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, is indicated in many territories worldwide for routine prophylactic management of bleeding episodes in persons with haemophilia A (PwHA).1,2
• 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.

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             | 18  |      
| 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
• Moderate impact activities
• High impact activities
• Typically fit into more than one category

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 (BT) management

• Around one third (17/50, 34%) of haematologists characterised their guidance on BT 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)

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.

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

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

DISCLOSURES

WGO: Employment (Pre-university), AHP: Employment and Board member (I. Hoffmann-La Roche Ltd., Generalist, Inc.)
AP: Employment (Shire US, Inc., Consultant (I. Hoffmann-La Roche Ltd., Generalist, Inc.)
JW: Employment (Genentech, Inc.)
ACKNOWLEDGMENTS

This manuscript was written by Sophie Nobes, BSc, of Gardiner-Caldwell Communications and was funded by I. Hoffmann-La Roche Ltd.

REFERENCES


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.

Immunotolerance induction (ITI)

• Over half of haematologists reported treating PwHA-Emi with ITI (47/50, 94%), 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)

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 starting emicizumab (36/50, 72%), and compared with PwHA receiving other treatments (39/50, 78%).
• Overall, 52% (26/50) of haematologists reported that non-routine care (i.e., trauma, major bleed, surgery) was required less frequently in PwHA-Emi after starting emicizumab.
• Over half (29/50) 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 reusing more or less support is ‘emicizumab is a new 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)

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