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Factor consumption and bleeding outcomes before and after switching to rIX-FP prophylaxis from other factor IX products for hemophilia B: 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, following a switch from another FIX product

Objective

- **To assess real-world FIX consumption and bleeding outcomes, and dosing frequency for PwHB, who switched to rIX-FP from other FIX prophylaxis**

Methods

- De-identified medical chart information was retrospectively obtained from USA healthcare institutions between 2019–2023
- The analysis included patients with a diagnosis of severe or moderate hemophilia B (FIX activity ≤5%) and at least 12 months of prophylactic treatment with rFIXFc, rFIX, or N9-GP before switching to rIX-FP for at least 12 months
- FIX consumption before and after switching 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
- Wilcoxon signed-rank tests were used to test statistical significance of the differences of consumption, ABR, AsBR, and AjBR before and after the switch

Results

PATIENTS

- In total, 16 PwHB were included, 10 switched from rFIX (nonacog alfa), four switched from rFIXFc, one switched from N9-GP, and one switched from nonacog gamma
- Mean age of patients was 26.1 years. Two patients were <12 years old; 11 (68.8%) patients had severe HB (**Table 1**)
- Mean observation period before and after switching was 25.6 and 23.4 months, respectively; all patients remained on rIX-FP until the end of the observation period

Table 1: Patient characteristics

	Before switch (n=16)	After switch (n=16)
Age (years) at switch, mean (SD)	26.1 (13.0)	
≥12 years, n (%)	14 (87.5)	14 (87.5)
Weight (kg), mean (SD)	79.2 (32.1)	83.3 (31.3)
Hemophilia B severity, n (%)		
Severe (≤1%)	11 (68.8)	11 (68.8)
Moderate (1–5%)	5 (31.3)	5 (31.3)
Observation period (months), mean (SD)	25.6 (7.5)	23.4 (7.5)

SD, standard deviation.

CONSUMPTION AND DOSING

- Mean consumption of FIX before and after switching to rIX-FP is shown in **Figure 1**
- Mean FIX consumption was significantly lower following a switch to rIX-FP (49.0 vs 94.2 IU/kg/week respectively; p=0.0004)
- Dosing intervals before and after switching to rIX-FP are shown in **Table 2**
- All PwHB were dosed once weekly or less frequently after switching to rIX-FP, versus seven PwHB before switching

Figure 1: FIX consumption

*Statistical significance was calculated using Wilcoxon signed-rank tests (p<0.05).

Table 2: Dosing intervals

	Before switch (n=16)	After switch (n=16)
Patient dosed once weekly or less frequently, n (%)	7 (43.8)	16 (100.0)
Patient dosed at intervals >10 days, n (%)	0 (0.0)	6 (37.5)

BLEEDING OUTCOMES

- Bleeding rates are presented in **Table 3**
- Mean ABR was significantly lower following a switch to rIX-FP across all PwHB (p=0.0009)
- AsBR and AjBR data were available for 12 PwHB, and were both also significantly lower after switching to rIX-FP

Table 3: Bleeding rates

	Before switch	After switch
ABR		
N	16	
Mean (SD)	3.2 (2.1)	3.2 (1.5)
p-value for ABR comparison*	0.0009	
AsBR		
N	12	
Mean (SD)	0.2 (0.4)	0
p-value for AsBR comparison*	0.0467	
AjBR		
N	12	
Mean (SD)	1.7 (1.3)	0.8 (0.5)
p-value for AjBR comparison*	0.0253	

*Statistical significance calculated using Wilcoxon signed-rank tests (p<0.05). Descriptive statistics (e.g., mean, median, SD, min, max, 1st and 3rd quartiles, frequency distributions) was performed for the variables relevant to this study. Histograms of the variables were generated to assess data distributions. Based on the distributions, generalized linear model (GLM) regression for parallel comparisons and Wilcoxon signed-rank tests were used for switch analysis.
†Four patients did not provide data for joint or spontaneous bleeds necessary for AsBR and AjBR calculations. ABR, annualized bleeding rate; AjBR, annualized joint bleeding rate; AsBR, annualized spontaneous bleeding rate; SD, standard deviation. N refers to the sample size with data available for the relevant outcome.

Conclusions

- **This analysis supports switching to rIX-FP from other FIX products at all dosing intervals, having shown significant reductions in ABR, AsBR, AjBR, and FIX consumption**
- **These findings continue to support the therapeutic effectiveness of rIX-FP**

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Disclosures

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