. 2.To describe the incidence of safety events occurring in patients who initiate eplontersen treatment, including onset of new clinical events, abnormal laboratory values and serious adverse events separately i) in patients with prior liver transplant, overall and by reason for liver transplant, if feasible; and ii) in patients with pre-existing severe hepatic impairment

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

WAINZUA

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

EPLONTERSEN SODIUM

Anatomical Therapeutic Chemical (ATC) code

(N07XX21) eplontersen

eplontersen

Medical condition to be studied

Cardiac amyloidosis

Familial amyloidosis

Population studied

Short description of the study population

The NCT06465810 study cohort includes individuals with confirmed diagnosis of ATTR, aged ≥18 years at the time of providing the informed consent. The sTTRing study population will be a subset of the NCT06465810 cohort who meet the additional criteria below.

Inclusion Criteria:

1. NCT06465810 participants who consented to have their data used for future related research studies.
2. NCT06465810 participants who initiated eplontersen treatment up to 1-year prior to enrolment into NCT06465810 study observation period, irrespective of ATTR phenotype or genotype. For the comparative analyses, patients unexposed to eplontersen treatment and who initiated another ATTR treatment during NCT06465810 study observation period will be included.

Exclusion criteria:

1. Patients with exposure to eplontersen more than 1-year prior to enrolment into NCT06465810 study.
2. Patients who participated in an interventional ATTR study in the 12-months prior to enrolment into NCT06465810 study.

Age groups

- **Adult and elderly population (≥18 years)**
  - Adults (18 to < 65 years)
    - Adults (18 to < 46 years)
    - Adults (46 to < 65 years)
  - Elderly (≥ 65 years)
    - Adults (65 to < 75 years)
    - Adults (75 to < 85 years)
    - Adults (85 years and over)

Special population of interest

Hepatic impaired

Special population of interest, other

Prior liver transplant

Study design details

Setting

The D8450R00003 study cohort includes individuals with confirmed diagnosis of ATTR , aged ≥18 years at the time of providing the informed consent. The overall sTTRing study population will be a subset of the D8450R00003 cohort who meet the additional inclusion criteria belowThe sTTRing study cohort will include those who meet the following additional inclusion criteria:

1. D8450R00003 participants who consented to have their data used for future related research studies.
2. D8450R00003 participants who initiated eplontersen treatment up to 1-year prior to enrolment into or during the D8450R00003 study observation period, irrespective of ATTR phenotype or genotype. For the comparative analyses, patients unexposed to eplontersen treatment and who initiated other ATTR treatments during D8450R00003 study observation period will be included.

The sTTRing study cohort will exclude those to whom the following additional exclusion criteria apply:

1. Patients with exposure to eplontersen more than 1-year prior to enrolment into D8450R00003 study.
2. Patients who participated in an interventional ATTR study in the 12-months prior to enrolment into D8450R00003 study.

Comparators

If feasible, a comparator cohort of non-eplontersen exposed patients who initiated another ATTR treatment will be identified and matched eplontersen initiators to achieve balance, and comparative analyses will be performed to evaluate long-term safety of eplontersen relative to other treatments.

Outcomes

1. The prevalence of liver transplant prior to eplontersen initiation (overall and by reason for liver transplant)
2. The prevalence of severe hepatic impairment at eplontersen initiation
3. Demographic and clinical characteristics (Laboratory markers, Clinical ATTR characteristics, Comorbidities, concomitant Medications, comorbidities.
4. ATTR Treatment details
5. Safety endpoints
6. Total duration of follow-up (person time observed)

Data analysis plan

The analysis will be descriptive in nature using descriptive statistics of counts, frequency, proportion and/or distribution characteristics (mean (SD), median (range)). Where possible, these characteristics will be described separately in new and prior eplontersen users; overall and by subgroups of interest, i.e., patients with prior liver transplant and patients with severe hepatic impairment at eplontersen initiation. Cohort event monitoring analysis will be performed to identify adverse events that require further characterisation including incidence densities and differences in incidence densities. If feasible, long-term safety of eplontersen will be compared to other treatments. An adequately balanced group of patients unexposed to eplontersen and new users of other ATTR treatments will be matched to patients initiating eplontersen treatment using propensity score matching methodology

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Summary results

Not applicable

Documents

https://www.clinicaltrials.gov/study/NCT06465810?term=EPLONTERSEN&rank=8

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 source(s), other

1. DD8450R00003
2. US claims data
3. AZ PV database (Argus)

Data sources (types)

Administrative healthcare records (e.g., claims)

Non-interventional study

Other

Data sources (types), other

Individual case safety reports (ICSRs)

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