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Factor consumption and bleeding outcomes of three commonly used factor IX products for hemophilia B prophylaxis in patients of any age: a retrospective medical chart analysis in the United States

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Introduction

- Hemophilia B (HB) is a rare X-linked congenital bleeding disorder caused by a deficiency of factor IX (FIX)[1]
- In the United States of America (USA), recombinant FIX albumin fusion protein (rIX-FP), recombinant FIX Fc fusion protein (rFIXFc) and recombinant FIX (rFIX) are commonly used to treat people with hemophilia B (PwHB)
- Clinical trials have demonstrated the efficacy and safety of each product;[2–7] although, there have been no direct head-to-head comparisons
- Real-world data will provide insights into the utilization and effectiveness of rIX-FP, rFIXFc and rFIX in clinical practice

Objective

- **To assess real-world FIX consumption and bleeding outcomes for PwHB of any age receiving prophylactic treatment with rIX-FP, rFIXFc or rFIX**

Methods

- Anonymized medical chart information was retrospectively obtained from USA healthcare institutions between 2020–2023
- The analysis included patients of any age with a diagnosis of severe or moderate hemophilia B (FIX activity ≤5%) and at least 12 months of prophylactic treatment with rIX-FP, rFIXFc, or rFIX
- FIX consumption was determined based on the most recently prescribed dosing frequency and dosage
- Annualized bleeding rate (ABR), annualized spontaneous bleeding rate (AsBR), and annualized joint bleeding rate (AjBR) were calculated based on the number of bleeding events over the observation period
- Generalized linear models adjusting for covariates were used to test statistical significance of the differences of consumption, ABR, AsBR, and AjBR between the products

Results

PATIENTS

- Patient characteristics are shown in **Table 1**
- A total of 253 PwHB were included with 99, 81, and 73 PwHB receiving prophylactic treatment with rIX-FP, rFIXFc, or rFIX, respectively
- Mean age of patients receiving rIX-FP, rFIXFc or rFIX was 28.2, 32.1, and 25.6 years, respectively; 16% of patients were <12 years old; 58% of patients had severe HB

Table 1: Patient characteristics

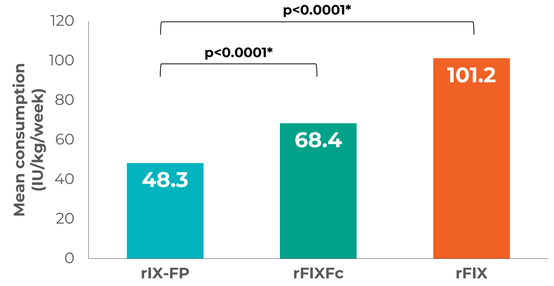
FIX product	rIX-FP (n=99)	rFIXFc (n=81)	rFIX (n=73)
Age (years), mean (SD)	28.2 (16.1)	32.1 (18.2)	25.6 (18.1)
≥12 years, n (%)	88 (88.9)	70 (86.4)	55 (75.3)
Weight (kg), mean (SD)	72.8 (22.4)	74.7 (21.0)	69.5 (22.2)
<1% FIX activity, n (%)	58 (58.6)	46 (56.8)	42 (57.5)
Observation period (months), mean (SD)	32.3 (7.6)	31.6 (8.0)	29.4 (8.9)

FIX, factor IX; rFIXFc, recombinant FIX Fc fusion protein; rFIX, recombinant FIX; rIX-FP, recombinant FIX albumin fusion protein; SD, standard deviation.

CONSUMPTION AND DOSING

- The mean consumption of rIX-FP, rFIXFc, and rFIX administered are shown in **Figure 1**
- PwHB treated with rIX-FP had a significantly lower (48.3 IU/kg/week) mean FIX consumption compared with those treated with rFIXFc, and rFIX (68.4 IU/kg/week, and 101.2 IU/kg/week, respectively; p<0.0001)
- Dosing intervals for each product are presented in **Figure 2**

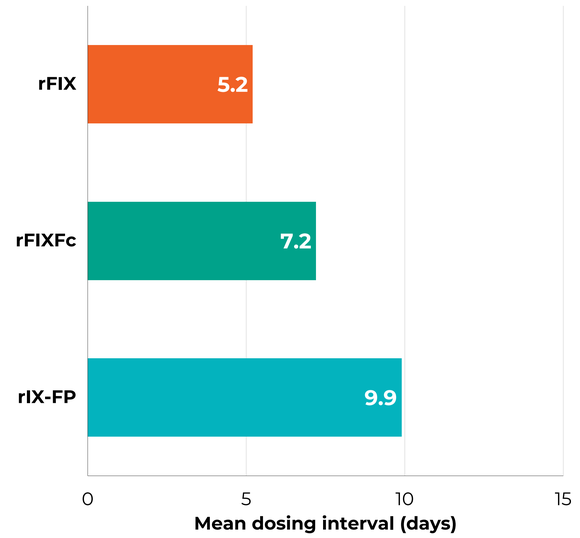
Figure 1: FIX consumption



FIX product	rIX-FP (n=99)	rFIXFc (n=81)	rFIX (n=73)
Median (Q1, Q3) IU/kg/week	45.9 (30.0, 60.5)	59.2 (50.0, 70.8)	100.0 (79.2, 101.8)

*The Generalized Linear Model for FIX consumption incorporated age, weight, and hemophilia severity as covariates, with the Gaussian family selected with an identity link function.
FIX, factor IX; IU, international units; Q1, first quartile; Q3, third quartile; rFIXFc, recombinant FIX Fc fusion protein; rFIX, recombinant FIX; rIX-FP, recombinant FIX albumin fusion protein; SD, standard deviation.

Figure 2: Mean dosing intervals



FIX product	rIX-FP (n=99)	rFIXFc (n=81)	rFIX (n=73)
Median (Q1, Q3), days	7.0 (7.0, 14.0)	7.0 (7.0, 7.0)	7.0 (3.5, 7.0)

FIX, factor IX; Q1, first quartile; Q3, third quartile; rFIXFc, recombinant FIX Fc fusion protein; rFIX, recombinant FIX; rIX-FP, recombinant FIX albumin fusion protein.

BLEEDING OUTCOMES

- Bleeding rates are presented in **Table 2**
- Mean ABR was significantly lower for PwHB receiving rIX-FP prophylaxis (1.2) versus rFIXFc (2.0; p=0.0058) and rFIX (2.1; p=0.0011)
- For those patients with available data, mean AsBR was significantly lower for rIX-FP prophylactic therapy (0.4) versus rFIXFc (1.0; p=0.0024) and rFIX (0.8; p=0.0056)
- For those patients with available data, mean AjBR was also significantly lower for prophylaxis with rIX-FP (0.6) versus rFIXFc (1.1; p=0.040) and rFIX (1.1; p=0.0210)

Table 2: Bleeding rates

FIX product	rIX-FP	rFIXFc	rFIX
ABR			
N	99	81	73
Mean (SD)	1.2 (1.5)	2.0 (1.9)	2.1 (2.5)
Median (Q1, Q3)	0.6 (0.0, 1.6)	1.3 (0.3, 3.7)	1.6 (0.5, 3.2)
p-value for ABR comparison*	NA	0.0058	0.0011
Patients with zero ABR, n (%)	32 (32.3)	15 (18.5)	15 (20.5)
AsBR			
N	70	57	62
Mean (SD)	0.4 (0.8)	1.0 (1.3)	0.8 (1.1)
Median (Q1, Q3)	0.0 (0.0, 0.3)	0.3 (0.0, 1.8)	0.0 (0.0, 1.5)
p-value for AsBR comparison*	NA	0.0024	0.0056
Patients with zero AsBR, n (%)	50 (71.4)	25 (43.9)	33 (53.2)
AjBR			
N	71	62	63
Mean (SD)	0.6 (0.9)	1.1 (1.3)	1.1 (1.2)
Median (Q1, Q3)	0.3 (0.0, 1.0)	0.6 (0.0, 1.7)	0.6 (0.0, 1.9)
p-value for AjBR comparison*	NA	0.0400	0.0210
Patients with zero AjBR, n (%)	35 (49.3)	19 (30.6)	19 (30.2)

*The Generalized Linear Model for ABR, AsBR and AjBR, incorporated age, weight, hemophilia severity, and factor consumption as covariates, with the Gaussian family selected with an identity link function.
ABR, annualized bleeding rate; AjBR, annualized joint bleeding rate; AsBR, annualized spontaneous bleeding rate; FIX, factor IX; Q1, first quartile; Q3, third quartile; rFIXFc, recombinant FIX Fc fusion protein; rFIX, recombinant FIX; rIX-FP, recombinant FIX albumin fusion protein; NA, not applicable; SD, standard deviation.
N refers to the sample size with data available for the relevant outcome.

Conclusions

- **Real-world data indicate that prophylaxis with rIX-FP is associated with reduced FIX consumption and improved protection against bleeds, compared to prophylaxis with rFIXFc and rFIX**

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Disclosures

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