Healthcare consumption and costs of Hemophilia B in France

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CONTEXT

Hemophilia B is an inherited bleeding disorder characterized by a deficiency in blood clotting factor IX (FIX). Its severity varies based on the degree of FIX deficiency, with the most severe cases experiencing frequent spontaneous hemorrhages (FIX levels < 1 IU/dL). Treatment options include on-demand administration following a hemorrhage or prophylaxis to prevent bleeding. A complication is the production of inhibitory antibodies against the coagulation factors in a few patients, leading to more aggressive and expensive treatments.

Treatment strategies for patients with inhibitors are based on the use of activated coagulation factor VII concentrates or activated prothrombin complex (known as bypassing agents).

OBJECTIVES

The aim of this study was to estimate the healthcare consumption and related costs of Hemophilia B according to severity in France in 2021.

METHODS

- This retrospective observational study used health insurance claims data from the French SNDS database, which covers the whole French population.
- Data relating to all patients diagnosed with Hemophilia B were extracted from the SNDS for the year 2021.
- Patients were categorized in 4 groups according to the pattern of treatment (on demand / in prophylaxis) as a proxy of the severity of the disease (mild or minor / severe), and to the presence of inhibitors: on demand without inhibitor, in prophylaxis without inhibitors, on demand with inhibitors and in prophylaxis with inhibitors
- Healthcare resource use and direct costs were estimated in a collective perspective (payer perspective plus the amount paid by the patients).

RESULTS

A total of **1,311** prevalent patients with Hemophilia B were identified in 2021. Mean age was 36 years and 83.5% were males.

TABLE 1: Patients' characteristics according to type of treatment and presence of inhibitors

	Without inhibitor – on demand	Without inhibitor – prophylaxis	With inhibitor – on demand	With inhibitor – prophylaxis	Total
HB prevalent population (01/01/2021)	996 (76.0%)	303 (23.1%)	6 (0.5%)	6 (0.5%)	1,311 (100%)
Gender (male)	785 (78.8%)	301 (99.3%)	NA	NA	1,095 (83.5%)
Age Mean (SD)	37.2 (22.0)	31.9 (20.4)	NA	NA	35.9 (21.8)

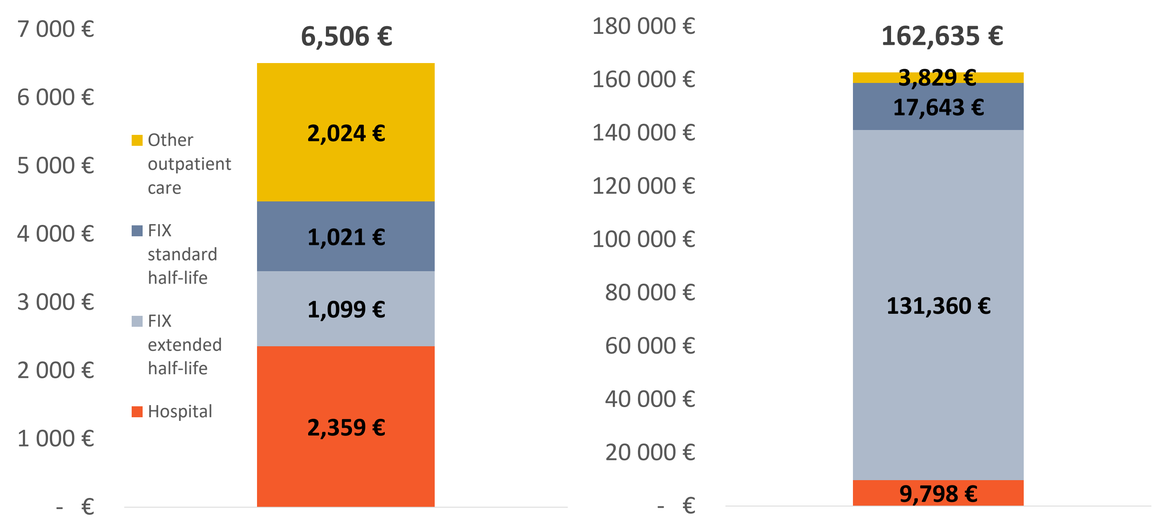
The number of patients was very low (<10) for 2 subgroups: with inhibitor – on demand and with inhibitor – prophylaxis.
For data privacy reason (n<10), it is not allowed to describe these 2 subgroups for age and gender.
Therefore, all results are presented only for 2 subgroups of patients: without inhibitor – on demand and without inhibitor – prophylaxis.

The mean and median annual direct medical costs (drugs, consultations, hospitalization, …) varied strongly according to treatment modalities (FIGURE 1).

- €6,506 and €1,120 per patient treated on demand without inhibitor (N=996, 76.0% of patients)
- €162,635 and €139,857 per patient treated in prophylaxis without inhibitor (N=303, 23.1% of patients)
- €147,677 and €104,487 for 6 patients treated on demand with inhibitors (0.5% of patients)
- €471,439 and €377,926 for 6 patients treated in prophylaxis with inhibitors (0.5% of patients).

Most of the costs were related to antihemophilic drugs: 33%, 92%, 71%, and 91% respectively for the 4 treatment groups.

FIGURE 1: Mean annual direct medical costs per patient



The TABLE 2 documents daily allowances and disabilities in 2021 for adults' patients of working age (18-65 years).

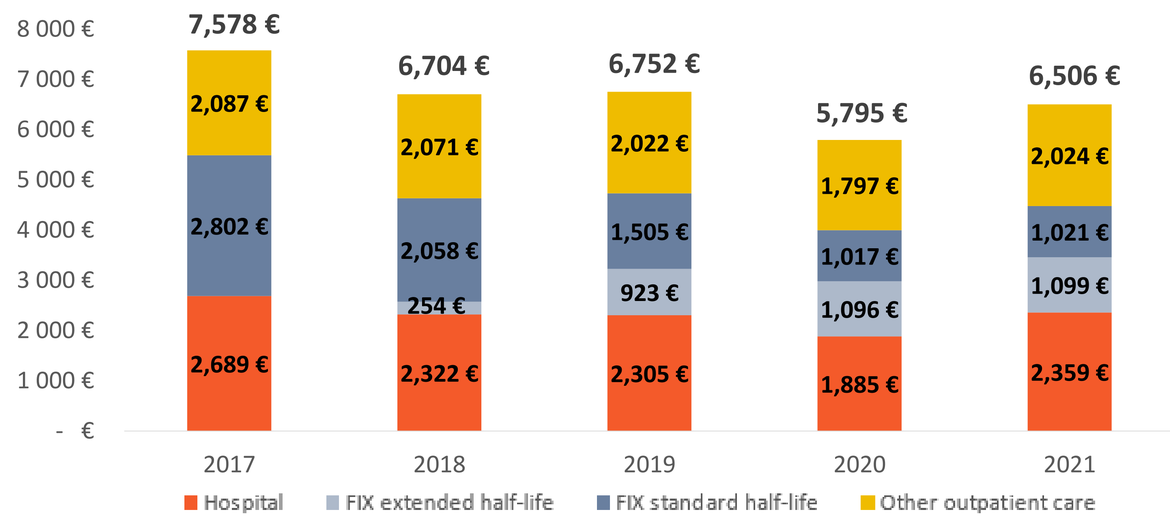
TABLE 2: Daily allowances (DA) and disabilities in 2021

	Without inhibitor – on demand	Without inhibitor – prophylaxis	Total*	P-value
Prevalent HB population at 01/01/2021	996 (76.0%)	303 (23.1%)	1,311 (100%)	
Patients aged 18-65 years	640 (76.9%)	189 (22.7%)	832 (100%)	
Daily allowances (DA) among patients with daily allowances				
At least 1 DA in 2021, n (%)	120 (18.8%)	39 (20.6%)	160 (19.2%)	0.4643
Number of DA in 2021 Mean (mean, SD)	52.7 (85.2)	77.7 (114.5)	59.4 (93.4)	0.3861
Amount paid in € for DA in 2021 (mean, SD)	2,386.8 (4 757.1)	3,149.0 (5 254.4)	2,589.7 (4,866.6)	0.3563
Disabilities in 2021 adults' patients of working age (18-65 years)				
Disability in 2021, n (%)	17 (2.7%)	18 (9.5%)	35 (4.2%)	0.0011
Amount paid in € (disability) in 2021 (mean, SD)	8,132.9 (3 624.2)	8,882.8 (4 279.9)	8,518.6 (3 935.0)	0.5808

* Total is including all patients of the 4 subgroups

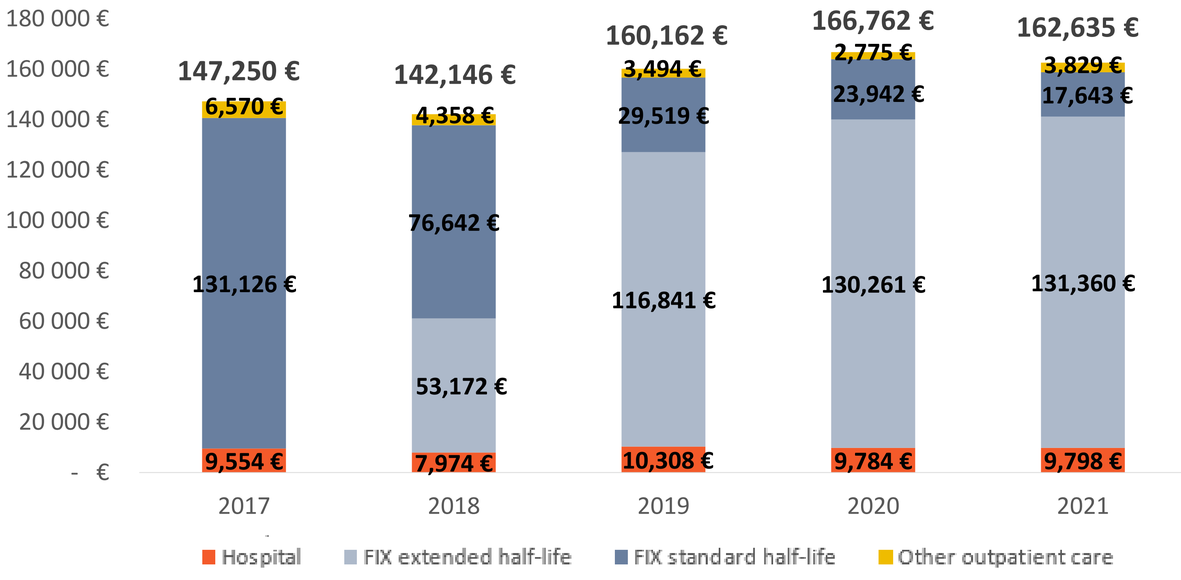
In patients treated without inhibitor and on demand, the overall cost slightly decreased between 2017 and 2021. The cost of FIX standard half-life decreased while the cost of FIX extended half-life increased (FIGURE 2).

FIGURE 2: Evolution of costs between 2017 and 2021 (without inhibitor – on demand)



In patients treated without inhibitor and in prophylaxis, the overall cost remained stable over the years 2017-2021. The cost of FIX standard half-life decreased while the cost of FIX extended half-life increased (FIGURE 3).

FIGURE 3: Evolution of costs between 2017 and 2021 (without inhibitor – prophylaxis)



CONCLUSION

The cost of Hemophilia B is high, varies greatly with disease severity and presence of inhibitors, and is mostly due to the antihemophilic drugs.

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