

Improved Quality of Life and Satisfaction With N8-GP in US Patients

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BACKGROUND & AIMS

- Severe hemophilia A is a chronic condition associated with pain, recurrent joint bleeding, limited mobility, and psychosocial and economic issues, all of which negatively affect quality of life (QoL)¹
- Prophylactic therapy can prevent bleeding episodes, leading to less pain and arthropathy and better mobility, which contribute to improved QoL¹
- N8-GP is an extended half-life recombinant factor VIII product indicated for routine prophylaxis in patients with hemophilia A²
- The objective of this analysis was to determine health-related QoL (HRQoL) and treatment satisfaction outcomes in US patients with severe hemophilia A receiving N8-GP for routine prophylaxis in the pathfinder trials

MATERIALS & METHODS

Treatment

- In **pathfinder2**, patients aged ≥12 years were administered N8-GP 50 IU/kg every 4 days (Q4D) as routine prophylaxis in the main phase and Q4D or Q7D during the extension phases
- In **pathfinder5**, patients aged <12 years received prophylaxis with N8-GP 60 IU/kg twice weekly
- In **pathfinder8**, patients from pathfinder2 and pathfinder5 continued treatment; regimen was allocated by investigator based on previous treatment response and bleeding tendency

HRQoL and Treatment Satisfaction

- Change in health-related, disease-specific, and age-specific QoL (HAEMO-QoL) as well as treatment satisfaction (Hemophilia Patient Satisfaction Scale [HEMO-SAT]) of patients were calculated from the initial screening visit (visit 1), end of trial visit, and once per year throughout the trial extension phase for pathfinder5 and the extension phases of pathfinder2
- In pathfinder8, change in treatment satisfaction was calculated from the initial screening visit and the end of trial visit using the HEMO-SAT(P), and HEMO-SAT(A) questionnaires
- Questionnaires were completed by patients and/or their parents. Scores ranged from 0 to 100; lower scores indicated better QoL and treatment satisfaction. Negative changes in scores indicated improvement

RESULTS

- In total, 177 patients in pathfinder2, 68 patients in pathfinder5, and 160 patients in pathfinder8 received prophylactic N8-GP. US patient demographics are summarized in **Table 1**

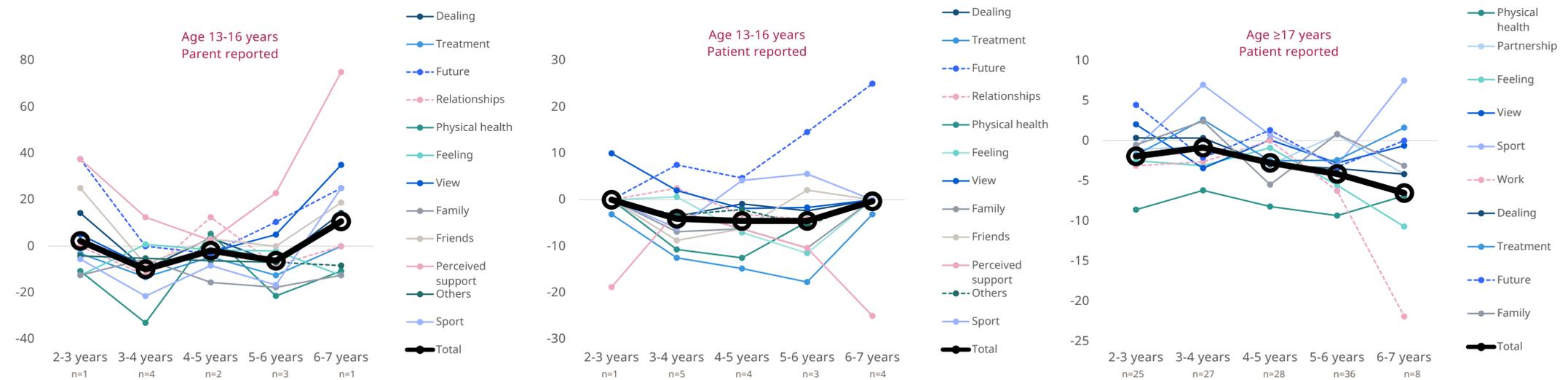
Table 1 pathfinder2, pathfinder5, and pathfinder8 patient characteristics

	pathfinder2		pathfinder5	pathfinder8	
	Q4D	Q7D		<12 y	≥12 y
Number of US patients	43	12	21	9	31
Age at baseline, mean (SD), years	25.4 (10.5)	21.4 (7.8)	5.4 (3.3)	7.9 (1.3)	30.7 (13.6)
Ethnicity, n (%)					
Hispanic or Latino	9 (20.9)	2 (16.7)	3 (14.3)	-	5 (16.1)
Not Hispanic or Latino	34 (79.1)	10 (83.3)	17 (81.0)	9 (100.0)	25 (80.6)
Not reported	-	-	1 (4.8)	-	1 (3.2)
Race, n (%)					
Asian	-	-	1 (4.8)	-	1 (3.2)
Black	6 (14.0)	1 (8.3)	3 (14.3)	2 (22.2)	2 (6.5)
White	36 (83.7)	11 (91.7)	14 (66.7)	7 (77.8)	26 (83.9)
Other	1 (2.3)	-	2 (9.5)	-	1 (3.2)
Not reported	-	-	1 (4.8)	-	1 (3.2)

HRQoL

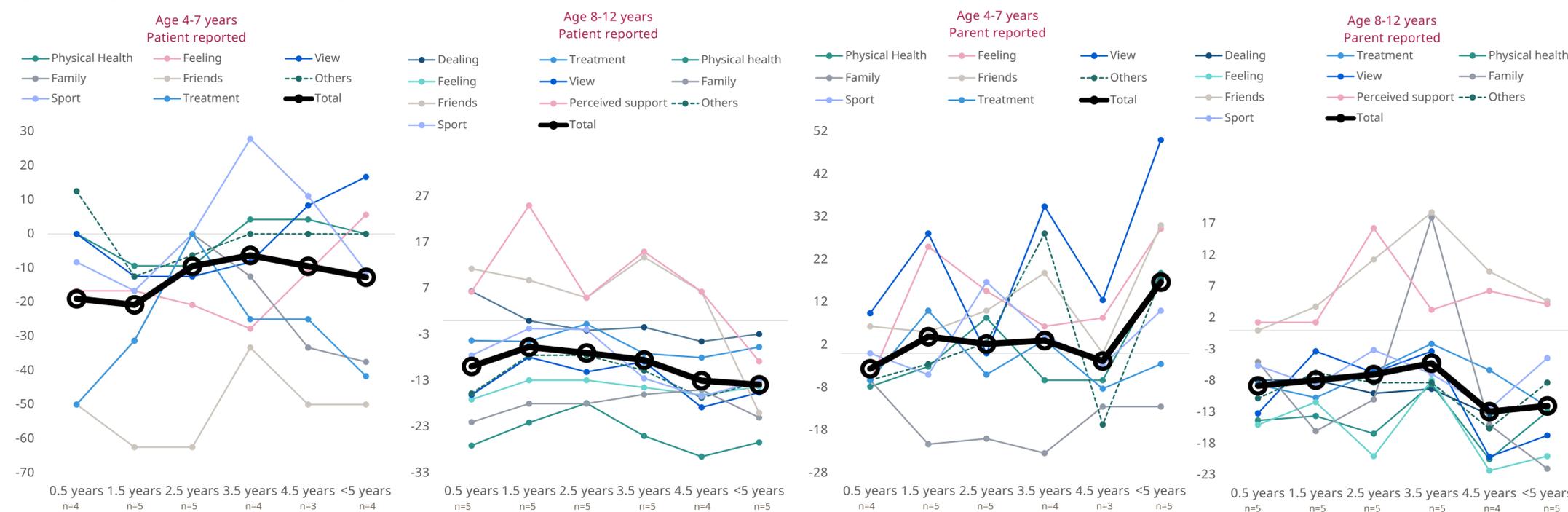
- In pathfinder2, improved QoL scores were reported by patients aged ≥17 years in physical health, work, feeling (emotional well-being), and partnership (**Figure 1**)
- The HAEMO-QoL total score changed by a mean (SD); number of questionnaires of -4.14 (12.69; 36) after 5 to <6 years of treatment and -6.53 (10.99; 8) after 6 to <7 years of treatment, indicating improved QoL

Figure 1 Change in health-related QoL according to mean HAEMO-QoL score for the duration of pathfinder2



- In pathfinder5, improved QoL scores were reported by patients aged 4 to 7 years in the domains of treatment, family, friends, and sports (**Figure 2**)
- The HAEMO-QoL total score changed by a mean of -12.7 (6.0; 4) at the end of trial visit. However, the parents of this group of patients reported higher scores in most of these domains, and only improvements in treatment and family were reported
- Patients aged 8 to 11 years reported improved QoL across all domains, with an HAEMO-QoL total score changing by a mean of -13.9 (13.4; 5). Parents of this patient group also reported improved QoL in most domains

Figure 2 Change in health-related QoL according to mean HAEMO-QoL score for the duration of pathfinder5



Treatment satisfaction

- Across all 3 studies, patients and parents reported improved treatment satisfaction with N8-GP in efficacy and burden (**Table 2**)
- Improved treatment satisfaction in ease and convenience was reported by patients aged >17 years in pathfinder2 and pathfinder8 and by parents of patients aged ≤16 years in pathfinder5
- Additionally, parents of patients aged ≤16 years and patients aged >17 years reported improved treatment satisfaction in specialist/nurse and total score in pathfinder8

Table 2 Mean change in patient-reported and parent-reported HEMO-SAT score (SD) from baseline to end of trial in pathfinder2, pathfinder5, and pathfinder8

	pathfinder2		pathfinder5		pathfinder8	
	Patient reported	Parent reported	Parent reported	Patient reported	Parent reported	Parent reported
Patient age	>17 years	13-16 years	≤16 years	>17 years	≤16 years	≤16 years
Ease and convenience	-16.7 (10.4)	0.0	-12.7 (16.9)	-3.0 (12.0)	0.21 (11.16)	
Efficacy	-3.1 (25.0)	-4.2 (-)	-1.8 (15.2)	-3.37 (9.19)	-4.17 (7.45)	
Burden	-3.9 (18.9)	-31.3 (-)	-10.7 (13.3)	-2.68 (12.27)	-5.11 (16.73)	
Specialist/nurse	3.1 (17.2)	0.0	2.3 (6.4)	-1.43 (5.84)	-0.32 (1.08)	
Center/hospital	7.5 (17.5)	0.0	3.6 (9.1)	0.25	-2.27 (7.54)	
General satisfaction	6.3 (21.1)	0.0	0.0	-2.38 (8.50)	3.41 (8.08)	
Total score	-	-	-	-2.58 (6.7)	-1.43 (4.87)	

Red shading indicates a positive change in HEMO-SAT score. Green shading indicates a negative change (improvement) in HEMO-SAT score. Grey shading indicates no change in score.

CONCLUSIONS

- Overall, US patients participating in the pathfinder2, pathfinder5, and pathfinder8 trials reported improved QoL and treatment satisfaction while receiving N8-GP for routine prophylaxis

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