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Changes in physical activity of pediatric patients treated with damoctocog alfa pegol during the Alfa-PROTECT clinical trial

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CONCLUSIONS

- **Hemophilia A patients aged 7 to <12 reported improvements in their physical and emotional health after initiating treatment with damoctocog alfa pegol**
- **Intensity and frequency of sport activities were reported to have increased for most patients within 1 year of damoctocog alfa pegol prophylaxis initiation**
- **Self-reported data on patient experiences and quality of life can supplement our understanding of the impact of treatment on patients' lives**

OBJECTIVE

Characterize the changes in physical health and activity levels of patients treated with damoctocog alfa pegol during the Alfa-PROTECT trial

INTRODUCTION

- Hemophilia patients experience excessive bleeding either spontaneously or after trauma, often causing joint and muscle damage [6]
- The current standard of care is prophylaxis treatment, aimed at preventing bleeding episodes
- Physical activity is essential for children in order to develop healthy bones, muscles and cardiovascular health[3]
- Hemophilia patients reportedly experience fear of falls and consequential pain and injury, therefore limit their engagement in physical activities[4,5]
- Damoctocog alfa pegol (Jivi®) was approved for the treatment of children aged 7 to <12 with hemophilia A in 2025
- Self-reported quality of life (QoL) and patient experience regarding physical activity in these patients provide valuable insights besides efficacy and safety data of damoctocog alfa pegol

METHODS

QoL in Alfa-PROTECT

- Alfa-PROTECT is a phase 3, multinational, open-label study to evaluate the safety of damoctocog alfa pegol in previously treated children aged 7 to <12 years with severe hemophilia A [1]
- Patients receive prophylaxis treatment with damoctocog alfa pegol [1]
- The study consists of two parts: (A) Main study for approx. 6 months with more frequent visits, followed by (B) Extension study for another 18 months of treatment, with less close monitoring
- Patients completed the Haemo-QoL Short-form II questionnaire for children aged 8-16 years old (SF II) at baseline and at the end of the Main study (at visit 11)
- The Haemo-QoL SF II measured impairment in QoL in 9 domains: Physical health, Feelings, Self perception, Family, Friends, Others, Sports, Dealings and Treatment; scoring is 0-100 where higher score means higher impairment (or lower QoL) [2]

VOICE caregiver interview study

- A cross-sectional qualitative interview study was conducted with caregivers of patients who were treated in the Alfa-PROTECT trial A 75-minute interview were performed with the patient's caregiver at approx. 12 months into treatment with damoctocog alfa pegol
- Caregivers of patients treated in study sites in Brazil and Canada were enrolled, ethical approval was obtained for all participating sites
- The objective of VOICE was to evaluate caregivers' perspectives of burden of disease for the child and caregiver, and to understand their experience in the Alfa-PROTECT clinical trial
- Primary endpoints included impacts on the child's daily life before and after treatment with damactocog alfa pegol; impacts to the caregiver pre- and post-trial as well as perspectives on the treatment itself.
- Descriptive statistical analysis was used to describe characteristics of participants. The answers were anonymized, and qualitatively assessed, focusing of concepts and themes as well as exemplary verbatim quotes
- When assessing physical activities, 3 intensity categories were used: High (e.g. football, judo); Medium (e.g. walking, chores); Low (e.g. video games, drawing).

RESULTS

Patients in Alfa PROTECT

- Total 35 patients received at least 1 dose of damoctocog alfa pegol and 32 of them continued treatment >3 months (mITT analysis set)
- Mean age at trial start was 8.6 (SD 1.36) years old
- Most patients had no target joint present at baseline (84%)
- 91% of patients received prior prophylaxis, 9% prior on-demand treatment
- No patient developed inhibitors during the trial

Haemo-QoL SF II QoL questionnaire

- QoL data was available for 33 children
- A mean reduction in impairment of 6.27 points was observed with damoctocog alfa pegol prophylaxis versus baseline (**Figure 1**)
- The highest improvements were reported in the Physical health (13.51 points) and Sports (10.28 points) domains (**Figure 1**)
- Considerable improvement was observed in the Feelings, Family and Other domains too (**Figure 1**)

VOICE

- Seven caregivers of eight pediatric patients participated in the interviews; one caregiver was interviewed twice representing two children
- Most participants were from Brazil (86%), parents of the patients (100%), from households of 1-2 adults and 1-3 children.
- Patients were aged mean 8.4 years old at the time of the interviews, receiving 3x weekly prophylaxis treatment before enrolling in the clinical trial
- Patients' physical activity levels increased during treatment with damoctocog alfa pegol, with most increase reported in high intensity activities (mean +3.2 activity performed and +110.0 minute spent on high intensity activities per week) (**Figure 2**)
- While 60% reported negative physical impacts of hemophilia before the trial, 100% described improvement in the child's physical state after initiating treatment with damoctocog alfa pegol (**Table 1**)
- >60% reported to observe improvement in their child's emotional status related to hemophilia, during the ~12 months of the trial (**Table 1**)

Table 1: CHANGE IN PERCEIVED PHYSICAL AND EMOTIONAL STATUS, AS REPORTED IN VOICE (n=8)

Change in perceived health status	Quotes from caregiver reports
100% improved physical status	*"However, with this treatment* [damoctocog alfa pegol]*, we have noticed that he has a better life. This issue of, for example, the times he gets hurt, and gets purple… Now, it is not like that anymore. Now, it is normal. He is basically a normal child."*
	"…[talks about joint pain]*…before the trial, I would be certain that he was coming home with a problem. Nowadays, … I allow him to play. Before, I would avoid him doing any activities."*
63% improved emotional status	*"he was very happy and that he is feeling a lot better about being able to participate and play. He does not feel as excluded as before"*
	"With his friends, for example, some don't even know he's hemophiliac because he doesn't see the need to say anything"

Figure 2: CHANGE FROM BASELINE IN PATIENT REPORTED QOL IN ALFA PROTECT CLINICAL TRIAL (n=8)

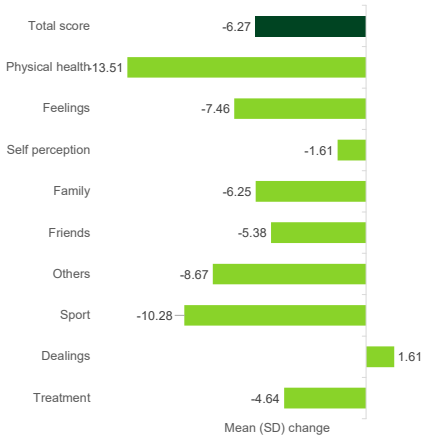
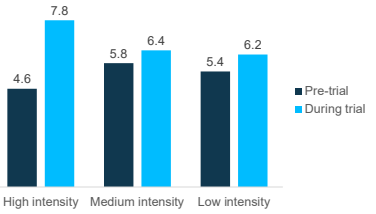


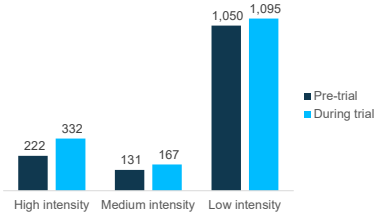
Figure 2: CHANGE IN WEEKLY PHYSICAL ACTIVITY DURING DAMACTOCOG ALFA PEGOL TREATMENT, AS REPORTED IN VOICE (n=8)

A) TOTAL NUMBER OF ACTIVITIES, PER INTENSITY



	High	Medium	Low
Pre-trial (Mean, SD, range)	4.6 (3.2) 2-12	5.8 (1.8) 4-9	5.4 (2.6) 0-7
During trial (Mean, SD, range)	7.8 (5.4) 2-16	6.4 (3.6) 1-10	6.2 (2.4) 2-8
Change	**+3.2**	**+0.6**	**+0.8**

B) TIME SPENT ON ACTIVITIES, PER INTENSITY (MINUTES)



	High	Medium	Low
Pre-trial (Mean, SD, range)	222 (116) 120-400	131.3 (77) 25-210	1,050 (905) 180-2100
During trial (Mean, SD, range)	332 (242) 120-620	166.7 (38) 140-210	1,095 (867) 180-2100
Change	**+110**	**+35.4**	**+45**

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Disclosures
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