Surveillance Of Emicizumab-Treated Patients: An Analysis of the EUHASS Pharmacovigilance Registry (Emicizumab Pharmacovigilance: EUHASS registry)

First published: 23/03/2018

Last updated: 12/06/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS23177

Study ID

49016

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ Belgium
- ☐ Bulgaria

- [ ] Croatia
- [ ] Cyprus
- [ ] Czechia
- [ ] Denmark
- [ ] Finland
- [ ] France
- [ ] Germany
- [ ] Greece
- [ ] Hungary
- [ ] Ireland
- [ ] Italy
- [ ] Latvia
- [ ] Lithuania
- [ ] Malta
- [ ] Netherlands
- [ ] Norway
- [ ] Poland
- [ ] Portugal
- [ ] Romania
- [ ] Russian Federation
- [ ] Slovakia
- [ ] Slovenia
- [ ] Spain
- [ ] Sweden
- [ ] Switzerland
- [ ] Türkiye
- [ ] United Kingdom

Study description

This is a cohort-surveillance, secondary data use, non-interventional, post authorization safety study (PASS) based on data collected by the European Haemophilia Safety Surveillance System (EUHASS) from participating centers. The main goal of this study is to assess the incidence of thromboembolism (TE), thrombotic microangiopathy (TMA), and anaphylaxis under real-world conditions in patients exposed to emicizumab with or without coagulation factor products. Secondary objectives are: to estimate the incidence of TE and TMA in patients exposed to emicizumab alone and concomitantly with each of the following drugs: activated prothrombin complex concentrate (aPCC), recombinant activated factor VII (rFVIIa), and factor VIII (FVIII) product, to describe individual cases of TE and TMA identified in EUHASS, to summarize the frequency of other adverse events collected by EUHASS in patients exposed to emicizumab, and to describe individual cases of “unexpected poor efficacy” reported to EUHASS based on the available information.

Study status

Ongoing

Research institutions and networks

Institutions

F. Hoffmann-La Roche

First published: 01/02/2024

Last updated: 01/02/2024

Institution

European Haemophilia Safety Surveillance System (EUHASS)

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Networks

EUHASS

Contact details

Study institution contact

Letizia Polito global.clinical_trial_registry@roche.com

Study contact

global.clinical_trial_registry@roche.com

Primary lead investigator

Letizia Polito

Primary lead investigator

Study timelines

Date when funding contract was signed
Actual: 14/02/2018

Study start date
Planned: 01/01/2019

Actual: 01/01/2019

Data analysis start date
Planned: 28/02/2026

Date of final study report
Planned: 30/06/2026

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

F. Hoffmann-La Roche, Ltd.

Study protocol

Prot GO40162 Hemlibra v1_Redacted.pdf (983.2 KB)

GO40162- Protocol v4_Redacted.pdf (1.36 MB)

Regulatory

Was the study required by a regulatory body?

No

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

EU RMP category 3 (required)

Other study registration identification numbers and links

GO40162

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

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Main study objective:

The primary objective for this study is to estimate the incidence of TE, TMA, and anaphylaxis under real-world conditions in patients exposed to emicizumab, with or without coagulation factor products.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Cohort surveillance

Study drug and medical condition

Medicinal product name

HEMLIBRA

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

EMICIZUMAB

Anatomical Therapeutic Chemical (ATC) code

(B02BX06) emicizumab

emicizumab

Medical condition to be studied

Haemophilia A with anti factor VIII

Haemophilia A without inhibitors

Population studied

Short description of the study population

The study population will consist of patients with Hemophilia A treated with emicizumab at centers participating in the EUHASS registry. Depending on the local approval/reimbursement decisions in the participating countries, the study population may include patients with any level of disease severity without FVIII inhibitors.

Age groups

- **Paediatric Population (< 18 years)**
  - Neonate
    - Preterm newborn infants (0 – 27 days)
    - Term newborn infants (0 – 27 days)
  - Infants and toddlers (28 days – 23 months)
  - Children (2 to < 12 years)
  - 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)

Special population of interest

Hepatic impaired

Immunocompromised

Pregnant women

Renal impaired

Estimated number of subjects

680

Study design details

Data analysis plan

Descriptive statistics on endpoints, including number and percentage of patients, will be provided. Continuous variables will be summarized by mean, median, minimum, maximum, and interquartile range (IQR).
Categorical variables will be summarized by counts, percentages, and corresponding 95% confidence intervals (CIs). Data generated within the first 7 calendar years post-marketing authorization throughout the European Union (2018-2024) will be analyzed annually.
Proportions of each adverse event, along with corresponding 95% CIs, will be calculated annually for all patients exposed to emicizumab. In addition, proportions of TE and TMA will be calculated annually for patients exposed to emicizumab and each of the following agents: aPCC, rFVII, and FVIII.

Documents

Study report

Interim CSR-3_GO40162-Annual Report_01Jul2022_Synopsis_Redacted.pdf (527.26 KB)
Interim_CSR GO40162_NI-PASS_Annual_Report_030Jun2020_Synopsis_Redacted.pdf (1.15 MB)
Interim_CSR_2-GO40162_Annual Report_23Jun2021_Synopsis_Redacted.pdf (443.45 KB)
Interim CSR-4, GO40162-Annual Report 2023_Synopsis_Redacted.pdf (1.48 MB)

Interim CSR-5, GO40162-Annual Report 2024_Synopsis_Redacted.pdf (384.64 KB)

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

EUHASS - Blood disorders

Data sources (types)

Other

Data sources (types), other

Spontaneous reporting system, prospective patient-based data collection

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