Early patient experiences with emicizumab in the United States: a qualitative study

Introduction

• CATCH is a new questionnaire developed to assess the impact of hemophilia and its treatment on PwHA and their families. There are three versions of CATCH: adult, pediatric, and caregiver.
• Adult and pediatric versions of CATCH measure the impact of hemophilia on daily activities (medical, emotional, social, work, school), participation in treatment, and treatment burden. The caregiver version assesses only participation and treatment burden.
• Emicizumab is a subcutaneously-administered, non-factor VII treatment for HA. Emicizumab was recently approved for prevention of bleeding (prophylaxis) in PwHA with or without FVIII inhibitors.
• Investigators hired PwHA and caregivers with the aim of better understanding the patient/caregiver experience with emicizumab vs previous HA treatment.
• The suitability of CATCH as a measure of hemophilia disease impact and change after starting emicizumab was also assessed.

Methods

• Participants completed either the adult (PwHA aged ≥18 years), pediatric (≤≤18 years) or caregiver (caregivers of pediatric PwHA) version of an online questionnaire.
• PwHA and their caregivers were then interviewed between June and October 2018, within 3 months of starting emicizumab and then again 3 months later (Figure 1).
• The study was approved by New England Independent Review Board; all participants completed consent or assent prior to participation.

Results

In total, 15 participants (Table 1) (5 children/teenagers, 4 adults and 6 caregivers) provided responses. FVIII inhibitors were present in 93% of PwHA.

<table>
<thead>
<tr>
<th>Variable</th>
<th>Adult (% N=10)</th>
<th>Pediatric (% N=4)</th>
<th>Caregiver (% N=6)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Frequency</td>
<td>22 (22/10)</td>
<td>12 (12/4)</td>
<td>6 (6/6)</td>
</tr>
<tr>
<td>Hemophilia severity, n (%)</td>
<td>Moderate</td>
<td>0 (0/10)</td>
<td>0 (0/4)</td>
</tr>
<tr>
<td>Mild</td>
<td>0 (0/10)</td>
<td>0 (0/4)</td>
<td>0 (0/6)</td>
</tr>
<tr>
<td>Severe</td>
<td>22 (22/10)</td>
<td>12 (12/4)</td>
<td>6 (6/6)</td>
</tr>
<tr>
<td>Previous HA treatment, n (%)</td>
<td>0-4</td>
<td>0 (0/10)</td>
<td>0 (0/4)</td>
</tr>
<tr>
<td>5-10</td>
<td>0 (0/10)</td>
<td>0 (0/4)</td>
<td>0 (0/6)</td>
</tr>
<tr>
<td>&gt;10</td>
<td>22 (22/10)</td>
<td>12 (12/4)</td>
<td>6 (6/6)</td>
</tr>
<tr>
<td>With or without FVIII inhibitors, n (%)</td>
<td>0-4</td>
<td>0 (0/10)</td>
<td>0 (0/4)</td>
</tr>
<tr>
<td>5-10</td>
<td>0 (0/10)</td>
<td>0 (0/4)</td>
<td>0 (0/6)</td>
</tr>
<tr>
<td>&gt;10</td>
<td>22 (22/10)</td>
<td>12 (12/4)</td>
<td>6 (6/6)</td>
</tr>
<tr>
<td>Frequency of the most recent bleed, n (%)</td>
<td>100</td>
<td>80 (80/4)</td>
<td>100 (100/6)</td>
</tr>
<tr>
<td>Frequency</td>
<td>0 (0/10)</td>
<td>0 (0/4)</td>
<td>0 (0/6)</td>
</tr>
<tr>
<td>Improvement in HA treatment, n (%)</td>
<td>0 (0/10)</td>
<td>0 (0/4)</td>
<td>0 (0/6)</td>
</tr>
<tr>
<td>Treatment burden</td>
<td>0 (0/10)</td>
<td>0 (0/4)</td>
<td>0 (0/6)</td>
</tr>
</tbody>
</table>

Conclusions

Switching to emicizumab treatment was associated with reductions in treatment burden, bleeding, and pain; less restriction and preoccupation; increased physical activity; and better performance at work or school.

• PwHA described “good days” and “bad days” differently when asked about the pre- and post-emicizumab periods.
• Themes associated with switching to emicizumab are shown in Figure 2.

References


Disclosures

CATCH was funded by Kedrion Biopharma, Inc. PwHA and/or caregivers’ participation in this research did not incur any financial incentive. The study was approved by New England Independent Review Board. No other competing interests declared.

Figure 1. Assessment of experiences with emicizumab and utility of the CATCH.

Table 1: Participant baseline characteristics

Evaluation of emicizumab in the United States: a qualitative study

Figure 2. Themes associated with switching to emicizumab for treatment of HA, based on qualitative data.

Participants A: adult, P: pediatric (children and adolescents), C: caregiver

Preoccupation

Worry about treatment efficacy
Caregiver more stressed
Consistently thinking about hemophilia
More caregiving
More worry
Needed to take care
Child less happy
Anticipation of negative outcomes
Less independence
Able to focus on other children

Change to an unrestricted lifestyle

Duties/worries
Less school due to bleeding
Caregiver burden of missed school
Caregiver using time in communication with school

Improved academic performance
Less bullying at school
Less school missed
Increased caregiver’s ability to work

Being active/social activities

Less social involvement
More active
Feeling more social, making more spontaneous plans
Feeling more “normal”

Restriction

Constantly sedated/inactive
Less sports participation
More sports gained
More restricted
Need to carry child

Less restricted
More independent
More ability to play sports
No longer need to use wheelchair

Treatmenf burden

Frequency more burdensome
Inflamed joints
Wake up early for treatment
More spare time
Caregiver lack of sleep

Easier treatment
Preferred subcutaneous injection
More sleep before school
Less frequency burdensome
Child scared of needles

Results

During interviews, participants described previous experiences with “good days” and “bad days” before and after treatment with emicizumab differently.

“Good days”

• Based on experiences with previous HA treatment, most participants described their good days as days without negative events.
• After switching to emicizumab, participants characterized good days by describing positive activities/experiences.

“Bad days”

• Based on experience with previous HA treatment, bad days were mostly characterized by the inability to move or leave the house due to a bleed or pain.
• After switching to emicizumab, participants reported that they either no longer experienced bad days, or that the bad days were less bad or not associated with HA.

Presented at the Hemophilia Federation of America (HFA) Virtual Annual Symposium | August 24–29, 2020