

Real-world Effectiveness of rFIXFc Prophylaxis in Patients with Haemophilia B Switched from Standard Half-life Therapy in Three European Countries

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SUPPLEMENTARY MATERIAL

Supplementary Table 1. Characteristics of the study population ($n = 30$) by country

Variable	Total population ($n = 30$)	Country		
		Denmark ($n = 9$)	Germany ($n = 9$)	United Kingdom ($n = 12$)
Male, n (%)	30 (100.0)	9 (100.0)	9 (100.0)	12 (100.0)
Age at index date, ¹ years				
Mean (SD)	31.7 (16.7)	25.3 (12.0)	20.9 (11.9)	44.7 (14.8)
Median (IQR)	30.5 (16.5, 45.0)	28.0 (19.0, 31.0)	16.0 (13.0, 30.0)	47.5 (41.5, 54.2)
Range	5–61	5–45	5–38	15–61
Weight at index date, ¹ kg				
Mean (SD)	72.1 (25.2)	74.9 (29.2)	58.8 (29.7)	80.1 (13.8)
Median (IQR)	79.0 (58.5, 86.5)	85.0 (75.0, 90.0)	56.0 (54.0, 85.0)	81.5 (74.2, 85.5)
Range	11.0–107.0	20.0–106.0	11.0–98.0	51.0–107.0
Haemophilia severity, n (%)				
Mild	2 (6.7)	0 (0)	1 (11.1)	1 (8.3)
Moderate	4 (13.3)	2 (22.2)	2 (22.2)	0 (0)
Severe	24 (80.0)	7 (77.8)	6 (66.7)	11 (91.7)
Target joints at index date, ¹ n (%)				
0	26 (86.7)	8 (88.9)	9 (100.0%)	9 (75.0)
1	2 (6.7)	1 (11.1)	0 (0.0)	1 (8.3)
2	2 (6.7)	0 (0.0)	0 (0.0)	2 (16.7)
Comorbidities at index date, ¹ n (%)				
HIV	1 (3.33)	0 (0.0)	0 (0.0)	1 (8.3)
HCV	4 (13.3)	1 (11.1)	1 (11.1)	2 (16.7)
Clinically significant liver disease	1 (3.33)	0 (0.0)	0 (0.0)	1 (8.3)

Variable	Total population (n = 30)	Country		
		Denmark (n = 9)	Germany (n = 9)	United Kingdom (n = 12)
Clinically significant renal disease	1 (3.33)	1 (11.1)	0 (0.0)	0 (0.0)
Clinically significant cardiovascular disease	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Clinical depression	2 (6.7)	2 (22.2)	0 (0.0)	0 (0.0)
Non-haemophilic medical condition causing mobility/joint problems	1 (3.3)	0 (0.0)	1 (11.1)	0 (0.0)
Coagulation disorder other than haemophilia B	1 (3.3)	1 (11.1)	0 (0.0)	0 (0.0)
Other	6 (20.0)	1 (11.1)	1 (11.1)	4 (33.3)
At least one comorbidity	10 (33.3)	3 (33.3)	2 (22.2)	5 (41.7)
No comorbidities	20 (66.7)	6 (66.7)	7 (77.8)	7 (58.3)
Prior regimen, n (%)				
On-demand	6 (20.0) ²	2 (22.2)	2 (22.2)	2 (16.7)
Prophylaxis	24 (80.0) ³	7 (77.8)	7 (77.8)	10 (83.3)
Previous SHL FIX product, n (%)				
Nonacog alfa	27 (90.0)	9 (100.0)	6 (66.7)	12 (100.0)
Plasma-derived product	3 (10.0)	0 (0.0)	3 (33.3)	0 (0.0)

¹First injection with rFIXFc; ²Includes 1 patient aged <12 years and 5 patients aged ≥18 years, 5 with severe haemophilia and 1 with moderate haemophilia;

³Includes 3 patients aged <12 years, 4 patients aged 12–17 years, and 17 patients aged ≥18 years; 19 with severe haemophilia, 3 with moderate haemophilia and 2 with mild haemophilia.

FIX, factor IX; IQR, interquartile range; rFIXFc, recombinant factor IX Fc fusion protein; HCV, hepatitis C virus; HIV, human immunodeficiency virus; SD, standard deviation; SHL, standard half-life.

Supplementary Table 2. Clinical outcomes and factor usage by completed months of rFIXFc prophylaxis

Variable	Completed months of rFIXFc prophylaxis			
	0–6 (<i>n</i> = 30)	6–12 (<i>n</i> = 30)	12–18 (<i>n</i> = 28)	18–24 (<i>n</i> = 20)
Prescribed prophylactic dose, IU/kg/week	<i>n</i> = 29	<i>n</i> = 29	<i>n</i> = 27	<i>n</i> = 20
Mean (SD)	44.3 (14.2)	45.7 (17.0)	49.2 (16.6)	53.5 (15.7)
Median (IQR)	45.5 (33.3, 53.3)	45.9 (33.3, 53.3)	49.0 (38.3, 55.6)	51.9 (45.3, 56.5)
Injection frequency per week	<i>n</i> = 29	<i>n</i> = 29	<i>n</i> = 27	<i>n</i> = 20
Mean (SD)	1.0 (0.1)	1.0 (0.2)	1.1 (0.2)	1.0 (0.3)
Median (IQR)	1.0 (1.0, 1.0)	1.0 (1.0, 1.0)	1.0 (1.0, 1.0)	1.0 (1.0, 1.0)
ABR				
Mean (SD)	1.9 (2.3)	2.5 (3.8)	1.1 (1.9)	1.1 (1.5)
Median (IQR)	1.0 (0.0, 3.5)	2.0 (0.0, 4.0)	0.0 (0.0, 2.0)	0.0 (0.0, 2.0)
AjBR				
Mean (SD)	0.9 (1.3)	1.0 (1.5)	0.5 (1.0)	0.4 (0.8)
Median (IQR)	0.0 (0.0, 2.0)	0.0 (0.0, 2.0)	0.0 (0.0, 0.0)	0.0 (0.0, 0.0)
AsBR				
Mean (SD)	0.8 (1.9)	0.6 (1.6)	0.3 (0.9)	0.1 (0.4)
Median (IQR)	0.0 (0.0, 0.0)	0.0 (0.0, 0.0)	0.0 (0.0, 0.0)	0.0 (0.0, 0.0)
Zero spontaneous bleeds n (%)	24 (80.0)	24 (80.0)	25 (89.3)	19 (95.0)

One paediatric patient was excluded from the analysis as data on injection frequency were missing.

ABR, annualised bleeding rate; AjBR, annualised joint bleeding rate; AsBR, annualised spontaneous bleeding rate; IQR, interquartile range; rFIXFc, recombinant factor IX Fc fusion protein; SD, standard deviation.