

CONTEXT

Hemophilia A is an inherited bleeding disorder characterized by a deficiency in blood clotting factor VIII (FVIII). Its severity varies based on the degree of FVIII deficiency, with the most severe cases experiencing frequent spontaneous hemorrhages (FVIII levels < 1 IU/dL). Treatment options include on-demand administration following a hemorrhage or prophylaxis to prevent bleeding. The most frequent complication is the production of inhibitory antibodies against the coagulation factors 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) or, since 2018, prophylactic treatment with Emicizumab in hemophilia A patients.

Since 2019, this treatment has also been indicated as first-line treatment for severe congenital HA without inhibitors, where prophylaxis is indicated. It represents an alternative to FVIII concentrates.

OBJECTIVES

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

METHODS

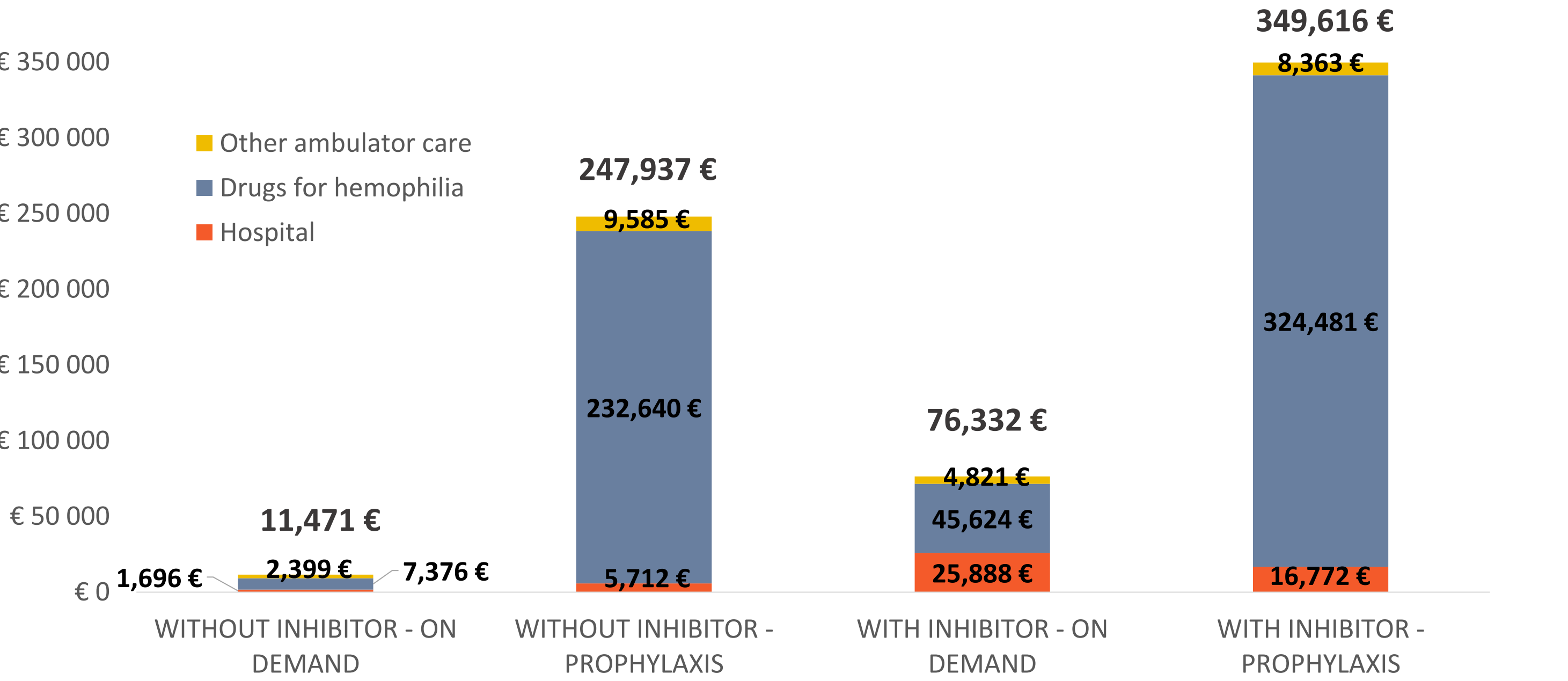
- This retrospective observational study used health insurance claims data from the French SNDS database, which covers the whole French population.
- Data relating to all patients diagnosed with Hemophilia A were extracted from the SNDS for the year 2021.
- Patients were categorized in 4 groups according 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 **5,507** prevalent patients with Hemophilia A were identified in 2021.

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
HA prevalent population (01/01/2021)	4,056 (73.7%)	1,243 (22.6%)	36 (0.7%)	172 (3.1%)	5,507 (100%)
Gender (male)	3,912 (96.4%)	1,240 (99.8%)	35 (97.2%)	172 (100.0%)	5,359 (97.3%)
Age Mean (SD)	38.5 (22.2)	27.9 (18.4)	43.5 (23.8)	26.0 (20.4)	35.8 (21.9)

FIGURE 1: Mean annual direct medical costs per patient



The mean and median annual direct medical costs (drugs, consultations, hospitalizations, ...) varied strongly according to treatment modalities and presence of inhibitors (Figure 1):

- €11,471 and €1,205 per patient treated on demand without inhibitor (N=4,056, 73.7% of patients).
- €247,937 and €211,928 per patient treated in prophylaxis without inhibitor (N=1,243, 22.6% of patients).
- €76,332 and €33,190 per patient treated on demand with inhibitors (N=36, 0.7% of patients).
- €349,616 and €369,777 per patient treated in prophylaxis with inhibitors (N=172, 3.1% of patients).

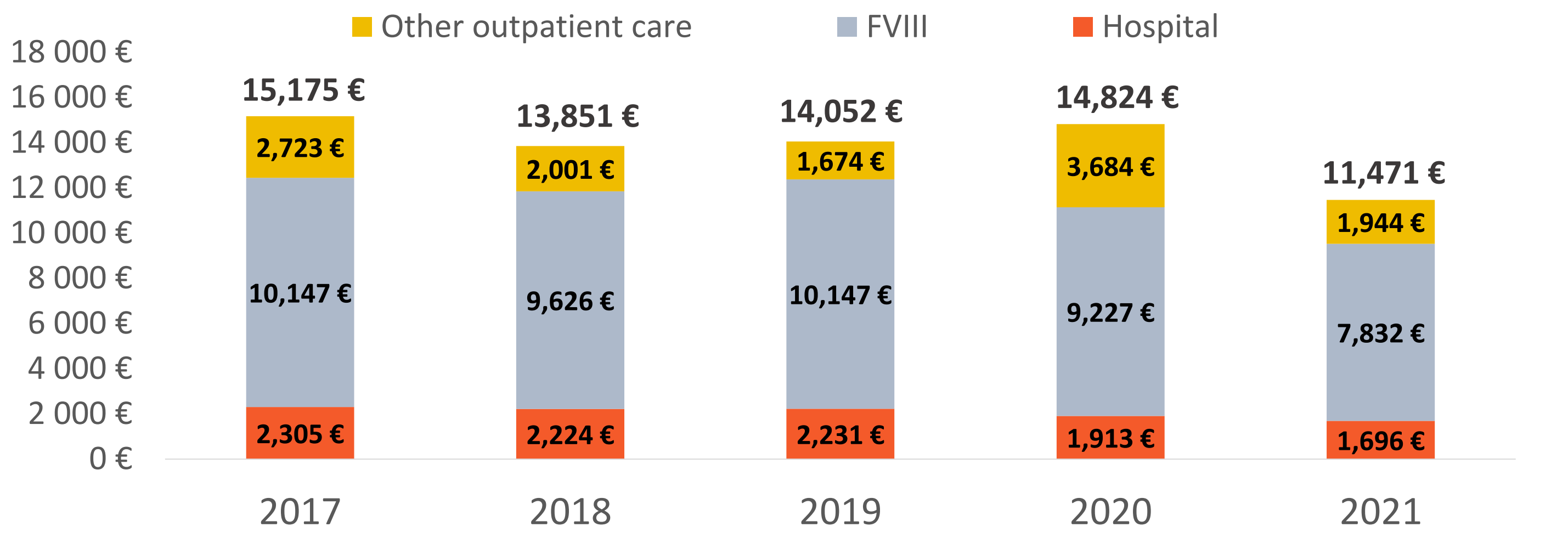
The majority of the costs was related to antihemophilic drugs: 64%, 94%, 60% and 93% respectively for the 4 treatment groups.

CONCLUSION

The cost of Hemophilia A is high, varies greatly with disease severity and presence of inhibitors, and is mostly due to the antihemophilic drugs. The availability of Emicizumab on the market modified the mean cost of HA patients treated in prophylaxis.

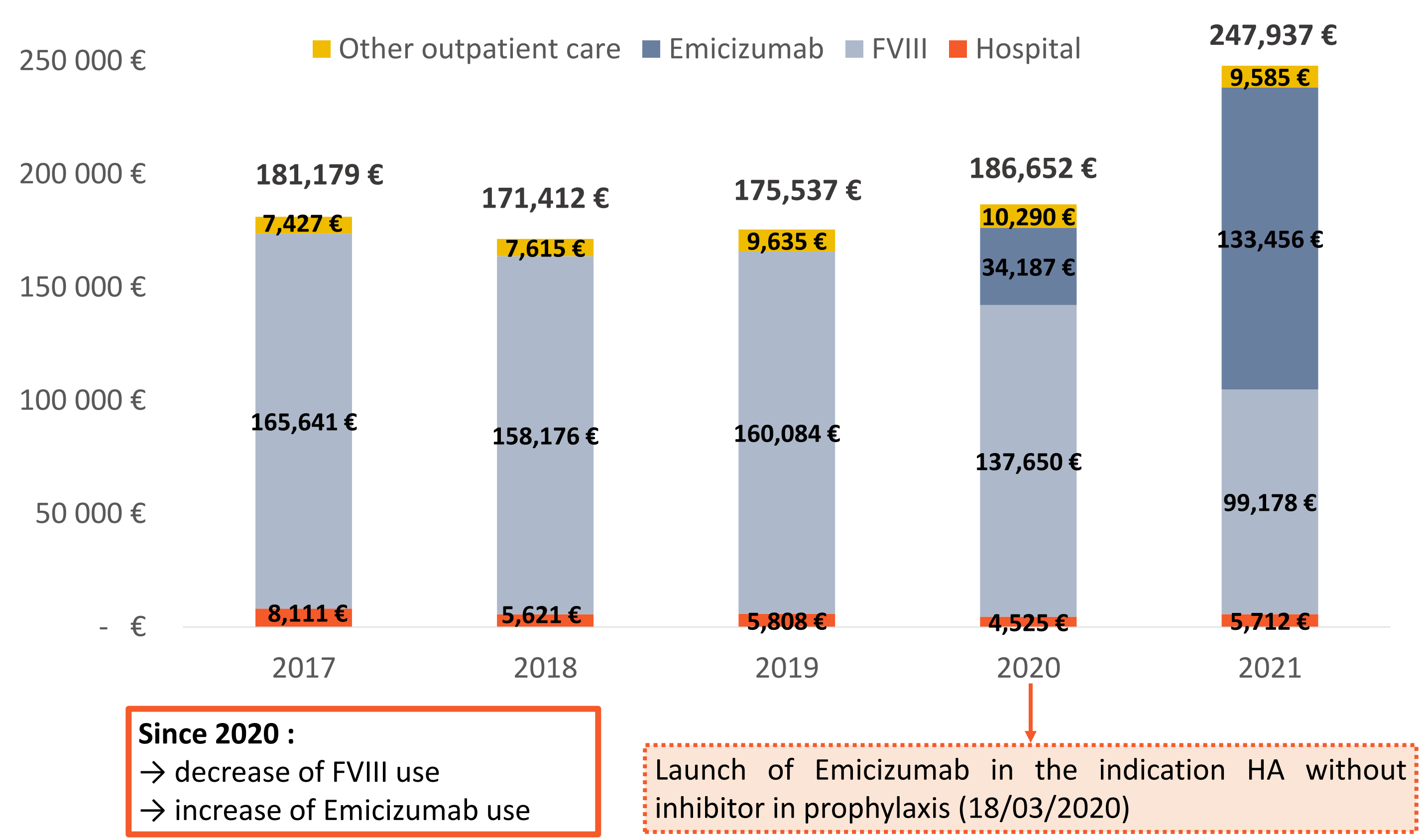
In patients treated without inhibitor and on demand, the overall cost slightly decreased between 2017 and 2021, mainly because of a decrease of the cost of factor VIII, but also a decrease of the cost of hospitalizations and other outpatient cares (FIGURE 2).

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



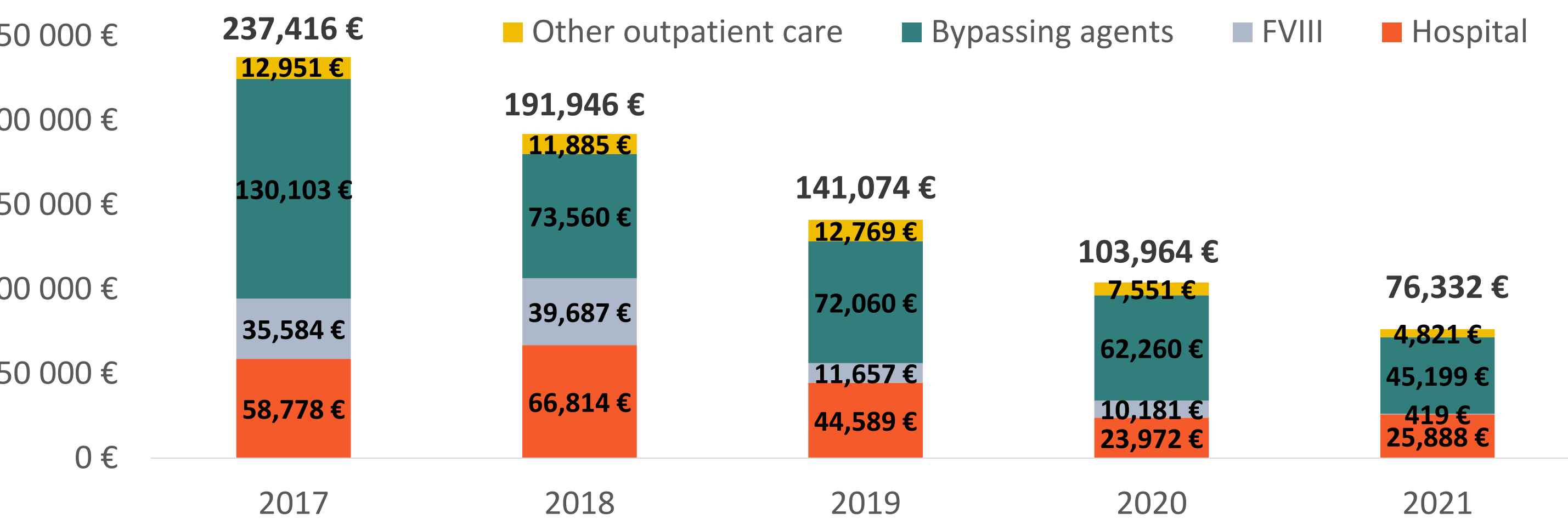
In patients treated without inhibitor and in prophylaxis, the overall cost remained stable over the years 2017-2019. In 2020, the cost of factor VIII decreased but the total cost increased due to the launch on the market of Emicizumab (FIGURE 3).

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



In patients treated with inhibitors and on demand, the overall cost decreased over the years 2017-2021, mainly due to the drop of bypassing agents use (FIGURE 5).

FIGURE 5: Evolution of costs between 2017 and 2021 (with inhibitors – on demand)



In patients treated with inhibitors and in prophylaxis, on the period 2017-2021, the overall cost decreased despite the launch of Emicizumab which allows a huge decrease of bypassing agents (FIGURE 4).

FIGURE 4: Evolution of costs between 2017 and 2021 (with inhibitors – prophylaxis)

