

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/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 | | Follow-up (Days) |
| | Bleeding Episodes (no.) | ABR (no./yr) | Bleeding Episodes (no.) | ABR (no./yr) | |
| 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.