

# Real-World Effectiveness and Usage of Recombinant Factor IX Fc (rFIXFc) in Haemophilia B by Age Groups: Final B-MORE Study Data

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## CONCLUSIONS

Real-world data from the B-MORE study confirm the effectiveness of recombinant factor IX Fc fusion protein (rFIXFc) with low annualised bleeding rates, annualised joint bleeding rates, injection frequency and factor consumption in people with haemophilia B across age groups.

## BACKGROUND

- Extended half-life recombinant factor IX Fc fusion protein (rFIXFc; Alprolix®) has an established efficacy and safety profile in people with haemophilia B (PwHB) across all ages.<sup>1-4</sup>
- Providing continuous, comprehensive real-world data can support informed treatment strategies with rFIXFc in routine practice.
- B-MORE (NCT03901755) was a 24-month, prospective, non-interventional study evaluating the real-world effectiveness and usage of rFIXFc in PwHB across Europe and the Middle East.<sup>5</sup>

## AIM

- To report final data from the prospective period of the B-MORE study in PwHB treated with rFIXFc prophylaxis, stratified by age groups.

## METHODS

- Eligible PwHB were prescribed rFIXFc (on-demand or prophylactically) prior to or at B-MORE study enrolment, regardless of study participation (Figure 1).
  - For this analysis, data are presented for prophylactically treated patients only, given the limited number of patients who received on-demand treatment.
- Final data are reported for PwHB grouped by those aged <12 years and ≥12 years, including those aged ≥50 years within the ≥12 years group.
- Baseline characteristics and follow-up data on rFIXFc during the prospective period are reported using descriptive statistics.
  - Retrospective data were collected as available but are not reported in this analysis.
- Outcomes measured during the prospective period included annualised bleeding rate (ABR) and annualised joint bleeding rate (AJBR), both analysed overall and by bleed type, along with weekly injection frequency (IF), weekly factor consumption (FC), and patient and physician satisfaction.
  - Annualised and weekly endpoints included PwHB with ≥6 months treatment during the prospective period only.
- Safety data, including serious adverse events and adverse events leading to permanent discontinuation of rFIXFc treatment, were collected for the retrospective and prospective periods.

## RESULTS

### Study population

- B-MORE enrolled 151 PwHB from 29 centres; this analysis focuses on 137 PwHB who received rFIXFc prophylaxis for ≥6 months during the prospective period.
- Baseline characteristics are shown in Table 1.
- Median (range) prospective rFIXFc treatment duration was 22.6 (7.3–29.4) months for PwHB aged <12 years, 23.4 (12.0–30.0) months for those aged ≥12 years and 23.3 (12.0–29.3) months for the ≥50 years subgroup.

### Annualised bleeding rates

- Overall ABRs (Figure 2) and AJBRs (Figure 3) were low across age groups (median ABR: ≤0.5; median AJBR: 0.0).
- PwHB aged <12 years had numerically lower rates of spontaneous bleeds than traumatic bleeds, whereas PwHB ≥12 years had similar rates of both bleed types (Table 2).
- These results are considered robust as few bleeds of unknown type were reported across age groups.

### Factor usage

- Median (interquartile range; IQR) IF and FC were similar across age groups, ranging from 1.0 (0.7–1.0) to 1.0 (1.0–1.0) injections/week and 45.6 (38.0–51.7) to 51.0 (38.9–60.3) IU/kg/week, respectively (Table 3).

### Treatment satisfaction

- Patient satisfaction with rFIXFc treatment was high across age groups, with a greater proportion highly satisfied in the <12 years age group compared to ≥12 years (Figure 4A).
- Physician satisfaction with rFIXFc treatment was also high across age groups, following a similar trend to patient satisfaction (Figure 4B).

### Safety

- No inhibitor development or serious adverse events related to rFIXFc were reported.
- Safety data were generally consistent with the established safety profile from phase 3 studies,<sup>1-4</sup> with no new safety concerns.

### 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).

### Disclosures

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### Abbreviations

ABR: annualised bleeding rate; AJBR: annualised joint bleeding rate; BMI: body mass index; FC: factor consumption; HCV: hepatitis C virus; HIV: human immunodeficiency virus; IF: injection frequency; IQR: interquartile range; IU: International Unit; kg: kilogram; PCR: polymerase chain reaction; PwHB: people with haemophilia B; rFIXFc: recombinant factor IX Fc fusion protein; SD: standard deviation.

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Table 1. Baseline characteristics			
n (%), unless otherwise specified	<12 years (N=51)	≥12 years	
		All (N=86)	≥50 years subgroup (n=23)
Age (years), median (range)	7.7 (1–12)	39.1 (12–81)	59.3 (50–81)
Gender, male	50 (98.0)	84 (97.7)	23 (100)
BMI (kg/m <sup>2</sup> ), median (range) [n]	17.3 (13–24) [43]	25.5 (11–46) [77]	27.0 (20–46) [21]
Haemophilia severity			
Severe	40 (78.4)	72 (83.7)	17 (73.9)
Moderate	11 (21.6)	14 (16.3)	6 (26.1)
Mild	0	0	0
Relevant comorbidities at enrolment*			
Yes	9 (17.7)	31 (36.1)	14 (60.9)
No	39 (76.5)	55 (64.0)	9 (39.1)
Missing	3 (5.9)	0	0
History of inhibitors	0	1 (1.2)	0

Baseline characteristics are reported for PwHB treated with rFIXFc prophylaxis for ≥6 months during the prospective period. Percentages may not sum to 100 due to rounding. [a] Relevant comorbidities present in the study population included clinically significant renal, liver and cardiovascular disease, HIV, HCV (PCR positive), clinical depression, non-haemophilic acute or chronic medical conditions causing mobility/joint problems and other coagulation disorders.

Table 2. Annualised traumatic and spontaneous bleeding rates			
Median (IQR)/mean	<12 years (N=51)	≥12 years	
		All (N=86)	≥50 years subgroup (n=23)
ABR			
Traumatic bleeds	0.0 (0.0–1.2)/0.7	0.0 (0.0–0.5)/0.4	0.0 (0.0–0.4)/0.4
Spontaneous bleeds	0.0 (0.0–0.0)/0.1	0.0 (0.0–0.5)/0.4	0.0 (0.0–0.8)/0.7
AJBR			
Traumatic bleeds	0.0 (0.0–0.0)/0.2	0.0 (0.0–0.0)/0.2	0.0 (0.0–0.0)/0.2
Spontaneous bleeds	0.0 (0.0–0.0)/0.0	0.0 (0.0–0.0)/0.3	0.0 (0.0–0.5)/0.5

Results are reported for PwHB treated with rFIXFc prophylaxis for ≥6 months during the prospective period. >80% of PwHB <12 years and >90% of PwHB ≥12 years and ≥50 years reported no bleeds of unknown type; the maximum number of bleeds of unknown type reported per year for these age groups were 1.8, 1.4 and 1.0, respectively.

Table 3. Factor usage			
Median (IQR)	<12 years (N=51)	≥12 years	
		All (N=86)	≥50 years subgroup (n=23)
Weekly IF	1.0 (1.0–1.0)	1.0 (1.0–1.0)	1.0 (0.7–1.0)
Weekly FC, IU/kg/week	51.0 (38.9–60.3)	45.6 (38.0–51.7)	47.3 (41.6–57.3)

Results are reported for PwHB treated with rFIXFc prophylaxis for ≥6 months during the prospective period.

