PO061

Moderate hemophilia A and FVIII prophylaxis: real-world data from the FranceCoag cohort

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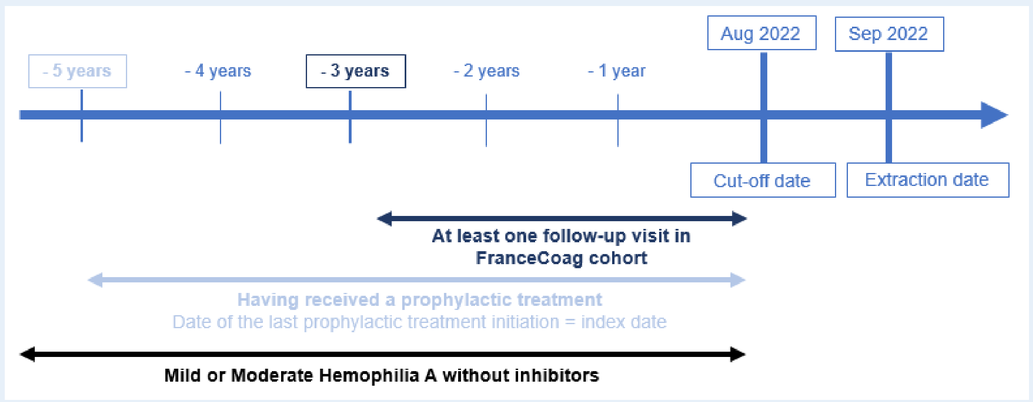
Introduction

- Few data are available in patients with moderate or mild hemophilia A (HA) without anti-factor VIII (FVIII) inhibitors requiring prophylaxis.
- The MIMOSA study (*MIld or MOderate hemophilia A without inhibitorS under prophylAxis in France: Description from the FranceCoag database*) was carried out to better characterize the French population of patients with moderate or mild HA without inhibitors, in the context of the extension of emicizumab's indication (granted on January 23, 2023) in patients with HA without inhibitors presenting a moderate form (FVIII ≥1% to ≤5%) with a severe bleeding phenotype[1].
- The description of the population with moderate HA without inhibitors under prophylactic treatment is presented.

Methods

- MIMOSA is a non-interventional study using data already collected in FranceCoag database, and additional variables collected from patient files.
- Patients included had moderate or mild HA (as assessed biologically and clinically) without inhibitors, had received prophylactic treatment in the last 5 years and completed at least one follow-up visit in the last 3 years, before September 2022, the date of data extraction (Figure 1).
- The objectives of the study were to describe patient characteristics, bleeding episodes (number and type) prior to initiation of the lastest period of prophylaxis, and reasons for last prophylaxis initiation.

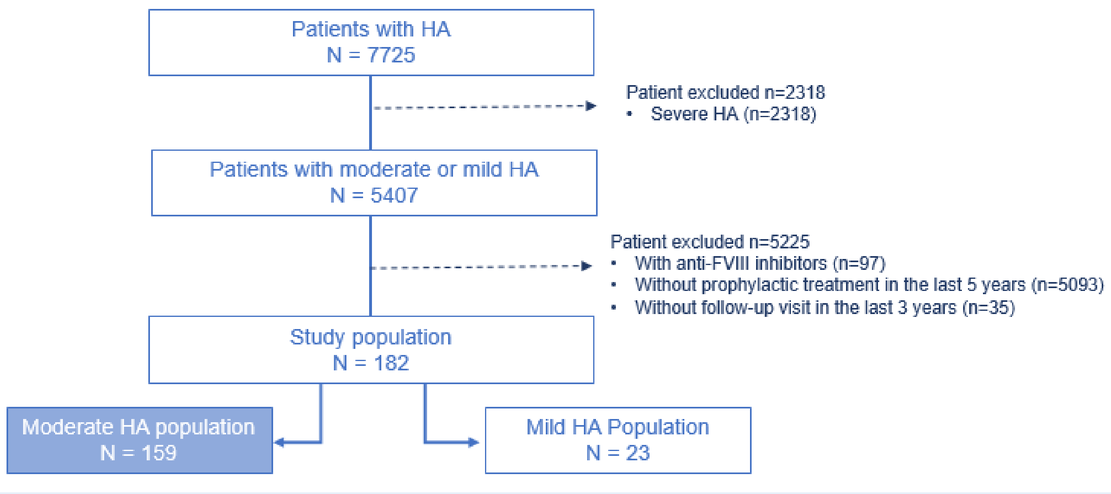
Figure 1: Study design



Results

- **Study population** (Figure 2)
  - In a cohort **of 5407 patients** with moderate or mild HA, 217 patients without inhibitors had received a prophylactic treatment in the last 5 years, 182 of whom had completed at least one follow-up visit in the last 3 years.
  - A total of **182 patients** on FVIII prophylaxis were included in the study, of whom **159 patients** (87.4%) had a moderate HA.

Figure 2. Study population



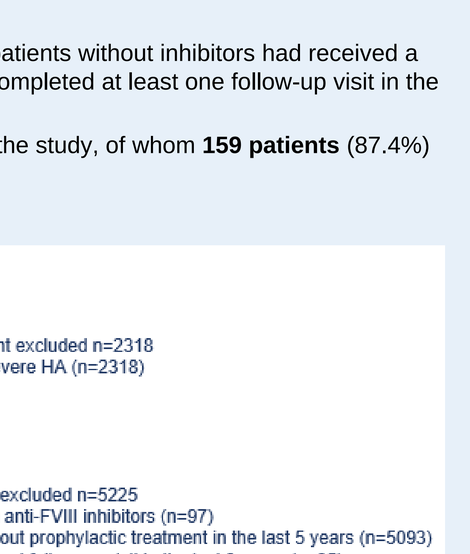
- **Patient characteristics**
  - Patient demographic characteristics are described in Table 1.
  - The last median basal FVIII level in the 6 months preceding the initiation of the last prophylaxis was 2.0% (Q1-Q3: 2.0-3.5) for the 138 patients for whom information was available (Figure 3).

Table 1. Patient characteristics

	Total moderate HA patients (N=159)
Gender	N=159
Men, n (%)	158 (99.4)
Women, n (%)	1 (0.6)
Age at diagnosis, years	N*=154
Median (Q1 - Q3)	0.9 (0.0 – 2.8)
Age at initiation of first prophylaxis, **years**	N*=157
Median (Q1 - Q3)	10.3 (6.0 – 31.8)
Age at initiation of last prophylaxis, years	N=159
Median (Q1 - Q3)	12.6 (6.6 – 32.8)
Age distribution at initiation of last prophylaxis	N=159
< 6 months, n (%)	0
[6 months - 2 years[, n (%)	1 (0.6)
[2 - 12[, n (%)	78 (49.1)
[12 - 18[, n (%)	12 (7.6)
[18 - 65[, n (%)	66 (41.5)
≥ 65 years, n (%)	2 (1.3)

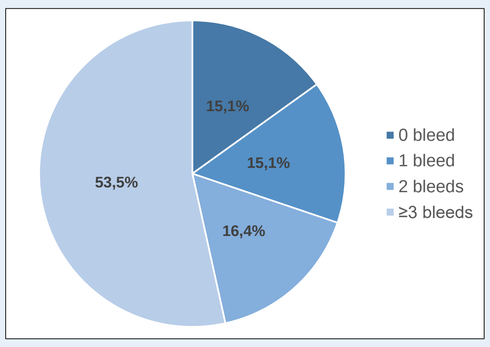
N*: Number of patients for whom information was collected.

Figure 3. Last median basal FVIII level before initiation of the last prophylaxis (N = 138)



- **Bleeding within the 24 weeks prior to initiation of the last prophylaxis**
  - The median number of treated bleeds per patient was 3.0 (Q1-Q3: 1.0-7.0) and of treated hemarthroses was 2.0 (Q1-Q3: 0.0-4.0).
  - The distribution of patients according to the number of treated bleeds is shown in Figure 4.

Figure 4: Distribution of patients (N = 159) by number of treated bleeds



  - At least 1 treated bleed was reported in 135/159 patients (84.9%). Bleeding types are described in Table 2.

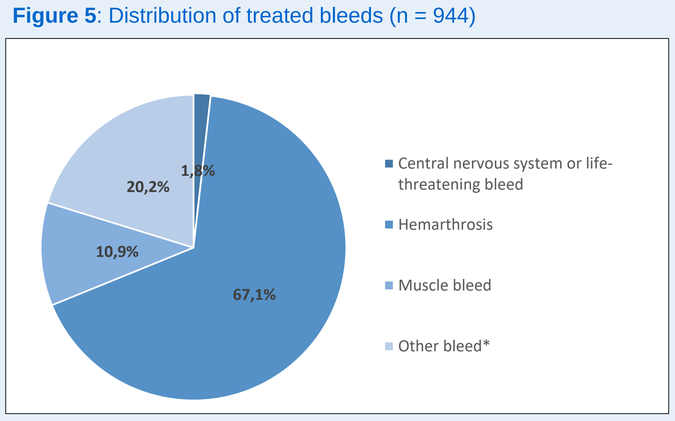
Table 2. Types of bleeding in patients with at least 1 treated bleed (N=135)

Type of bleeding	Patients for whom the type of bleeding was collected N	Patients with at least 1 bleed of this type n (%)
Central nervous system or life-threatening bleed	135	15 (11.1)
Hemarthrosis	119	107 (90.0)
Muscle bleed	124	46 (37.1)
Other bleed*	116	44 (37.9)

** Hematoma, Hematuria, Mucosal/dental bleed, Wound/trauma, Other/Not specified*

  - The distribution of the 944 treated bleeds by type of bleed is shown in Figure 5.

Figure 5: Distribution of treated bleeds (n = 944)



** Hematoma, Hematuria, Mucosal/dental bleed, Wound/trauma, Other/Not specified*

  - A target joint was reported by clinicians in 29 (20.1%) of the 144 patients for whom information was collected.

- **Reasons for initiating the last prophylaxis**
  - The reasons reported for initiating the last prophylaxis are described in Table 3. One or more reasons could be given.

Table 3. Reasons for initiation of the last prophylaxis in total moderate HA patients (N=159)

Reason	Patients for whom the reason was collected N	Patients having initiated the last prophylaxis for this reason n (%)
History of frequent bleeding	158	100 (63.3)
History of frequent hemarthroses	158	111 (70.2)
History of severe bleeding	157	7 (4.4)
Prevention of traumatic bleeding	157	34 (21.6)
Other reasons*	158	33 (20.9)

**Arthropathy, Chronic skin lesion, Concomitant treatment affecting hemostasis (antiaggregant or anticoagulant therapy), Hemarthrosis, Hemarthrosis with severe localization (hip), Pseudo-tumor, Situation with over-risk of hemorrhage (tumor, radiotherapy, chemotherapy), Specific situation (relay of ITI).*

- **Last prophylactic treatment**
  - Among the 159 patients in the study, 79 were receiving FVIII prophylaxis, 78 patients were receiving extended half-life FVIII, and for 2 patients the information was not available.
    Of note, among the 159 patients, 10 initiated a prophylaxis with emicizumab (before September 2022, the date of data extraction).

Discussion / Conclusions

- This real-world study using FranceCoag data contributes to better characterize patients with moderate HA undergoing prophylactic treatment.
- This study shows the need for prophylaxis in patients with moderate HA patients and a severe bleeding phenotype, as well as in severe HA patients.

EAHAD February 5-7th, 2025, Milan - Italy

References

1. RCP emicizumab. https://www.ema.europa.eu/en/documents/product-information/hemlibra-epar-product-information_en.pdf

Conflict of interest

BG: Biomarin, CSL Behring, LFB, NovoNordisk, Octapharma, Roche/Chugai, Sobi; **HC**: Biomarin, CSL Behring, NovoNordisk, Octapharma, Pfizer, Roche/Chugai, Sobi/Sanofi; **AL**: Bayer, CSL Behring, LFB, Novonordisk, Octapharma, Pfizer, Roche, Sobi, Takeda; **DP**: Roche SAS employee; **JV**: Roche SAS employee; **SP**: Roche SAS employee, **AG**: Chugai Pharma France employee; **RDO**: Shire/Takeda, Biomarin, CSL Behring, LFB, NovoNordisk, Octapharma, Pfizer, Roche, Sobi/Sanofi, Spark and UniQure