



Acceptance Code:
EE156

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 2022.

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 2022.
- 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 **1,022** prevalent patients with Hemophilia B were identified in 2022. Mean age was 44.8 years and 81.2% were males. Among the patient's population, 78.2%, 0.6%, 20.9% and 0.3% patients were treated on demand without and with inhibitors and in prophylaxis without and with inhibitors, respectively.

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 (01/01/2022)	799 (78.2%)	214 (20.9%)	6 (0.6%)	3 (0.3%)	1,022 (100%)
Gender (male)	611 (76.5%)	213 (99.5%)	NA	NA	830 (81.2%)
Age Mean (SD)	45.6 (18.8)	41.7 (16.5)	NA	NA	44.8 (18.3)

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.

Compared to controls, patients with HB had more frequently consultations with general practitioners (79.8%/66.1%), hospital specialists (58.5%/25.9%), nurses (69.1%/42.7%), physiotherapists (22.2%/13.6%) and visits to emergency room visits (26.4%/15.9%). They were more frequently treated with analgesics (93.2%/81.5%) and corticosteroids (51.8%/44.7%) but less frequently with non-steroid anti-inflammatory drugs (52.4%/63.6%).

The proportion of HB patients hospitalized was higher than controls, overall (26.7% vs 12.8%) and for bleeding (1.7% vs 0.2%) and orthopedic surgery (8.3% vs 5.7%).

The mean annual direct medical costs varied according to treatment patterns:

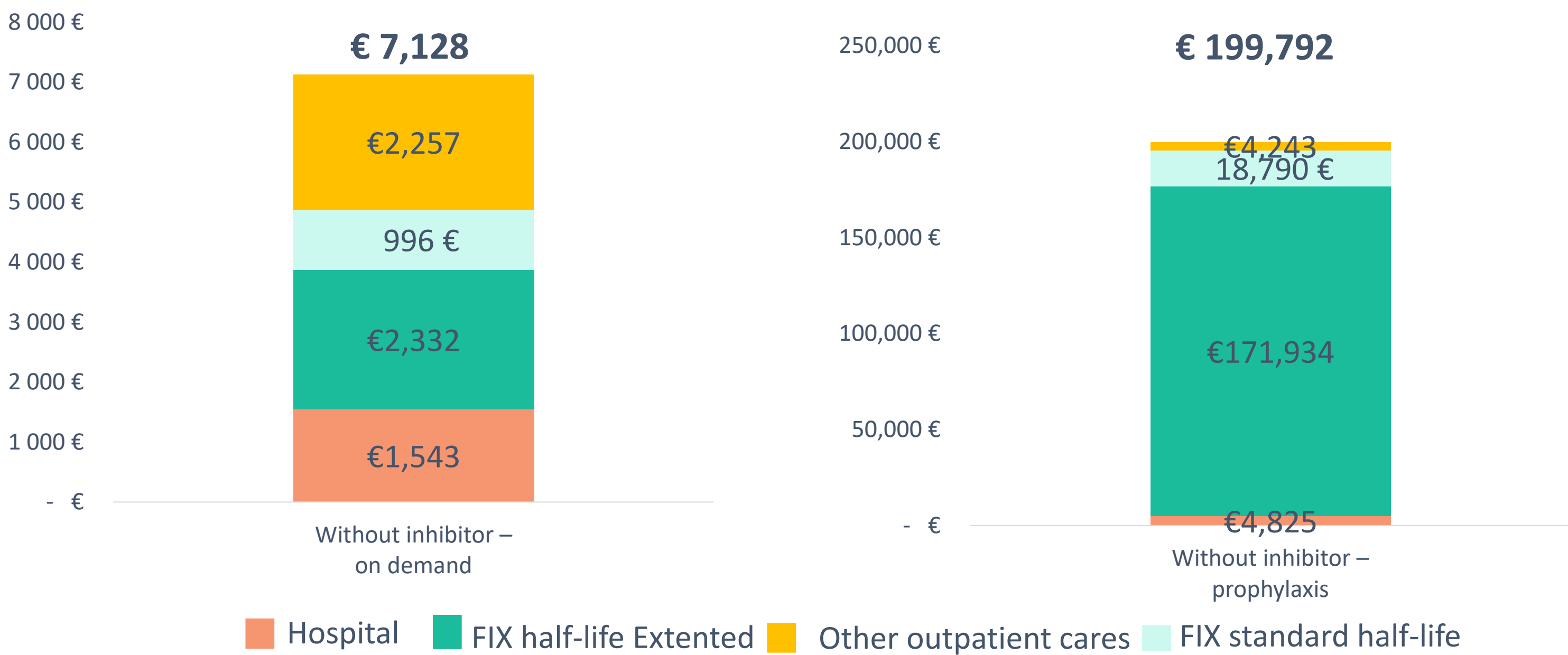
- €7,128 for patients treated on demand without inhibitors (€1,907 for controls)
- €199,792 for patients treated in prophylaxis without inhibitors (€1,428 for controls)

The majority of the costs was related to antihemophilic drugs: 47% and 95% respectively for the 2 treatment groups.

CONCLUSION

The cost of HB is high, varies greatly with treatment regimen and presence of inhibitors, and is mostly due to the antihemophilic drugs.

FIGURE 1: Mean annual direct medical costs in € per patient in 2022 (without inhibitor on demand / prophylaxis)



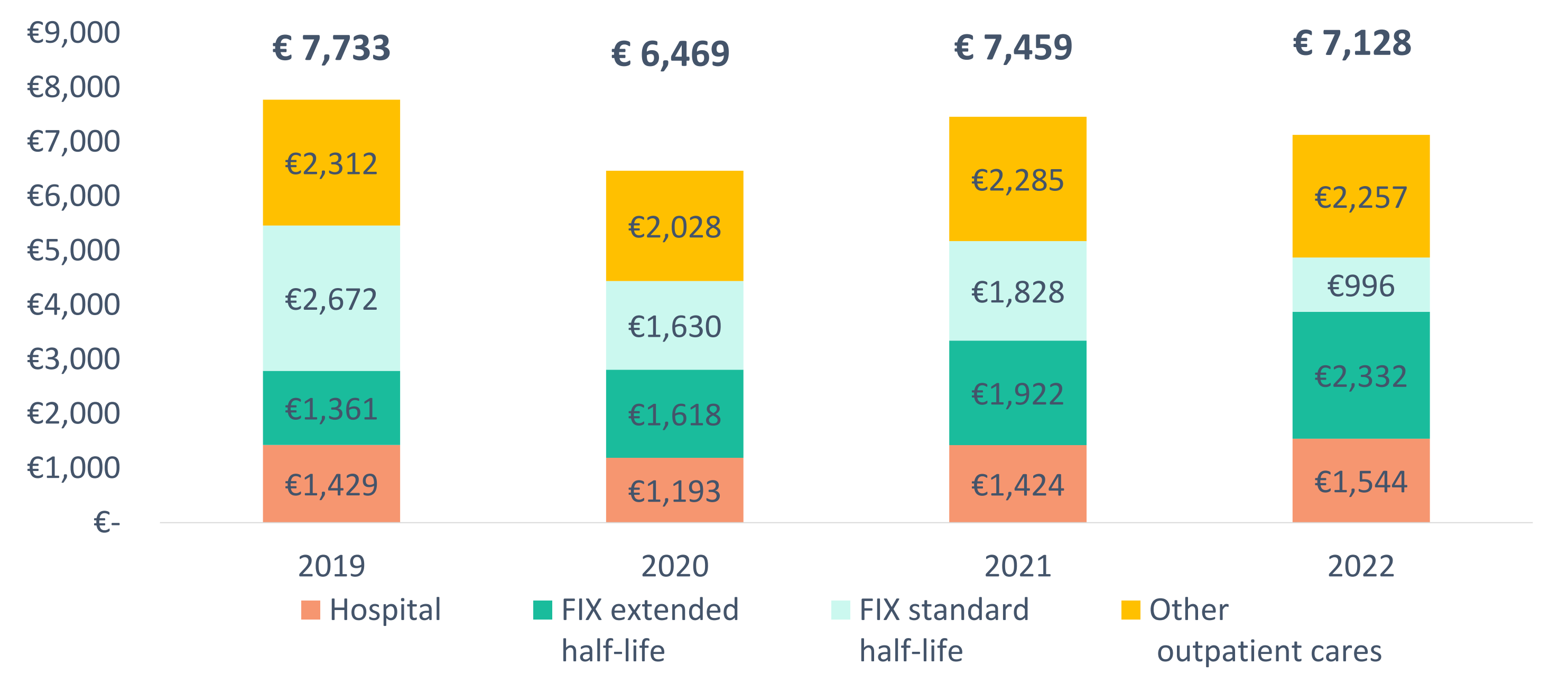
The TABLE 2 documents daily allowances and disabilities in 2022 for adult patients of working age (18-65 years).

TABLE 2: Daily allowances (DA) and disabilities in 2022				
	Without inhibitor – on demand	Without inhibitor – prophylaxis	Total*	P-value
Prevalent HB population at 01/01/2022	799 (78,2%)	214 (20,9%)	1,022 (100%)	
Patients aged 18-65 years	646 (80,9%)	191 (89,3%)	846 (100%)	
Daily allowances (DA) among patients with daily allowances				
At least 1 DA in 2022, n (%)	185 (23.2%)	55 (25.7%)	242 (23.7%)	0.7292
Number of DA in 2022 Mean (mean, SD)	34.9 (69)	62.9 (98)	41.8 (77.4)	0.0842
Amount paid in € for DA in 2022 (mean, SD)	1,570 (3 308)	2,724 (4,819)	1,856 (3,729.5)	0.2804
Disabilities in 2022 adults' patients of working age (18-65 years)				
Disability in 2022, n (%)	17 (2.1%)	19 (8.9%)	36 (3.5%)	0.08566
Amount paid in € (disability) in 2022 (mean, SD)	8,998 (3,857)	8,242 (4,819)	8,518.6 (3 935.0)	0.6096

* Total is including all patients of the 4 subgroups

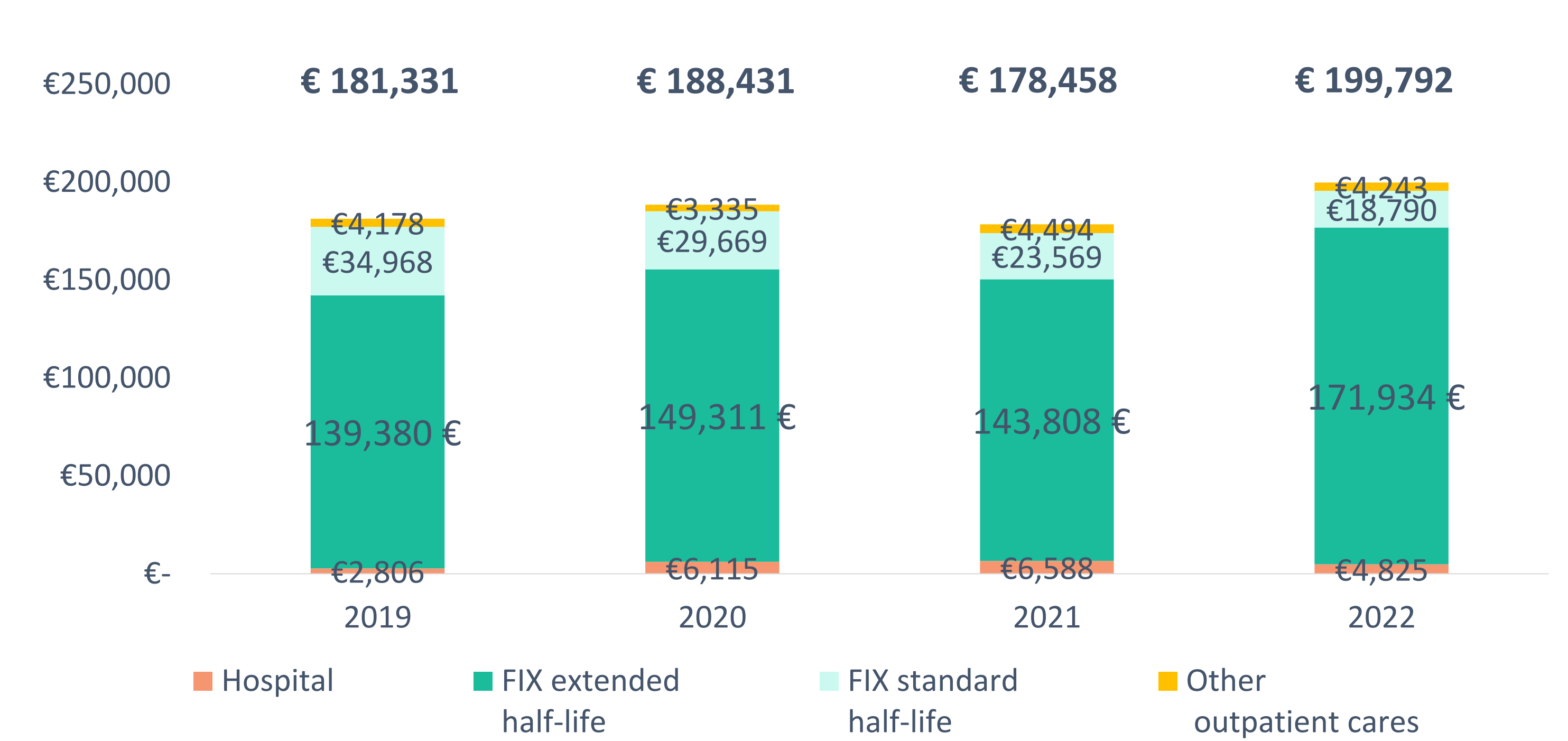
In patients treated without inhibitor and on demand, the overall cost was stable between 2019 and 2022. During this study period, the cost of FIX standard half-life slightly decreased and the cost of extended half-life slightly increased (FIGURE 2).

FIGURE 2: Evolution of costs between 2019 and 2022 (without inhibitor – on demand)



In patients treated without inhibitor and in prophylaxis, the overall cost remain stable over the years 2019-2021 and increased in 2022. 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 2019 and 2022 (without inhibitor – prophylaxis)



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