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

Heidi Glosli,¹ Anna Olsson,² Cristina Santoro,³ Ester Zapotocka,⁴ Martin Scott,⁵ Eva Bednar,⁶ Sabine Lauer,⁶ Stefan Lethagen⁶

<sup>1</sup>Centre for Rare Disorders, Oslo University Hospital, Oslo, Norway; <sup>1</sup>Department of Medicine, Region Västra Götaland, Sahlgrenska University Hospital, Gothenburg, Sweden; <sup>1</sup>Department of Translational and Precision Medicine, Sapienza University Hospital, March Program, March Party, Living Kingdom; <sup>1</sup>Schi, Stockholm, Sundan Stockholm, Sundan Hospital, March Party, March Party, Living Kingdom; <sup>1</sup>Schi, Stockholm, Sundan Stockholm, Sundan Hospital, March Party, March

#### **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>

#### ΔΙΜ

 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.</p>
- 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).

# (38.9–60.3) IU/kg/week Treatment satisfaction

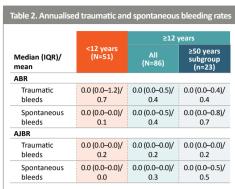
- Patient satisfaction with rFIXFc treatment was high across age groups, with a greater proportion highly satisfied in the <12 years age group compared to ≥12 years (Figure 4A).
- Physician satisfaction with rFIXFc treatment was also high across age groups, following a similar trend to patient satisfaction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

### Safety

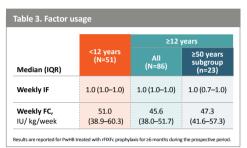
- No inhibitor development or serious adverse events related to rFIXFc were reported.
- Safety data were generally consistent with the established safety profile from phase 3 studies,<sup>1-d</sup> with no new safety concerns.

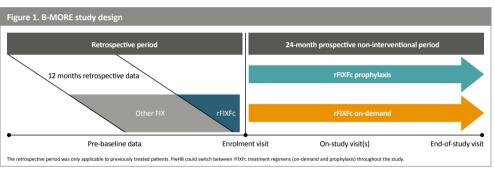
#### n (%), unless specified Age (vears). 7.7 (1-12) 39.1 (12-81) 59.3 (50-81) median (range) Gender, male 50 (98.0) 84 (97.7) 23 (100) BMI (kg/m²), median (range) [n] (13-24) [43] (11-46) [77] (20-46) [21] Haemophilia severity 72 (83 7) Severe 40 (78.4) 17 (73.9) 11 (21.6) 14 (16.3) 6 (26.1) Moderate 0 Relevant comorbidities at enrolment Yes 9 (17.7) 31 (36.1) 14 (60.9) 39 (76.5) 55 (64.0) 9 (39.1) No Missing 3 (5.9) 0 0 History of 0 1 (1.2) 0 inhibitors

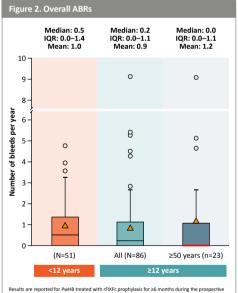
Baseline characteristics are reported for PwHB treated with FFIVFc prophylaxis for 26 months during the prospective period. Percentages may not sum to 100 due to rounding, [a] Relevant comorbidities present in the study population included clinically significant renal, liver and cardiovascular disease, HIV, HCV (PCR positive), clinical depression, non-haemophilic acute or chronic medical conditions causing mobility/pion troblems and other coagulation disorders.



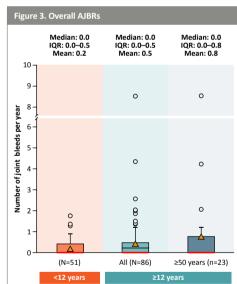
Results are reported for PwHB treated with FIXFc prophylaxis for 26 months during the prospective period. 280% of PwHB <12 years and 250 years reported no bleeds of unknown type; the maximum number of bleeds of unknown type reported per year for these age groups were 1.8, 1.4 and 1.0, respectively.



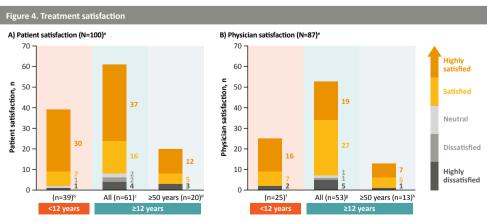




Results are reported for PwHB treated with rFBXFc prophylaxis for 26 months during the prospective period. Figures show: means (orange triangles) and IQR (box boundaries); ends of whisters represent the largest and smallest values excluding the outliers; a blick red line inclinates equal medians', quartiles; cricles represent outliers. Outlier values are defined as >1.5x the IQR above the upper quartile or 1.5x the IQR below the lower quartile. Some outlier data points may overlap.



Results are reported for PwHB treated with rFIXFc prophylaxis for ≥6 months during the prospective period. Figures show means (orange triangles) and IQR (box boundaries); ends of whisters represent the largest and smallest values excluding the outliers; a "bitch red line inclicates equal medians/ quartiles; circles represent outliers. Outlier values are defined as >1.5± the IQR above the upper quartile or >1.5± the IQR above the upper quartile or >1.5± the IQR above the upper quartile or >1.5± the IQR below the IQR above the upper quartile or >1.5± the IQR below the IQR above the Upper quartile or >1.5± the IQR below the IQR above the Upper quartile or >1.5± the IQR below the IQR above the Upper Quartile or >1.5± the IQR below the IQR above the Upper Quartile or >1.5± the IQR



Results are reported for PwHB treated prophylactically for at least 6 months since rFRXTe initiation (N=137) with available data. Patient satisfaction and physician satisfaction data were available for 100 PwHB and 87 PwHB, respectively, across all age groups, [a] Patients statisfaction with rFIXTE treatment as measured at last documentation. [b] n=12 missing, [c] n=25 missing, [d] n=3 missing, [d] Physician satisfaction with rFIXTE treatment as measured at 22 missing minority from the common the form enrollment. [d] n=25 missing, [d] n=3 missi

### References

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

Closures

\*\*Principal investigator for studies by Baxaita (Takeda), Bayer, Novo Nordisk, Octapharma, Roche and Sobi; writing support from Bayer and Sobi; AO: Research grant from CSL Behring; CS: Received honoraria for consulting/speaker bureau from Amgen, Bayer, BioMarin, CSL Behring, Grifols, Novartis, Novo lisk, Piters, Roche, Sobi and Takeda; EZ: Board member and speaking/lecture fees from CSL Behring, Novo Nordisk, Roche and Sobi; MS: Research grant from Bayer; speaker/honoraria for Novo Nordisk, Roche and Sobi; consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consultation fees from CSL Behring, Novo Nordisk, Roche and Sobi; Consul

R: annualised bleeding rate; ABR: annualised joint bleeding rate; BMI: body mass index; PC 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 the haemophilia B; FFIXFc: recombinant factor IX Fc fusion protein; SD: standard deviation.

Acknowledgements

We thank the patients and investigators who participated in the study. The authors acknowledge Daniela Bruni, PharmD, PhD, Sobi for publication coordination, Riddhi Naik, MSG, Costello Medical, UK for medical writing and editorial assistance, and Peter Lang, Costello Medical, UK, for design assistance, full by Sob. Sob. and Soanfi reviewed and provided feetback on the poster. The authors had full editorial control of the poster and provided their final approval of all content. This research is funded by Sobi.

