

# Nordic Country Patient Registry for Romiplostim (NCPRR): Population-Based Prospective Annual Assessment of Safety of Romiplostim Treatment in Adult Patients with Chronic Idiopathic (Immune) Thrombocytopenic Purpura (ITP) Based on National Health Registry Systems in Denmark, Sweden and Norway (20070797)

**First published:** 30/04/2020

**Last updated:** 13/03/2024

Study

Finalised

## Administrative details

### **PURI**

<https://redirect.ema.europa.eu/resource/45344>

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### **EU PAS number**

EUPAS34701

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## Study ID

45344

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## DARWIN EU® study

No

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## Study countries

Denmark

Norway

Sweden

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## Study description

The study is a European Medicines Agency (EMA) mandated category 3 Post Authorization Safety Study (PASS) investigating the long-term safety of Nplate® (romiplostim) in the treatment of adults diagnosed with chronic immune thrombocytopenia (cITP). The primary objective addresses possible changes in bone marrow, and the secondary objectives address a number of other specific postulated safety endpoints. The study is a retrospective observational study which takes the form of a registry encompassing the vast majority of adult cITP patients in Denmark, Norway, and Sweden over the period 2009 to 2019. Data is primarily extracted from patient medical notes, with some elements obtained through linkage to National Health Registry Systems (NHRS) in each country. The study is wholly undertaken on behalf of Amgen by Aarhus University, Denmark, with key collaborators in each country. The investigators are responsible of the collection of data, its management, and its analysis: the Aarhus University team are then responsible for interpretation of the cumulative data in an annual report which is submitted to the EMA. There is a two-year lag between data collection and production of the report: the final report, encompassing analysis of the full 2009-2019 dataset, will be submitted to the EMA in 2021. Independent oversight of the study is provided by a Scientific

Advisory Committee (SAC) whose members are Key Opinion Leaders (KOLs) in Denmark, Finland, Norway, and Sweden.

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## Study status

Finalised

## Research institutions and networks

### Institutions

#### Amgen

United States

**First published:** 01/02/2024

**Last updated:** 21/02/2024

**Institution**

#### Centre for Pharmacoepidemiology, Karolinska Institutet (CPE-KI)

Sweden

**First published:** 24/03/2010

**Last updated:** 23/04/2024

**Institution**

**Educational Institution**

**Laboratory/Research/Testing facility**

**Not-for-profit**

**ENCePP partner**

Department of Clinical Epidemiology, Aarhus  
University Hospital Olof Palmes Allé 43-45. 8200  
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## Contact details

### Study institution contact

Global Development Leader Amgen Inc.

Study contact

[medinfo@amgen.com](mailto:medinfo@amgen.com)

### Primary lead investigator

Global Development Leader Amgen Inc.

Primary lead investigator

## Study timelines

### Date when funding contract was signed

Actual: 10/06/2009

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### Study start date

Actual: 01/09/2009

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### Data analysis start date

Planned: 31/05/2021

Actual: 03/06/2021

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### **Date of final study report**

Planned: 31/12/2021

Actual: 24/01/2022

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

Amgen

## Study protocol

[EUPAS34701-35135.pdf](#)(731.5 KB)

## Regulatory

### **Was the study required by a regulatory body?**

Yes

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

EU RMP category 3 (required)

## Methodological aspects

### Study type

### Study type list

**Study topic:**

Human medicinal product

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**Study type:**

Non-interventional study

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**Scope of the study:**

Assessment of risk minimisation measure implementation or effectiveness

Disease epidemiology

Safety study (incl. comparative)

**Data collection methods:**

Secondary use of data

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**Main study objective:**

The overarching aim of the study is to characterize the long-term safety profile of romiplostim therapy in adult chronic ITP patients.

## Study Design

**Non-interventional study design**

Cohort

Other

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**Non-interventional study design, other**

Post-marketing safety surveillance

## Study drug and medical condition

**Name of medicine**

NPLATE

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

ROMIPLOSTIM

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**Anatomical Therapeutic Chemical (ATC) code**

(B02BX04) romiplostim

romiplostim

## Population studied

**Short description of the study population**

The study population includes chronic ITP patients, defined as patients who have been diagnosed with ITP for more than 6 months, and who continually receive ITP treatment (eg, ITP medication) and/or have had a splenectomy procedure. All qualified adult chronic ITP patients will be the source population, regardless of romiplostim therapy status.

**Inclusion Criteria**

Subjects in the NCPRR will be those whose health information is recorded by the NHRS of Denmark, Norway, or Sweden.

The eligible adult cITP romiplostim-exposed patients for the NCPRR must meet all of the following criteria:

- Patients must be 18 years or older at the time of chronic ITP diagnosis,
- Patients will have romiplostim therapy between 01 January 2009 and 31 December 2018, and
- Patients will have at least 6 months of medical information prior to initial date of receiving romiplostim treatment (Index Date), data from which will establish

a

baseline of study subjects.

The eligible adult cITP romiplostim-unexposed patients for the NCPRR must meet all following criteria:

- Patients must be 18 years or older at the time of chronic ITP diagnosis,
- Patients will not have received romiplostim therapy prior to or during the study period between 01 January 2009 and 31 December 2019, and
- Patients will have at least 6 months of medical information in the study period. The data from this period will establish baseline of subjects in the comparison group.

#### Exclusion Criteria

Patients who are enrolled in a clinical trial for other TPO receptor agonists (eg, clinical trials for eltrombopag) will be excluded from the study.

Patients are excluded from the cohorts of chronic ITP patients if either of the following is present:

1. Any diagnosis of another condition associated with thrombocytopenia other than chronic ITP listed in Appendix 2 at any time prior to or within 6 months after the qualifying diagnosis for chronic ITP. Examples of thrombocytopenia associated with other diseases and conditions include system

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#### **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)

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#### **Estimated number of subjects**

623

## Study design details



## Outcomes

To estimate the incidence rate of increased bone marrow (BM) reticulin and/or BM fibrosis with associated clinical signs (any of the following: splenomegaly, hepatomegaly, leukocytosis, and/or, cytopenia), confirmed by BM biopsy findings of adults with chronic ITP receiving romiplostim, Describe worsened thrombocytopenia Study incidence rate of thrombocytosis Describe incidence rate of thrombotic events Assess incidence rate of hematological malignancies/pre-malignant states Describe bleeding and/or use of rescue medication Describe incidence rate of concurrent leukocytosis/anemia Describe safety in patients with renal impairment Measure impact of EU dosing

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## Data analysis plan

The study is descriptive in nature. Number and frequency distributions of patients who experience events will be reported. Rates of occurrence of events over available follow-up time will be reported.

## Documents

### Study results

[20070797\\_Executive Summary\\_04JAN2022.pdf](#)(122.78 KB)

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## Data management

## Data sources

### Data sources (types)

[Other](#)

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## **Data sources (types), other**

Retrospective observational study: data is primarily extracted from patient medical notes, with some elements obtained through linkage to National Health Registry Systems (NHRS) in each country.

## Use of a Common Data Model (CDM)

### **CDM mapping**

No

## Data quality specifications

### **Check conformance**

Unknown

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### **Check completeness**

Unknown

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### **Check stability**

Unknown

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### **Check logical consistency**

Unknown

## Data characterisation

### **Data characterisation conducted**

No