Third Interim Analysis of the Phase 4 HEM-POWR Study Evaluating the Real-World Effectiveness of Damoctocog Alfa Pegol in Previously Treated Patients with Hemophilia A

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Mark T Reding,[1]* María Teresa Alvarez Román,[2] Martin Sanabria,[3] Giancarlo Castaman,[4] Maissaa Janbain,[5] Tadashi Matsushita,[6] Karina Meijer,[7] Kathrin Schmidt,[8] Johannes Oldenburg[9]

[1]University of Minnesota Medical Center, Minneapolis, Minnesota, US; [2]Hospital Universitario La Paz, Madrid, Spain; [3]Bayer, Basel, Switzerland; [4]Careggi University Hospital, Florence, Italy; [5]Tulane School of Medicine, New Orleans, Louisiana, US; [6]Nagoya University Hospital, Nagoya, Japan; [7]University Medical Center Groningen, Groningen, the Netherlands; [8]Bayer, Berlin, Germany; [9]University Clinic Bonn, Bonn, Germany

*Presenting author

CONCLUSIONS

- **These updated results from the ongoing HEM-POWR study provide further evidence for the real-world effectiveness of damoctocog alfa pegol in previously treated patients with mild, moderate, and severe hemophilia A.**

INTRODUCTION

- Damoctocog alfa pegol (BAY 94-9027, Jivi®) is a PEGylated extended half-life FVIII replacement product approved for use in previously treated patients aged ≥12 years with hemophilia A.[1,2]
- The effectiveness and safety of damoctocog alfa pegol has been demonstrated in previous interim analyses of the ongoing real-world HEM-POWR study, as well as in the Phase 2/3 PROTECT VIII clinical trial.[3-5]
- The aim of these analyses is to present updated real-world effectiveness data of damoctocog alfa pegol in previously treated patients with hemophilia A from the ongoing HEM-POWR study.

METHODS

- HEM-POWR (NCT03932201) is a prospective, multinational, observational, open-label Phase 4 study of damoctocog alfa pegol in previously treated patients with mild, moderate, and severe hemophilia A in a real-world clinical setting.
- This is the third interim analysis of the HEM-POWR study.
- The primary endpoints were annualized bleeding rate (ABR) and joint health.
- Patients in the Full Analysis Set had to fulfill all inclusion criteria with a documented first study drug dose and ≥1 infusion during the observation period.
- Previously treated patients starting or currently receiving damoctocog alfa pegol with any kind of treatment modality (ie, on-demand, prophylaxis, or intermittent prophylaxis) are eligible for enrollment to the study.
- Statistical analyses were descriptive and explorative. Data were collated from patient e-diaries and physician records, and ethical approval was obtained at all sites.

RESULTS

- At data cut-off (August 17, 2022), 161 previously treated patients were included in the Full Analysis Set. The median (min, max) age at enrollment was 35.0 (13.0, 78.0) years and most patients (141/161; 87.6%) presented with severe hemophilia. The median (Q1, Q3) prescribed dose per infusion per kg of damoctocog alfa pegol at baseline was 37.5 IU/kg (30.6, 47.6). A total of 133/161 (82.6%) patients were pre-treated with damoctocog alfa pegol (prophylaxis [96.2%], on demand [2.3%], or intermittent prophylaxis [0.8%]). Other baseline characteristics are summarized in **Table 1** and **Figure 1**.

Table 1. DEMOGRAPHICS AND BASELINE CHARACTERISTICS

Characteristic	Full Analysis Set (n=161)
Observation period, days, median (min, max)	362.0 (2.0, 944.0)
Age at enrollment, years, median (min, max)	35.0 (13.0, 78.0)
Sex, male, n (%)	160 (99.4)
Race, n (%)*	
White	76 (47.2)
Asian	60 (37.3)
Black or African American	6 (3.7)
Native Hawaiian or other Pacific Islander	1 (0.6)
Disease severity at diagnosis, n (%)†	
Mild	3 (1.9)
Moderate	15 (9.3)
Severe	141 (87.6)
Family history of hemophilia, yes, n (%)	81 (50.3)
Patient self-infusion, yes, n (%)	138 (85.7)
Prescribed dose per infusion per kg of damoctocog alfa pegol at baseline, IU/kg, median (Q1, Q3)	37.5 (30.6, 47.6)
Prescribed dosing modality, n (%)	
On demand	3 (1.9)
Prophylaxis	158 (98.1)
Intermittent prophylaxis	0 (0.0)
Patients pre-treated with damoctocog alfa pegol	**n=133**
Most recent prescribed dosing modality of damoctocog alfa pegol prior to initial visit, n (%)‡	
On demand	3 (2.3)
Prophylaxis	128 (96.2)
Intermittent prophylaxis	1 (0.8)
Most recent damoctocog alfa pegol prophylaxis regimen prior to initial visit in pre-treated patients, n (%)	
Every day	3 (2.3)
Every 2 days	22 (17.2)
Every 3–4 days	66 (51.6)
Every 5 days	21 (16.4)
Every 7 days	16 (12.5)

*Missing/not reported for 18 (11.2%) patients; †Missing for 2 (1.2%) patients; ‡Missing for 1 (0.8%) patient. IU, international units; Q1, 1st quartile; Q3, 3rd quartile; SD, standard deviation.

- All patients across all joint types, and the total, had a mean decrease in bleeds during observation vs prior to study (**Figure 2**).
- The mean difference of total ABR during the observation period and prior to damoctocog alfa pegol initiation was –0.9 (9.4) (**Table 2**).
- Previously treated patients had a mean (SD) of 1.8 (2.2) infusions for bleeding control, and 68.7% of bleeding events were controlled with a single damoctocog alfa pegol infusion.
- During the observation period, 97/161 (60.3%) patients had no bleeding events requiring treatment, 121/161 (75.2%) had no spontaneous bleeds, and 110/161 (68.3%) had no joint bleeds (**Figure 3**).

Figure 1. COUNTRIES OF RECRUITMENT INTO THE FULL ANALYSIS SET (N=161)

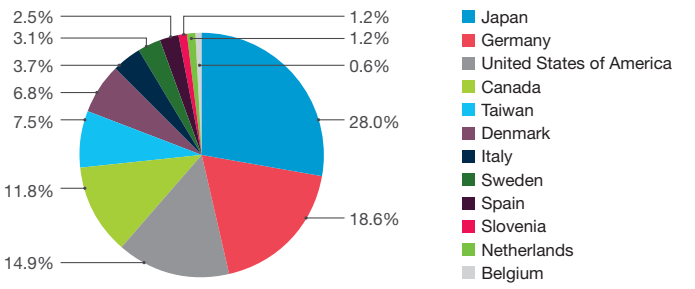
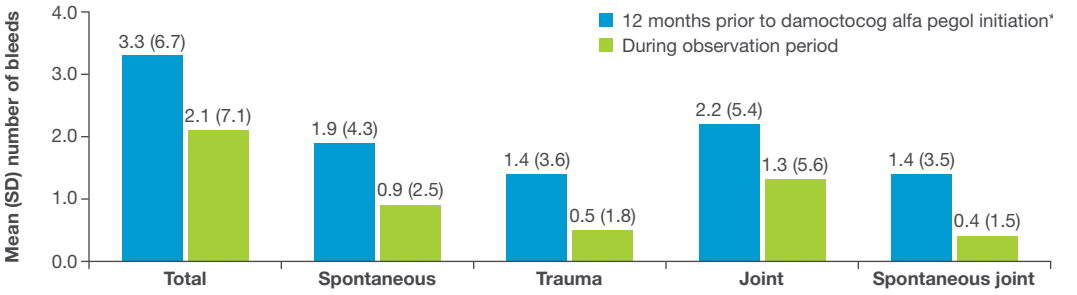


Figure 2: MEAN (SD) NUMBER OF BLEEDS WITHIN 12 MONTHS PRIOR TO DAMOCTOCOG ALFA PEGOL INITIATION AND DURING THE OBSERVATION PERIOD (FULL ANALYSIS SET, N=161)



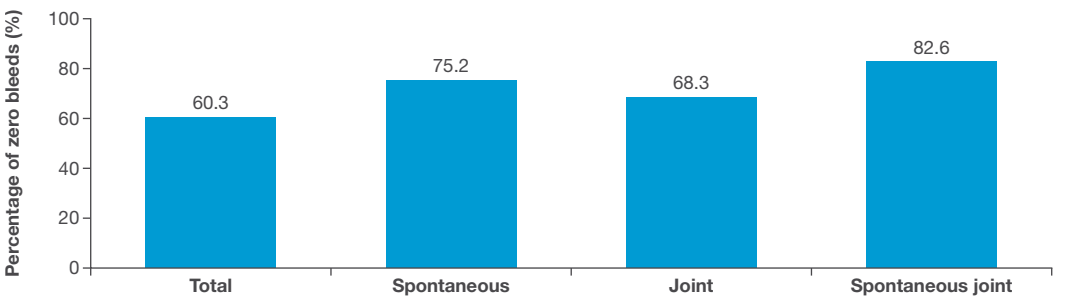
*Missing/not reported for 5 (3.1%) patients; SD, standard deviation.

Table 2: DIFFERENCE OF ABR DURING THE OBSERVATION PERIOD AND PRIOR TO DAMOCTOCOG ALFA PEGOL INITIATION BY BLEED TYPE (FULL ANALYSIS SET, N=161)

Median (Q1, Q3); mean (SD) difference of ABR				
Total	Spontaneous	Trauma	Joint	Spontaneous joint
0.0 (–2.1, 0.0); –0.9 (9.4)	0.0 (–1.0, 0.0); –0.5 (7.7)	0.0 (–1.0, 0.0); –0.9 (3.3)	0.0 (–1.0, 0.0); –0.9 (5.5)	0.0 (–1.0, 0.0); –0.9 (3.6)

Data during the observation period were calculated based on an annualized rate; data prior to initiation were the average number of bleeds over 12 months. ABR, annualized bleeding rate; Q1, 1st quartile; Q3, 3rd quartile; SD, standard deviation.

Figure 3: PERCENTAGE OF ZERO BLEEDS IN THE OBSERVATION PERIOD BY BLEED TYPE (FULL ANALYSIS SET, N=161)



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Disclosures

MTR: receipt of institutional research support from Bayer and BioMarin, member of advisory boards and/or speaker bureaus for Bayer, CSL Behring, Novo Nordisk, Sanofi Genzyme, Takeda, and HEMA Biologics; **MTAR:** speaker in advisory boards and sponsored symposia with Novo Nordisk, Bayer, Takeda, Roche, Pfizer, Octapharma, Amgen, Novartis, CSL Behring, and Sobi; **MS:** employee of Bayer; **GC:** speaker at satellite symposia during scientific meetings for Bayer, Grifols, LFB, Roche, Sobi, Novo Nordisk, Werfen, and Kedrion, member of steering committee of uniQure, participant of advisory boards for Alexion, Bayer, BioMarin, Takeda, CSL Behring, LFB, Novo Nordisk, Pfizer, Roche, Sanofi, Sobi, and uniQure, consultant for Roche; **MJ:** member of speaker bureau for Takeda, BioMarin, CSL Behring, and Sanofi, consultancy for Takeda, CSL Behring, Sanofi, BioMarin, Genentech, and Octapharma, member of Bayer steering committee; **TM:** member of advisory boards for Takeda, Bayer, Novo Nordisk, Chugai, and Pfizer, receipt of educational and investigational support from Chugai, and Novo Nordisk, received honoraria from Takeda, Bayer, Sanofi, Chugai, CSL Behring, JB Pharma, KMB Pharma, Novo Nordisk, Octapharma, and Sysmex; **KM:** receipt of speaker fees from Bayer and Alexion, participation in trial steering committee for Bayer, receipt of consulting fees from uniQure; **KS:** employee of Bayer; **JO:** reimbursed for attending symposia/congresses and/or received honoraria and/or funds for research from Bayer, Biogen Idec, Biotest, Chugai, CSL Behring, Novo Nordisk, Octapharma, Pfizer, Roche, Swedish Orphan Biovitrum, and Takeda.

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