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First real-world evidence study of patients with haemophilia in Algeria

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BACKGROUND & OBJECTIVES

Background

- A patient registry under the ownership of the Algerian Ministry of Health was designed to collect local real-world evidence to monitor innovative care in congenital bleeding disorders, including haemophilia

Objectives

- The primary objective of this analysis was **to describe the progress of patients with haemophilia or other rare haematological disorders in Algeria** over a 3-year period
- Secondary objectives include:
  - To study the epidemiological profile of patients with haemophilia
  - Evaluate haemophilia patient care at inclusion
  - To estimate the incidence of complications occurring during follow-up in patients with haemophilia
  - Describe associated comorbidities
  - Assess quality of life of patients

MATERIALS & METHODS

Study design

- "Barometer" is a national, multicentre, observational registry that began including patients with haemophilia and other congenital bleeding disorders at treatment centres between 01 July 2018 and 24 April 2022
- Input from the cases was captured by the different study centres using a platform developed for this analysis
- Epi-Info 7 and R software were used for data analysis
- Parameters collected and evaluated by a multidisciplinary team included demographic and geographical characteristics, haematological diagnoses, disease severity and treatment modalities
- Informed consent was obtained from the patient or guardian prior to any study-related activities

Descriptive analysis

- Qualitative parameter results were expressed as percentages calculated based on the total sample number with a confidence interval of 95%
- Quantitative variable results were expressed as means with the standard deviation

RESULTS

Patient distribution

- 1134 patients with haemophilia were recruited, representing approximately 40% of known patients recorded in Algeria in 2020[1]
- 31% of these patient were under the age of 12
- 49.9% of the cases were recruited from the central region of the country, 34.7% from the eastern region and 15.4% from the western region
- The distribution of patients by inclusion centre is presented in **Table 1**
- Haemophilia occupied the top position with 1134 cases, representing a frequency of 84.12% (95% CI [82.08%–85.98%]) of all hereditary bleeding diseases
- The number of patients affected by other rare hereditary haematological disorders was 214, or 15.9% (95% CI [14.0%–17.9%])
- The breakdown of all hereditary bleeding disease cases are presented in **Table 2**
- The distribution of haemophilia patients according to the circumstances of discovery is presented in **Table 3**
- The majority of cases were haemophilia A (n = 934, 82%), with haemophilia B being less prevalent (n = 200, 18%)
- 13 patients were female
- The severe form was predominant and affected approximately more than 2/3 of the cases (95% CI: [65.3%–70.5%])
- Patient distribution according to haemophilia type, severity and age are presented in **Figure 1**
- Inhibitors were reported in 126 patients with haemophilia A and 2 patients with haemophilia B

Table 1: Distribution of patients by inclusion centre

Inclusion Centre	Effective	%
CHU Beni Messous	584	43.3
CHU Annaba	187	13.9
CHU Constantine	141	10.5
CHU Sétif	140	10.4
CHU Tlemcen	126	9.3
EHS Canastel Oran	73	5.4
CHU Mustapha	52	3.9
Centre Tizi Ouzou	36	2.7
Centre Oran	9	0.7
Total	**1348**	**100**

EHS, Établissement hospitalier spécialisé (Hospital establishment specialising); CHU, Centre hospitalo-universitair (University hospital centre)

Table 2: Breakdown of cases by disease type

Disease type	Effective	%
Haemophilia	1134	84.1
Factor VII deficiency	80	5.9
Willebrand factor deficiency	66	4.9
Fibrinogen deficiency	13	1.0
Glanzmann Thrombasthenia	13	1.0
Other combined deficits	11	0.8
Factor V deficiency	8	0.6
Factor XIII deficiency	7	0.5
Factor XI deficiency	6	0.5
Jean Bernard and Soulier Syndrome	4	0.3
Other disorders of primary haemostasis	3	0.2
Factor X deficiency	3	0.2
Total	**1348**	**100**

Table 3: Distribution of haemophilia patients according to the circumstances of discovery

Circumstances of discovery	Number of haemophilia patients	%
Secondary to a haemorrhagic manifestation	470	68.2
Family investigation	83	12.0
Circumcision	71	10.3
Fortuitously during a haemostasis assessment	31	4.5
Other	24	3.5
During a surgical procedure	10	1.5
Total	**389**	**100**

Figure 1: Patient distribution according to haemophilia type, severity and age

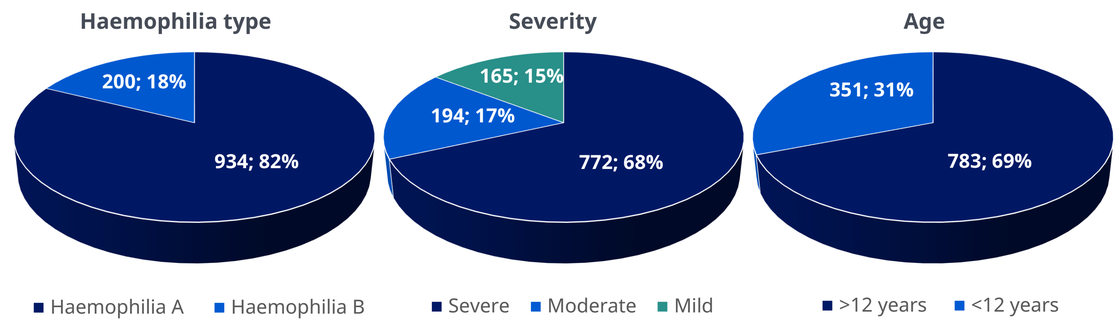
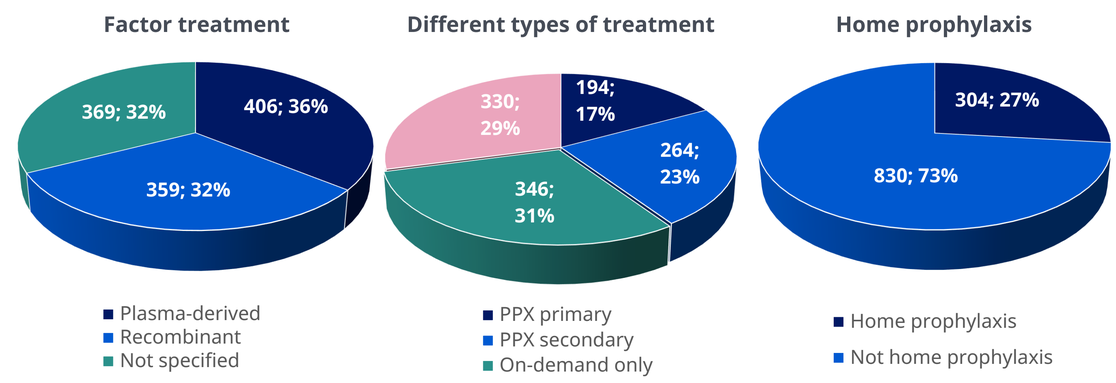


Figure 2: Type of treatment



Type of treatment

- Approximately one-third of haemophilia patients received plasma-derived factor treatment (95% CI: [33.0%–38.6%]; 74 <12 years of age) or recombinant factor treatment (95% CI: [29.0%–34.4%]; 192 <12 years of age)
- Among all patients, only 458, 40.4% (95% CI: [37.6%–43.3%]) were on prophylaxis (primary or secondary)
  - The proportion of cases under secondary prophylaxis was 23.3% (95% CI: [10.9%–25.8%]) higher than that of primary prophylaxis
- Only 300, 26.8% of haemophilia patients (95% CI: [24.3%–29.4%]), took home prophylaxis
  - Home injections were mostly administered by patients themselves or a home nurse
  - Mothers and fathers also administered treatment in equal measure, with a small number of children who also administered treatment themselves
- The proportion of patients not receiving treatment was high
- Details on the type of treatments administered are presented in **Figure 2**

CONCLUSIONS

- This first real-world study **highlights a remaining unmet medical need among patients with haemophilia in Algeria**, including the use of **plasma-derived factor replacement products**
- **29% of patients received no treatment**
- **Haemophilia was the most abundant disease type** with 1134 cases (84.1%)
- **Severe haemophilia is predominant** and affects over 2/3 of the cases
- There is a **dire need to upgrade haemophilia care, especially in children,** to international standards

REFERENCES

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