

Demographic and Baseline Characteristics

Table 11.2.1: Demographics (All Enrolled Participants)

Demographic Variable	6E12 vg/kg (N=1)	2E13 vg/kg (N=1)	4E13 vg/kg (N=6)	6E13 vg/kg (N=7)	Total (N=15)
Age at enrollment, years					
n	1	1	6	7	15
Mean (SD)	25.0 (NA)	43.0 (NA)	31.3 (9.6)	30.4 (5.8)	31.3 (7.8)
Min, Max	25, 25	43, 43	22, 45	23, 42	22, 45
Age at enrollment, n (%)					
18 to < 40 years	1 (100)	0	5 (83.3)	6 (85.7)	12 (80.0)
40 to < 60 years	0	1 (100)	1 (16.7)	1 (14.3)	3 (20.0)
Sex, n (%)					
Male	1 (100)	1 (100)	6 (100)	7 (100)	15 (100)
Race, n (%)					
Asian	1 (100)	0	0	1 (14.3)	2 (13.3)
Black or African American	0	0	1 (16.7)	0	1 (6.7)
White	0	1 (100)	5 (83.3)	6 (85.7)	12 (80.0)

Baseline Characteristics

Table 11.2.2: Baseline Characteristics (All Enrolled Participants)

Demographic Variable	6E12 vg/kg (N=1)	2E13 vg/kg (N=1)	4E13 vg/kg (N=6)	6E13 vg/kg (N=7)	Total (N=15)
Time since diagnosis of hemophilia A, years					
n	1	1	6	7	15
Mean (SD)	25.0 (NA)	43.0 (NA)	31.3 (9.3)	30.6 (6.1)	31.3 (7.7)
Median	25.0	43.0	30.0	30.0	30.0
Min, Max	25.0, 25.0	43.0, 43.0	22.0, 45.0	23.0, 43.0	22.0, 45.0
Type of FVIII treatment for hemophilia A, n (%)					
On-demand	0	0	0	1 (14.3)	1 (6.7)
Prophylaxis	1 (100)	1 (100)	6 (100)	6 (85.7)	14 (93.3)
Baseline FVIII activity (IU/dL) (CSA)					
n	1	1	5	7	14
Mean (SD)	30.30 (NA)	0.00 (NA)	16.64 (32.01)	15.43 (20.75)	15.82 (23.45)
Median	30.30	0.00	0.00	6.60	3.30
Min, Max	30.3, 30.3	0.0, 0.0	0.0, 73.4	0.0, 51.1	0.0, 73.4
Baseline annualized FVIII usage, IU/kg/year					
n	1	1	6	7	15
Mean (SD)	3486.66 (NA)	3022.48 (NA)	4704.16 (2345.75)	4444.48 (1969.50)	4389.70 (1965.51)
Median	3486.66	3022.48	4944.56	5085.90	4107.51
Min, Max	3486.7, 3486.7	3022.5, 3022.5	1241.4, 7682.7	573.2, 6438.5	573.2, 7682.7
Baseline annualized number of FVIII infusions, infusions/year					
n	1	1	6	7	15
Mean (SD)	87.35 (NA)	103.77 (NA)	142.83 (48.78)	120.12 (45.94)	125.93 (45.11)
Median	87.35	103.77	155.83	121.38	124.75
Min, Max	87.3, 87.3	103.8, 103.8	53.8, 184.3	27.4, 158.5	27.4, 184.3
Baseline ABR, treated bleeds/year					
n	1	1	6	7	15
Mean (SD)	2.00 (NA)	3.00 (NA)	12.17 (15.35)	17.57 (14.71)	13.40 (14.26)
Median	2.00	3.00	8.00	24.00	9.00
Min, Max	2.0, 2.0	3.0, 3.0	0.0, 41.0	0.0, 40.0	0.0, 41.0

Secondary: Annualized Bleeding Rate Requiring Exogenous Factor VIII Replacement Treatment during Week 5 and Beyond

Table 14.2.2.1.2.1
Summary of Annualized Bleeding Rate Requiring Exogenous Factor VIII Replacement Treatment (ISTH)
Analysis population: Full Analysis Set

Cohort Results	Post-Baseline				
	Weeks 5-52		Week 5 and Beyond		
	Bleeding Episodes (no.)	ABR (no./yr)	Bleeding Episodes (no.)	ABR (no./yr)	Follow-up (Days)
BMN 270 6E12 vg/kg (N = 1)					
n	1	1	1	1	1
Mean (SD)	10.00 (NA)	10.87 (NA)	13.00 (NA)	1.87 (NA)	2534.00 (NA)
Median	10.00	10.87	13.00	1.87	2534.00
Q1, Q3	10.00, 10.00	10.87, 10.87	13.00, 13.00	1.87, 1.87	2534.00, 2534.00
Min, Max	10.0, 10.0	10.9, 10.9	13.0, 13.0	1.9, 1.9	2534.0, 2534.0
BMN 270 2E13 vg/kg (N = 1)					
n	1	1	1	1	1
Mean (SD)	2.00 (NA)	2.17 (NA)	43.00 (NA)	6.20 (NA)	2534.00 (NA)
Median	2.00	2.17	43.00	6.20	2534.00
Q1, Q3	2.00, 2.00	2.17, 2.17	43.00, 43.00	6.20, 6.20	2534.00, 2534.00
Min, Max	2.0, 2.0	2.2, 2.2	43.0, 43.0	6.2, 6.2	2534.0, 2534.0

Max, maximum; Min, minimum; SD, standard deviation; Q1: 25% Percentile; Q3: 75% Percentile; ABR, annualized bleeding rate.
A bleeding episode is defined as a bleed or symptoms associated with the development of a bleed (or multiple bleeds occurred in the same day) requiring FVIII replacement treatment.

Secondary: Annualized Factor VIII Utilization during Week 5 and Beyond

Table 14.2.2.2.1
Summary of Annualized Factor VIII Utilization
Analysis population: Full Analysis Set

Cohort Results	Post-Baseline			
	Week 5 and Beyond			
	FVIII Utilization (IU/kg)	Annualized FVIII Utilization (IU/kg/yr)	Follow-up (Days)	CFB (IU/kg/yr)
BMN 270 6E12 vg/kg (N=1)				
n	1	1	1	1
Mean (SD)	17597.27 (NA)	2536.47 (NA)	2534.00 (NA)	-950.20 (NA)
Median	17597.27	2536.47	2534.00	-950.20
Min, Max	17597.3, 17597.3	2536.5, 2536.5	2534.0, 2534.0	-950.2, -950.2
Q1, Q3	17597.27, 17597.27	2536.47, 2536.47	2534.00, 2534.00	-950.20, -950.20

FVIII, Coagulation factor VIII; Max, maximum; Min, minimum; SD, standard deviation; Q1: 25% Percentile; Q3: 75% Percentile; CFB: Change from baseline.
Annualized FVIII utilization (IU/kg/yr) = (sum(FVIII replacement treatment (IU/kg)) of the period/sum(follow-up days of the period))*365.25.

Table 14.2.2.2.1
Summary of Annualized Factor VIII Utilization
Analysis population: Full Analysis Set

Cohort Results	Post-Baseline			
	Week 5 and Beyond			
	FVIII Utilization (IU/kg)	Annualized FVIII Utilization (IU/kg/yr)	Follow-up (Days)	CFB (IU/kg/yr)
BMN 270 2E13 vg/kg (N=1)				
n	1	1	1	1
Mean (SD)	1937.47 (NA)	279.27 (NA)	2534.00 (NA)	-2743.22 (NA)
Median	1937.47	279.27	2534.00	-2743.22
Min, Max	1937.5, 1937.5	279.3, 279.3	2534.0, 2534.0	-2743.2, -2743.2
Q1, Q3	1937.47, 1937.47	279.27, 279.27	2534.00, 2534.00	-2743.22, -2743.22

FVIII, Coagulation factor VIII; Max, maximum; Min, minimum; SD, standard deviation; Q1: 25% Percentile; Q3: 75% Percentile; CFB: Change from baseline.
Annualized FVIII utilization (IU/kg/yr) = (sum(FVIII replacement treatment (IU/kg)) of the period/sum(follow-up days of the period))*365.25.

Secondary: Annualized Factor VIII Infusion Rate during Week 5 and Beyond

Table 14.2.2.3.1
Summary of Annualized Factor VIII Infusion Rate
Analysis population: Full Analysis Set

Cohort Results	Post-Baseline			
	Week 5 and Beyond			
	FVIII infusion rate (no.)	Annualized FVIII infusion rate (no./yr)	Follow-up (Days)	CFB (no./yr)
BMN 270 6E12 vg/kg (N=1)				
n	1	1	1	1
Mean (SD)	541.00 (NA)	77.98 (NA)	2534.00 (NA)	-9.37 (NA)
Median	541.00	77.98	2534.00	-9.37
Min, Max	541.0, 541.0	78.0, 78.0	2534.0, 2534.0	-9.4, -9.4
Q1, Q3	541.00, 541.00	77.98, 77.98	2534.00, 2534.00	-9.37, -9.37

FVIII, Coagulation factor VIII; Max, maximum; Min, minimum; SD, standard deviation; Q1: 25% Percentile; Q3: 75% Percentile; CFB: Change from baseline.
Annualized FVIII infusion rate (count/yr) = (sum(Number of FVIII replacement infusions during calculation period / sum(follow-up days of the period)) * 365.25.

Table 14.2.2.3.1
Summary of Annualized Factor VIII Infusion Rate
Analysis population: Full Analysis Set

Cohort Results	Post-Baseline			
	Week 5 and Beyond			
	FVIII infusion rate (no.)	Annualized FVIII infusion rate (no./yr)	Follow-up (Days)	CFB (no./yr)
BMN 270 2E13 vg/kg (N=1)				
n	1	1	1	1
Mean (SD)	70.00 (NA)	10.09 (NA)	2534.00 (NA)	-93.68 (NA)
Median	70.00	10.09	2534.00	-93.68
Min, Max	70.0, 70.0	10.1, 10.1	2534.0, 2534.0	-93.7, -93.7
Q1, Q3	70.00, 70.00	10.09, 10.09	2534.00, 2534.00	-93.68, -93.68

FVIII, Coagulation factor VIII; Max, maximum; Min, minimum; SD, standard deviation; Q1: 25% Percentile; Q3: 75% Percentile; CFB: Change from baseline.
Annualized FVIII infusion rate (count/yr) = (sum(Number of FVIII replacement infusions during calculation period / sum(follow-up days of the period)) * 365.25.