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

**First published:** 23/03/2018

**Last updated:** 10/06/2024

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

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**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.

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**Study status**

Ongoing

# Research institutions and networks

## Institutions

### 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

# 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

Safety study (incl. comparative)

**Data collection methods:**

Secondary use of data

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**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

**Name of medicine**

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.

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**Age groups**

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)

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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**Special population of interest**

Hepatic impaired

Immunocompromised

Pregnant women

Renal impaired

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**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)

# Data management

## 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