PB2106 Retrospective, Real-World Study of Annual Bleeding Rate Changes Reported in the Microhealth Digital App by Patients with Hemophilia A Transitioning to Efanesoctocog Alfa

M. CHITLUR[1], A. GIERMASZ[2], M. JANBAIN[3], J.C. ROBERTS[4,5], A.C. WEYAND[6], A. STYHLER[7], M. LARA[8], A. WILSON[9], A. SUBAGIARTA[9], D. BOZKAYA[9], S. AHUJA[10]

1 Central Michigan University, Children's Hospital of Michigan, Hemostasis and Thrombosis Center, MI, USA; 2 UC Davis Hemostasis and Thrombosis Center, CA, USA; 3Tulane University, Louisiana Center for Bleeding and Clotting Disorders, LA, USA; 4 Bleeding & Clotting Disorders Institute, Dills Family Foundation Center for Research at BCDI, Peoria, IL, USA; 5 University of Illinois College of Medicine at Peoria, Peoria, IL, USA; 6 University of Michigan, Hemophilia and Coagulation Disorders, MI, USA; 7 Gloval LLC, CO, USA; 8 Microhealth Inc, DE, USA; 9 Sanofi, Cambridge, MA, USA; 10 Indiana Hemophilia and Thrombosis Center, IN, USA

INTRODUCTION

- Efanesoctocog alfa is a first-in-class, ultra-long half-life FVIII replacement therapy. It provides FVIII activity in the near-to-normal range (>40 IU/dL) for up to 4 days after the dose and at 15 IU/dL on day 7 in adults and adolescents.[1]
- In clinical trials, efanesoctocog alfa was efficacious for the prevention and treatment of bleeds in adults and children with severe hemophilia A (HA).[1,2]
- Real-world evidence regarding outcomes for patients switching to efanesoctocog alfa remains limited.

AIM

To evaluate real-world, patient-reported hemostatic outcomes and adherence in US patients transitioning from standard half-life (SHL) products, extended half-life (EHL) products, or FVIIIa mimetic (emicizumab), to efanesoctocog alfa.

METHOD

- Study design: Retrospective analysis
- Data source: Deidentified patient data collected by the Microhealth Digital Hematology mobile application between May 2022 and October 2025
- Inclusion criteria:
  - US residents
  - Patients with HA (PwHA) without inhibitors
  - Prophylaxis regimen
  - Any disease severity, age, and gender
  - 24 continuous months of treatment (12 months on SHL or EHL products, or FVIIIa mimetic (emicizumab), followed by 12 months of treatment with efanesoctocog alfa)
- Primary endpoint: Change in annual bleeding rate (ABR) from pre-transition to post-transition
  - ABR statistically modelled using a generalized linear model (GLM)
  - GLMs expand upon linear regression and model how a dependent variable is affected by independent variables
  - GLM outputs: Rate ratio (how much ABR changes from the pre- to post-transition period) and p-value
- Secondary endpoints:
  - ABR by medication class
  - Adherence (number of infusions reported/number of infusions expected per treatment regimen) pre- and post-transition

RESULTS

- 47 patients met eligibility criteria for this analysis, of all ages and hemophilia severity (Table 1)
  - 11 switched from SHL, 24 from EHL, and 12 from FVIIIa mimetic (emicizumab) (Table 2)
    - ABR was numerically lower post-transition compared to all prior treatment groups
    - Reduction in ABR was statistically significant overall (0.87 [2.09] vs 1.89 [2.35], p = 0.006), including among patients who transitioned from EHL products (0.46 [1.25] vs 1.87 [1.96], p = 0.002)
- Significantly more patients reported zero bleeds post-transition versus pre-transition (70% vs 43%, p < 0.05) (Figure 1)
- Adherence for all 47 patients who actively reported treatment for the duration of the observation was 94% pre-transition and 97% post-transition (Figure 2)

Table 1. Patient characteristics (N=47)

Category	n (%*)
Age group	
0-9 years	9 (19%)
10-19 years	5 (11%)
20-59 years	22 (47%)
60+ years	5 (11%)
Unknown	6 (13%)
Males	39 (83%)
Disease severity	
Severe	37 (79%)
Moderate	6 (13%)
Unknown	4 (9%)

*Rounded

Table 2. ABR for PwHA without inhibitors who transitioned to efanesoctocog alfa as reported in the Microhealth Digital Hematology application

From product class	n	Pre-transition ABR Mean (SD)	Post-transition ABR Mean (SD)	Rate ratio (p-value)
Overall	47	1.89 (2.35)	0.87 (2.09)	0.46 (0.006)
SHL	11	0.91 (1.45)	0.64 (1.80)	0.70 (0.584)
EHL	24	1.87 (1.96)	0.46 (1.25)	0.24 (0.002)
FVIIIa mimetic	12	2.83 (3.35)	1.92 (3.23)	0.68 (0.425)

Statistical significance set at p < 0.05

Figure 1. Proportion of overall study population with zero bleeds

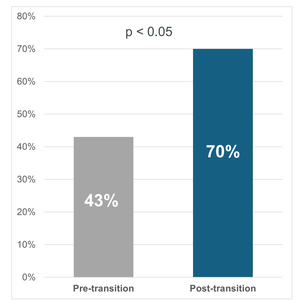
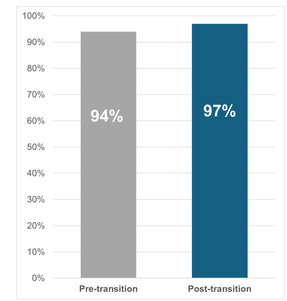


Figure 2. Adherence to treatment for overall study population



CONCLUSIONS

- A reduction of ABR was observed in this cohort of PwHA without inhibitors who transitioned to efanesoctocog alfa from their previous treatment (SHL FVIII products, EHL FVIII products, or FVIIIa mimetic [emicizumab]).
- The proportion of PwHA reporting zero bleeds increased significantly following transition to efanesoctocog alfa, with 70% of study participants reporting no breakthrough bleeds in the 12 months post transition.
- Adherence was high before (94%) and after (97%) transition, strengthening the reliability of the reported bleed rates. This finding may reflect the high engagement of participants, who were already compliant with treatment and logging in the Microhealth mobile application.

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CONTACT INFORMATION

Meera Chitlur, MD

mchitlur@med.wayne.edu