A Prospective, Non-interventional, Long-term, Multinational Cohort Safety Study of Patients with Hereditary Transthyretin Amyloidosis with Polyneuropathy (hATTR-PN) (TEG4001)

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Study Ongoing

Administrative details

EU PAS number

EUPAS37728

Study ID

41080

DARWIN EU® study

No

Study countries

Bulgaria

Cyprus

France
Germany
Greece
Italy
Portugal
Spain

Study description

This is a non-interventional, international, prospective cohort safety study of TEGSEDI-exposed and TEGSEDI-unexposed patients. Data will be extracted from medical records via secondary data collection. The TEGSEDI-exposed cohort will consist of patients diagnosed with hATTR-PN who are receiving TEGSEDI. Data from the TEGSEDI-exposed cohort will be compared to data collected prospectively from a TEGSEDI-unexposed cohort which will consist of patients diagnosed with hATTR-PN who have not taken any dose of TEGSEDI within 25 weeks prior to enrollment and are eligible for TEGSEDI treatment per the applicable product label. Patients may take other drugs to treat hATTR-PN. Among the patients enrolled in the TEGSEDI-exposed cohort, at least 96 adult subjects will meet the criteria of a “new user.” A new user is defined as a patient who has had no exposure to TEGSEDI prior to receiving commercial TEGSEDI. Patients in the TEGSEDI-unexposed cohort who begin TEGSEDI following enrollment will be analyzed as part of the TEGSEDI-exposed cohort from the time of initiation of TEGSEDI. The overarching goal of this study is to further characterize the long-term safety of TEGSEDI in patients with hATTR-PN under real-world conditions.

Study status

Ongoing

Research institutions and networks

Institutions

Akcea Therapeutics, Inc.

Contact details

Study institution contact

Ionis Global Regulatory Affairs

globalregulatoryaffairs@ionis.com

Study contact

globalregulatoryaffairs@ionis.com

Primary lead investigator

Akcea Therapeutics, Inc.

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 16/12/2019

Actual: 16/12/2019

Study start date

Planned: 20/10/2021

Actual: 20/10/2021

Date of final study report

Planned: 30/06/2037

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Akcea Therapeutics, Inc.

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

TEG4001

Methodological aspects

Study type

Study type list

Study topic:

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:

This is a non-interventional, international, prospective cohort safety study of TEGSEDI-exposed and TEGSEDI-unexposed patients. Data will be extracted from the medical record via secondary data collection.

Main study objective:

To further characterize the long-term safety of TEGSEDI in patients with hATTR-PN under real-world conditions.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Cohort Driven, Non-interventional observational study

Study drug and medical condition

Medicinal product name

TEGSEDI

Additional medical condition(s)

Hereditary Transthyretin Amyloidosis with Polyneuropathy (hATTR-PN)

Population studied

Short description of the study population

Patients with hATTR-PN in Europe, US, and Canada

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)

Estimated number of subjects

240

Study design details

Setting

This study will be conducted in Europe, the US, and Canada at centers which manage patients with hATTR such as specialist centers as well as physicians treating amyloidosis (e.g., neurologists, cardiologists, nephrologists, and hematologists).

All patients from each participating site will be invited to participate in the study in order to minimize selection bias. For eligible patients who do not enroll, reason for non-enrollment will be collected.

Comparators

NA

Outcomes

Primary Outcome Measure:

1) Further characterization of the long-term safety of TEGSEDI in patients with hATTR-PN under real-world conditions.

• Determination of the incidence rate of thrombocytopenia in patients with hATTR-PN treated with TEGSEDI (TEGSEDI-exposed cohort)

• Comparison of the relative rates of thrombocytopenia in hATTR-PN patients treated with TEGSEDI (TEGSEDI exposed) to hATTR-PN patients unexposed to TEGSEDI (TEGSEDI- unexposed)

[Time Frame: 10 years]

Secondary Outcome Measures:

2) Description of the incidence rate of the Adverse Events of Special Interest (AESI) in the TEGSEDI-exposed and TEGSEDI-unexposed patients.

To describe the incidence rate of the following AESIs:

• severe thrombocytopenia (platelet counts <25 x 109/L and separately, <50 x 109/L)

• serious and non-serious bleeding events

• glomerulonephritis

• hepatotoxicity/serious hepatobiliary events

• composite of stroke and/or cervicocephalic arterial dissection
• central nervous system (CNS) vasculitis
• ocular toxicity due to vitamin A deficiency
[Time Frame: 10 years]

3) Description of the time to onset of Adverse Events of Special Interest (AESI) in the TEGSEDI-exposed and TEGSEDI-unexposed patients.
To describe the time to onset of the following AESIs:
• severe thrombocytopenia
• serious and non-serious bleeding events
• hepatotoxicity/serious hepatobiliary events
• glomerulonephritis
• composite of stroke and/or cervicocephalic arterial dissection
• central nervous system (CNS) vasculitis
• ocular toxicity due to vitamin A deficiency
[Time Frame: 10 years]

Data analysis plan
Demographic and medical history (including but not limited to disease stage, time since diagnosis, prior and concurrent medications use, history of severe thrombocytopenia, hepatotoxicity/serious hepatobiliary events, glomerulonephritis, stroke, cervicocephalic arterial dissection, central nervous system vasculitis, and ocular toxicity due to vitamin A deficiency will be summarized using descriptive statistics, with number and percent for categorical variables, and n, mean, standard deviation (SD), standard error (SE) of the mean, median, minimum, and maximum for continuous variables. Demographics and medical history will also be summarized using descriptive statistics on different exposure groups (patients who have already been treated with TEGSEDI versus patients entering this study who are initiating TEGSEDI treatment.

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 data source

Data sources (types)

Electronic healthcare records (EHR)

Other

Data sources (types), other

For both cohorts, data will be extracted from the patient's medical records by site personnel. Once written informed consent has been obtained, study site personnel will complete the baseline data collection for each patient. Follow-up data for patient visits will be recorded in the patient chart in accordance with the clinical site's standard of care or clinical judgment. All data will be abstracted from medical records and entered into the EDC on an ongoing basis.

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