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

EU PAS number
EUPAS34701

Study ID
45344

DARWIN EU® study
No

Study countries
Denmark
Norway
Sweden

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.

Study status
Finalised

Research institution 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

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Contact details

Study institution contact
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Study contact

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Primary lead investigator
Global Development Leader Amgen Inc.
Primary lead investigator

Study timelines

Date when funding contract was signed
Actual:
10/06/2009

Study start date
Actual:
01/09/2009

Data analysis start date
Planned:
31/05/2021
Actual:
03/06/2021

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

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

Study type:
Non-interventional study

Scope of the study:
Assessment of risk minimisation measure implementation or effectiveness

Disease epidemiology
Safety study (incl. comparative)

Data collection methods:
Secondary data collection

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

Non-interventional study design, other
Post-marketing safety surveillance

Study drug and medical condition

Name of medicine
Nplate

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

Anatomical Therapeutic Chemical (ATC) code
(B02BX04) 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)

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 thrombocytopeniaStudy incidence rate of thrombocytosisDescribe incidence rate of thrombotic eventsAssess incidence rate of hematological malignancies/pre-malignant statesDescribe bleeding and/or use of rescue medicationDescribe incidence rate of concurrent leukocytosis/anemiaDescribe safety in

patients with renal impairmentMeasure impact of EU dosing

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)

Data management

Data sources

Data sources (types)
Other

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

Check completeness
Unknown

Check stability
Unknown

Check logical consistency
Unknown

Data characterisation

Data characterisation conducted
No