Hemlibra Survey to Prescribers and Patients/Carers to Evaluate Awareness, Knowledge and Compliance to Additional Risk Minimization Measures

First published: 11/07/2019

Last updated: 02/07/2024

Study Finalised

Administrative details

EU PAS number

EUPAS30451

Study ID

45024

DARWIN EU® study

No

Study countries

- [ ] Austria
- [ ] Belgium
- [ ] Czechia

- [ ] France
- [ ] Germany
- [ ] Hungary
- [ ] Italy
- [ ] Netherlands
- [ ] Poland
- [ ] Portugal
- [ ] Romania
- [ ] Spain
- [ ] United Kingdom

Study description

The study will utilize anonymous, cross-sectional, multinational surveys conducted among prescribers (health care providers HCPs) and patients with haemophilia A receiving Hemlibra, or their carers, in European countries, using primary data collection in the form of online questionnaires. The surveys to HCPs and patients/carers will assess their awareness of the Hemlibra educational program, knowledge of important identified risks (thrombotic microangiopathy TMA and thrombotic events TEs) and important potential life-threatening risk of bleeding due to misinterpretation of the standard coagulation tests associated with Hemlibra use, and adherence to additional risk minimization measures. Only relevant HCPs that have treated haemophilia patients with Hemlibra at least once outside of clinical trials will be targeted for the survey. Patients/carers will be recruited via their physicians.

Study status

Finalised

Research institutions and networks

Institutions

F. Hoffmann-La Roche

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

Nives Selak Bienz

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 24/08/2018

Study start date

Planned: 08/01/2020

Actual: 12/02/2020

Data analysis start date
Actual: 15/01/2021

Date of final study report
Planned: 30/09/2021

Actual: 22/07/2021

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

F. Hoffmann-La Roche, Ltd.

Study protocol

Prot BO40853 Emicizumab v2_Redacted.pdf (780.24 KB)

Prot BO40853 emicizumab v3_Redacted.pdf (863.48 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)

Other study registration identification numbers and links

BO40853

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Data collection methods:

Primary data collection

Main study objective:

The primary objective for this study is to evaluate the awareness (of risks of TMA and TE and the potential risk of life-threatening bleeding due to misinterpretation of standard coagulation tests), knowledge, and adherence of prescribers (HCPs) and patients/carers to the additional risk minimization

measures (RMMs) for Hemlibra.

Study Design

Non-interventional study design

Cross-sectional

Other

Non-interventional study design, other

Survey

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 is HCPs who have prescribed Hemlibra® and patients with haemophilia A who have been treated with Hemlibra® or their carers, all of whom must meet the following inclusion/exclusion criteria:

Inclusion criteria

HCPs

- HCPs who prescribed Hemlibra® at least once outside the context of a clinical trial to patients with haemophilia A with FVIII inhibitors or patients with severe haemophilia A without FVIII inhibitors
- HCPs who are willing to participate in this survey

Patients/Carers

- Patients who received at least 4 doses of Hemlibra® within the last 6 months, including patients on ongoing therapy
- Patients who are willing to participate in this self-administered survey, or their carers (parent or legal guardian) if the patient is under the age of 18

Exclusion criteria

HCPs

- HCPs who may have conflicts of interest with the study (e.g., HCPs employed by Roche or IQVIA)

Patients/Carers

- Patients/Carers who may have conflicts of interest with the study (e.g., patients/carers employed by Roche or IQVIA)
- Patients who have participated in a Hemlibra® clinical trial
- Patients with non-severe (i.e., mild or moderate) haemophilia A without FVIII inhibitors

Age groups

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

110

Study design details

Data analysis plan

Statistical analyses will be described and further detailed in the Statistical Analysis Plan. The following described analysis might be revised, and adjustments might occur. All the analyses will be descriptive and results will be presented separately for HCPs and patients/carers, overall, by region, and other. Continuous variables will be presented by their number (of valid cases, of missing data if applicable), mean, standard deviation, median, first quantile, third quantile, minimum, and maximum. No missing data will be imputed. Categorical variables will be presented as the total number and relative percentage per category. Confidence intervals of 95% will be evaluated, when relevant. The percentage of correctly answered survey questions will be calculated. Success on the educational materials for additional RMMs will be

defined a priori as achieving overall scores of >/=75% for awareness and adherence and >/=60% for knowledge for HCPs and >/=60% in all areas for patients/carers.

Documents

Study results

Final_CSR,_Study_BO40853,_,_Published_Output-1_Redacted.pdf (315.16 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 sources (types)

Other

Data sources (types), other

Primary data collection by survey

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