# 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)

**First published:** 22/10/2020

**Last updated:** 17/09/2025

Study Ongoing

## Administrative details

**EU PAS number**

EUPAS37731

**Study ID**

37732

**DARWIN EU® study**

No

**Study countries**

Bulgaria

Cyprus

France

Greece

Italy

Portugal

Spain

---

**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. 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: 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

**Medicinal product name**

TEGSEDI

**Medicinal product name, 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

# 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 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