PO136

Target Joint Resolution in Children With Haemophilia A Treated With Damoctocog Alfa Pegol: A *Post Hoc* Analysis of the PROTECT VIII Kids Study

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CONCLUSIONS

- **This *post hoc* analysis shows that treatment with damoctocog alfa pegol resolved all pre-existing target joints and prevented the development of persistent target joints in children aged <12 years with severe haemophilia A**

INTRODUCTION

- Recurrent bleeding into joints may result in irreversible joint damage and arthropathy[1]
- In the PROTECT VIII Kids phase 3 study (NCT01775618), damoctocog alfa prophylaxis was efficacious in preventing and treating bleeds in pre-treated patients with severe haemophilia A, aged <12 years at study entry[2]
- This *post hoc* analysis aimed to assess target joint resolution and the maintenance of joint health in previously treated patients receiving damoctocog alfa pegol in PROTECT VIII Kids and its extension

METHODS

- Male patients aged <12 years with severe haemophilia A received damoctocog alfa pegol prophylaxis either twice weekly, every 5 days or every 7 days in the PROTECT VIII Kids study[2,3]
- In this *post hoc* analysis, we evaluated target joint resolution for patients previously receiving factor VIII treatment, who were followed for ≥1-year observation in PROTECT VIII Kids
- Target joints were as reported by the investigator at baseline
  - New target joints were those that developed following study entry, defined as joints with ≥3 spontaneous bleeds within a 6-month interval, as per the International Society on Thrombosis and Haemostasis (ISTH) definition[4]
  - Target joints were defined as resolved if a joint had ≤2 spontaneous bleeds during the patient's last 12 months in the study[4]

RESULTS

- In total, 46 male patients who had received previous prophylaxis were followed up for ≥1 year in the PROTECT VIII Kids study
- Median time of follow-up was 2183 (range: 630–2396) days
- Baseline patient demographics for the 46 patients previously receiving prophylaxis are presented in **Table 1**
  - The mean age of patients across treatment groups was 6.1 years
  - Most patients (89.1%) were White, and patients had a mean body mass index of 16.3
  - Six (13.0%) of the 46 patients (range: 8–10 years old) had pre-existing target joints at baseline, one target joint each per patient
  - One patient (4 years old) developed a new transient target joint in the ankle, following 1.1 years into the study (**Table 2**)
  - Five patients had previously received on-demand treatment; of these, four had target joints at baseline; one new transient target joint developed in this cohort during the study
  - For all patients (previously treated on demand or with prophylaxis), all target joints resolved during the patients' last 12 months in the PROTECT VIII Kids study

Table 1: BASELINE DEMOGRAPHICS

Variable	Treatment frequency				
	Twice a week (n = 11)	**Every 5 days (n = 19)**	**Every 7 days (n = 5)**	**Variable frequency (n = 11)**	**Total (N = 46)**
Age, years, mean	6.8	5.4	5.6	6.7	6.1
Race, n (%)					
White	10 (90.9)	17 (89.5)	4 (80.0)	10 (90.9)	41 (89.1)
Black	1 (9.1)	1 (5.3)	0	1 (9.1)	3 (6.5)
Asian	0	1 (5.3)	1 (20.0)	0	2 (4.3)
BMI, mean	16.8	16.0	15.9	16.5	16.3

BMI, body mass index.

Table 2: TARGET JOINTS DURING AND FOLLOWING THE PROTECT VIII KIDS STUDY AND EXTENSION WITH OBSERVATION OF ≥1 YEAR

Patient		Pre-study		Main study and extension		Last 12 months
	Dosing Main study/ extension	**Number of joint bleeds***	**Target joints**	**Annualised joint bleed rate**	**New target joints**	**Resolved target joints**
1	Every 5 days/ Every 5 days	6	Left ankle	1.5	NA	Left ankle
2	Every 5 days/ Variable frequency	0	Right knee	1.5	NA	Right knee
3	Every 5 days/ Every 5 days	3	Left ankle	3.0	NA	Left ankle
4	Twice a week/ Twice a week	3	Right knee	1.8	NA	Right knee
5	Twice a week/ Twice a week	15	Right knee	1.0	NA	Right knee
6	Every 7 days/ Variable frequency	0	NA	1.5	Left ankle	Left ankle
7	Variable frequency/ Every 7 days	2	Left ankle	0.5	NA	Left ankle

*In the 12 months prior to screening.

NA, not applicable.

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Disclosures

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