Real-World Effectiveness Data on Mild and Moderate Hemophilia A Patients Using BAY 81-8973 in the ATHNdataset

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CONCLUSION

- This observational, retrospective analysis provides descriptive data contributing to the understanding of mild or moderate hemophilia A (MMHA) patient care.
- 39% of all BAY 81-8973 users in the ATHNdataset were mild and moderate patients. 79% and 68% were treating with an on demand (OD) modality, respectively.
- The remaining patients treated prophylactically (PPX), with 48% moderate vs 30% mild patients using a ≥3x/week regimen.
- Annualized bleeding rates (ABRs) were low across treatment modalities and disease severities, supporting the use of PPX treatment in all hemophilia A patients.
- Without clinical evidence, the numerically slightly higher ABR in the PPX vs OD groups, might be explained by a potentially more severe bleeding phenotype, requiring PPX treatment in MMHA patients.

OBJECTIVE

- To evaluate treatment effectiveness, regimens and characteristics of MMHA patients that are being treated with BAY 81-8973 in a real-world setting.

INTRODUCTION

- Octocog alfa is an unmodified, full-length, standard half-life recombinant Factor VIII (rFVIII) product approved in March 2016, indicated for prophylaxis and on-demand treatment of bleeding events in adults and children with congenital hemophilia A.[1]
- Real-world data on rFVIII product effectiveness are sparse for MMHA patients.[2]
- The ATHNdataset, sponsored by the American Thrombosis and Hemostasis Network, includes 15,304 people with hemophilia A as of 11/30/23, a potential source of data to evaluate therapeutic effectiveness.

METHODS

- The ATHNdataset was queried for hemophilia A patients with a baseline FVIII severity classified as mild or moderate using BAY 81-8973.
- Data included demographics, disease and treatment history, bleed rates, treatment regimens, inhibitor and target joint status.
- Query dates were between January 1, 2010 and November 30, 2023.

Acknowledgments
This analysis was supported by Bayer. Medical writing services were provided by Kushali Patel, PharmD, RPh, an employee of Bayer, in accordance with Good Publication Practices (GPP) 2022.

Data Availability
The data that support the findings of this study originate from the ATHNdataset and are available from the ATHN. Restrictions apply to the availability of these data, which were used under the license for this study. Data inquiries can be made by emailing ATHN at support@athn.org

Disclosures
MC, none to declare. JC, Bayer employee. TS, research funding support to employer from Spark, BioMarin, Pfizer; speakers bureau/honoraria for non-CME from Octapharma, Novo Nordisk, CSL Behring, Genentech, BioMarin, Takeda, Grifols; consultation/advisory board fee Octapharma, Genentech, Novo Nordisk, CSL Behring, Bayer, BioMarin, Takeda, HEMA Biologics, Kedrion, Pfizer; patent holder of Octapharma, Genentech, CSL Behring, Novo Nordisk, Takeda, BPL, BioMarin, Grifols, Pfizer

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1. Kovaltry® prescribing information. Available from: https://www.fda.gov/media/96215/download?attachment. Accessed January 2025
2. Castaman, et al. Journal of Clinical Medicine 2023; 12, 1368

RESULTS

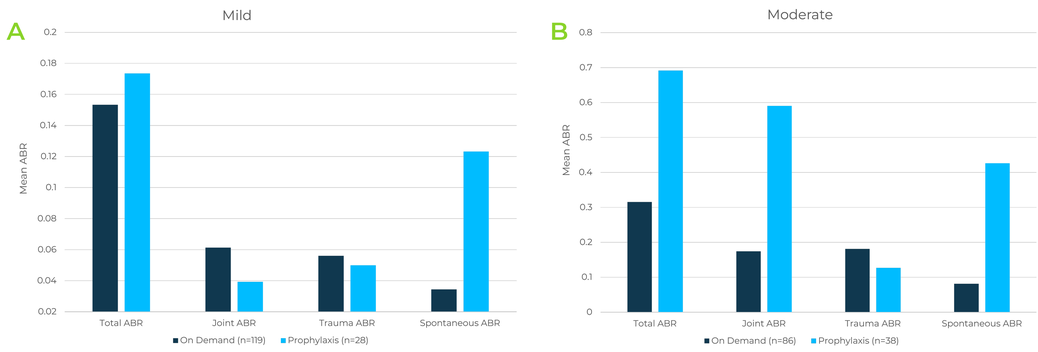
TABLE 1: PATIENT DEMOGRAPHICS, DISEASE CHARACTERISTICS, DISEASE HISTORY, AND TREATMENT DURATIONS IN PATIENTS TREATED WITH BAY 81-8973

All patients (N=291)	Moderate patients (n=132)		Mild patients (n=159)	
	On demand (n=92)	Prophylaxis (n=40) Continuous (n=32) Intermittent (n=8)	On demand (n=126)	Prophylaxis (n=33) Continuous (n=15) Intermittent (n=18)
Age				
Mean	35.3	37.3	38.4	37.0
Median	34.2	36.0	37.2	36.0
Weight (kg)				
Mean	81	85.0	81	89
Median	81	87.1	84	87
BMI				
Mean	27	29.0	28	29
Median	26	28.4	27	27.4
Sex, n (%)				
Male	90 (98)	40 (100)	108 (86)	25 (76)
Female	2 (2)	0 (0)	18 (14)	8 (4 intermittent) (24)
Race, n (%)				
White	70 (76)	29 (73)	105 (83)	29 (88)
Black or African/American	14 (15)	5 (13)	9 (7)	2 (6)
Asian	5 (5)	3 (8)	7 (6)	0 (0)
Mixed Race	0 (0)	1 (3)	1 (1)	1 (3)
American Indian or Alaska Native	0 (0)	1 (3)	0 (0)	0 (0)
Unknown	3 (3)	1 (3)	4 (3)	1 (3)
Ethnicity, n (%)				
Not Hispanic	79 (86)	32 (80)	104 (83)	24 (73)
Hispanic	13 (14)	8 (20)	21 (17)	9 (27)
Unknown	0 (0)	0 (0)	1 (1)	0 (0)
Time taking BAY 81-8973, years				
Mean	2.0	3.2	2.4	3.1
Median	1.1	2.5	1.3	2.4
BAY 81-8973 IU/dose				
Mean	2911	3312	2726	2785
Median	3000	3170	2693	3000
BAY 81-8973 IU/kg				
Mean	37	40	35	33
Median	38	40	38	31
History of Infection, n (%)				
HBV	2 (2)	3 (8)	4 (3)	1 (3)
HCV	23 (25)	12 (30)	28 (22)	3 (9)
HIV	8 (9)	4 (10)	2 (2)	1 (3)
History of FVIII inhibitor, n (%)				
Yes	7 (8)	3 (8)	4 (3)	1 (3)
No	85 (92)	37 (92)	122 (97)	32 (97)
Patients with target joints, n (%)	3 (3)	1 (3)	1 (0.8)	0 (0)
Target joints prior taking BAY 81-8973, n	3	2	1	0
Target joints while taking BAY 81-8973, n	2	2	0	0
Target joints after stopping BAY 81-8973, n	0	1	0	0

Out of 746 patients, 291 (39%) MMHA patients were using BAY 81-8973 either OD (n=218, 75%) or PPX (n=73, 25%), including intermittent PPX, at data cut-off. **(Table 1)**

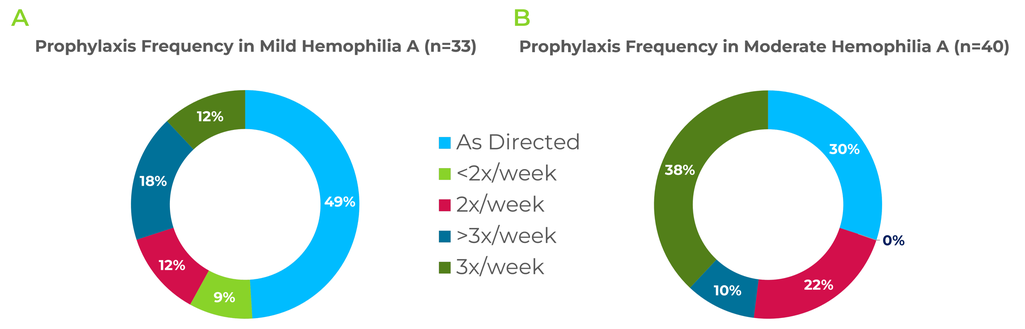
- Of the 159 (55%) patients with mild disease, 126 (79%) were treating OD and 33 (21%) PPX (15 continuously and 18 intermittently).
- Hepatitis C was the most common infection patients ever had, compared to Hepatitis B and HIV.
- The prevalence of ever having had FVIII inhibitors was at 3% for OD and PPX treating mild patients.
- <1% of patients in either treatment modality had recorded target joints at some point in time.
- Mild patients treating OD, were using BAY 81-8973 in average for 2.4 years, while those on prophylaxis, used BAY 81-8973 for an average of 3.1 years with mean doses of 35 and 33 International Units (IU)/kg, respectively. **(Table 1)**
- Of the 132 (45%) patients with moderate disease, 92 (68%) were treating OD and 40 (32%) PPX (32 continuously and 8 intermittently).
- Hepatitis C was the most common infection patients ever had, compared to Hepatitis B and HIV.
- The prevalence of ever having had FVIII inhibitors was at 8% for OD and PPX treating moderate patients.
- 3% in either treatment modality had recorded target joints at some point in time.
- Moderate patients treating OD, were using BAY 81-8973 in average for 2.0 years, while those on PPX, used BAY 81-8973 for an average of 3.2 years with mean doses of 37 and 40 IU/kg, respectively. **(Table 1)**

FIGURE 1: MEAN ANNUALIZED BLEEDING RATES IN PATIENTS WITH MILD AND MODERATE DISEASE TREATING ON DEMAND AND PROPHYLACTICALLY



- In patients with mild disease, total ABRs were 0.15 for OD treating patients and 0.17 for PPX. **(Figure 1A)**
- In patients with moderate disease, total ABRs were 0.32 for OD treating patients and 0.70 for PPX. **(Figure 1B)**

FIGURE 2: FREQUENCY OF PROPHYLAXIS REGIMENS IN PATIENTS TREATED WITH BAY 81-8973



- Of the 33 patients with mild disease treating PPX, 21% used a ≤2x/week regimen and 30% ≥3x/week; the rest were unknown. **(Figure 2A)**
- Of the 40 patients with moderate disease treating PPX, 22% used a 2x/week regimen and 48% ≥ 3x/week; the rest were unknown. **(Figure 2B)**

Poster presented at the HTRS congress, Coronado, CA, USA, March 13-15, 2025