

HEMONIS - Therapeutic management of adult or child patients with hemophilia A : A retrospective observational study - HEMONIS study

Head :Roche Medical data center

Last update : 11/26/2021 | Version : 1 | ID : 74089

General	
Identification	
Detailed name	Therapeutic management of adult or child patients with hemophilia A : A retrospective observational study - HEMONIS study
Sign or acronym	HEMONIS
CNIL registration number, number and date of CPP agreement, AFSSAPS (French Health Products Safety Agency) authorisation	ML39418
General Aspects	
Medical area	Hematology
Study in connection with Covid-19	No
Pathology (details)	Hemophilia A
Health determinants	Medicine
Keywords	emicizumab
Scientific investigator(s) (Contact)	
Name of the director	Roche Medical data center
Address	4 cours de l'Ile Seguin - 92100 Boulogne-Billancourt
Email	data_sharing.france@roche.com
Organization	Roche SAS
Collaborations	
Participation in projects, networks and consortia	No

Funding

Funding status	Private
----------------	---------

Governance of the database

Sponsor(s) or organisation(s) responsible	Roche SAS
---	-----------

Organisation status	Private
---------------------	---------

Presence of scientific or steering committees	Yes
---	-----

Additional contact

Main features

Type of database

Type of database	Study databases
------------------	-----------------

Study databases (details)	Cohort study
---------------------------	--------------

Database recruitment is carried out by an intermediary	A selection of health care professionals
--	--

Database recruitment is made on the basis of:	Medication(s) taken
---	---------------------

Database recruitment is carried out as part of an interventional study	No
--	----

Database objective

Main objective	Primary Objective: To describe the current therapeutic regimen for moderate and severe HA patients (ITI / on-demand / short-term prophylaxis / long-term prophylaxis) in the MS population. Secondary Objectives: - To describe the current therapeutic regimen (ITI / on-demand / short-term prophylaxis / long-term prophylaxis) in severe HA patients in the Sev population. In both Moderate or Severe (MS) population and Severe (Sev) population : - To describe the profile of patients with HA - To describe the current treatment type according to therapeutic regimen, inhibitors status and severity - To describe patient status regarding medical
----------------	--

conditions and disease characteristics (severity, presence of inhibitors)

- To describe the patient's musculoskeletal complications

In Severe (Sev) population:

- To describe medical history and related conditions
- To describe thrombosis events
- To describe therapeutic management administered to patients with severe HA from January 1, 2000 to the last visit (therapeutic regimens in the 2 years preceding the index date , therapeutic regimen from January 1, 2000 until 2 years preceeding the last visit)

- To describe ITI prescribed treatment from initial diagnosis

- To describe significant events (bleeding) or prevention of physical activities and their management occurring in the 2 years preceeding the last visit.

- To describe all surgeries occurring in the 2 years preceeding the last visit.

Exploratory Objectives:

- To identify factors associated with choice of therapeutic regimen (on-demand vs prophylaxis) in the Sev population with inhibitors status at last visit: "Never inhibitor" or "Tolerized inhibitor".

- To identify factors associated with choice of therapeutic regimen (Prophylaxis vs on-demand, ITI vs on-demand) in the Sev population with inhibitors status at last visit: "Current inhibitor" .

- To identify factors associated with choice of ITI regimen (no ITI vs ITI) in the Sev population among patients with inhibitors.

Inclusion criteria

Inclusion criteria:

- o Patient aged ≥ 5 years old
- o Patient with moderate or severe constitutional
- o Patient with a last visit within the last 2 years
- o Patient followed in the same center for 2 years previous to the last visit, or patient followed in the same center from initial diagnosis, whichever is the shortest as collected in eCRF

Non inclusion criteria:

- o Patient who has received written information and expressed his/her refusal to participate in the study, or minor patient for whom at least one parent or guardian has expressed a refusal to participate in the study as checked by programming (patients without information date)
- o Patient included in a clinical trial at the date of last visit as collected in eCRF

Population type	
Age	Childhood (6 to 13 years) Adolescence (13 to 18 years) Adulthood (19 to 24 years) Adulthood (25 to 44 years) Adulthood (45 to 64 years) Elderly (65 to 79 years) Great age (80 years and more)
Population covered	Sick population
Pathology	D66 - Hereditary factor VIII deficiency
Gender	Male Woman
Geography area	National
Data collection	
Dates	
Date of first collection (YYYY or MM/YYYY)	2016
Date of last collection (YYYY or MM/YYYY)	2018
Size of the database	
Size of the database (number of individuals)	< 500 individuals
Details of the number of individuals	430
Data	
Database activity	Data collection completed
Type of data collected	Clinical data Declarative data
Clinical data (detail)	Medical registration
Details of collected clinical data	Type of data collected : verification of the selection criteria before inclusion, hemophilia A characteristics, demography, history of hemophilia A, medical conditions, therapeutic regimen of moderate and severe hemophilia A at last visit date, musculoskeletal complications, medical history and related conditions at the last visit, therapeutic

regimens for hemophilia A in the 2 years preceding the last visit, therapeutic regimen of hemophilia A from January 1, 2000 until 2 years preceding the last visit, ITI from initial diagnosis, events (bleeding) or prevention of physical activities occurring in the 2 years preceding the last visit, all surgeries occurring in the 2 years preceding the last visit.

Declarative data (detail)	Internet self-questionnaire
---------------------------	-----------------------------

Presence of a biobank	No
-----------------------	----

Procedures

Data collection method	eCRF
------------------------	------

Classifications used	CDISC like
----------------------	------------

Quality procedure(s) used	GCP/GVP
---------------------------	---------

Participant monitoring	Yes
------------------------	-----

Monitoring procedures	Monitoring by contact with the referring doctor
-----------------------	---

Followed pathology	D66 - Hereditary factor VIII deficiency
--------------------	---

Links to administrative sources	No
---------------------------------	----

Promotion and access

Promotion

Access

Dedicated website	https://www.roche.fr/fr/innovation-recherche-medicale/data-sharing-portail-d-information-partage-des-donnees.html
-------------------	---

Presence of document that lists variables and coding procedures	Yes
---	-----

Access to aggregated data	Access on specific project only
---------------------------	---------------------------------

Access to individual data	Access on specific project only
---------------------------	---------------------------------