A Retrospective, Non-interventional, Multi-centre Study of TEGSEDI-treated Patients to Evaluate Real-world Adherence to, and Effectiveness of the Recommendations for Platelet Monitoring, Dose Adjustment, and Steroid Initiation to Manage Risk of Thrombocytopenia (TEG4002)

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Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/37732

EU PAS number

EUPAS37731

Study ID

37732

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ Bulgaria
- ☐ Cyprus
- ☐ France
- ☐ Germany
- ☐ Greece
- ☐ Italy
- ☐ Portugal
- ☐ Spain
- ☐ Sweden
- ☐ United Kingdom

Study description

This is a retrospective chart review study of patients who have commenced commercial TEGSEDI, at any time and for any duration, between the date of relevant marketing authorization of TEGSEDI and the date of data extraction. Patients will be enrolled in the study in 2025 and data will then be extracted from the medical records for the period from the start of commercial TEGSEDI treatment until 1) eight weeks after discontinuation of treatment with TEGSEDI, or 2) death.

For those patients who are still on TEGSEDI at the time of enrollment, retrospective data collection will be censored from the time of informed consent. It is anticipated that at least 100 patients will be recruited.

The goal of this study is to assess the real-world adherence to, and effectiveness of the proposed schedule for platelet monitoring, TEGSEDI dose adjustment, and initiation of steroids for platelet recovery in accordance with

the schedule outlined in the Summary of Product Characteristics (SmPC).

Study status

Ongoing

Research institutions and networks

Institutions

Akcea Therapeutics, Inc.

Contact details

Study institution contact

Akcea Therapeutics, Inc.

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: 01/01/2025

Actual: 26/03/2025

Date of final study report
Planned: 31/12/2026

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

TEG4002

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Drug utilisation

Data collection methods:

Secondary use of data

Main study objective:

To assess the real-world adherence to, and effectiveness of the proposed schedule for platelet monitoring, TEGSEDI dose adjustment, and initiation of steroids for platelet recovery in accordance with the schedule outlined in the SmPC.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Retrospective chart review study

Study drug and medical condition

Name of medicine

TEGSEDI

Name of medicine, other

TEGSEDI

Anatomical Therapeutic Chemical (ATC) code

(N07XX15) inotersen

inotersen

Medical condition to be studied

Thrombocytopenia

Additional medical condition(s)

Hereditary transthyretin amyloidosis (hATTR)

Population studied

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

100

Study design details

Outcomes

Primary outcome measure:

1. Evaluation of real-world adherence to, and effectiveness of the recommended schedule for platelet monitoring, dose adjustment, and initiation of steroids for platelet recovery.

Secondary Outcome Measures:

2. Evaluation of real-world thrombocytopenia-related outcomes (e.g. incidence rates of thrombocytopenia, serious and non-serious bleeding events associated with thrombocytopenia) in patients who receive TEGSEDI based on level(s) of adherence with recommendations for platelet monitoring and TEGSEDI dose adjustment per the SmPC.
3. Evaluation of potential predictors of adherence with recommendations for platelet monitoring, TEGSEDI dose adjustment, and initiation of steroids for platelet recovery per the SmPC in patients who receive TEGSEDI.

Data analysis plan

The primary analysis will include descriptive statistics to tabulate compliance with platelet monitoring and dose adjustments. Additionally, adherence will be summarized as a continuous measure as a % and will also be summarized as a dichotomous measure where adherence is defined as ≥90% compliance with the platelet monitoring and dose adjustment schedule and non-adherence defined as <90% compliance.

Data management

Data sources

Data sources (types)

Other

Data sources (types), other

retrospective cases will be collected

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