Introduction

• HA is caused by a deficiency in coagulation factor (F) VIII activity and is characterized by spontaneous or traumatic bleeding.
• PwHA are prescribed prophylactic treatment, taken to prevent a bleed from occurring, or treatment to take on demand when needed. Efficacy and safety data are limited.

• Emicizumab is a novel treatment for PwHA that replaces the function of missing activated factor VIII (FVIII) [1].
• It was first approved in the United States for prophylaxis in PwHA with FVIII inhibitors in 2017 with the label broadened to include those without FVIII inhibitors in 2018 [2].
• Real-world data can provide valuable information regarding the clinical characteristics of the group of individuals prescribed emicizumab, and allow evaluation of the effect of FVIII inhibitor status on disease burden.

• This study presented here aims to provide an early view of individuals treated with emicizumab, according to FVIII inhibitor status.

Methods

This retrospective analysis used data from US commercial insurance claims (Figure 1).

Inclusion criteria

• PwHA with evidence of emicizumab use (‘emicizumab-claim’; ≥2 months of continuous enrollment prior to the index date [i.e. the year prior to taking emicizumab]).

Outcomes

• Individual information and disease characteristics (such as major bleeds, arthropathy, and pain), use of FVIII and bypassing agents (BPAs), and healthcare resource use were examined in the 12-month period before first use of emicizumab.

• An algorithm developed by Shrestha et al. [2017] was used to identify major bleeds, defined as a cluster of bleeding claims for the same body part in a 7-day period; arthropathy and pain were identified using International Classification of Diseases Ninth and Tenth Revisions Clinical Modification (ICD-9-CM/ICD-10-CM) diagnosis codes.

• National Drug Codes or Healthcare Common Procedure Coding System codes were used to identify FVIII and BPAs.

Results

Among 107 individuals identified, those with FVIII inhibitors were younger, and a larger proportion had major bleeds, pain, and arthropathy than those without FVIII inhibitors (Table 1).

Table 1. Individual and disease characteristics before starting emicizumab

<table>
<thead>
<tr>
<th>Outcome</th>
<th>PwHA with FVIII inhibitors</th>
<th>PwHA without FVIII inhibitors</th>
<th>All PwHA</th>
<th>N = 107</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age (y)</td>
<td>18 (100)</td>
<td>89 (100)</td>
<td>107 (100)</td>
<td></td>
</tr>
<tr>
<td>Mean (±SD) years</td>
<td>18 (6.7)</td>
<td>18 (6.4)</td>
<td>18 (6.5)</td>
<td></td>
</tr>
<tr>
<td>Coverage with a Preferred Provider Organization plan (%)</td>
<td>15 (83)</td>
<td>62 (70)</td>
<td>77 (72)</td>
<td></td>
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<tr>
<td>Major bleed in the year before taking emicizumab (%)</td>
<td>8 (44)</td>
<td>22 (25)</td>
<td>30 (28)</td>
<td></td>
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</tbody>
</table>
| In the 12 months prior to taking emicizumab, the mean ±SD number of FVIII and BPA prescriptions were 10.3 ± 12.3 vs 6.8 ± 7.9, respectively. The mean ±SD number of FVIII use was 20 ± 60 vs 14 ± 34, respectively.

Conclusions

To our knowledge, this is the first real-world claims study that describes the characteristics of individuals prescribed emicizumab. Results show utilization of emicizumab in persons with HA with a broad range of clinical characteristics, and across various age groups. Persons with HA and FVIII inhibitors had greater disease burden and higher healthcare resource use in the year prior to taking emicizumab than those without FVIII inhibitors.

Longer follow-up data will enable further examination of these real-world outcomes.