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Health-related quality of life in young children with haemophilia B treated with the novel long-acting nonacog beta pegol recombinant factor IX

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Objective

- To describe the impact of once weekly 40 IU/kg nonacog beta pegol, given prophylactically for 1 yr, on health-related quality of life (HRQoL) in 8-12 yr old boys.

Conclusions

- HRQoL for children treated with nonacog beta pegol tended to improve in key domains that reflect relationships with others and acceptance of the disease and its treatment.
- For both children and parents, once weekly treatment with 40 IU/kg nonacog beta pegol maintained or improved haemophilia-specific HRQoL of children.
- For parents, once weekly prophylactic treatment with 40 IU/kg nonacog beta pegol improved satisfaction with treatment efficacy and burden over the 1-yr study period.

Introduction

- Nonacog beta pegol is an extended half-life glycoPEGylated factor IX (FIX) designed to enable once weekly prophylaxis in patients with haemophilia B.[1]
- In previously treated boys aged ≤12 yrs with severe haemophilia B, once weekly 40 IU/kg nonacog beta pegol given prophylactically for 1 yr was well tolerated, demonstrated a median spontaneous annualised bleeding rate of 0, and mean steady state weekly trough levels of 17%.[2]

Methods

Study Design and Sample

- HRQoL was assessed at baseline (BL) and at end of trial (ET) in paradigm™5, an open label, single-arm, multi-national trial in boys with haemophilia B.
- The sample for the current analysis study comprised a subsample from the overall trial for boys age 8-12 yrs.

Measures

- HRQoL was assessed by children and their parents using the Haemophilia-Quality of Life questionnaire (HAEMO-QOL-II) for ages 8-12 yrs that contains 64 items covering 10 HRQoL domains; scores range from 0 to 100, with lower scores reflecting better QoL.[3]
- Treatment satisfaction was assessed by parents using the Hemophilia Satisfaction (HEMO-SAT) questionnaire that contains 35 items covering 6 domains; scores range from 0 to 100, with lower scores reflecting greater treatment satisfaction.[4]

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Statistical Analyses

- Descriptive analyses: changes in scores calculated by subtracting BL from ET; thus a negative change in score indicates improvement in HRQoL or satisfaction.

Results

Patients' Characteristics

- 25 boys were included in paradigm™5; of whom 12 were aged between 8 and 12 yrs
- Characteristics of 8-12 yr old boys are presented in Table 1.

Table 1 Patients' characteristics.

Variable	8-12 yrs (N=12)
Age at baseline, yrs	
Mean (SD) / Median	9.8 (1.5) / 10.0
Previous regimen prior to study entry	
Prophylaxis	10 (83.3%)
On-demand	2 (16.7%)
Mean annualised total bleeding rate 12 months prior to study entry*	2.6
Annualised total bleeding rate on study (bleed/yr/pt)*	
Estimated (95%CI)**	1.9 (1.1- 3.1)
Median (Min – Max)	2.0 (0.0- 6.5)

Annualised total bleeding rates calculated from the total study sample in children aged 7-12 yrs (n=13).
***Poisson regression model*

HRQoL - HAEMO-QOL-II

- Low HAEMO-QoL-II median scores at BL reflected an overall good haemophilia-related HRQoL. The most impacted domain scores at BL were 'Friend' and 'Perceived support' (children and parents reported a median score above 50), and 'Dealing' (children reported a median score above 50).

Table 2 Description of HAEMO-QOL-II scores at baseline and median change over paradigm™5.

HAEMO-QOL-II Median (Q1-Q3)	Children 8-12 yrs Baseline (N=12)	Children 8-12 yrs Change (N=12)	Parents of children 8-12 yrs Baseline (N=12)	Parents of children 8-12 yrs Change (N=12)
Physical health	14.3 (10.7-25.0)	-5.4 (-8.9-7.1)	19.6 (1.8-33.9)	-3.6 (-17.9-7.1)
Feeling	10.7 (0.0-17.9)	-3.6 (-14.3-5.4)	19.6 (7.1-35.7)	-14.3 (-17.9-8.9)
View	26.4 (8.3-33.9)	1.4 (-22.2-6.4)	26.4 (15.3-30.6)	-8.9 (-20.8-8.3)
Family	22.5 (15.0-37.5)	-10.0 (-20.0-0.0)	25.0 (20.0-47.5)	-10.0 (-12.5-10.0)
Friend	59.4 (37.5-65.6)	-12.5 (-18.8-0.0)	56.3 (40.6-78.1)	-6.3 (-25.0-12.5)
Perceived support	68.8 (46.9-100)	0.0 (-6.3-4.2)	50.0 (40.6-68.8)	0.0 (-18.8-9.4)
Others	12.5 (4.2-20.8)	0.0 (-8.3-8.3)	18.8 (10.4-22.9)	4.2 (-8.3-6.3)
Sport & school	20.1 (9.4-23.4)	-1.6 (-15.6-17.4)	39.1 (18.8-45.3)	-3.8 (-18.8-0.0)
Dealing	64.3 (35.7-75.0)	-3.6 (-25.0-10.7)	32.1 (25.0-35.7)	-7.1 (-25.0- -1.8)
Treatment	39.3 (14.3-50.0)	-7.1 (-21.4-0.0)	23.2 (14.3-37.5)	-8.9 (-14.3- -1.8)
Total score	30.9 (22.7-39.1)	-1.6 (-9.8-0.2)	30.3 (24.6-33.6)	-7.0 (-11.2- -0.0)

In light blue, domains for which ≥6 children or parents reported improvements

- Median changes for the scores for which ≥6 children reported improvements were: -12.5 ('Friend'), -10.0 ('Family'), -3.6 ('Dealing'), -7.1 ('Treatment') and -1.6 ('total HAEMO-QOL' score) (Table 2)
- Median changes for the scores for which ≥6 parents reported improvements for their children were: -8.9 ('Treatment'), -7.1 ('Dealing'), -7.0 ('total HAEMO-QOL' score), -6.3 ('Friend') and -3.8 ('Sport') (Table 2).

Treatment satisfaction - HEMO-SAT

- Reduction in median scores between BL and ET (improved satisfaction) was observed in the 'Burden' and 'Efficacy' domains (Table 3).

Table 3 Description of HEMO-SAT scores and median change over paradigm™5.

HEMO-SAT Median (Q1-Q3)	Parents of children 8-12 yrs Baseline (N=12)	Parents of children 8-12 yrs End of trial (N=11)	Parents of children 8-12 yrs Change (N=11)
Ease & convenience	25.0 (18.2-29.6)	20.5 (6.8-31.8)	0.0 (-11.4-6.8)
Efficacy	16.7 (6.3-29.2)	4.2 (0.0-8.3)	-5.8 (-16.7-0.0)
Burden	15.6 (0.0-40.6)	6.3 (0.0-18.8)	0.0 (-25.0-0.0)
Specialist/Nurses	0.0 (0.0-1.8)	0.0 (0.0-3.6)	0.0 (0.0-3.6)
Centre/Hospital	0.0 (0.0-0.0)	0.0 (0.0-0.0)	0.0 (0.0-0.0)
General satisfaction	0.0 (0.0-0.0)	0.0 (0.0-0.0)	0.0 (0.0-0.0)

In light blue, domains for which a reduction ≥5 is observed between BL and ET median scores

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Conflict of interest disclosure

MC has received honoraria, speaker's fees and research support from Baxter/Baxalta, Bayer, Biogen, Biotest, CSL Behring, Grifols, Novo Nordisk, Octapharma and Pfizer. SK has been study local principal investigator for several Novo Nordisk and Biogen clinical trials and has served on advisory boards for Biogen Idec. ES has received speaker's fees from Kedrion, acted as a payed consultant to Bayer, Pfizer, CSL Behring, NovoNordisk, Grifols, Baxter, Baxalta, Biogen Idec, Sobi,Octapharma and Roche and received unrestricted research grant from Pfizer. JOOO declares Novo Nordisk. Baxter/Baxalta, Bayer, CSL, Pfizer Advisory Boards Member; Novo Nordisk, Pfizer, Bayer, CSL Sponsorship for Conferences/Meetings. TC and CSH are employees at Novo Nordisk A/S. JM, an employee of Mapi, was a paid consultant to Novo Nordisk A/S to conduct the HRQoL analysis of the study. VSB is Chair of the International Prophylaxis Study Group (IPSG) which is supported by grants from Bayer Healthcare, Baxalta, Biogen Idec, CSL-Behring, Novo Nordisk and Pfizer to the Hospital for Sick Children Foundation, Toronto, Canada. VSB does not receive any personal remuneration for his role as Chair of the IPSG. VSB has received speaker fees and fees for participation in Advisory Boards from Bayer Healthcare, Baxalta, Biogen Idec, Novo Nordisk and Pfizer. VSB has received research support from Baxalta and Bayer. CZ and NY had no disclosures to report.

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