

Real-World Effectiveness and Usage of rFIXFc in Haemophilia B: Final Paediatric Data from the B-MORE Study

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CONCLUSIONS

- Final data from the B-MORE study confirm that recombinant factor IX Fc fusion protein (rFIXFc) is effective and well-tolerated in children and adolescents with haemophilia B.
- With up to nearly 7 years of total follow-up in a real-world setting, bleeding rates were low, as were injection frequency and factor consumption.

INTRODUCTION

- Extended half-life (EHL) recombinant factor IX Fc fusion protein (rFIXFc; eftrenonacog alfa; Alprolix®) has an established safety and efficacy profile in people with haemophilia B (PwHB).¹⁻⁴
- Continuous and comprehensive real-world data can help inform treatment strategies with rFIXFc in routine practice.
- B-MORE (NCT03901755) was a 24-month, prospective, non-interventional, international study evaluating the real-world effectiveness and usage of rFIXFc in PwHB across Europe and the Middle East.⁵

AIM

- To report final data from the B-MORE study in paediatric PwHB (aged <18 years) treated with rFIXFc.

METHODS

- Eligible PwHB (previously treated and untreated) were prescribed rFIXFc on demand or as prophylaxis, either prior to or at B-MORE study enrolment (Figure 1).
- Final data are reported for paediatric PwHB (age groups: <6 years, 6-12 years and 12-18 years) with ≥6 months of rFIXFc prophylactic treatment since initiation, including both retrospective and prospective periods.
- Outcomes measured in this analysis included annualised bleeding rate (ABR), annualised joint bleeding rate (AJBR), weekly injection frequency and weekly factor consumption as well as patient and physician treatment satisfaction.

RESULTS

Study population

- B-MORE enrolled 151 PwHB from 29 centres, including 73 paediatric PwHB. Of these, 65 paediatric PwHB were included in this analysis (7 were excluded due to starting with on-demand treatment, while 1 was excluded due to having <6 months prophylactic treatment).
- Most included PwHB were male (63/65; 96.9%) with severe disease (51/65; 78.5%) and there was no history of FIX inhibitors at enrolment (Table 1).
- Overall median (range) rFIXFc treatment duration from initiation to end of study was 35.4 (22.9-54.1) months for PwHB aged <6 years, 38.2 (7.3-79.7) months for those aged 6-12 years and 35.4 (19.1-81.3) months for those aged 12-18 years.
 - Notably, some PwHB received up to nearly 7 years of rFIXFc treatment across the retrospective and prospective periods.
- No target joints were developed in paediatric PwHB during the study.

Bleeding rates

- Overall ABRs (Figure 2A) and AJBRs (Figure 2B) were low across age groups (median ABR: ≤0.8; median AJBR: 0.0).

Factor usage

- Median injection frequency was 1 per week for all age groups and median factor consumption was similar between age groups (41.3-46.4 IU/kg/week; Table 2).

Treatment satisfaction

- Patient satisfaction with rFIXFc treatment was high across age groups, with the largest proportion of highly satisfied PwHB (90.9%) in the <6 years age group (Figure 3A).
- Physician satisfaction with rFIXFc treatment was also most commonly rated highly satisfied or satisfied across age groups (Figure 3B).

Safety

- No FIX inhibitor development or serious adverse events related to rFIXFc were reported.

References

1. Powell JS, et al. *N Engl J Med* 2013;369:2313-23; 2. Fischer K, et al. *Lancet Haematol* 2017;4:e75-82; 3. Pasi KJ, et al. *Haemophilia* 2020;26:e262-71; 4. Nolan B, et al. *Blood Adv* 2021;5:2732-9; 5. ClinicalTrials.gov (NCT03901755).

Abbreviations

ABR: annualised bleeding rate; AJBR: annualised joint bleeding rate; BMI: body mass index; EHL: extended half-life; FIX: factor IX; IQR: interquartile range; IU: International Unit; PwHB: people with haemophilia B; rFIXFc: recombinant factor IX Fc fusion protein; SD: standard deviation.

Disclosures

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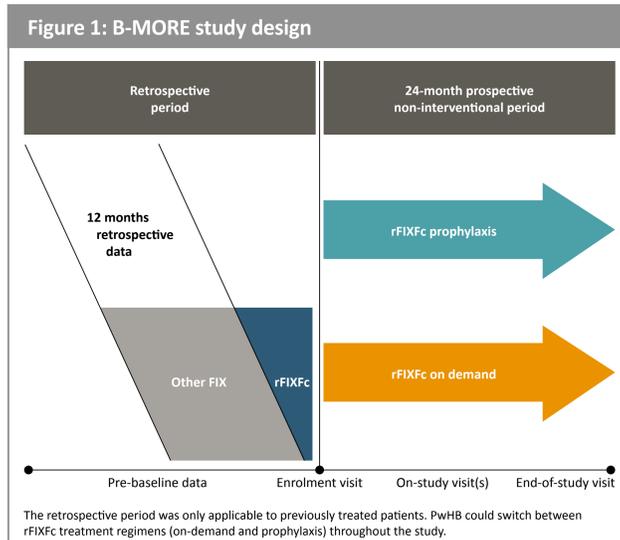


Table 2. Factor usage

Median (IQR)	<6 years (n=13)	6-12 years (n=38)	12-18 years (n=14)
Weekly injection frequency	1.0 (0.9-1.0)	1.0 (1.0-1.0)	1.0 (1.0-1.0)
Weekly factor consumption, IU/kg/week	44.6 (37.5-63.2)	46.4 (40.2-58.2)	41.3 (35.9-49.2)

Table 1: Baseline characteristics

n (%) unless otherwise specified	<6 years (n=13)	6-12 years (n=38)	12-18 years (n=14)
Age (years), median (range)	4.1 (1-6)	8.5 (6-12)	13.8 (12-18)
Gender			
Male	13 (100)	37 (97.4)	13 (92.9)
Female	0	1 (2.6)	1 (7.1)
BMI (kg/m ²) at enrolment, median (range)	17.7 (15-24)	17.0 (13-24)	21.6 (16-40)
Haemophilia severity			
Severe	13 (100)	27 (71.1)	11 (78.6)
Moderate	0	11 (29.0)	3 (21.4)
Mild	0	0	0
Type of previous non-Fc FIX treatment ≤12 months prior to rFIXFc initiation			
Only plasma-derived product	0	8 (21.1)	1 (7.1)
Only recombinant product	6 (46.2)	19 (50.0)	12 (85.7)
Only investigational medicinal product	1 (7.7)	5 (13.2)	1 (7.1)
Plasma derived and recombinant products	0	1 (2.6)	0
None	6 (46.2)	5 (13.2)	0
Treatment regimen prior to rFIXFc initiation			
Prophylaxis	4 (30.8)	28 (73.7)	12 (85.7)
On-demand	0	4 (10.5)	1 (7.1)
Both prophylaxis and on-demand	3 (23.1)	1 (2.6)	1 (7.1)
Previously untreated	6 (46.2)	3 (7.9)	0
Missing	0	1 (2.6)	0
Previously treated without documentation ≤12 months prior to rFIXFc initiation	0	1 (2.6)	0
≥1 target joints at rFIXFc initiation before enrolment ^{a,b}	0	0	0
History of FIX inhibitors at enrolment	0	0	0

Percentages may not sum to 100 due to rounding. [a] Patients with rFIXFc treatment before enrolment: <6 years: n=11; 6-12 years: n=32; 12-18 years: n=13. [b] A target joint is defined as a joint in which ≥3 spontaneous bleeds have occurred within a consecutive 6-month period in the previous year.

