A Post-Authorisation Safety Study to Evaluate the Safety of Marstacimab Among Patients with Haemophilia A or B using Real-World Data in Haemophilia Registers

First published: 10/06/2026

Last updated: 10/06/2026

Study Planned

Administrative details

EU PAS number

EUPAS1000000947

Study ID

1000000947

DARWIN EU® study

No

Study countries

United Kingdom

United States

Study description

The study will address the research question:
What are the incidence rates of TEs in patients with haemophilia A or B with or without inhibitors treated with marstacimab during routine clinical care in real-world settings in the UK and the US?

The primary study objective is:
- To describe the incidence rates of TEs among patients with haemophilia A or B with or without inhibitors in the patient cohort treated with marstacimab during routine clinical care.

The secondary study objectives are:
- To describe the incidence rates of TEs among patients with haemophilia A or B with or without inhibitors in the patient cohort unexposed to marstacimab and receiving routine prophylaxis.
- To describe clinical characteristics of patients with haemophilia A or B with or without inhibitors (those exposed to marstacimab and those unexposed to marstacimab and receiving routine prophylaxis) who experience a TE.
- To describe clinical characteristics of patients with haemophilia A or B with or without inhibitors (those exposed to marstacimab and those unexposed to marstacimab and receiving routine prophylaxis) who did not experience a TE.

Study status

Planned

Research institutions and networks

Institutions

United BioSource Corporation (UBC)

Switzerland

First published: 25/04/2013

Last updated: 06/03/2024

Institution | Non-Pharmaceutical company | ENCePP partner

Pfizer

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Li Wang

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 10/10/2023

Actual: 10/10/2023

Study start date
Planned: 27/02/2026

Date of interim report, if expected
Planned: 01/02/2030

Date of final study report
Planned: 01/02/2035

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer 100%

Study protocol

B7841016_PROTOCOL V1.1 CLEAN_20OCT2025.pdf (365.84 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:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Disease epidemiology

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Study design:

This is a multi-country, non-interventional, prospective cohort study to evaluate the incidence of TEs in patients with haemophilia A or B with or without inhibitors treated with marstacimab in real-world settings in the UK and the US.

Main study objective:

To describe the incidence rates of TEs among patients with haemophilia A or B with or without inhibitors in the patient cohort treated with marstacimab during routine clinical care.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

HYMPAVZI

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

MARSTACIMAB

Anatomical Therapeutic Chemical (ATC) code

(B02BX11) marstacimab

marstacimab

Population studied

Short description of the study population

Two patient cohorts will be included in the study:

- Patients 12 years of age and older with haemophilia A or B with or without inhibitors who have been treated with marstacimab in a routine care setting

after marstacimab has become commercially available in the UK and the US.
- Patients 12 years of age and older with haemophilia A or B with or without inhibitors who are unexposed to marstacimab and are receiving routine prophylaxis in a routine care setting.

Patients in the unexposed cohort will be matched within their originating register in a 1:1 ratio to the marstacimab exposure cohort by age group, index date, haemophilia severity, inhibitor status, history of TE, and history of cardiovascular disease.

Inclusion Criteria:
The study inclusion criteria are specific for each cohort, as indicated below. The inclusion and exclusion criteria are subject to amendments, and final criteria will be based on the approved local labels. These eligibility criteria are intended to make the two cohorts as similar as possible.
Patients must meet all the following inclusion criteria specific to the applicable cohort to be eligible for inclusion in the study.
Marstacimab Exposure Cohort:
- Patients 12 years of age and older with haemophilia A or B with or without inhibitors who have been treated with commercial marstacimab in a routine care setting in the UK or the US.
Unexposed Cohort:
- Patients 12 years of age and older with haemophilia A or B with or without inhibitors who have not been treated with marstacimab and are receiving routine prophylaxis in a routine care setting in the UK or the US.
Exclusion Criteria:
There are no exclusion criteria.

Age groups

- Adolescents (12 to < 18 years)
- **Adult and elderly population (≥18 years)**
  - Adults (18 to < 65 years)
    - Adults (18 to < 46 years)
    - Adults (46 to < 65 years)
  - Elderly (≥ 65 years)
    - Adults (65 to < 75 years)
    - Adults (75 to < 85 years)
    - Adults (85 years and over)

Estimated number of subjects

220

Study design details

Setting

Routine clinical care in real-world settings in the UK and the US.

Outcomes

New diagnoses of selected medical conditions

TEs (deep vein thrombosis, myocardial infarction, pulmonary embolism, stroke, angina, thrombotic microangiopathy, pulmonary infarction)

All Serious Adverse Events

All-Cause Mortality, including cause and date of death when available

- Intracranial haemorrhage, Bleeding (excluding intracranial)

- Thromboembolic event

- Liver disease, specify

- Cancer, specify

- Cardiac

- Infection, including pneumonia
- HIV
- Other, specify.

Data analysis plan

This is a descriptive study with a focus on estimation and not hypothesis testing. Descriptive statistics will be presented for each cohort. For categorical variables, the frequency and percentage will be reported. For continuous variables, the number of patients with available data, mean, median, standard deviation, range, minimum, and maximum values will be reported. In addition, the number of patients with missing data will be summarized for each variable. No imputation of missing values will be performed, except in the case of partial dates which will be outlined in the SAP. Patients who switch between treatments during the study will contribute patient years to each cohort as applicable. See Protocol for additional details

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

The American Thrombosis and Hemostasis Network (ATHN)

United Kingdom Haemophilia Centers Doctor’s Organization (UKHCDO)

Data sources (types)

Disease registry

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