A survey study of haematologists in the United States to understand disease management of patients with haemophilia A treated with emicizumab ▼

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INTRODUCTION

- Emicizumab, a bispecific monoclonal antibody,[1] is indicated in many territories worldwide for routine prophylactic management of bleeding episodes in persons with haemophilia A (PwHA).[2,3]
- A survey of haematologists was conducted in the United States (US) to understand the characteristics of PwHA receiving emicizumab (PwHA-Emi), and to assess any changes in disease management practices in response to the availability of emicizumab.

METHODS

- A descriptive analysis was conducted on data collected in May 2019 via a 30-minute online survey of 50 haematologists from 22 states.
- Haematologists must have met the following criteria:
  - Board-certified in haematology;
  - Have ≥2 years post-residency experience;
  - Treat ≥3 PwHA-Emi per month.
- All data are as reported by the surveyed haematologists.

CONCLUSIONS

- **Most haematologists have retained standard disease management practices when treating PwHA-Emi.**
- **As more PwHA are treated with emicizumab, disease management practices, immune tolerance induction (ITI) treatment, and surgical management should continue to be monitored to edify treatment and care.**

RESULTS

Demographics and characteristics

- Surveyed haematologists (N=50) reported that most PwHA-Emi had severe haemophilia A (**Table 1**).

Table 1. Characteristics of PwHA-Emi

Characteristic, %	N=50
Patient age, years	
0–11	18
12–17	16
18–65	55
≥66	11
Previously treated patients*	90
Disease severity	
Mild	13
Moderate	21
Severe	66
Physical activity level	
Low impact activities	24
Moderate impact activities	34
High impact activities	30
Typically fit into more than one category	12

*Prior FVIII treatment.

Initiating treatment with emicizumab

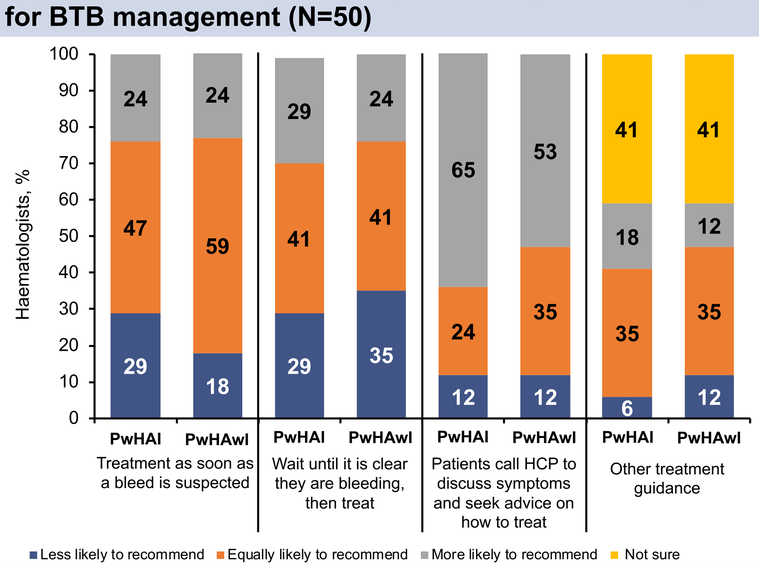
- '*Presence of inhibitors*' (20/50, 40%) and '*high treatment burden with prior treatment*' (8/50, 16%) were reported as the top reasons for treating with emicizumab.

Management of PwHA-Emi

Breakthrough bleed (BTB) management

- Around one third (17/50, 34%) of haematologists changed their guidance on BTB management in PwHA-Emi; 42% (21/50) advised PwHA-Emi to keep 3–4 doses of bypassing agent/FVIII at hand for BTB treatment.
- The majority of haematologists recommend that PwHA-Emi should call their HCP to discuss symptoms and seek advice on treatment when a BTB is suspected (**Figure 1**).

Figure 1. Treatment guidance provided by haematologists for BTB management (N=50)



HCP, healthcare provider; PwHAI, persons with haemophilia A with FVIII inhibitors; PwHAwI, persons with haemophilia A without FVIII inhibitors.

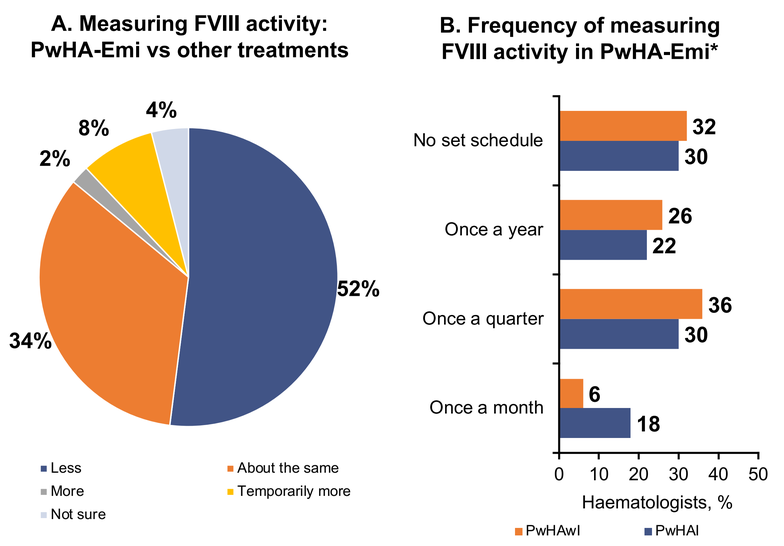
Missed dose guidance

- Most haematologists (47/50, 94%) provided guidance on what to do if PwHA-Emi missed a dose of emicizumab; 44% (22/50) recommended that emicizumab should be administered as soon as possible after the missed dose and that the normal dosing schedule should then be resumed.

Monitoring

- Overall, 52% (26/50) and 28% (14/50) of haematologists reported '*less*' frequent testing for FVIII activity (**Figure 2**) and FVIII inhibitors, respectively, following initiation of emicizumab.

Figure 2. Frequency of measuring FVIII activity (N=50)



PwHAI, persons with haemophilia A with FVIII inhibitors; PwHAwI, persons with haemophilia A without FVIII inhibitors.
*No respondents selected 'other frequency'.

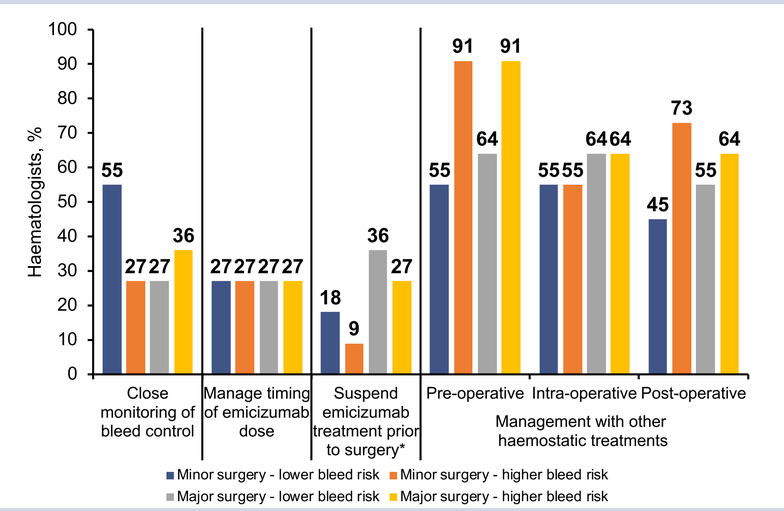
Activity guidance

- Most haematologists (42/50, 84%) reported the physical activity of PwHA-Emi to be the same or higher following initiation of emicizumab; 48% (24/50) recommend a gradual increase in activity levels after starting emicizumab.

Surgical management

- Overall, 11/50 (22%) haematologists had treated PwHA-Emi who had undergone surgery while receiving emicizumab; 91% (10/11) of those reported pre-operative prophylactic management with other haemostatic agents in PwHA-Emi with a high risk of bleeding (**Figure 3**).

Figure 3. Surgical management of PwHA-Emi (n=11)



*Due to the long half-life of emicizumab (around 30 days), effects of emicizumab can remain for at least 6 months after discontinuation.[2,3]

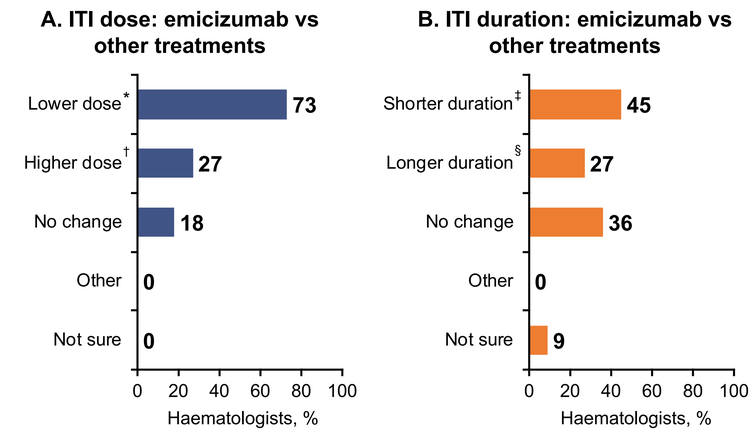
Treatment access

- Around half of haematologists (23/50, 46%) reported PwHA-Emi having regular issues with their emicizumab insurance coverage; 38% (19/50) reported that PwHA-Emi regularly have issues with FVIII/bypassing agent coverage for BTB treatment.

Immune tolerance induction (ITI)

- Over half of haematologists reported treating PwHA-Emi with ITI (11/50, 22%), or considering it in the future (19/50, 38%).
- Of those using ITI, 73% (8/11) reported using lower dose ITI, and 45% (5/11) shortened ITI duration in PwHA-Emi versus PwHA receiving other treatments (**Figure 4**).

Figure 4. Use of ITI in PwHA-Emi (n=11)



*e.g. standard half-life or extended half-life FVIII 50–100 IU/kg two or three times per week.
†e.g. standard half-life or extended half-life FVIII >100 IU/kg three or more times per week.
‡e.g. <12 months. §e.g. >12 months.

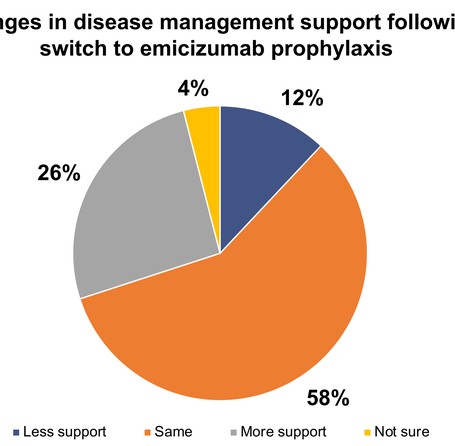
Resource use and care

- Haematologists indicated that the level of routine care required (i.e., annual visits, scheduled appointments) was generally unchanged in PwHA-Emi compared with before starting emicizumab (36/50, 72%), and compared with PwHA receiving other treatments (39/50, 78%).
- Overall, 32% (16/50) of haematologists reported that non-routine care (i.e., trauma, major bleed, surgery) was required less frequently in PwHA-Emi after starting emicizumab.
- Overall, 40% (20/50) and 50% (25/50) of haematologists reported '*similar*' or '*better or significantly better*' adherence among PwHA-Emi compared with PwHA on other treatments, respectively.

Changes in overall management

- Over half (29/50, 58%) of haematologists see no change in disease management support following a switch to emicizumab (**Figure 5**); the most common reason for PwHA-Emi requiring more or less support is '*emicizumab is a newer agent with limited long-term safety and efficacy data*' and '*patients have fewer bleeds*', respectively.

Figure 5. Change in overall disease management support (N=50)



DISCLOSURES

WEO: Employment (Pro Unlimited); **AMP**: Employment and Shareholder (F. Hoffmann-La Roche Ltd./Genentech, Inc.); **KP**: Grant/Research Support (MedPanel Inc.); **JB**: Consultancy (F. Hoffmann-La Roche Ltd.); **JP**: None; **RHK**: Employment (Genentech, Inc.).

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