

Real-world evidence study on Hemophilia B treatment with Factor IX and corresponding treatment costs in Finland

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CONCLUSIONS

- This real-world evidence study reveals 40-50 Hemophilia B patients per year purchased FIX replacement products in Finland
- Most Hemophilia B patients purchasing FIX replacement products were 18-44 years old
- Annual FIX treatment costs were highest in the group of adult patients on FIX prophylactic treatment modality
- FIX replacement product costs are the main driver for the high economic burden for Hemophilia B treatment

INTRODUCTION

- Hemophilia B (HB) is a rare bleeding disorder characterized by deficiency or complete lack of coagulation factor IX (FIX).¹
- HB prevalence rate is estimated with 1 in 30 000 male live births.¹
- HB is associated with a high clinical, humanistic, and economic burden, with substantial morbidity, and lifelong healthcare needs.²
- About two thirds of persons have HB classified based on plasma FIX activity as moderate (activity of 1 to 5% of the normal value) or severe (activity of <1%).³
- Dependent on disease severity, HB patients are at risk for spontaneous bleeding into the joints and muscles, leading to synovitis and joint arthropathy⁴ and may also result in gastrointestinal bleeds or intracranial hemorrhage.⁵
- Moderate to severe HB is treated with lifelong, continuous FIX replacement therapy to prevent bleeding.⁷
- In Finland, four different FIX replacement products are reimbursed, i.e., human coagulation factor IX, nonacog alfa, efrenonacog alfa, and nonacog beta pegol.

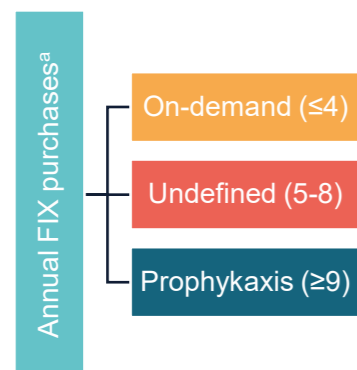
AIM

- This real-world evidence study aimed to provide up-to-date information on HB patient numbers, treatment modalities and FIX costs in Finland.

METHODS

- Data from the Social Insurance Institution of Finland (SII) were used to assess the total number of HB patients based on FIX product purchases (ATC-B02BD04) per calendar year.
 - For each patient, treatment initiation was defined as the date of their first ever purchase of any of the reimbursed FIX products.
- Patients were stratified by age groups and categorized according to three different treatment modalities based on their number of annual FIX purchases (Figure 1) for each calendar year during 2019-2022.

Figure 1. Treatment modality categorization based on FIX purchases 2019-2022^a.



^aBased on data for all reimbursed FIX products (ATC-B02BD04) from SII.

Total annual treatment costs were calculated based on all individual FIX purchases made i.e., cost of any FIX product purchased [€].

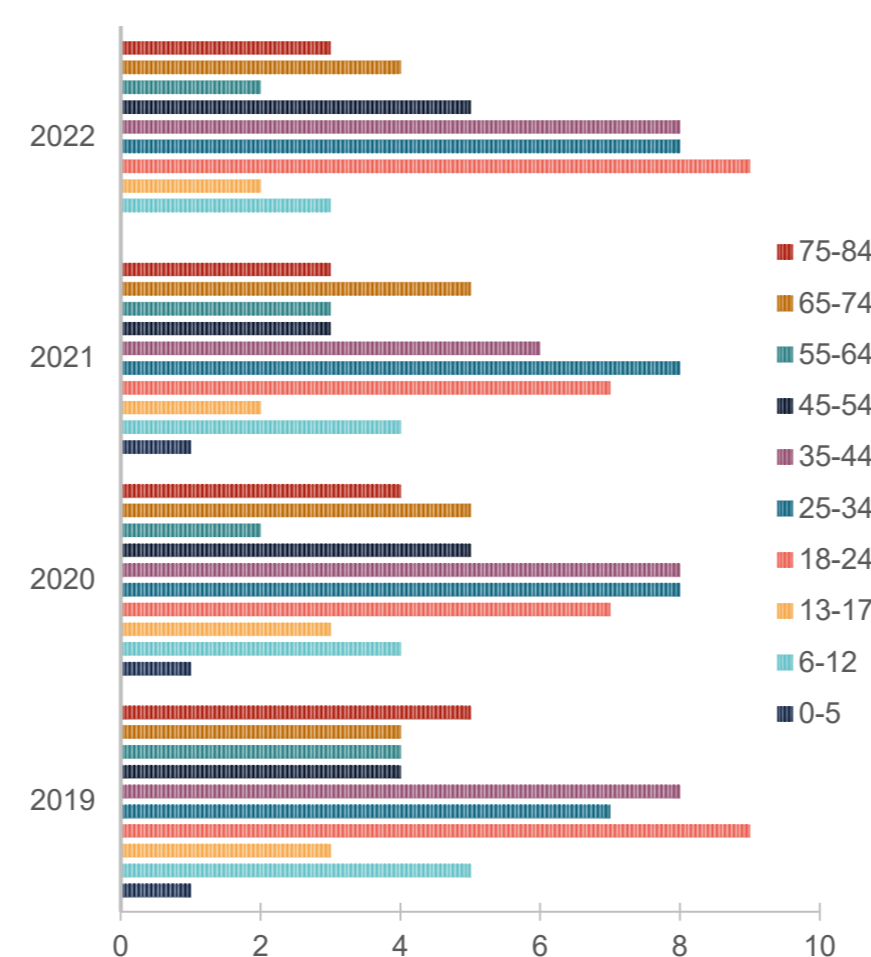
- Annual FIX treatment costs per patient were calculated based on the “total FIX costs [€]” and “total time of follow-up” as per calendar year.
- Annual FIX treatment costs were further stratified by age group and treatment modality.
- Data was reported as summary statistics.

RESULTS

Patient population by age

- In total, n=50 (2019), n=47 (2020), n=42 (2021), and n=44 (2022) unique HB patients purchased FIX products (Figure 2).
- All patients were males of which most were 18-44 years of age.

Figure 2. Annual number of unique HB patients purchasing FIX replacement products per age group 2019-2022^a.



^aBased on SII data for all reimbursed FIX product purchases (ATC-B02BD04) from the open access portal.

Patient population by treatment modality

- Patients were stratified by age groups <18, 18-65 and >65 years and treatment modality (Table 1).
- Only a few patients were <18 years and most were on prophylactic treatment.
- Most patients were 18-65 years and of these, about half were treated on-demand, while 25% were treated prophylactically while treatment modality was undefined for about one third of the patients (2022).
- About the same number of patients in the >65 years age group was treated on-demand or prophylactically.

Table 1. Annual number of HB patients per pooled age group and treatment modality 2019-2022^a.

Age Group	Treatment modality	2019	2020	2021	2022
<18 years	All	9	8	7	5
<18 years	On-demand	2	1	1	0
<18 years	Prophylaxis	1	4	5	3
<18 years	Undefined	6	3	1	2
18-65 years	All	33	32	27	32
18-65 years	On-demand	12	11	9	14
18-65 years	Prophylaxis	9	8	6	8
18-65 years	Undefined	12	13	12	10
>65 years	All	8	7	8	7
>65 years	On-demand	2	3	3	3
>65 years	Prophylaxis	3	3	2	2
>65 years	Undefined	3	1	3	2
Total	All	50	47	42	44

^aBased on SII data for all reimbursed FIX product purchases (ATC-B02BD04). Patients 65 years old at data request were included in the group 18-65 years.

Total annual FIX treatment costs

- The overall annual FIX treatment costs for all patients independent of age group and treatment modality were 6,4m€, 6,3m€, 6,2m€, and 5,9m€ for years 2019, 2020, 2021, and 2022 respectively (SII data).

Annual FIX treatment costs per patient and treatment modality

- In 2022, the mean annual FIX treatment cost per patients <18 years of age on prophylactic treatment was 114,173€ (n=3, no patients with evident on-demand treatment); 18-65 years 230,150€ (n=8, prophylaxis) and 104,611€ (n=14, on-demand); and >65 years 215,569€ (n=2, prophylaxis) and 92,452€ (n=3, on-demand) (Table 2).
- Mean annual FIX costs for adult patients (18-65 years) with undefined treatment modality were 143,153€ (n=10) and 119,767€ (n=2) for patients >65 years.

Table 2. Mean and median annual costs per patient for FIX replacement products stratified by age group and treatment modality 2022^a.

Age Group	Treatment modality	Mean (SD)	Median (IQR)
<18 years	All	83,753 € (56,149 €)	59,181 € (49,416 €-120,705 €)
<18 years	On-demand	-	-
<18 years	Prophylaxis	114,173 € (52,035 €)	120,705 € (89,943 €-141,669 €)
<18 years	Undefined	38,122 € (15,972 €)	38,122 € (32,475 €-43,769 €)
18-65 years	All	146,055 € (93,000 €)	135,205 € (72,784 €-199,686 €)
18-65 years	On-demand	99,772 € (99,084 €)	70,045 € (37,065 €-105,755 €)
18-65 years	Prophylaxis	230,149 € (60,202 €)	200,190 € (195,620 €-256,933 €)
18-65 years	Undefined	143,577 € (56,272 €)	135,205 € (105,251 €-176,630 €)
>65 years	All	134,153 € (89,263 €)	159,683 € (68,250 €-189,290 €)
>65 years	On-demand	89,468 € (103,401 €)	56,651 € (31,554 €-130,973 €)
>65 years	Prophylaxis	215,568 € (59,797 €)	215,568 € (194,427 €-236,710 €)
>65 years	Undefined	119,767 € (56,451 €)	119,767 € (99,808 €-139,725 €)

^aBased on SII data for all reimbursed FIX product purchases (ATC-B02BD04).

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COI statements:

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